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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2021

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission file number: 001-37625

Voyager Therapeutics, Inc.
(Exact name of Registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	**46-3003182** (I.R.S. Employer Identification No.)
75 Sidney Street, Cambridge, Massachusetts (Address of principal executive offices)	**02139** (Zip Code)

(857) 259-5340
(Registrant's telephone number, including area code)

Not Applicable
(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	VYGR	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☒
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of October 28, 2021 was 37,957,396.

Forward-Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “predict,” “project,” “target,” “potential,” “contemplate,” “anticipate,” “goals,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, among other things, statements about:

- our plans to develop and commercialize our product candidates based on adeno-associated virus, or AAV, gene therapy;
- our ability to identify and optimize product candidates and novel AAV gene therapy capsids;
- our strategic collaboration with and funding from our collaboration partner Neurocrine Biosciences, Inc., or Neurocrine, and our licensing agreement with Pfizer Inc., or Pfizer, regarding AAV gene therapy capsids;
- our ongoing and planned clinical trials and related timelines, and our preclinical development efforts and studies;
- formulation changes to our product candidates that may require us to conduct additional clinical studies to bridge our modified product candidates to earlier versions;
- our ability to determine the appropriate path forward for the development of VY-AADC (NBIb-1817) as a treatment for Parkinson’s disease;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for our product candidates, including the ability to file IND applications for our programs;
- our estimates regarding expenses, future revenues, capital requirements, and needs for additional financing;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ AAV capsid screening platform and our vectorized antibody platform;
- our ability to develop a manufacturing capability compliant with current good manufacturing practices for our product candidates;
- our ability to access, develop, and obtain regulatory clearance for devices to deliver our AAV gene therapies to critical targets of neurological disease;
- our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection for our proprietary assets;
- our estimates regarding the size of the potential markets for our product candidates and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates for any indication once approved;
- our plans and ability to raise additional capital, including through equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements;

- our competitive position and the success of competing products that are or become available for the indications that we are pursuing;
- the impact of government laws and regulations including in the United States, the European Union, and other important geographies such as Japan;
- our ability to enter into future collaborations, strategic alliances, or licensing arrangements;
- our ability to reduce costs and reprioritize our product candidate pipeline successfully in connection with our strategic initiatives; and
- the potential impact of the COVID-19 pandemic on our clinical trials and other business operations.

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements. You should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in "Part II, Item 1A — Risk Factors," and in "Part I, Item 1A — Risk Factors" included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 25, 2021 that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

RISK FACTOR SUMMARY

Investment in our securities involves risk and uncertainties that you should be aware of when evaluating our business. The following is a summary of what we believe to be the principal risks facing our business, as more fully described under "Part II, Item 1A—Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations.

- We have a history of incurring significant losses and anticipate that we will incur losses for the foreseeable future and may never achieve or maintain consistent profitability. We may not be able to generate sufficient revenue from the commercialization of our product candidates and may never be consistently profitable.
- We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.
- To date, all of our revenue has been derived from our prior collaborations with Sanofi Genzyme Corporation, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, our ongoing collaboration with Neurocrine Biosciences, Inc., and our licensing agreement with Pfizer Inc. If any ongoing or future collaboration or licensing agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.
- Our AAV gene therapy product candidates are based on a novel technology, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and of subsequently obtaining regulatory approval for, our product candidates.

- Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.
- We are early in our development efforts. Our product candidates are in the discovery and preclinical development stages. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.
- Our product candidates or the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.
- Gene therapies and their companion diagnostics are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.
- Our gene therapy approach utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.
- If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.
- Our inability to recruit, or the loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and could harm our business, financial condition, results of operations and prospects.
- We have recently reduced the size of our organization, and we may encounter difficulties in managing our business as a result of this reduction, or the attrition that may occur following this reduction, which could disrupt our operations. In addition, we may not achieve anticipated benefits and savings from the reduction.
- A widespread outbreak of an illness or other health issue could significantly disrupt our operations. The current COVID-19 pandemic and the response to it have had, and we expect they will continue to have, an adverse effect on our business, operations, and future results.

VOYAGER THERAPEUTICS, INC.

FORM 10-Q

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PART I. FINANCIAL INFORMATION

Voyager Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(amounts in thousands, except share and per share data)
(unaudited)

	September 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 111,475	$ 104,440
Marketable securities, current	10,024	76,698
Related party collaboration receivable	1,165	8,012
Prepaid expenses and other current assets	3,540	8,619
Total current assets	126,204	197,769
Property and equipment, net	22,992	25,435
Deposits and other non-current assets	1,779	2,316
Operating lease, right-of-use asset	33,799	36,064
Total assets	$ 184,774	$ 261,584
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 592	$ 634
Accrued expenses	11,324	14,205
Other current liabilities	5,248	4,198
Deferred revenue, current	9,537	7,729
Total current liabilities	26,701	26,766
Deferred revenue, non-current	29,639	36,088
Other non-current liabilities	41,012	44,410
Total liabilities	97,352	107,264
Commitments and contingencies (see note 8)		
Stockholders' equity:		
Preferred stock $0.001 par value: 5,000,000 shares authorized at September 30, 2021 and December 31, 2020; no shares issued and outstanding at September 30, 2021 and December 31, 2020	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized at September 30, 2021 and December 31, 2020; 37,781,786 and 37,368,027 shares issued and outstanding at September 30, 2021 and December 31, 2020, respectively	38	37
Additional paid-in capital	440,325	430,324
Accumulated other comprehensive loss	(128)	(134)
Accumulated deficit	(352,813)	(275,907)
Total stockholders' equity	87,422	154,320
Total liabilities and stockholders' equity	$ 184,774	$ 261,584

The accompanying notes are an integral part of these condensed consolidated financial statements.

Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Income (Loss)
(amounts in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Collaboration revenue	$ 1,482	$ 117,843	$ 9,342	$ 164,591
Operating expenses:				
Research and development	17,914	25,039	59,767	86,757
General and administrative	8,714	8,277	28,895	26,721
Total operating expenses	26,628	33,316	88,662	113,478
Operating (loss) income	(25,146)	84,527	(79,320)	51,113
Other income (expense) :				
Interest income	121	254	253	1,578
Other (expense) income	(112)	830	2,161	(24)
Total other income, net	9	1,084	2,414	1,554
Net (loss) income	$ (25,137)	$ 85,611	$ (76,906)	$ 52,667
Other comprehensive (loss) income				
Net unrealized (loss) gain on available-for-sale securities	(2)	(205)	6	44
Total other comprehensive (loss) income	(2)	(205)	6	44
Comprehensive (loss) income	$ (25,139)	$ 85,406	$ (76,900)	$ 52,711
Net (loss) income per share, basic	$ (0.67)	$ 2.30	$ (2.04)	$ 1.42
Net (loss) income per share, diluted	$ (0.67)	$ 2.27	$ (2.04)	$ 1.40
Weighted-average common shares outstanding, basic	37,780,547	37,242,504	37,623,309	37,079,242
Weighted-average common shares outstanding, diluted	37,780,547	37,672,328	37,623,309	37,500,155

The accompanying notes are an integral part of these condensed consolidated financial statements.

Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(amounts in thousands, except share data)
(unaudited)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Stockholders' Equity
Balance at December 31, 2019	36,865,116	$ 37	$ 412,227	$ (104)	$ (312,648)	$ 99,512
Exercises of vested stock options	3,035	—	34	—	—	34
Vesting of restricted stock units	108,600	—	—	—	—	—
Stock-based compensation expense	—	—	3,949	—	—	3,949
Unrealized gain on available-for-sale securities, net of tax	—	—	—	525	—	525
Net loss	—	—	—	—	(24,263)	(24,263)
Balance at March 31, 2020	36,976,751	$ 37	$ 416,210	$ 421	$ (336,911)	$ 79,757
Exercises of vested stock options	160,478	—	1,651	—	—	1,651
Vesting of restricted stock units	21,403	—	—	—	—	—
Issuance of common stock under ESPP	44,995	—	638	—	—	638
Stock-based compensation expense	—	—	3,795	—	—	3,795
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(276)	—	(276)
Net loss	—	—	—	—	(8,681)	(8,681)
Balance at June 30, 2020	37,203,627	$ 37	$ 422,294	$ 145	$ (345,592)	$ 76,884
Exercises of vested stock options	50,774	—	510	—	—	510
Vesting of restricted stock units	8,302	—	—	—	—	—
Stock-based compensation expense	—	—	3,436	—	—	3,436
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(205)	—	(205)
Net income	—	—	—	—	85,611	85,611
Balance at September 30, 2020	37,262,703	$ 37	$ 426,240	$ (60)	$ (259,981)	$ 166,236
Balance at December 31, 2020	37,368,027	$ 37	$ 430,324	$ (134)	$ (275,907)	$ 154,320
Exercises of vested stock options	3,811	1	27	—	—	28
Vesting of restricted stock units	184,217	—	—	—	—	—
Stock-based compensation expense	—	—	3,498	—	—	3,498
Unrealized gain on available-for-sale securities, net of tax	—	—	—	11	—	11
Net loss	—	—	—	—	(21,649)	(21,649)
Balance at March 31, 2021	37,556,055	$ 38	$ 433,849	$ (123)	$ (297,556)	$ 136,208
Vesting of restricted stock units	114,412	—	—	—	—	—
Issuance of common stock under ESPP	101,752	—	580	—	—	580
Stock-based compensation expense	—	—	3,871	—	—	3,871
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(3)	—	(3)
Net loss	—	—	—	—	(30,120)	(30,120)
Balance at June 30, 2021	37,772,219	$ 38	$ 438,300	$ (126)	$ (327,676)	$ 110,536
Vesting of restricted stock units	9,567	—	—	—	—	—
Stock-based compensation expense	—	—	2,025	—	—	2,025
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(2)	—	(2)
Net loss	—	—	—	—	(25,137)	(25,137)
Balance at September 30, 2021	37,781,786	$ 38	$ 440,325	$ (128)	$ (352,813)	$ 87,422

The accompanying notes are an integral part of these condensed consolidated financial statements.

Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows
(amounts in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2021	**2020**
Cash flow from operating activities		
Net (loss) income	$ (76,906)	$ 52,667
Adjustments to reconcile net loss (income) to net cash used in operating activities:		
Stock-based compensation expense	9,670	11,432
Depreciation	3,683	2,830
Amortization of premiums and discounts on marketable securities	324	(57)
Other non-cash items	(2,460)	24
Changes in operating assets and liabilities:		
Related party collaboration receivable	6,847	5,807
Prepaid expenses and other current assets	1,731	1,379
Operating lease, right-of-use asset	2,265	2,341
Other non-current assets	69	207
Accounts payable	(42)	(684)
Accrued expenses	(2,912)	(3,440)
Operating lease liabilities	(2,348)	(2,370)
Lease incentive benefit	—	3,947
Deferred revenue	(4,641)	(148,822)
Net cash used in operating activities	(64,720)	(74,739)
Cash flow from investing activities		
Purchases of property and equipment	(1,262)	(8,541)
Proceeds from sale of equipment	—	31
Purchases of marketable securities	—	(24,979)
Proceeds from sales and maturities of marketable securities	72,632	168,000
Net cash provided by investing activities	71,370	134,511
Cash flow from financing activities		
Proceeds from the exercise of stock options	28	2,195
Proceeds from the purchase of common stock under ESPP	357	482
Net cash provided by financing activities	385	2,677
Net increase in cash, cash equivalents, and restricted cash	7,035	62,449
Cash, cash equivalents, and restricted cash beginning of period	106,219	86,777
Cash, cash equivalents, and restricted cash end of period	$ 113,254	$ 149,226
Supplemental disclosure of cash and non-cash activities		
Capital expenditures incurred but not yet paid	$ 22	$ 454

The accompanying notes are an integral part of these condensed consolidated financial statements.

VOYAGER THERAPEUTICS INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of business

Voyager Therapeutics, Inc. (the "Company") is a gene therapy company focused on developing life-changing treatments and next-generation platform technologies. The Company is focused on diseases where the Company believes a single dose adeno-associated virus ("AAV") gene therapy approach that either increases or decreases the production of a specific protein can either halt or slow disease progression or reduce the symptom severity, therefore providing clinically meaningful impact to patients. The Company's gene therapy platforms enable it to engineer, optimize, manufacture and deliver its AAV-based gene therapies that the Company believes have the potential to safely provide durable efficacy following a single administration. The Company's team of experts in the fields of AAV gene therapy and neuroscience first identifies and selects diseases with target tissues that have tropism for AAV gene therapy. The Company then engineers and optimizes AAV vectors for delivery of the virus payload to the targeted tissue or cells. The Company believes its single dose gene therapies have the potential to be delivered directly, with targeted infusions or systemically, in conjunction with its novel capsids.

The Company is identifying novel AAV capsids, outer viral protein shells that enclose genetic material of a virus payload. The Company's team has developed a proprietary system called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) to facilitate the selection of AAV capsids with blood brain barrier ("BBB") crossing and cell-specific transduction properties for particular therapeutic applications. The TRACER system is a broadly-applicable, functional RNA-based AAV capsid screening platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in wild-type animals. The Company is also applying the TRACER system towards further capsid variant libraries and selection for tropism and transduction in additional cell and tissue types, such as cardiac and skeletal muscle.

The Company's quality and manufacturing processes employ an established system capable of enabling production of high quality AAV vectors at clinical scale. In addition to the Company's capsid screening platform, the Company has developed a vectorized antibody platform which the Company believes will overcome many of the challenges of passive immunization.

The Company's business strategy focuses on discovering, developing, manufacturing and commercializing its gene therapy programs. As part of this strategy, the Company has developed core competencies specific to AAV gene therapy development and manufacturing. This business strategy also includes business development activities that may include in-licensing activities or partnering certain programs in certain geographies with collaborators, as the Company has demonstrated through its ongoing collaboration with Neurocrine Biosciences, Inc. ("Neurocrine") under a collaboration agreement that became effective in January 2019 (the "Neurocrine Collaboration Agreement"), or out-licensing activities including license agreements related to the Company's AAV capsids. The Company is devoting substantially all of its efforts to product research and development, market development, and raising capital. The Company is subject to risks common to companies in the biotechnology and gene therapy industries, including but not limited to, the need to obtain sufficient capital to continue to fund its operations, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for its product candidates, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary information and technology, protection against data breaches and other cybersecurity threats, compliance with government regulations, development by competitors of technological innovations, and ability to transition from pilot-scale manufacturing to large-scale production of products.

The Company has a history of incurring annual net operating losses. As of September 30, 2021, the Company had an accumulated deficit of $352.8 million. The Company has not generated any product revenue and has financed its operations primarily through public offerings and private placements of its equity securities and funding from its prior collaborations with Sanofi Genzyme Corporation ("Sanofi Genzyme") and AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company (collectively, "AbbVie") and its ongoing collaboration with Neurocrine, and, in October 2021, its licensing agreement with Pfizer Inc. ("Pfizer").

Through September 30, 2021, the Company has raised approximately $640.0 million of proceeds from sales of convertible preferred stock and common stock, including its initial public offering and follow-on public offering, and proceeds from collaboration agreements. As of September 30, 2021, the Company had cash, cash equivalents, and marketable debt securities of $121.5 million. Based upon its current operating plan, the Company expects that its existing cash, cash equivalents, and marketable debt securities will be sufficient to meet the Company's planned operating expenses and capital expenditure requirements into early 2023.

There can be no assurance that the Company will be able to obtain additional debt or equity financing or generate product revenue or revenue from collaboration partners on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of significant accounting policies and basis of presentation

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of the Company have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial reporting and as required by Regulation S-X, Rule 10-01. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. For further information, refer to the consolidated financial statements and footnotes included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2020 as filed with the Securities and Exchange Commission ("SEC") on February 25, 2021. These interim condensed consolidated financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company's financial position and results of operations for the periods presented. Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

Principles of Consolidation

The unaudited interim consolidated financial statements include the accounts of the Company and its wholly owned subsidiary as disclosed in Note 2, under the headings "Summary of Significant Accounting Policies" and "Basis of Presentation", within the "Notes to Consolidated Financial Statements" accompanying the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2020. Intercompany accounts and transactions have been eliminated.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, accrued expenses, stock-based compensation expense, and income taxes. The Company bases its estimates on historical experience and other market specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions. Certain reclassifications have been made to prior periods to conform to current period presentation.

Summary of Significant Accounting Policies

There have been no changes in the Company's significant accounting policies as described in Note 2, "Summary of Significant Accounting Policies," within the "Notes to Consolidated Financial Statements" accompanying the Company's Annual Report on Form 10-K for the year ended December 31, 2020.

3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of September 30, 2021 and December 31, 2020 are as follows:

Assets	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
		(in thousands)		
September 30, 2021				
Money market funds included in cash and cash equivalents	$ 105,359	$ 105,359	$ —	$ —
Marketable securities - U.S. Treasury notes	10,024	10,024	—	—
Total	$ 115,383	$ 115,383	$ —	$ —
December 31, 2020				
Money market funds included in cash and cash equivalents	$ 103,992	$ 103,992	$ —	$ —
Marketable securities:				
U.S. Treasury notes	70,342	70,342	—	—
Equity securities	6,356	6,356	—	—
Total marketable securities	$ 76,698	$ 76,698	$ —	$ —
Warrants to purchase equity securities	3,816	—	3,816	—
Total	$ 184,506	$ 180,690	$ 3,816	$ —

The Company measures the fair value of money market funds, U.S. Treasury notes and equity securities based on quoted prices in active markets for identical securities. The Level 2 equity securities included warrants used to purchase equity securities that were valued using the Black-Scholes model. The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of expected term of the awards, (c) the risk-free interest rate, and (d) expected dividends. All warrants were exercised, and the shares of common stock received following exercise were subsequently sold, by the Company during the three months ended June 30, 2021.

4. Cash, cash equivalents, restricted cash, and available-for-sale marketable securities

Cash, cash equivalents, and marketable securities included the following at September 30, 2021 and December 31, 2020:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
		(in thousands)		
As of September 30, 2021				
Money market funds included in cash and cash equivalents	$ 105,359	$ —	$ —	$ 105,359
Marketable securities - U.S. Treasury notes	10,024	—		10,024
Total money market funds and marketable securities	$ 115,383	$ —	$ —	$ 115,383
As of December 31, 2020				
Money market funds included in cash and cash equivalents	$ 103,992	$ —	$ —	$ 103,992
Marketable securities:				
U.S. Treasury notes	70,348	—	6	70,342
Equity securities	1,220	5,136	—	6,356
Total marketable securities	$ 71,568	$ 5,136	$ 6	$ 76,698
Total money market funds and marketable securities	$ 175,560	$ 5,136	$ 6	$ 180,690

All of the Company's marketable debt securities as of September 30, 2021, have a contractual maturity of one year or less.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows:

	As of September 30, 2021	As of December 31, 2020
	(in thousands)	
Cash and cash equivalents	$ 111,475	$ 104,440
Restricted cash included in deposits and other non-current assets	1,779	1,779
Total cash, cash equivalents, and restricted cash	$ 113,254	$ 106,219

5. Accrued expenses

Accrued expenses as of September 30, 2021 and December 31, 2020 consist of the following:

	As of September 30, 2021	As of December 31, 2020
	(in thousands)	
Employee compensation costs	$ 5,862	$ 5,857
Research and development costs	4,068	6,624
Accrued goods and services	326	496
Professional services	1,068	1,228
Total	$ 11,324	$ 14,205

6. Lease obligation

Operating Leases

As of September 30, 2021, the Company has leases for office and lab space at 75 and 64 Sidney Street in Cambridge, Massachusetts through November 30, 2026.

In March 2020, the Company entered into an agreement to lease additional laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031. The Company gained control of and occupied the space in November 2020.

In September 2021, the Company entered into an agreement with BioNTech US, Inc. to sublease part of the office and lab space leased by the Company at 75 Sidney Street in Cambridge, Massachusetts (the "Sublease Agreement"). The sublease term is for approximately 3.3 years and the Company expects to receive $8.5 million from the sublessee over the term of the sublease. The sublease did not relieve the Company of its original obligation under the lease, and therefore the Company did not adjust the operating lease right-of-use asset as a result of the sublease and accounted for the sublease as a separate lease. Sublease payments received are classified within operating expenses to offset the related operating lease payments.

The Company received leasehold improvement incentives from the landlord totaling $5.3 million for the 75 Sidney Street and 64 Sidney Street leases and $5.6 million of leasehold improvement incentives from the landlord for the 75 Hayden Avenue lease. The leasehold improvements have been capitalized as fixed assets and the Company recorded the incentives as a component of its right-of-use assets and is amortizing them as a reduction of lease expense over the respective lease terms.

The Company's lease agreements require the Company to maintain a cash deposit or irrevocable letter of credit in the aggregate amount of $1.8 million payable to its landlords as security for the performance of its obligations under

the leases. These amounts are recorded as restricted cash and included in deposits and other non-current assets in the accompanying condensed consolidated balance sheets.

The following table summarizes the Company's significant contractual obligations under operating leases as of payment due date by period as of September 30, 2021:

	Total Minimum Lease Payments
	(in thousands)
2021 (remainder of year)	2,117
2022	8,698
2023	8,958
2024	9,227
2025	9,644
Thereafter	19,945
Total future minimum lease payments	$ 58,589
Less: imputed interest	(13,330)
Total lease liability	$ 45,259
Reported as:	
Other current liabilities	$ 5,248
Other non-current liabilities	40,011
Total lease liability	$ 45,259

During the three and nine months ended September 30, 2021, the Company incurred lease expense of $1.7 million and $5.4 million, respectively, for operating leases. During the three and nine months ended September 30, 2020, the Company incurred lease expense of $1.6 million and $5.0 million, respectively, for operating leases. As of September 30, 2021, the weighted average remaining lease term was 6.2 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 7.9%.

The following table summarizes the operating sublease income generated under the Sublease Agreement for the three and nine months ended September 30, 2021 and 2020:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating sublease income	*(in thousands)*			
	$ 204	$ —	$ 204	$ —

7. Other liabilities

As of September 30, 2021 and December 31, 2020, other current and non-current liabilities consisted of the following:

	As of September 30, 2021	As of December 31, 2020
	(in thousands)	
Other current liabilities		
Lease liability	5,248	4,198
Total other current liabilities	$ 5,248	$ 4,198
Other non-current liabilities		
Lease liability	$ 40,011	$ 43,409
Other	1,001	1,001
Total other non-current liabilities	$ 41,012	$ 44,410

Strategic Restructuring

On August 6, 2021, the board of directors of the Company approved a strategic restructuring plan to eliminate a portion of its workforce as part of an initiative to reduce expenses and enhance operations. The strategic restructuring plan was approved in connection with its portfolio reevaluation efforts and its strategic shift to invest additional resources in the Company's novel capsid development efforts.

During the three and nine months ended September 30, 2021, the Company incurred restructuring costs of approximately $2.0 million, which consists of severance-related costs. Approximately $0.9 million of these restructuring costs were paid as of September 30, 2021.

8. Commitments and contingencies

Significant Agreements

Neurocrine Collaboration Agreement

Summary of Agreement

In January 2019, the Company signed the Neurocrine Collaboration Agreement for the research, development and commercialization of certain of its AAV gene therapy products. The Neurocrine Collaboration Agreement became effective in March 2019. Under the Neurocrine Collaboration Agreement, the Company agreed to collaborate on the conduct of four collaboration programs (the "Neurocrine Programs") which include: (i) the VY-AADC (NBIb-1817) for Parkinson's disease (the "VY-AADC Program"), (ii) the VY-FXN01 for Friedreich's ataxia (the "FA Program") (collectively with the VY-AADC Program, the "Legacy Programs"), and (iii) two programs to be determined by the Company and Neurocrine at a later date (the "Discovery Programs").

In June 2019, in conjunction with the termination of the collaboration agreement with Sanofi Genzyme (the "Sanofi Genzyme Collaboration Agreement"), the Company gained ex-U.S. rights to the FA Program. The Company's ex-U.S. rights to the FA Program were subsequently transferred to Neurocrine under the terms of the Neurocrine Collaboration Agreement. To facilitate the transfer of the ex-U.S. rights to the FA Program to Neurocrine, the Company and Neurocrine executed an amendment to the Neurocrine Collaboration Agreement (the "June 2019 Modification"), and Neurocrine paid $5.0 million to the Company. There were no other changes in pricing or scope of the obligations required to be performed under the Neurocrine Collaboration Agreement.

In February 2021, Neurocrine notified the Company that it had elected to terminate the Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program, effective August 2, 2021 (the "Neurocrine VY-AADC Program Termination Effective Date"). The Neurocrine Collaboration Agreement remains in full force and effect

for each other program thereunder. As a result of the termination, subsequent to the Neurocrine VY-AADC Program Termination Effective Date, Neurocrine is no longer be obligated to reimburse the Company for research and development activities related to the VY-AADC Program.

Under the terms of the Neurocrine Collaboration Agreement, the Company originally agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of its intellectual property rights, for all human and veterinary diagnostic, prophylactic, and therapeutic uses, for the research, development, and commercialization of gene therapy products (the "Collaboration Products") on a worldwide basis under (i) the VY-AADC Program; (ii) the FA Program; and (iii) each Discovery Program. As a result of the termination of the Neurocrine Collaboration Agreement with regards to the VY-AADC Program, in accordance with the terms of the Neurocrine Collaboration Agreement, the licenses granted by the Company to Neurocrine regarding the VY-AADC Program have expired, and the Company has regained worldwide intellectual property rights regarding the VY-AADC Program, in each case as of the VY-AADC Termination Effective Date.

Pursuant to development plans agreed by the parties, which are overseen by a joint steering committee ("JSC"), the Company has operational responsibility, subject to certain exceptions, for the conduct of each Neurocrine Program prior to the occurrence of a specified event for such Neurocrine Program (a "Transition Event"), as described below, and is required to use commercially reasonable efforts to develop the corresponding Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by the Company in conducting these activities for each Neurocrine Program, in accordance with an agreed budget for each Neurocrine Program. If the Company breaches its development responsibilities or in certain circumstances upon a change in control, Neurocrine has the right but not the obligation to assume the activities under such Neurocrine Program.

Upon the occurrence of a Transition Event for each Neurocrine Program, Neurocrine has agreed to assume responsibility for development, manufacturing and commercialization activities for such Neurocrine Program from the Company and to pay milestones and royalties on future net sales as described further below. As a result of Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the Transition Event with respect to the VY-AADC Program is no longer applicable. The Transition Events for the remaining programs are (i) with respect to the FA Program, the Company's receipt of topline data for the initial Phase 1 clinical trial for an FA Program product candidate; and (ii) with respect to each Discovery Program, the preparation by the Company and the approval by Neurocrine of an investigational new drug ("IND") application to be filed with the U.S. Food and Drug Administration (the "FDA") by Neurocrine for the first development candidate in such Discovery Program. For the FA Program, the Company was granted the option (the "FA Co-Co Option") to co-develop and co-commercialize the FA Program upon the occurrence of a specified event (a "FA Co-Co Trigger Event"). The Company agreed, upon its exercise of the FA Co-Co Option, to enter into a cost- and profit-sharing arrangement with Neurocrine (the "FA Co-Co Agreement"), and (i) jointly develop and commercialize the Collaboration Products for the FA Program ("FA Collaboration Products"), (ii) share in its costs, profits and losses, and (iii) forfeit certain milestones and royalties on net sales in the United States during the effective period of the FA Co-Co Agreement. The FA Co-Co Trigger Event is the receipt of topline data for the initial Phase 1 clinical trial for an FA Program product candidate.

Under the Neurocrine Collaboration Agreement, subject to exceptions specified therein, the Company and Neurocrine agreed that profits and losses under the Company's FA Co-Co Option would be allocated 60% to Neurocrine and 40% to the Company for any FA Collaboration Product. The parties agreed that FA Co-Co Agreement would provide the Company the right to terminate for any reason upon prior written notice to Neurocrine and Neurocrine the right to terminate in certain circumstances upon change of control.

The Company's research and development activities under the Neurocrine Collaboration Agreement are to be conducted pursuant to plans agreed to by the parties, on a program-by-program basis, and overseen by the JSC, as detailed in the Neurocrine Collaboration Agreement.

The parties have agreed on a list of up to eight target genes (the "Targets") from which Neurocrine had the right to nominate Targets for the two Discovery Programs. The Targets nominated for the Discovery Programs were approved by a consensus of the JSC or the executive officers.

The Neurocrine Collaboration Agreement provides for an upfront non-refundable payment of $115.0 million, as well as for aggregate development and regulatory milestone payments from Neurocrine to the Company for Collaboration Products under (i) the VY-AADC Program of up to $170.0 million; (ii) the FA Program of up to $195.0 million, and (iii) each of the two Discovery Programs of up to $130.0 million per Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for each Collaboration Product of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all Neurocrine Programs of $1.1 billion. As a result of Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the Company is no longer entitled to receive the development, regulatory and commercial milestone payments related to the VY-AADC Program upon the achievement of specified milestones. Furthermore, in connection with the Neurocrine Collaboration Agreement, Neurocrine purchased 4,179,728 shares of the Company's common stock at a price of $11.9625 per share, for an aggregate purchase price of $50.0 million.

Neurocrine also agreed to pay the Company royalties, based on future net sales of the Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (i) for the VY-AADC Program, from the mid-teens to low thirties and the low-teens to low twenties, respectively; (ii) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (iii) for each Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a Collaboration Product and terminate on the later of (a) the expiration of the last patent covering the Collaboration Product or its method of use in such country, (b) ten years from the first commercial sale of the Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country (the "Royalty Term"). Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patents rights related to a Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any Collaboration Product. As a result of Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the Company is no longer entitled to receive royalties related to the VY-AADC Program. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the Royalty Term applicable to such Collaboration Product in such country.

Under the terms of the Neurocrine Collaboration Agreement and subject to specified exceptions therein, each party owns the entire right, title and interest in and to all intellectual property rights made solely by its employees or agents in the course of the collaboration. The parties jointly own all rights, title and interest in and to all intellectual property rights made or invented jointly by employees or agents of both parties.

During the term of the Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly exploit any AAV-based gene therapy products directed to a Target to which a Collaboration Product is directed, subject to specified exceptions including the parties' conduct of basic research activities.

Unless earlier terminated, the Neurocrine Collaboration Agreement expires on the later of (i) the expiration of the last to expire royalty term with respect to a Collaboration Product in all countries in the relevant territory or (ii) the expiration or termination of any FA Co-Co Agreement. Neurocrine may terminate the Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least (x) 180-day advance notice if such notice is provided prior to the first commercial sale of the Collaboration Product to which the termination applies or (y) one-year advance notice if such notice is provided after the first commercial sale of the Collaboration Product to which the termination applies. The Company may terminate the Neurocrine Collaboration Agreement, subject to specified conditions, if Neurocrine challenges the validity or enforceability of certain of the Company's intellectual property rights. Subject to a cure period, either party may terminate the Neurocrine Collaboration Agreement in the event of a material breach by the other party in whole or in part, subject to specified conditions.

Upon termination in certain cases, Neurocrine has agreed to grant to the Company licenses to certain Neurocrine intellectual property, subject to a negotiation between the parties to establish royalty rates for use of such intellectual property. In the event of a breach by the Company with respect to a Neurocrine Program, if such termination were to occur after a Transition Event, then (i) with respect to the FA Program, if an FA Co-Co Agreement is in effect, Neurocrine can terminate the FA Co-Co Agreement for such program and the Company would no longer have co-

development and co-commercialization rights with respect to the FA Collaboration Products and (ii) subject to any license agreements, Neurocrine would no longer have any obligations with respect to any Collaboration Products resulting from such program.

Termination of VY-AADC Program

As described above, as of the Neurocrine VY-AADC Program Termination Effective Date, the license granted by the Company to Neurocrine thereunder regarding the VY-AADC Program expired, the Company regained worldwide intellectual property rights regarding the VY-AADC Program, and the restrictions on the Company to develop, manufacture or commercialize a gene therapy product directed to the Target of the VY-AADC Program terminated, in each case in accordance with the terms of the Neurocrine Collaboration Agreement. As of the Neurocrine VY-AADC Program Termination Effective Date, Neurocrine no longer is obligated to reimburse the Company for research and development activities related to the VY-AADC Program, and the Company is no longer entitled to receive future milestone or royalty payments related to the VY-AADC Program. The Company intends to support Neurocrine, the study sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the Data Safety and Monitoring Board (the "DSMB"), and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial.

The Company continues to believe that the VY-AADC program may hold promise for Parkinson's disease patients as evidenced by the positive multi-year safety and efficacy data from the two Phase 1b clinical trials presented in September 2020 at the MDS Virtual Congress. As a result of portfolio reevaluation efforts and a strategic shift to invest in novel capsid development efforts, however, the Company has determined that it will not advance the VY-AADC Program on its own. The Company is exploring potential options for continuing the future development and commercialization of the VY-AADC Program as a partnered program.

Accounting Analysis

At inception, the Neurocrine Collaboration Agreement included the following performance obligations: (i) research and development services for each Legacy Program combined with a development and commercialization license for each such program and (ii) research and development services for each Discovery Program combined with a development and commercialization license for each program. The research services and license on a program by program basis are not distinct as Neurocrine cannot benefit from such license on its own or from other resources commonly available in the industry, without the corresponding research services due to the unique and specialized expertise of the Company that is not readily available in the marketplace.

The Company identified $92.4 million of fixed transaction price consisting of the $115.0 million upfront fee and $5.0 million payment from the June 2019 Modification, offset by a discount of $27.6 million related to the $50.0 million equity investment of 4,179,728 shares when measured at fair value on the date of issuance. The Company is also entitled to reimbursement of costs incurred by the Company prior to the Transition Events associated with each Neurocrine Program. These amounts are determinable based on program plans and budgets, and the Company has a contractual right to the payment of costs incurred under the agreed upon program plans. The Company utilized the most likely amount approach and estimated the expected cost reimbursement to be $431.1 million at inception. The Company concluded that these amounts do not require a constraint and are included in the transaction price at inception. The Company considers this estimate at each reporting date and updates the estimate based on information available. As of September 30, 2021, the estimate of the expected reimbursement is $284.7 million based on expectations as of such date. Additional consideration to be paid to the Company upon reaching certain milestones are excluded from the transaction price at inception due to the uncertainty of achieving the development and regulatory milestones.

The Company allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation or in the case of certain variable consideration to one or more performance obligations. The estimated standalone selling prices for performance obligations, that include a license and research services, were developed using the estimated selling price of the license, using comparable and market data, and an estimate of the overall effort to perform the research services along with a reasonable profit for research services.

The Company has concluded that the variable consideration related to the cost reimbursement of each program will be allocated to each respective program as the cost reimbursement relates specifically to the respective program services being performed under the Neurocrine Collaboration Agreement. The reimbursement of research services is considered to be at a market rate and the allocation of the fixed consideration to all of the performance obligations depicts the estimated amounts in which it would expect to receive for these obligations, absent the variable consideration related to the research reimbursement. The total variable consideration allocated to each program related to the expected cost reimbursement was as follows as of September 30, 2021:

Performance Obligation	Amount
	(in thousands)
Variable Consideration	
VY-AADC Program	$ 53,397
FA Program	88,533
Discovery Program 1	72,782
Discovery Program 2	70,040
Total	$ 284,752

Based on the relative standalone selling price allocation, the allocation of the transaction price, exclusive of the variable consideration allocated to the individual performance obligations, to the separate performance obligations was as follows:

Performance Obligation	Amount
	(in thousands)
Fixed Consideration	
VY-AADC Program	$ 49,045
FA Program	20,647
Discovery Program 1	14,443
Discovery Program 2	8,247
Total	$ 92,382

The Company recognizes the transaction price associated with each performance obligation on a proportional performance basis over the period of service using input-based measurements such as costs incurred to date, to estimate proportion performed, and remeasures its progress towards completion at the end of each reporting period.

The Company determined the partial termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program represented a modification of the arrangement under ASC 606 and that the remaining fixed transaction price at the Neurocrine VY-AADC Program Termination Effective Date of $42.2 million should be re-allocated to the FA Program and Discovery Program 1 and 2 based on their standalone selling prices. Accordingly, the Company recorded a cumulative adjustment to revenue of approximately $0.9 million on the partially satisfied remaining performance obligations, as the remaining services to be performed under each of the performance obligations are not distinct from the services prior to the modification. The Company determined that reasonable changes to the Company's estimates of standalone selling prices for the FA Program, Discovery Program 1 and Discovery Program 2 performance obligations did not have a material impact on the re-allocation or the amount of revenue recorded pursuant to the cumulative catch-up adjustment.

During the three and nine months ended September 30, 2021, the Company recognized $1.5 million and $9.3 million, respectively, of revenue associated with its collaboration with Neurocrine related to research and development services performed during the period and the corresponding cost reimbursement receivable. During the three and nine months ended September 30, 2020, the Company recognized $11.2 million and $50.1 million of revenue, respectively, associated with its collaboration with Neurocrine related to research and development services performed during the period and the corresponding cost reimbursement receivable.

The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities during the nine months ended September 30, 2021:

	Balance at December 31, 2020	Additions	Deductions	Balance at September 30, 2021
		(in thousands)		
Related party collaboration receivable	$ 8,012	$ 6,418	$ (13,265)	$ 1,165
Contract liabilities:				
Deferred revenue	$ 43,689	$ -	$ (4,512)	$ 39,176

The change in the receivable balance for the nine months ended September 30, 2021 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected from Neurocrine during the period.

As of September 30, 2021, there was $39.2 million of deferred revenue related to the Neurocrine Collaboration Agreement, which is classified as either current or non-current in the accompanying condensed consolidated balance sheet based on the period the services are expected to be delivered.

Costs incurred relating to the Company's collaboration programs under the Neurocrine Collaboration Agreement consist of internal and external research and development costs, which primarily include: salaries and benefits, lab supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's condensed consolidated statements of operations during the three and nine months ended September 30, 2021.

The Company incurred approximately $0.8 million of costs to obtain the Neurocrine Collaboration Agreement which were payable only upon the close of the deal and therefore considered incremental costs of obtaining a contract with a customer and capitalized. The costs are recorded in prepaid expenses and other non-current assets and are being amortized over the period in which the research services will be provided.

Other Agreements

The Company has entered into various agreements with contract research organizations and institutions to license intellectual property. In consideration for the rights licensed under such agreements, the Company generally made upfront payments, which were recorded as research and development expense as the acquired technologies were considered in-process research and development. The license agreements obligate the Company to make additional payments that are contingent upon specific clinical trial and regulatory approval milestones being achieved as well as royalties on future product sales. The agreements to license intellectual property include potential milestone payments that are dependent upon the development of products licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. The Company reached a milestone related to first patient dosing on the RESTORE-1 Phase 2 clinical trial which resulted in a $0.1 million milestone payment to one of its licensors in 2019. The Company can generally terminate the license agreements upon 30 to 90 days' prior written notice.

Additionally, certain license agreements require the Company to reimburse the licensor for certain past and ongoing patent related expenses.

During the year ended December 31, 2016, the Company entered into a research and development funding arrangement with a non-profit organization that provides up to $4.0 million in funding to the Company upon the achievement of clinical and development milestones. The agreement provides that the Company repay amounts received under certain circumstances including termination of the agreement, and to pay an amount up to 2.6 times the funding received upon successful development and commercialization of any products developed. During the year ended December 31, 2017, the Company earned a milestone payment of $1.0 million. The Company has evaluated the arrangement and has concluded that it represents a research and development financing arrangement as it is probable that

the Company will repay amounts received under the arrangement. As a result, the $1.0 million earned to date is recorded as a non-current liability in the condensed consolidated balance sheet.

Litigation

Except as described below, the Company was not a party to any material legal matters or claims as of September 30, 2021 or December 31, 2020. The Company did not have contingency reserves established for any litigation liabilities as of September 30, 2021 or December 31, 2020.

On January 22, 2021, a putative class action lawsuit was filed in the U.S. District Court for the Eastern District of New York against the Company and certain of its current and former officers and directors, captioned *Karp v. Voyager Therapeutics, Inc. et al.*, No. 1:21-cv-00381. The complaint generally alleged that the defendants violated Sections 10(b) and/or 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder by making material misstatements or omissions concerning the Company's Huntington's disease program and the Company's investigational new drug application for VY-HTT01. On April 19, 2021, the court appointed the lead plaintiff for the action, and on April 30, 2021, the action was transferred to the U.S. District Court for the District of Massachusetts (where it was assigned case number 1:21-cv-10727). On July 2, 2021, the lead plaintiff voluntarily dismissed the action without prejudice against all defendants and as to all claims. This matter is no longer pending.

9. Stock-based compensation

Stock-Based Compensation Expense

Total compensation cost recognized for all stock-based compensation awards in the condensed consolidated statements of operations and comprehensive income (loss) is as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(in thousands)			
Research and development	$ 702	$ 1,524	$ 3,409	$ 4,973
General and administrative	1,375	2,008	6,261	6,459
Total stock-based compensation expense	$ 2,077	$ 3,532	$ 9,670	$ 11,432

Stock-based compensation expense by type of award included within the condensed consolidated statements of operations and comprehensive income (loss) was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(in thousands)			
Stock options	$ 1,372	$ 2,696	$ 6,375	$ 8,834
Restricted stock awards and units	651	740	3,018	2,345
Employee stock purchase plan awards	54	96	277	252
Total stock-based compensation expense	$ 2,077	$ 3,532	9,670	11,432

Restricted Stock Units

A summary of the status of and changes in unvested restricted stock unit activity under the Company's equity award plans for the nine months ended September 30, 2021 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2020	638,471	$ 12.74
Granted	1,264,651	$ 6.20
Vested	(308,196)	12.88
Forfeited	(647,661)	$ 9.08
Unvested restricted stock units as of September 30, 2021	947,265	$ 7.52

Stock-based compensation of restricted stock units is based on the fair value of the Company's common stock on the date of grant and is recognized over the vesting period. Restricted stock units granted by the Company typically vest in equal amounts, annually over three years. In the nine months ended September 30, 2021, the Company granted 534,651 restricted stock units vesting in equal amounts, annually over three years, and 730,000 restricted stock units vesting in equal amounts, annually over two years. All of the restricted stock units granted in the nine months ended September 30, 2020 vest in equal amounts, annually over three years. The stock-based compensation expense related to restricted stock units and awards was $0.7 million and $3.0 million for the three and nine months ended September 30, 2021, respectively. The stock-based compensation expense related to restricted stock units and awards was $0.7 million and $2.3 million for the three and nine months ended September 30, 2020, respectively.

As of September 30, 2021, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $0.3 million, which is expected to be recognized over the remaining average vesting period of 1.7 years.

Stock Options

The following is a summary of stock option activity for the nine months ended September 30, 2021:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value *(in thousands)*
Outstanding at December 31, 2020	5,485,078	$ 14.77		
Granted	1,411,929	$ 6.57		
Exercised	(3,811)	$ 7.27		
Cancelled or forfeited	(1,487,124)	$ 13.41		
Outstanding at September 30, 2021	5,406,072	$ 13.01	6.0	$ —
Exercisable at September 30, 2021	3,750,020	$ 14.48	4.7	$ —

As of September 30, 2021, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $9.4 million which is expected to be recognized over the remaining weighted-average vesting period of 2.4 years.

10. Net loss per share

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net loss per share because to include them would be anti-dilutive:

	As of September 30,	
	2021	2020
Unvested restricted common stock awards	156,863	117,647
Unvested restricted common stock units	947,265	—
Outstanding stock options	5,406,072	4,013,244
Total	6,510,200	4,130,891

Basic net income (loss) and diluted weighted-average shares outstanding are as follows for the three and nine months ended September 30, 2021 and 2020.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(in thousands, except share data)			
Numerator:				
Net (loss) income	$ (25,137)	$ 85,611	$ (76,906)	$ 52,667
Denominator for basic net (loss) income per share:				
Weighted average shares outstanding-basic	37,780,547	37,242,504	37,623,309	37,079,242
Denominator for diluted net (loss) income per share:				
Weighted average shares outstanding	37,780,547	37,242,504	37,623,309	37,079,242
Common stock options and restricted stock units	—	429,824	—	420,913
Weighted average shares outstanding-diluted	37,780,547	37,672,328	37,623,309	37,500,155

Basic net income (loss) per share for the three and nine months ended September 30, 2021, is the same as diluted net income (loss) per share as shown on the Company's condensed consolidated statement of operations.

11. Related-party transactions

During the three and nine months ended September 30, 2021 and 2020, the Company received board and scientific advisory services from one of its prior executives, Dinah Sah, Ph.D., the Company's former Chief Scientific Officer. The total amount of fees paid to Dr. Sah for services provided during the three and nine months ended September 30, 2021, was $30,000 and $120,000, respectively. The total amount of fees paid to Dr. Sah for services provided during the three and nine months ended September 30, 2020 was $0.2 million and $0.3 million, respectively.

During the three and nine months ended September 30, 2021, the Company received consulting and advisor services from Third Rock Ventures. The total fees paid was de minimus.

Under the Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to conduct research, development and commercialization activities for certain of the Company's AAV gene therapy products (Note 8). Amounts due from Neurocrine are reflected as related party collaboration receivables. As of September 30, 2021, the Company had approximately $1.2 million in related party collaboration receivables associated with Neurocrine.

12. Subsequent Events

On October 1, 2021, the Company entered into an option and license agreement with Pfizer pursuant to which the Company has granted Pfizer options to receive an exclusive license to novel capsids generated from the Company's TRACER screening technology to develop and commercialize certain AAV gene therapy candidates comprised of a

novel capsid and specified transgenes to help treat respective central nervous system and cardiovascular diseases. Under the terms of this agreement, the Company received an upfront payment of $30.0 million and is eligible to receive future option exercise payments of $10.0 million upon each of up to two option exercises; specified development, regulatory, and commercialization milestone payments following each option exercise of up to an aggregate of $115.0 million for the first licensed product to achieve such milestones; specified sales milestone payments of up to an aggregate of $175.0 million per licensed product; and tiered, escalating royalties in the mid- to high-single digit percentages of annual net sales of each licensed product.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2020, which was filed with the Securities and Exchange Commission, or the SEC, on February 25, 2021.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report on Form 10-Q. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, including those risks identified under "Part II, Item 1A-Risk Factors."

These forward-looking statements are made under the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. These statements are neither promises nor guarantees. We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Overview

We are a gene therapy company focused on developing life-changing treatments and next-generation platform technologies. We focus on diseases where we believe a single dose adeno-associated virus, or AAV, gene therapy approach that either increases or decreases the production of a specific protein can either halt or slow disease progression or reduce symptom severity, therefore providing clinically meaningful impact to patients. Our gene therapy platforms enable us to engineer, optimize, manufacture and deliver AAV-based gene therapies that we believe have the potential to safely provide durable efficacy. Our team of experts in the fields of AAV gene therapy and neuroscience first identifies and selects diseases that are well-suited for treatment using AAV gene therapy. We then engineer and optimize AAV vectors for delivery of the virus payload to the targeted tissue or cells. We believe our single dose gene therapies have the potential to be delivered directly, with targeted infusions or systemically, in conjunction with our novel capsids.

We are identifying novel AAV capsids, the outer viral protein shells that enclose genetic material of a virus payload. Our team has developed a proprietary system called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) to facilitate the selection of AAV capsids with blood brain barrier, or BBB, crossing and cell-specific transduction properties for particular therapeutic applications. The TRACER system is a broadly-applicable, functional RNA-based AAV capsid screening platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates (NHPs). We are also applying the TRACER system towards further capsid variant libraries and selection for tropism and transduction in additional cell and tissue types, such as cardiac and skeletal muscle. We are actively engaged in discussions with multiple parties to make novel AAV capsids identified and developed under the TRACER system available to third parties for use in their drug development programs through potential licensing and other arrangements.

Our quality and manufacturing processes employ an established system capable of enabling production of high quality AAV vectors at clinical scale. In addition to our capsid screening platform, we have developed a vectorized antibody platform which we believe will overcome many of the challenges of passive immunization.

Our business strategy focuses on discovering, developing, manufacturing and commercializing our gene therapy programs. As part of this strategy, we have developed core competencies specific to AAV gene therapy development and manufacturing. This business strategy also includes business development activities that may include in-licensing activities or partnering certain programs in specific geographies with collaborators, as we have demonstrated through our ongoing collaboration with Neurocrine Biosciences, Inc., which we refer to as Neurocrine, or out-licensing activities including license agreements related to our novel capsids such as our October 2021 licensing agreement with Pfizer Inc., which we refer to as Pfizer. We believe there is significant opportunity for out-licensing transactions related to the novel capsids identified by our TRACER system. To maximize the potential of novel capsids identified by our TRACER system for our own programs and out-licensing transactions, we have retained to date, and expect to retain in the future, all rights associated with such novel capsids other than the rights specific to their use in combination with a particular licensee's transgenes. Since our inception, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio, determining which neurological diseases to pursue, advancing our product candidates including delivery and manufacturing, and conducting preclinical studies and early-phase clinical trials. We do not have any product candidates approved for sale and have not generated any revenue from product sales.

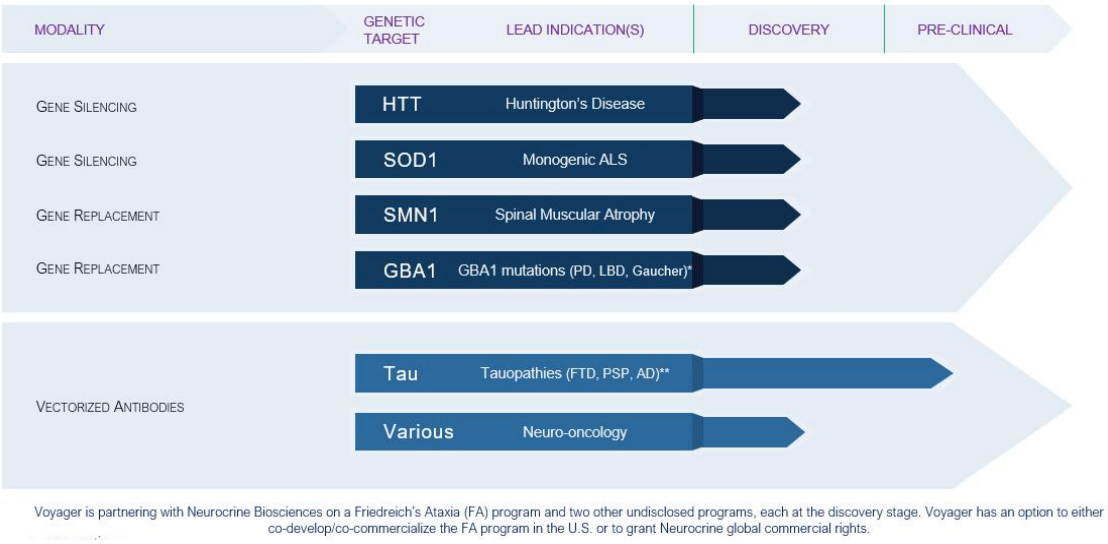
We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings of our common stock, and our strategic collaborations, including our prior collaboration with Sanofi Genzyme Corporation, or the Sanofi Genzyme Collaboration, which commenced in February 2015 and was terminated in June 2019, our prior collaboration with AbbVie Biotechnology Ltd. focusing on tau-related disease, or the AbbVie Tau Collaboration, which commenced in February 2018 and was terminated in August 2020, our prior collaboration with AbbVie Ireland Unlimited Company focusing on pathological species of alpha-synuclein, or the AbbVie Alpha-Synuclein Collaboration, which commenced in February 2019 and was terminated in August 2020, our ongoing collaboration with Neurocrine, which commenced in March 2019, and our licensing agreement with Pfizer, which commenced in October 2021. We refer to our collaboration agreement with Neurocrine as the Neurocrine Collaboration Agreement and the collaboration with Neurocrine as the Neurocrine Collaboration.

In August 2021, we initiated a strategic initiative and reevaluated our existing product candidate portfolio. As a result of this reevaluation, we intend to invest additional resources in our novel capsid screening technology to expand discovery of novel capsids with broad tissue tropism in CNS, cardiac and skeletal tissues. We also plan to advance innovative gene therapy programs that leverage these novel capsids as well as our vectorized antibody technology.

As a result of these efforts, we have determined that we will not advance our VY-AADC program for the treatment of Parkinson's disease on our own. Additionally, to leverage our novel capsid development efforts, we have decided to discontinue our current first-generation Huntington's disease program and to initiate a second-generation program leveraging a novel, proprietary AAV capsid that may enable intravenous administration and achieve broad distribution to affected tissue. We have also initiated gene therapy programs using our novel capsids in treatment programs for monogenic amyotrophic lateral sclerosis, or ALS; spinal muscular atrophy, or SMA; and various diseases linked to GBA1 mutations, including Parkinson's disease, Lewy body dementia and Gaucher's disease. Additionally, we

continue to advance our vectorized antibody platform capability with programs for tauopathies and indications in neuro-oncology. We continue to partner with Neurocrine on programs for diseases including Friedreich's ataxia. All of our current product candidates are in the early stages of development. We continue to evaluate additional diseases that could be treated using AAV gene therapy and are also actively exploring additional potential treatment methods that can utilize our proprietary novel capsids.

Our pipeline of early-stage gene therapy programs is summarized in the table below:



As part of the Neurocrine Collaboration, we and Neurocrine have been developing VY-AADC (NBIb-1817) for the treatment of Parkinson's disease, or the VY-AADC Program. The FDA has granted VY-AADC (NBIb-1817) its regenerative medicine advanced therapy, known as RMAT, designation, which provides for an enhanced level of interactions between the company sponsor and the FDA throughout a development program, and has granted VY-AADC (NBIb-1817) fast-track designation.

VY-AADC (NBIb-1817) is currently being evaluated in the RESTORE-1 Phase 2 clinical trial. In December 2020, the FDA notified Neurocrine that the FDA had placed a clinical hold on the RESTORE-1 Phase 2 trial. In February 2021, Neurocrine notified us of its termination of the Neurocrine Collaboration with regards to the VY-AADC Program, effective August 2, 2021.

We continue to believe that the VY-AADC program may hold promise for Parkinson's disease patients as evidenced by the positive multi-year safety and efficacy data from the two Phase 1b clinical trials presented previously. As a result of the portfolio reevaluation efforts and a strategic shift to invest in novel capsid development efforts, however, we have determined that we will not advance the VY-AADC program on our own and are currently evaluating potential options for partnering the future development and commercialization of the VY-AADC program.

VY-AADC (NBIb-1817) Phase 1 Clinical Development

We evaluated the delivery of VY-AADC (NBIb-1817) in a transfrontal (i.e., top of the head) surgical delivery route in a Phase 1b clinical trial, which we refer to as PD-1101, and we are exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory (i.e., back of the head) surgical delivery route in a Phase 1 clinical trial, which we refer to as PD-1102. PD-1101 was an open-label, dose-ranging, Phase 1b clinical trial for VY-AADC (NBIb-1817) to evaluate safety and efficacy. We enrolled 15 patients with advanced Parkinson's disease and assessed increased volume or concentration of VY-AADC (NBIb-1817) in three separate cohorts consisting of five patients in each cohort. PD-1102 is a separate, open-label Phase 1 clinical trial for VY-AADC (NBIb-1817) to evaluate the posterior trajectory that enrolled

eight patients with advanced Parkinson's disease. We have completed PD-1101, and have completed three-year follow-up for patients in PD-1102. Data to date from both trials demonstrate that VY-AADC (NBIb-1817) has been generally well-tolerated and that administration with VY-AADC (NBIb-1817) resulted in stable or improved motor function and quality of life as measured by standard scores and measures used in Parkinson's disease trials.

VY-AADC (NBIb-1817) RESTORE-1 Program

In connection with our continued clinical development of VY-AADC (NBIb-1817), we sought to shift from production using a mammalian cell system consisting of triple-transfection of HEK293 cells, which was used in our two Phase 1 clinical trials, to production using insect-derived cells and our baculovirus/Sf9 manufacturing process. We designed our baculovirus/Sf9 manufacturing process to produce AAV vectors at clinical and commercial scale, with the potential for increased yields and more efficient scalability compared with mammalian-based systems.

In December 2017, we submitted an IND to the FDA to evaluate VY-AADC (NBIb-1817) in the RESTORE-1 Phase 2 clinical trial, a randomized, double-blind, sham-surgery controlled trial evaluating the safety and efficacy of VY-AADC (NBIb-1817) for the treatment of moderate to advanced Parkinson's disease in patients with motor fluctuations. As part of the IND application for VY-AADC (NBIb-1817), the chemistry, manufacturing, and controls section included data demonstrating comparability between VY-AADC (NBIb-1817) produced using our baculovirus/Sf9 manufacturing process and VY-AADC (NBIb-1817) produced using the mammalian cell system. In each case, the VY-AADC was produced under cGMP. The data demonstrated that this production platform change resulted in comparable vector quality and activity. As a result, we decided to use VY-AADC (NBIb-1817) manufactured in our baculovirus/Sf9 system in the RESTORE-1 Phase 2 clinical trial.

In December 2018, we announced randomization of the first patient in the RESTORE-1 Phase 2 clinical trial. We received written feedback from the FDA, including FDA guidance received during the Type B meeting, that in a disease such as Parkinson's, two adequate and well-controlled clinical trials are suggested. Based upon feedback received from the FDA, we and Neurocrine amended the RESTORE-1 clinical trial protocol to support a potential future registration filing for VY-AADC (NBIb-1817) for the treatment of Parkinson's disease in the United States. The protocol amendments included increasing the planned enrollment to approximately 85 patients from the previously planned 42 patients and adjusting future enrollment in the trial to randomize patients 2:1 to VY-AADC (NBIb-1817) or sham-surgery, respectively, as compared to the previous 1:1 randomization. The eligibility criteria remained substantially the same: the trial is potentially available to patients who have been diagnosed with Parkinson's disease for at least four years, are not responding adequately to oral medications, and have at least three or more hours of OFF time during the day as measured by a validated self-reported patient diary. The protocol amendments were anticipated to facilitate enrollment and patient convenience.

A dose of up to 3.6×10^{12} vector genomes, which we refer to as the maximum total bilateral dose, was selected for the RESTORE-1 Phase 2 clinical trial. This dose is between the maximum total vector genome doses administered in Cohorts 2 and 3 from PD-1101 when considering the higher volume administered with the posterior trajectory and vector produced using the baculovirus system.

The primary efficacy endpoint of the RESTORE-1 Phase 2 clinical trial is the mean improvement from baseline to 12 months in good ON time as measured by a validated self-reported patient diary at 12 months compared to sham surgery. Secondary endpoints include mean improvement in diary OFF time, other motor function and quality of life measures from the UPDRS (UPDRS-II and -III scores), assessments from the Parkinson's Disease Questionnaire, or PDQ-39, and patient's global function as measured by the proportion of participants with improvement on the Clinical Global Impression, or CGI, score. The trial will also measure non-motor symptoms from the Non-Motor Symptom Scale, or NMSS, as well as safety.

Changes in patients' daily doses of oral levodopa and related medications will also be recorded. Biomarker data collected during the RESTORE-1 Phase 2 clinical trial includes measurements of the coverage of the putamen, the specific region of the brain targeted with VY-AADC (NBIb-1817), and measurements of AADC enzyme expression and activity in the putamen measured by positron emission tomography using 18-F-fluorodopa.

In November 2020, the sponsor medical monitor and surgical core requested that the DSMB for the RESTORE-1 Phase 2 clinical trial, review certain patient MRI abnormalities observed in some clinical trial participants in the ongoing clinical trial. Following this review, the DSMB requested additional information about magnetic resonance imaging abnormalities observed in trial participants and recommended a pause in the dosing of patients in the RESTORE-1 Phase 2 clinical trial pending review by the DSMB of these additional data. The DSMB informed Neurocrine that patient screening could continue for the trial and that the trial should remain blinded. Trial sites participating in the RESTORE-1 clinical trial were not screening, enrolling, or dosing patients at the time of this DSMB request as a result of the COVID-19 pandemic. In response to the DSMB's recommendation to pause the dosing of patients, we and Neurocrine decided to delay the planned resumption of patient screening in the RESTORE-1 Phase 2 clinical trial until Neurocrine had submitted the required expedited IND safety report related to these matters and the DSMB was able to complete its evaluation.

In December 2020, the FDA notified Neurocrine that it had placed a clinical hold on the RESTORE-1 clinical trial. In January 2021, the FDA informed Neurocrine of the information required to provide a complete response to the FDA in connection with the clinical hold. Information required by the FDA includes an assessment of how the investigational product may have given rise to the adverse findings, a mitigation plan to manage the adverse findings, and supportive data to justify that a favorable benefit/risk profile remains for the product.

The DSMB met to review additional patient data in January 2021 and has characterized the MRI abnormalities observed in the RESTORE-1 Phase 2 clinical trial as having uncertain clinical significance. The DSMB requested that Neurocrine obtain and provide additional information on past and current patients in the VY-AADC (NBIb-1817) clinical program. The clinical implications of this observation are currently unknown and are being evaluated.

In February 2021, Neurocrine notified us of its decision to terminate the Neurocrine Collaboration with respect to the VY-AADC Program, effective August 2, 2021. The Collaboration Agreement remains in full force and effect for each other program thereunder. Upon the termination of the VY-AADC Program, the license granted by us to Neurocrine expired, we regained worldwide intellectual property rights to the VY-AADC Program in accordance with the collaboration agreement, and the restrictions on us to develop, manufacture or commercialize a gene therapy product directed to the specified target of the VY-AADC Program terminated. We intend to support Neurocrine, the study sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the DSMB and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial.

As stated above, we continue to believe that the VY-AADC program may hold promise for Parkinson's disease patients, but we have determined that we will not advance the VY-AADC program on our own. We are currently evaluating potential options for partnering the future development and commercialization of VY-AADC program.

Preclinical Pipeline Programs

We are pursuing additional product candidates in the early stages of development. As part of our portfolio reevaluation and a strategic shift to invest in novel capsid development efforts, we intend to focus on validated gene therapy targets that can be treated using AAV gene therapy through application of either a gene replacement or a gene knockdown approach utilizing our novel, proprietary capsids to enable intravenous administration or leveraging our vectorized antibody platform capabilities. We are currently evaluating treatment programs for Huntington's disease; monogenic amyotrophic lateral sclerosis, or ALS; tau-related neurodegenerative diseases; spinal muscular atrophy, or SMA; diseases linked to GBA1 mutations including Parkinson's disease, Lewy body dementia and Gaucher's disease; and indications in neuro-oncology. We are also evaluating treatment programs for Friedreich's ataxia and other diseases under the Neurocrine Collaboration Agreement. We continue to evaluate additional diseases that could be treated using AAV gene therapy and are also actively increasing our investment in our TRACER capsid screening platform technology to expand discovery of novel capsids with broad tissue tropism in CNS, cardiac, and skeletal tissues.

VY-HTT01 is our first-generation gene therapy candidate for the treatment of Huntington's disease. VY-HTT01 is composed of an AAV capsid (AAV1) and a proprietary transgene that harnesses the RNA interference pathway to selectively knock down, or reduce, levels of huntingtin, or HTT, mRNA.

In non-human primate studies, one-time administration of VY-HTT01 resulted in robust and durable reduction of HTT mRNA and protein with knock-down stabilization between six and twelve months, and widespread distribution of VY-HTT01 vector genome across the striatum and cortex, which are core areas of disease pathology. VY-HTT01 treatment demonstrated robust reduction of HTT mRNA and protein in the YAC128 and BACHD transgenic mouse models of Huntington's disease, with significant improvements in motor function.

In September 2020, we submitted an IND application to evaluate VY-HTT01 in a planned Phase 1/2 clinical trial in patients with Huntington's disease. In October 2020, the FDA placed a clinical hold on our IND application pending the resolution of certain chemistry, manufacturing and controls, or CMC, information requests, and we subsequently received written feedback from the FDA requesting additional information on specific CMC topics, including drug device compatibility and drug substance and product characterization. We provided our complete response to the additional requests from the FDA regarding the IND application for VY-HTT01 and, in April 2021, were notified that the FDA removed the clinical hold on VY-HTT01.

As part of our portfolio reevaluation and to leverage our novel capsid development efforts, we have decided to discontinue our current first-generation Huntington's disease program and not to proceed with the initiation of our planned Phase 1/2 clinical trial of VY-HTT01, which we referred to as VYTAL. We withdrew the IND for VYTAL. We have initiated a second-generation program for the treatment of Huntington's disease using a novel, proprietary AAV capsid that may enable intravenous administration and achieve broad distribution to affected tissue.

As part of the Neurocrine Collaboration, we are developing a gene therapy for the treatment of Friedreich's ataxia, a debilitating neurodegenerative disease resulting in poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing, and speech. We and Neurocrine are evaluating potential development candidates that will comprise a capsid, promoter, and FXN transgene. We are evaluating capsids that effectively transduce disease target tissues in non-human primates following intravenous injection. Criteria for evaluating these capsids include safety, the overall level of transgene expression achieved, and the anatomic and cellular distribution of the transgene expression. To evaluate the therapeutic potential of our vectors, we have conducted testing in a new genetic mouse model of Friedreich's ataxia. In this preclinical model of Friedreich's ataxia, our gene therapy candidates durably improved sensory function and rescued the disease phenotype based on multiple functional tests. In physiological and behavioral assays, our gene therapy candidates demonstrated dose-dependent and durable responses for more than 10 months after a single administration, preventing central and peripheral disease progression. As part of our portfolio reevaluation and strategic shift to invest in novel capsid development efforts, we and Neurocrine are evaluating the potential use of our proprietary novel capsids to allow for enhanced transduction across the disease target tissues. If we and Neurocrine successfully identify a lead candidate and an AAV capsid for this program and reach agreement on a product profile and product development program, we plan to complete IND enabling studies to evaluate its safety and efficacy.

In 2018, we began collaborating with AbbVie on the research and development of specified vectorized antibody compounds comprised of an AAV or other viral capsid and a virus vector genome that encodes one or more antibodies that target and bind to a tau protein, which we refer to as the AbbVie Tau Collaboration. In early 2019, we presented on the use of therapeutic antibodies targeting various forms of tau to prevent, reduce, or slow the development of tau pathology as an important potential therapeutic strategy for Alzheimer's disease and other tauopathies. Because of the blood-brain barrier, or BBB, only very low levels of antibody distribute to the brain from the systemic circulation after passive immunization, resulting in modestly reduced tau pathology in animal models. Separately, in 2019, we began collaborating with AbbVie on the research and development of specified vectorized antibody compounds comprised of an AAV or other viral capsid and a virus vector genome that encodes one or more antibodies and development against pathological species of alpha-synuclein for the potential treatment of Parkinson's disease and other synucleinopathies which we refer to as the AbbVie Alpha-Synuclein Collaboration. Our vectorized antibody approach aims to circumvent this limitation by delivering, with a potential one-time intravenous, or IV, administration, the genes that encode for the production of therapeutic antibodies utilizing our novel BBB-penetrant AAV capsids. This approach could potentially result in higher levels of therapeutic antibodies in the brain compared with current systemic administration of antibodies.

Both of our collaborations with AbbVie were terminated by AbbVie, effective as of August 3, 2020, or the AbbVie Collaboration Termination Date. As a result of such terminations, we were relieved of future research and

development obligations under each collaboration. Exclusivity provisions restricting either party or any of its respective affiliates from directly or indirectly exploiting any vectorized antibody compound targeting a tau protein or an alpha-synuclein protein and restricting us, alone or jointly with any third party, from directly or indirectly exploiting specified antibodies targeting a tau protein or contributed by AbbVie to the AbbVie Alpha-Synuclein Collaboration have also terminated. Each party retains a royalty-free, exclusive license to the other's interest in certain intellectual property rights developed by either party under the collaborations, or Joint IP, to exploit antibodies it contributed to each collaboration as well as royalty-free, non-exclusive licenses to Joint IP for any other purpose. Further, AbbVie has granted us, effective as of the AbbVie Collaboration Termination Date, in the case of the AbbVie Tau Collaboration, a worldwide, royalty-free, transferable, sublicensable (though multiple tiers), exclusive license to AbbVie's interest in Joint IP to exploit research compounds or product candidates that were investigated under the collaborations and do not encode antibodies contributed by AbbVie or include active pharmaceutical ingredients owned by AbbVie or its affiliates and, in the case of the AbbVie Alpha-Synuclein Collaboration, a worldwide, royalty-free, transferable, sublicensable (though multiple tiers), non-exclusive license to AbbVie's interest in Joint IP for any purpose, in each case for all human diagnostic, prophylactic and therapeutic uses. We are not obligated to repay the upfront payment we received from AbbVie in connection with entering into either collaboration agreement, but we are no longer eligible to receive option payments, milestone payments or royalties thereunder. We expect to continue to advance our research and development efforts related to vectorized antibodies, including vectorized antibody compounds comprised of an AAV or other viral capsid and a virus vector genome that encodes one or more antibodies that target and bind to a tau protein, and we are currently evaluating our options for advancing these efforts individually or with other potential collaborators. We are evaluating our options for potentially advancing our alpha-synuclein program in the future.

In addition to the programs described above, we continue to evaluate additional diseases that could be treated using AAV gene therapy through application of either a gene replacement or a gene knockdown approach and are also actively exploring additional potential treatment methods that can utilize an AAV vector. In early 2019, we presented on our discovery and development of novel AAV capsids that cross the BBB after IV administration with improved transduction of the brain and spinal cord and enhanced cellular specificity using libraries under the control of either the neuron-specific synapsin, or SYN, promoter or the astrocyte-specific glial fibrillary acidic protein, or GFAP, promoter to apply selective pressure for capsid variants that transduce the cell type of interest.

Novel Capsid Discovery

Our scientists have developed TRACER, a proprietary system to facilitate the selection of AAV capsids with BBB crossing and cell-specific transduction properties for particular therapeutic applications. In May 2021, we presented new data demonstrating that we have developed a series of novel AAV capsids which, following intravenous administration, achieve up to 1000-fold higher RNA expression in the brain and 100-fold higher expression in the spinal cord of non-human primates than AAV9, the current natural AAV serotype with the best ability to ability to cross the BBB. We also identified a capsid which displayed strong cardiac transduction and significant dorsal root ganglia detargeting in non-human primates, which may avoid toxicities associated with AAV9 delivery. We believe these capsids may allow for significantly enhanced gene delivery to specific types of cells in the brain at lower doses. These capsids are now in advanced stages of characterization for deployment in our gene therapy development programs. We are also applying the TRACER system towards further capsid variant libraries and selection for tropism and transduction in additional cell and tissue types, such as cardiac and skeletal muscle. We are actively engaged in discussions with multiple parties to make novel AAV capsids identified and developed under the TRACER system available to third parties for use in their drug development programs through potential licensing and other arrangements.

Pfizer Option and License Agreement

In connection with our continued development of the TRACER system, on October 1, 2021, we entered into an option and license agreement with Pfizer, which we refer to as the Pfizer Agreement, pursuant to which we have granted Pfizer options to receive an exclusive license, or the License Options, to novel capsids generated from the TRACER system to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes, which we refer to as the Pfizer Transgenes. Under the terms of the Pfizer Agreement, Pfizer intends to evaluate the potential use of the capsids in combination with up to two Pfizer Transgenes to help treat respective central nervous system and cardiovascular diseases.

Under the Pfizer Agreement, we have agreed to provide Pfizer with certain quantities of materials encoding specified existing capsids for Pfizer's evaluation. During the research term, which extends until October 1, 2022, or, in the event Pfizer exercises a License Option, until October 1, 2024, we may, at our sole discretion and expense, conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases. We have agreed to disclose to Pfizer, on a rolling basis, the performance characteristics identified during the research term for all such capsid candidates. Following such disclosure, Pfizer has the right, in its sole discretion, to select any capsid candidate for evaluation to determine its interest in exercising a License Option with respect to such capsid candidate. Pfizer may exercise up to two License Options, provided that it may exercise only one License Option for each Pfizer Transgene. We have granted Pfizer, effective upon Pfizer's exercise of a License Option, with respect to a capsid candidate for the Pfizer Transgene identified therein, an exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize the applicable licensed capsid as incorporated into products containing the corresponding Pfizer Transgene, or the Licensed Products. Additionally, upon such option exercise, we and Pfizer have agreed that we shall provide certain additional know-how that has not been previously provided to Pfizer to enable Pfizer to exploit such licensed capsid and the corresponding Pfizer Transgene for use in a Licensed Product. Pfizer may, during the research term, conduct additional evaluation of capsid candidates and has the right to substitute any other capsid candidate for the capsid it previously elected to license.

Subject to our obligations to disclose new capsids candidates and certain know-how, we and Pfizer have agreed to conduct our respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of us and Pfizer.

Under the Pfizer Agreement, Pfizer is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Licensed Products. In the event Pfizer exercises a License Option, Pfizer is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Licensed Product for each Pfizer Transgene for which Pfizer has exercised its License Option in (i) the United States and (ii) at least one of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan, each of which we refer to as a Major Market Country, subject to certain limitations. Pfizer is also required to use commercially reasonable efforts to commercialize each Licensed Product in the United States and at least one Major Market Country where Pfizer or its designated affiliates or sublicensees has received regulatory approval for such Licensed Product, subject to certain limitations.

We have agreed to provide to Pfizer materials encoding the existing capsid candidates for Pfizer's evaluation. During the research term, if we identify a new capsid candidate through a specified screening campaign that has not been previously identified and disclosed to Pfizer, we have agreed to, at Pfizer's request, provide plasmids to Pfizer for the production of such new capsid candidates for evaluation as requested by Pfizer. We have also granted Pfizer, effective upon an option exercise and in addition to its exclusive license under certain of our intellectual property described above, a non-exclusive license, on a licensed capsid-by-licensed capsid basis, under certain of our know-how to exploit the applicable licensed capsid as incorporated into Licensed Products containing the corresponding Pfizer Transgene.

Under the terms of the Pfizer Agreement, Pfizer agreed to pay us an upfront payment of $30.0 million. We received this upfront payment in October 2021. Pfizer has also agreed to pay us, upon each option exercise, a fee of $10.0 million. Following each option exercise with respect to a Pfizer Transgene, we are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115.0 million for the first corresponding Licensed Product to achieve the corresponding milestone. On a Licensed Product-by-Licensed Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175.0 million per Licensed Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Licensed Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Under the terms of the Pfizer Agreement, each of us and Pfizer owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the effective date of the Pfizer Agreement, or invented, developed, created, generated or acquired solely by or on behalf of such party after such effective date.

Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Pfizer Agreement and in the course of our and Pfizer's activities under the Pfizer Agreement will follow inventorship under U.S. patent law.

Subject to certain limitations and exceptions, we have agreed (i) during the research term, not to conduct any internal program or program on behalf of a third party that is directed to development or commercialization of any capsid candidates, or grant any third party or affiliate any right or license under our rights in such capsid candidates to exploit any therapeutic product, in combination with any Pfizer Transgene in any indication for therapeutic, diagnostic and prophylactic human and veterinary use; and (ii) after Pfizer's exercise of a License Option, not to grant any third party or affiliate any right or license under our patents to exploit any licensed capsid in combination with any Pfizer Transgene.

Unless earlier terminated, the Pfizer Agreement expires on the earlier to occur of (i) the first anniversary of the effective date of the Pfizer Agreement, if no License Option is exercised, and (ii) the expiration of the last-to-expire royalty term with respect to all Licensed Products in all countries if at least one License Option is exercised. Subject to a cure period, either party may terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Pfizer may also terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, for our insolvency, the occurrence of a violation of global trade control laws, or for our non-compliance with certain anti-bribery or anti-corruption covenants. Pfizer may also terminate the Pfizer Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Pfizer, the licenses that we have granted to Pfizer under the Pfizer Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Pfizer under such licenses had the Pfizer Agreement remained in effect would be substantially reduced.

We believe there is significant opportunity for out-licensing transactions related to the novel capsids identified by our TRACER system. To maximize the potential of novel capsids identified by our TRACER system for our own programs and out-licensing transactions, we have retained to date, and expect to retain in the future, all rights associated with such novel capsids other than the rights specific to their use in combination with a particular licensee's transgenes.

Devices

Finally, we have developed our own real-time, intra-operative, MRI compatible device, the variable trajectory array guide, or V-TAG™, that can be used with other neuro-navigational systems for the administration of drugs via direct intraparenchymal delivery and other surgical procedures, to avoid blood vessels and reduce the risk of potential hemorrhage during surgery, and to maximize drug coverage of the targeted structures. In July 2018, the Center for Devices and Radiological Health, or the CDRH, of the FDA provided 510(k) clearance for V-TAG. We have worked with ClearPoint Neuro, Inc. (formerly known as MRI Interventions, Inc.), or CLPT, on manufacturing of the device for relevant clinical trials, and in March 2019, we transferred our premarket notification (510(k)) clearance for V-TAG to CLPT. Investigators have used an alternative MRI-compatible device called the ClearPoint® System in our Phase 1 and Phase 1b clinical trials and in the RESTORE-1 Phase 2 clinical trial of VY-AADC (NBIb-1817).

We have a history of incurring annual net operating losses. Our net losses were $76.9 million for the nine months ended September 30, 2021. As of September 30, 2021, we had an accumulated deficit of $352.8 million. We expect to continue to incur significant expenses and operating losses in the near term. We expect our expenses to decrease from current levels in the near term as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, and our strategic shift to invest in novel capsid development efforts, including our decision on whether to proceed with the VY-AADC Program for Parkinson's disease with a collaborative partner and our decision to terminate our current VY-HTT01 program for Huntington's disease and to advance a second generation Huntington's disease program, and our initiation of other cost-saving initiatives. We anticipate that our expenses will increase significantly over time if and as we:

- continue investing in our gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques;

- determine the appropriate path forward, if any, for VY-AADC (NBIb-1817) as a treatment for Parkinson's disease;
- develop a second-generation program for Huntington's disease using a novel, proprietary AAV capsid to enable enhanced transduction and broad distribution with intravenous administration;
- increase our investment in and support for TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA), our proprietary system to facilitate the selection of AAV capsids and expand our investment to discover novel capsids with broad tropism in CNS, cardiac, and skeletal tissues with cell-specific transduction properties for particular therapeutic applications;
- enter into licensing agreements regarding our novel capsids, such as the Pfizer Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;
- expand our manufacturing capabilities;
- seek marketing and regulatory approvals for product candidates or devices that arise from our programs that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- develop a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- expand over time our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- increase our product liability and clinical trial insurance coverage as we expand our clinical trials and commercialization efforts; and
- continue to operate as a public company.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future. For the nine months ended September 30, 2021, we recognized $9.3 million of collaboration revenue from the Neurocrine Collaboration.

For additional information about our revenue recognition policy related to collaborations and a description of the key terms of our collaboration arrangement with Neurocrine, refer to Note 8, *Commitments and Contingencies*, of our condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

For the foreseeable future, we expect substantially all of our revenue will be generated from our existing collaboration agreement with Neurocrine, our existing option and license agreement with Pfizer, and from any other strategic relationships we may enter into. If our development efforts are successful, we may also generate revenue from product sales in the future.

Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our program discovery efforts, and the development of our programs and gene therapy platform, which include:

- employee-related expenses including salaries, benefits, and stock-based compensation expense;
- costs of funding research performed by third parties that conduct research and development, clinical and preclinical activities, manufacturing and production design on our behalf;
- the cost of purchasing lab supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation and maintenance expenses; and
- fees for maintaining licenses under our third-party licensing agreements.

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing, preclinical studies, and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales of our product candidates. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- identifying additional product candidates;
- completing preclinical studies successfully;
- designing, initiating, enrolling and completing clinical trials successfully;
- establishing an appropriate safety profile;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- receiving marketing approvals from applicable regulatory authorities;

- commercializing the product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- continued acceptable safety profiles of the products following approval; and
- recruiting and retaining key research and development personnel.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs, timing and viability associated with the development of that product candidate.

Research and development activities are central to our business model. We are in the early stages of development of our product candidates. We expect research and development costs to decrease from current levels in the near term as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, and our strategic shift to invest in novel capsid development efforts, including our decision on whether to proceed with the VY-AADC Program for Parkinson's disease with a collaborative partner and our decision to terminate our VY-HTT01 program for Huntington's disease and to advance a second generation Huntington's disease program, and our initiation of other cost-saving initiatives. Following the completion of our restructuring, as our development programs progress and as we identify product candidates and initiate preclinical studies and clinical trials, we expect research and development costs to increase.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will decrease from current levels in the near term as a result of our strategic restructuring including the elimination of a portion of our workforce associated with general and administrative functions to align our resources with the needs of our ongoing business. These decreases include a reduction in personnel costs and fees paid to outside consultants, as well as other cost-saving initiatives including a reduction in facility-related expenditures. Following the completion of our restructuring, we expect general and administrative expenses to increase to support continued research and development activities and increased expenses associated with being a public company, including accounting and audit, legal, regulatory, and tax, compliance, insurance (including director and officer insurance) business development and investor relations costs.

Other Income (Expense), net

Interest and other income (expense) consists primarily of interest income on our marketable debt securities and the gain or loss on the equity securities investment in CLPT. All equity securities in CLPT held by us were sold or otherwise disposed of during the nine months ended September 30, 2021.

Critical Accounting Policies and Estimates

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate. There were no changes to our critical accounting policies during the nine months ended September 30, 2021 as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020. It is important that the discussion of our operating

results that follow be read in conjunction with the critical accounting policies disclosed in our Annual Report on Form 10-K, as filed with the SEC on February 25, 2021.

Results of Operations

Comparison of the three months ended September 30, 2021 and 2020

The following table summarizes our results of operations for the three months ended September 30, 2021 and 2020, together with the changes in those items in dollars:

	Three Months Ended September 30,		
	2021	2020	Change
	(in thousands)		
Collaboration revenue	$ 1,482	$ 117,843	$ (116,361)
Operating expenses:			
Research and development	17,914	25,039	(7,125)
General and administrative	8,714	8,277	437
Total operating expenses	26,628	33,316	(6,688)
Other income, net:			
Interest income	121	254	(133)
Other (expense) income	(112)	830	(942)
Total other income	9	1,084	(1,075)
Net (loss) income	$ (25,137)	$ 85,611	$ (110,748)

Collaboration Revenue

Collaboration revenue was $1.5 million and $117.8 million for the three months ended September 30, 2021 and 2020, respectively. The decrease in collaboration revenue in the three months ended September 30, 2021 was largely a result of our reduced performance of research and development services related to the Neurocrine Collaboration including reduced services resulting from Neurocrine's decision to terminate the Neurocrine Collaboration Agreement with respect to VY-AADC and its participation in the VY-AADC Program. Additionally, in the three months ended September 30, 2020, we recognized collaboration revenue on the AbbVie Tau Collaboration and AbbVie Alpha-Synuclein Collaboration, both of which were terminated in August 2020. During the three months ended September 30, 2021, collaboration revenue was entirely comprised of $1.5 million related to research services and cost reimbursement from the Neurocrine Collaboration. During the three months ended September 30, 2020, collaboration revenue included $47.2 million related to research services from the AbbVie Tau Collaboration, which consists of $0.9 million related to research services provided prior to the termination date and $46.3 million of deferred revenue remaining under the agreement at the termination date for which all of our obligations were complete as of September 30, 2020, $59.4 million related to research services from the AbbVie Alpha-Synuclein Collaboration, which consists of $0.5 million related to research services provided prior to the termination date and $58.9 million of deferred revenue remaining under the agreement at the termination date for which all of our obligations were complete as of September 30, 2020, and $11.2 million related to research services and cost reimbursement from the Neurocrine Collaboration. Our collaboration revenues were not materially impacted by the coronavirus disease 2019, or COVID-19, pandemic during the three months ended September 30, 2021.

As a result of the terminations of the AbbVie Tau Collaboration and the AbbVie Alpha-Synuclein Collaboration and Neurocrine's termination of its participation in the VY-AADC Program under the Neurocrine Collaboration, we expect collaboration revenues will continue to be lower in 2021 than in comparable periods in 2020.

Research and Development Expense

Research and development expense decreased by $7.1 million from $25.0 million for the three months ended September 30, 2020, to $17.9 million for the three months ended September 30, 2021. The following table summarizes

our research and development expense for the three months ended September 30, 2021 and 2020, together with the change in those items in dollars:

	Three Months Ended September 30,		
	2021	2020	Change
	(in thousands)		
Employee and consultant	$ 8,697	$ 12,347	$ (3,650)
External research and development	4,436	8,547	(4,111)
Facilities and other	2,547	1,964	583
Professional fees	2,234	2,181	53
Total research and development expenses	$ 17,914	$ 25,039	$ (7,125)

The decrease in research and development expense for the three months ended September 30, 2021 was primarily attributable to the following:

- approximately $4.1 million for external research and development costs primarily related to a reduction in clinical and manufacturing activities for the VY-AADC Program for Parkinson's disease; and
- approximately $3.7 million for employee and consultant related costs associated with lower headcount in research and development functions as compared to the prior year; partially offset by
- approximately $0.6 million for increased facility and other costs including rent, depreciation, maintenance and other expenses due to the additional space leased at 75 Hayden Avenue.

As a result of the terminations of the AbbVie Tau Collaboration and the AbbVie Alpha-Synuclein Collaboration and Neurocrine's termination of its participation in the VY-AADC Program under the Neurocrine Collaboration; our strategic restructuring; our decisions to terminate our VY-HTT01 program for Huntington's disease and to advance a second-generation Huntington's disease program; and other activities associated with our portfolio reevaluation efforts, we expect research and development costs will continue to be lower in 2021 than in comparable periods in 2020.

General and Administrative Expense

General and administrative expense increased by $0.4 million from $8.3 million for the three months ended September 30, 2020 to $8.7 million for the three months ended September 30, 2021. The increase in general and administrative expense was primarily attributable to the following:

- approximately $0.4 million for increased compensation costs and stock-based compensation associated with recognition of the severance expensed as a result of the reduction in force;
- approximately $0.2 million for increased facility and other costs including rent, depreciation, maintenance and other expenses due to the additional space leased at 75 Hayden Avenue; partially offset by
- approximately $0.2 million for decreased professional fees and related expenses to support the pipeline programs.

Other (Expense) Income, net

Interest and other expense of approximately $9.0 thousand and interest and other income of approximately $1.1 million was recognized during the three months ended September 30, 2021 and 2020, respectively, related to interest income on marketable securities balances, in addition to gains and losses on our common stock investment in and

warrants to purchase shares of common stock of CLPT. All equity securities in CLPT held by us were sold or otherwise disposed of during the nine months ended September 30, 2021.

Comparison of the nine months ended September 30, 2021 and 2020

The following table summarizes our results of operations for the nine months ended September 30, 2021 and 2020, together with the changes in those items in dollars:

	Nine Months Ended September 30,		
	2021	2020	Change
	(in thousands)		
Collaboration revenue	$ 9,342	$ 164,591	$ (155,249)
Operating expenses:			
Research and development	59,767	86,757	(26,990)
General and administrative	28,895	26,721	2,174
Total operating expenses	88,662	113,478	(24,816)
Other income, net:			
Interest income	253	1,578	(1,325)
Other income (expense)	2,161	(24)	2,185
Total other income, net	2,414	1,554	860
Net (loss) income	$ (76,906)	$ 52,667	$ (129,573)

Collaboration Revenue

Collaboration revenue was $9.3 million and $164.6 million for the nine months ended September 30, 2021 and 2020, respectively. The decrease in collaboration revenue in the nine months ended September 30, 2021 was largely a result of our reduced research and development services related to the Neurocrine Collaboration including as a result of Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program. Additionally, in the nine months ended September 30, 2020, we recognized revenue on the AbbVie Tau Collaboration and AbbVie Alpha-Synuclein Collaboration, both of which were terminated in August 2020. During the nine months ended September 30, 2021 collaboration revenue was entirely comprised of $9.3 million related to research services and cost reimbursement from the Neurocrine Collaboration. During the nine months ended September 30, 2020, collaboration revenue included $50.8 million related to research services from the AbbVie Tau Collaboration, which consisted of $4.5 million related to research services provided under the AbbVie Tau Collaboration prior to the termination date and $46.3 million of deferred revenue remaining under the agreement at the termination date for which all of our obligations were complete as of September 30, 2020, $63.7 million related to research services from the AbbVie Alpha-Synuclein Collaboration, which consisted of $4.8 million related to research services provided under the AbbVie Tau Collaboration prior to the termination date and $58.9 million of deferred revenue remaining under the agreement at the termination date for which all of our obligations were complete as of September 30, 2020, and $50.1 million related to research services and cost reimbursement from the Neurocrine Collaboration. Our collaboration revenues were not materially impacted by the COVID-19 pandemic during the nine months ended September 30, 2021.

Research and Development Expense

Research and development expense decreased by $27.0 million from $86.8 million for the nine months ended September 30, 2020, to $59.8 million for the nine months ended September 30, 2021. The following table summarizes our research and development expense, for the nine months ended September 30, 2021 and 2020:

	Nine Months Ended September 30,		
	2021	2020	Change
	(in thousands)		
Employee and consultant	$ 30,382	$ 41,333	$ (10,951)
External research and development	14,784	32,741	(17,957)
Facilities and other	7,525	5,986	1,539
Professional fees	7,076	6,697	379
Total research and development expenses	$ 59,767	$ 86,757	$ (26,990)

The decrease in research and development expense for the nine months ended September 30, 2021 was primarily attributable to the following:

- approximately $18.0 million for external research and development costs primarily related to a reduction in clinical and manufacturing activities for the VY-AADC Program for Parkinson's disease; and
- approximately $11.0 million for employee and stock-based compensation associated with lower headcount in research and development functions compared to the prior year; partially offset by
- approximately $1.5 million of increased facility costs, including rent, depreciation, maintenance and other expenses due to additional space leased at 75 Hayden Avenue

General and Administrative Expense

General and administrative expense increased by $2.2 million from $26.7 million for the nine months ended September 30, 2020 to $28.9 million for the nine months ended September 30, 2021. The increase in general and administrative expense was primarily attributable to the following:

- approximately $1.8 million for increased employee and contractor related costs, including $1.3 million for compensation associated with the departure in June of our Chief Executive Officer, in addition to $0.4 million associated with the recognition of the severance expensed as a result of the reduction in force;
- approximately $0.6 million for increased facility costs including rent, depreciation, maintenance and other expenses due to additional space leased at 75 Hayden Avenue; partially offset by
- approximately $0.2 million for a decrease in professional fees and related expenses to support the pipeline programs.

Other Income, net

Interest and other income of approximately $2.4 million and $1.6 million were recognized during the nine months ended September 30, 2021 and 2020, respectively, related to interest income on marketable securities balances in addition to gains and losses on our common stock investment in and warrants to purchase shares of common stock of CLPT.

COVID-19

The COVID-19 pandemic continues to evolve rapidly. We have and will continue to be guided by applicable guidelines and safety measures, including stay-at-home policies for certain non-essential employees, consultants, contractors, and staff. Certain of our clinical trial sites, collaboration partners, suppliers and consultants have experienced facility closures or been subject to quarantines, travel restrictions and other governmental restrictions and have appropriately diverted attention and resources to respond to the impacts of COVID-19 on their own operations and personnel. Some have even become involved in research and development efforts related to COVID-19.

The current workplace safety measures that we have enacted in response to COVID-19 have required a reduction in on-site activity at our facilities in Massachusetts, including in our laboratories in which preclinical experiments are conducted. As a result, we have had to prioritize our preclinical experiments and terminate or delay some non-critical experiments in order to maintain critical experiments for our preclinical programs.

We will continue to monitor the issues raised by the global spread of COVID-19 and have put in place and will continue to put in place measures as appropriate and necessary for, or that we believe to be in the best interest of, our business, employees, collaborators, stockholders, and the community.

Strategic Restructuring – August 2021

On August 6, 2021, our board of directors approved a strategic restructuring plan to eliminate positions affecting a portion of our workforce as part of an initiative to reduce expenses and enhance operations. The strategic restructuring plan was approved in connection with our portfolio reevaluation efforts and our strategic shift to invest additional resources in our novel capsid development efforts.

During the three months ended September 30, 2021, we incurred restructuring costs of approximately $2.0 million, which primarily consists of severance related costs. Approximately $0.9 million of these restructuring costs were paid as of September 30, 2021.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings of our common stock, our strategic collaborations, including our prior Sanofi Genzyme Collaboration, AbbVie Tau Collaboration, and AbbVie Alpha-Synuclein Collaboration, our ongoing Neurocrine Collaboration, and the Pfizer Agreement.

In February 2021, Neurocrine notified us it had elected to terminate the Neurocrine Collaboration with respect to VY-AADC and its participation in the VY-AADC Program, effective August 2, 2021. The Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. We intend to support Neurocrine, the study sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the DSMB and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial. As a result of the termination, subsequent to the Neurocrine VY-AADC Program Termination Effective Date, Neurocrine is no longer obligated to reimburse us for research and development activities related to the VY-AADC Program, other than incidental expenses incurred by us in conducting wind-down activities.

As of September 30, 2021, we had cash, cash equivalents, and marketable debt securities of $121.5 million. Based upon our current operating plans, we expect that our existing cash, cash equivalents, and marketable debt securities will be sufficient to meet our planned operating expenses and capital expenditure requirements into early 2023.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2021 and 2020:

	Nine Months Ended September 30,	
	2021	2020
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (64,720)	$ (74,739)
Investing activities	71,370	134,511
Financing activities	385	2,677
Net increase in cash, cash equivalents, and restricted cash	$ 7,035	$ 62,449

Net Cash Used in Operating Activities

Net cash used in operating activities was $64.7 million during the nine months ended September 30, 2021 compared to $74.7 million of net cash used in operating activities during the nine months ended September 30, 2020. The decrease in cash used in operating activities for the nine months ended September 30, 2021 compared to the nine months ended September 30, 2020 was primarily due to lower expenditures related to the reduction of VY-AADC Program activities following the delivery by Neurocrine of notice of termination of its participation in the VY-AADC Program in February 2021.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was $71.4 million during the nine months ended September 30, 2021 compared to $134.5 million of net cash provided by investing activities during the nine months ended September 30, 2020. The net cash provided by investing activities for the nine months ended September 30, 2021 was primarily due to $60.0 million from maturities of marketable securities and $12.6 million from proceeds of sales of marketable securities partially offset by $1.2 million for purchases of property and equipment. The net cash provided by investing activities for the nine months ended September 30, 2020 was primarily due to $168.0 million from maturities of marketable securities partially offset by $25.0 million for purchases of marketable securities and $8.5 million for purchases of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $0.4 million and $2.7 million during the nine months ended September 30, 2021 and September 30, 2020, respectively, due to the proceeds from the exercise of stock options.

Funding Requirements

We expect our expenses to decrease from current levels in the near term as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, and our strategic shift to invest in novel capsid development efforts, including our decision on whether to proceed with the VY-AADC Program for Parkinson’s disease with a collaborative partner and our decision to terminate our VY-HTT01 program for Huntington’s disease and to advance a second generation Huntington’s disease program, and our initiation of other cost-saving initiatives. We expect our expenses to increase in the future, however, as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates and as we continue to enter into or expand efforts on our collaboration agreements. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we expect to incur increasing costs associated with operating as a public company, meeting financial controls, satisfying regulatory and quality standards, fulfilling healthcare compliance requirements, and maintaining product, clinical trial and directors’ and

officers' liability insurance coverage. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable debt securities will be sufficient to meet our planned operating expenses and capital expenditure requirements into early 2023. Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates and any required companion devices;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and licensing agreements, including any research and development costs for which we are responsible, our collaborators' or licensors' willingness and ability to approve desirable budgets for research and development costs for which they are responsible, the potential exercise by our collaboration partners or licensees of any options to develop or license certain products and product candidates, capsids or other technologies that they might have, our potential receipt of future milestone payments and royalties from our collaboration partners or licensors, and any decisions by our collaborators or licensors to exercise their rights to terminate a collaboration or licensing agreement in whole or in part;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger payments, under any other collaboration agreement to which we might become a party;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;
- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company, meeting applicable financial, regulatory and quality control standards, fulfilling healthcare compliance requirements, and maintaining adequate product, clinical trial and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete. We may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration or licensing agreements, will be derived from sales of products that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing and business development transactions to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate product revenues sufficient to achieve profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partners or licensors for reimbursement of research and development expenses, potential option exercises, the achievement of specified regulatory and commercial milestones, and royalty payments under our collaboration or licensing agreements. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted. The amount of stockholder dilution will be affected by the size of each securities offering and the offering price for the securities sold. The offering price will likely reflect the prevailing market price for our securities, with dilution increasing as the prevailing market price for our securities decreases. The terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

We enter into agreements in the normal course of business with clinical research organizations, contract manufacturing organizations, and institutions to license intellectual property. These contracts are cancelable at any time by us, generally upon 30 to 90 days prior written notice.

Our agreements to license intellectual property include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. We may also be required to pay annual maintenance fees or minimum amounts payable ranging from low-four digits to low five-digits depending upon the terms of the applicable agreement.

We also have non-cancelable operating lease commitments arising from our leases of office and laboratory space at our facilities in Cambridge and Lexington, Massachusetts. We expect lease payments under these commitments to total $2.1 million for the remainder of 2021 and increase annually; in 2025, we expect total lease payments of approximately $9.6 million. We expect to receive sublease payments of $7.1 million from our sublessee over the term of the sublease.

Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable SEC rules.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to market risk related to changes in interest rates. We have policies requiring us to invest in high-quality issuers, limit our exposure to any individual issuer, and ensure adequate liquidity. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of money market funds and marketable securities and are invested in U.S. Treasury notes. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We are not currently exposed to market risk related to changes in foreign currency exchange rates; however, we may contract with vendors that are located in Asia and Europe in the future and may be subject to fluctuations in foreign currency rates at that time.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition, or results of operations during the three and nine months ended September 30, 2021.

ITEM 4. CONTROLS AND PROCEDURES

Management's Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) or 15d-15(e) under the Exchange Act to mean controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Our disclosure controls and procedures include, without limitation, controls and other procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2021. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2021, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Changes in Internal Control over Financial Reporting

During the three months ended September 30, 2021, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

As of the date of this Quarterly Report on Form 10-Q, we were not party to any material legal matters or claims. In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution

of which we do not anticipate would have a material adverse impact on our financial position, results of operations, or cash flows.

On January 22, 2021, a putative class action lawsuit was filed in the U.S. District Court for the Eastern District of New York against us and certain of our current and former officers and directors, captioned *Karp v. Voyager Therapeutics, Inc. et al.*, No. 1:21-cv-00381. The complaint generally alleged that the defendants violated Sections 10(b) and/or 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder by making material misstatements or omissions concerning our Huntington's disease program and our investigational new drug application for VY-HTT01. On April 19, 2021, the court appointed the lead plaintiff for the action, and on April 30, 2021, the action was transferred to the U.S. District Court for the District of Massachusetts (where it was assigned case number 1:21-cv-10727). On July 2, 2021, the lead plaintiff voluntarily dismissed the action without prejudice against all defendants and as to all claims. This matter is no longer pending.

ITEM 1A. RISK FACTORS

The following risk factors and other information in this Quarterly Report on Form 10-Q, including our condensed consolidated financial statements and related notes thereto, should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see page 1 of this Quarterly Report on Form 10-Q for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Capital

We have a history of incurring significant losses and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain consistent profitability.

We are an early-stage gene therapy company with a limited operating history and have not yet generated revenues from the sales of our product candidates. All of our product candidates are in the early stages of development. Investment in biotechnology companies is highly speculative because it entails substantial upfront capital expenditures and significant risk that any product candidates will fail to be safe and efficacious, obtain regulatory approval or become commercially viable. We have not yet demonstrated the ability to complete any clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. We continue to incur significant expenses related to research and development, and other operations in order to commercialize our product candidates. We have a history of incurring significant operating losses. We reported a net loss of $76.9 million for the nine months ended September 30, 2021 and net income of $52.7 million for the nine months ended September 30, 2020. As of September 30, 2021, we had an accumulated deficit of $352.8 million.

We historically have financed our operations primarily through private placements of our redeemable convertible preferred stock, public offerings of our common stock, and strategic collaborations, including our prior collaborations with Sanofi Genzyme Corporation, or Sanofi Genzyme, and AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, or collectively, AbbVie, and our ongoing collaboration with Neurocrine Biosciences, Inc., or Neurocrine, and our option and license agreement with Pfizer Inc., or Pfizer, which commenced in October 2021. We refer to the Pfizer option and license agreement as the Pfizer Agreement.

To date, we have devoted substantially all of our financial resources to building our gene therapy platform, selecting product programs, conducting research and development, including preclinical development of our product candidates, building our intellectual property portfolio, building our team, and establishing strategic collaborations. We expect that it could be at least several years before we have a commercialized product, if we ever succeed in doing so.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter.

Although our expenses may decrease in the near term as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, and our strategic shift to invest in novel capsid development efforts, we anticipate that our expenses will increase substantially if, and as, we:

- continue investing in our gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques;
- determine the appropriate path forward, if any, for VY-AADC (NBIb-1817) as a treatment for Parkinson's disease;
- develop a second-generation program for Huntington's disease using a novel, proprietary adeno-associated virus, or AAV capsid to enable enhanced transduction and broad distribution with intravenous administration;
- increase our investment in and support for TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA), our proprietary system to facilitate the selection of AAV capsids and expand our investment to discover novel capsids with broad tropism in CNS, cardiac, and skeletal tissues with cell-specific transduction properties for particular therapeutic applications;
- enter into licensing agreements regarding our novel capsids, such as the Pfizer Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;
- expand our manufacturing capabilities;
- seek marketing and regulatory approvals for product candidates or devices that arise from our programs that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- develop a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- expand over time our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;

- increase our product liability and clinical trial insurance coverage as we expand our clinical trials and commercialization efforts; and

- continue to operate as a public company.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve consistent profitability. Our expenses will increase if:

- we are required by the U.S. Food and Drug Administration, or FDA, or the European Medicines Agency, or EMA, or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;

- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs or to use companion devices required in such clinical programs; or

- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

To become and remain profitable, we must develop and commercialize, alone or with our collaborators, product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities include completing preclinical studies and clinical trials of our product candidates; obtaining marketing approval for these product candidates; developing and obtaining marketing approval of any required companion devices; manufacturing at clinical and commercial scale; marketing and selling those products that are approved; satisfying any post-marketing requirements and achieving an adequate level of market acceptance of and obtaining and maintaining adequate coverage and reimbursement from third-party payors for such products; and protecting our rights to our intellectual property portfolio. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause our stockholders to lose all or part of their investment.

We may not be able to generate sufficient revenue from the commercialization of our product candidates and may never be consistently profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with our collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future product candidates. All of our product candidates are in the early stages of development. We do not anticipate generating revenues from product sales for at least the next several years, and we may never succeed in doing so. Our ability to generate future revenues from product sales depends heavily on our and our collaborators' and licensors' success in:

- completing preclinical and clinical development of our product candidates or product candidates incorporating our licensed capsids or other technologies and any required companion devices and identifying new product candidates;

- seeking and obtaining regulatory and marketing approvals for product candidates for which we or they complete clinical trials;

- launching and commercializing product candidates for which we or they obtain regulatory and marketing approval by establishing a sales, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;

- obtaining and maintaining adequate coverage and reimbursement by government and third-party payors for our product candidates if and when approved;

- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;

- establishing and maintaining supply and manufacturing relationships with third parties that have the financial, operating and technical capabilities to provide adequate products and services, in both amount and quality, to support clinical development and the market demand for our or their product candidates, if and when approved;

- obtaining an adequate level of market acceptance of our or their product candidates as a viable treatment option;

- addressing any competing technological and market developments;

- implementing additional internal systems and infrastructure, as needed;

- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;

- obtaining, maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;

- avoiding and defending against third-party claims of interference or infringement; and

- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, EMA, or other regulatory authorities to redesign or modify preclinical studies or clinical trials or to perform preclinical studies or clinical trials in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.

Although our expenses may decrease in the near term as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, and our strategic shift to invest in novel capsid development efforts, we expect our expenses to increase over time in connection with our ongoing and planned activities, particularly as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Since the completion of our IPO on November 16, 2015, we have also incurred costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we

are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our research and development programs or any future commercialization efforts.

Our operations have consumed significant amounts of cash since inception. As of September 30, 2021, our cash, cash equivalents, and marketable debt securities were $121.5 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable debt securities will be sufficient to meet our planned operating expenses and capital expenditure requirements into early 2023.

Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates and any required companion devices;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and licensing agreements, including any research and development costs for which we are responsible, our collaborators' or licensors' willingness and ability to approve desirable budgets for research and development costs for which they are responsible, the potential exercise by our collaboration partners or licensors of any options to develop or license certain products and product candidates, capsids or other technologies that they might have, our potential receipt of future milestone payments and royalties from our collaboration partners or licensors, and any decisions by our collaborators or licensors to exercise their rights to terminate a collaboration in whole or in part;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger payments, under any other collaboration agreement to which we might become a party;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;
- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company, meeting applicable financial, regulatory and quality control standards, fulfilling healthcare compliance requirements, and maintaining adequate product, clinical trial and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete. We may never generate the necessary data or results required to maintain the financial support of our collaborators or obtain marketing approval and achieve product sales. In the event we are unable to achieve milestones necessary to demonstrate progress on those programs, a current or future collaboration partner or licensor may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Our ability to develop a second-generation program for the treatment of Huntington's disease may take longer than we anticipate, may not happen at all, and could require funding at a level higher than we expect. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration or licensing agreements will be derived from sales of products that may not be commercially available for many years, if at all. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing and business development to achieve our business objectives. Adequate additional financing or business development transactions may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate product revenues sufficient to achieve consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partner Neurocrine for the reimbursement of certain research and development expenses, the achievement of specified regulatory and commercial milestones, and royalty payments under our collaboration agreement and the amounts we are entitled to receive from our licensor Pfizer for the achievement of specified development, regulatory, and commercialization milestones and royalty payments under the Pfizer Agreement. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted. The amount of stockholder dilution will be affected by the size of each securities offering and the offering price for the securities sold. The offering price will likely reflect the prevailing market price for our securities, with dilution increasing as the prevailing market price for our securities decreases. The terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our common stock to decline. Further, our existing stockholders may not agree with the terms of such financings.

If we raise additional funds through collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves. Such collaborations, alliances, or licensing arrangements could therefore cause the market price of common stock to decline.

Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

Our operating history is short, and to date has been limited to building our team, business planning, raising capital, establishing our intellectual property portfolio, determining which neurological diseases to pursue, advancing our product candidates including delivery and manufacturing and conducting preclinical studies and early-phase clinical trials. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors such as the regulatory actions that occurred in connection with the VY-AADC Program for Parkinson's disease and the VY-HTT01 Program for Huntington's disease. These and other events that are part of our operating history, and ongoing activities such as our decision to conduct a reevaluation of our product candidate pipeline and to restructure our workforce, may impact our ability to operate our business and to raise capital. All of our product candidates are in the early stages of development. To achieve our current goals, we will need to transition in the future from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, our stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

Risks Related to the Development and Regulatory Approval of Our Product Candidates

Our AAV gene therapy product candidates are based on a novel technology, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and subsequently obtaining regulatory approval for, our product candidates. Only two AAV gene therapy products have been approved in the United States. In Europe, only two AAV gene therapy products have been approved.

We have concentrated our research and development efforts to date on our gene therapy platform, identifying our initial targeted disease indications, and our initial product candidates. Our future success depends on our successful development of viable AAV gene therapy product candidates. We currently only have one product candidate, VY-AADC (NBIb-1817), in clinical development, and the RESTORE-1 Phase 2 clinical trial in which we are evaluating VY-AA DC (NBIb-1817) is on clinical hold. Our collaboration partner Neurocrine is the study sponsor and IND holder for our VY-AADC program, and it terminated its participation in the program, effective August 2, 2021. We have determined that we will not advance the VY-AADC program unless the program is partnered with another collaborator.

The remainder of our product candidates are in preclinical development. In connection with our strategic restructuring, we determined not to initiate our planned VYTAL Phase 1/2 clinical trial for VY-HTT01 and have instead initiated efforts to develop a second-generation program for Huntington's disease using a novel, proprietary AAV capsid to enable enhanced transduction and broad distribution with intravenous administration. While we believe the decision to transition to a second-generation program for Huntington's disease to be in our best interest, it also extends the timeline for the development and potential approval and commercialization of a product for Huntington's disease.

AAV gene therapies are a relatively new technology. We cannot accurately predict when or if any of our product candidates will prove effective or safe in humans or whether these product candidates will receive marketing approval. There can be no assurance that we will not experience problems or delays in the preclinical testing or development of our product candidates and that such problems or delays will not cause unanticipated costs, or that any such problems or delays can be solved in a timely or profitable basis, if at all. We also may experience unanticipated problems or delays in expanding our manufacturing capacity.

The clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the product candidate. The regulatory approval process for novel product candidates such as gene therapies can be more expensive and take longer than for other, better known or more extensively studied product candidates. Until August 2017, the FDA had never approved a gene therapy product. Since that time, it has approved Luxturna, an AAV gene therapy product by Spark Therapeutics, Inc. (acquired by F. Hoffmann-La Roche Ltd., or Roche, in 2019), or Spark, for patients with an inherited form of vision loss, and Zolgensma, an AAV gene therapy product by Avexis, a Novartis company, for pediatric patients with spinal muscular atrophy. In Europe, Luxturna and Zolgensma, as well as Glybera by uniQure N.V., or uniQure, have been granted marketing authorization; however, uniQure decided not to pursue renewal of such authorization in 2017 and has since withdrawn Glybera from the European market.

It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or how long it will take to commercialize our

product candidates. The few regulatory approvals to date may not be indicative of what the FDA, European Commission, or other regulatory authorities may require for approval or whether different or additional preclinical studies or clinical trials may be required to support regulatory approval in a particular jurisdiction. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects may be harmed.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.

The Center for Biologics Evaluation and Research, or CBER, of the FDA regulates biological products for human use. The Office of Tissues and Advanced Therapies, or OTAT, formerly known as the Office of Cellular, Tissue and Gene Therapies, within CBER reviews gene therapy and related products and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review.

NIH-funded institutions need to have their institutional biosafety committee, or IBC, as well as their institutional review board, or IRB, review proposed clinical trials to assess the safety of the trial. The PD-1101 Phase 1b clinical trial of VY-AADC (NBIb-1817), the PD-1102 Phase 1 trial exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory, and the RESTORE-1 Phase 2 clinical trial have been conducted at multiple sites, and therefore are subject to oversight by these authorities. Such trials will need to be re-reviewed by the respective institutional IRBs if the protocols for the trials are amended, and any delay in or failure to obtain institutional IRB approval for any protocol or protocol amendment could delay, interrupt, or limit the conduct of the clinical trial at one or more participating clinical trial sites. For example, in connection with the RESTORE-1 Phase 2 clinical trial, we and our collaboration partner Neurocrine paused screening of new patients, in part to facilitate IRB reviews of certain amendments made to the clinical trial protocol. As the FDA imposed a clinical hold on the RESTORE-1 Phase 2 clinical trial in December 2020, the resumption of any patient screening or dosing in the trial is now subject to the resolution of the clinical hold currently in effect.

Adverse or unforeseen developments in clinical trials of gene therapy products conducted by us or others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, EMA and local health authorities of individual countries within the European Union may issue new guidelines concerning the clinical development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. The EMA and agencies at both the federal and state level in the United States have expressed an interest in further regulating new biotechnologies, including gene therapy. In addition, gene therapy products are considered genetically-modified organism, or GMO, products and are regulated as such in each country. Designation of the type of GMO product and subsequent handling and disposal requirements can vary across countries and is variable throughout the European Union. Addressing each specific country requirement and obtaining approval to commence a clinical trial in these countries could result in delays in starting, conducting, or completing a clinical trial. Similar issues could be faced in other regions of the world including the Asia-Pacific region.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. For example, we have requested feedback from the FDA on, among other matters, the regulatory pathway for VY-AADC (NBIb-1817) and the design of the proposed pivotal program. We had multiple interactions with the FDA throughout 2018 and received certain written feedback requiring additional clarification. In December 2018, we held a Type B meeting with the FDA to discuss the overall development and pivotal program for VY-AADC (NBIb-1817). We received written feedback from the FDA, including FDA guidance received during the Type B meeting that in a disease such as Parkinson’s two adequate and well-controlled clinical trials is suggested.

For example, in connection with our collaboration and license agreement with Neurocrine, or the Neurocrine Collaboration Agreement, we agreed to transfer sponsorship of the clinical research program for VY-AADC (NBIb-1817) in Parkinson's disease, or the VY-AADC Program, to Neurocrine, which required the related IND application to be transferred to Neurocrine. The transition process required additional regulatory filings with and review by the FDA. Based upon feedback received from the FDA, we and Neurocrine amended the RESTORE-1 clinical trial protocol. Following Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program, Neurocrine remains the sponsor of the RESTORE-1 Phase 2 clinical trial and the holder of the applicable IND. If sponsorship of the RESTORE-1 Phase 2 clinical trial is transferred back to us and we chose to advance the program with a collaboration partner, such transition would require additional regulatory filings with and review by the FDA, and would likely lead to additional costs and delays in the enrollment of patients in any potential future clinical trial.

In October 2020, the FDA notified us that the IND application for our planned Phase 1/2 clinical trial to evaluate VY-HTT01 in patients with Huntington's disease was placed on clinical hold pending the resolution of certain information requests regarding chemistry, manufacturing and controls, or CMC, matters. We had previously sought and received FDA feedback on the VY-HTT01 development program in a pre-IND meeting in 2017. Because the FDA only grants one pre-IND meeting per product in a given indication, however, we were unable to have additional formal consultations with the FDA prior to our submission of our IND application in September 2020 concerning changes to the program since our 2017 meeting. Although we have since resolved the clinical hold, we have decided not to commence the VYTAL Phase 1/2 clinical trial for VY-HTT01 and to develop a second-generation program for the treatment of Huntington's disease using a novel, proprietary AAV capsid to enable enhanced transduction and broad distribution with intravenous administration. These and other regulatory delays may require us to incur additional clinical development costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue from our product candidates.

We plan to continue to seek and incorporate FDA guidance in our ongoing development plans for each of our potential clinical candidates. If we fail to consult or solicit guidance from regulators or are unable to obtain sufficiently frequent or detailed guidance from regulators, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of increased or lengthier regulatory approval process and further restrictions on development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Results from preclinical studies and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials.

All of our product candidates are in early stages of development, and the risk of failure is high. Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Our product candidates may fail to show the desired safety and efficacy in preclinical testing or clinical development despite demonstrating promising results in earlier preclinical studies or clinical trials. In addition, the outcome of preclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials. Similarly, interim results generated from clinical trials do not necessarily predict final results, and results from one completed clinical trial may not be replicated in a subsequent clinical trial with a similar study design. For example, data from the PD-1101 Phase 1b clinical trial and from the separate PD-1102 Phase 1 clinical trial exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory suggest that one-time treatment with VY-AADC (NBIb-1817) could result in sustained improvement in motor function in patients with Parkinson's disease. These results, however, may not be predictive of the results of the RESTORE-1 Phase 2 clinical trial or any future clinical trials that we or a collaboration partner may choose to conduct on VY-AADC (NBIb-1817). Some of our clinical trials, including the PD-1101 and PD-1102 clinical trials, were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be sustained or repeated in larger and more advanced clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

There is a high failure rate for product candidates proceeding through preclinical studies and clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results, if these results are not reproducible, or if our products show diminishing activity over time, our products may not receive approval from the EMA or the FDA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late-stage clinical trials with larger patient populations could harm our business and we may never succeed in commercialization or generating product revenue.

We may in the future conduct, and intend to conduct, clinical trials for product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations.

To date, we have only conducted clinical trials in the United States. However, we may in the future choose to conduct, one or more of our clinical trials or include sites in current or future clinical trials outside the United States.

Although the FDA may accept data from sites or clinical trials outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, the clinical trial must be well-designed and conducted and performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials or trial sites are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials or trial sites also complied with all applicable U.S. laws and regulations. If the FDA does not accept the data from any trial or trial site outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and would delay or permanently halt our development of the applicable product candidates.

Other risks inherent in conducting international clinical trials or using international trial sites include:

- foreign regulatory requirements that could restrict or limit our ability to conduct our clinical trials;
- the administrative burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment;
- the failure of enrolled patients to adhere to clinical protocols or inadequate collection and assessment of clinical data as a result of differences in healthcare services or cultural customs;
- foreign exchange fluctuations;
- diminished or loss of protection of intellectual property in the relevant jurisdiction; and
- political, economic, environmental, and health risks relevant to specific foreign countries, including risks related to natural disasters or disease outbreaks, including the current coronavirus disease 2019, or COVID-19, pandemic.

We are early in our development efforts. Our primary product candidates are in preclinical development. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.

We are early in our development efforts, and our primary product candidates are in preclinical development. Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. To

conduct clinical trials, we must first complete preclinical testing and studies to support IND applications or similar applications in other jurisdictions. We cannot be certain of the timely completion or successful outcome of our preclinical testing and studies. Our ability to complete our preclinical testing and studies is contingent on our ability to source animals and other supplies required for the conduct of such testing and studies. If we are unable to obtain all necessary animals and other supplies required for the conduct of our preclinical testing and studies, we may be unable to complete such preclinical testing and studies in a timely manner or at all. For example, some of our IND-enabling toxicology and other studies require certain non-human primates that are customarily imported from the People's Republic of China, or the PRC, and current trade relations between the United States and the PRC has made the sourcing of these non-human primates challenging. We have encountered, and may continue to encounter, delays in obtaining a sufficient supply of such non-human primates to enable the conduct of our preclinical studies and testing. In addition, we may need to conduct preclinical studies utilizing non-human primates located in testing facilities outside of the United States. Utilizing such facilities will require us to observe export control regulations for the shipment of vectors and transgenes and import controls for the shipment of samples to us for evaluation and storage, which controls we may not be to satisfy, or may result in delay or additional expense. Our inability to obtain access to a sufficient supply of these non-human primates in a timely manner or at all may impair our ability to complete preclinical testing and studies to support IND applications or similar applications in other jurisdictions or delay the submission of such applications.

Additionally, we cannot predict if the FDA or similar regulatory authorities outside the United States will accept our planned clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our preclinical and clinical programs.

For example, in September 2020, we submitted an IND to the FDA in connection with the proposed initiation of a planned Phase 1/2 clinical trial for VY-HTT01 in patients with Huntington's disease. In October 2020, the FDA notified us that the IND application for VY-HTT01 was placed on clinical hold pending the resolution of certain CMC information requests. We provided a complete response to the additional requests from the FDA regarding the IND application for VY-HTT01 in the first quarter of 2021, and the FDA removed its clinical hold on the IND and confirmed that we could proceed with our planned VYTAL Phase 1/2 clinical trial in April 2021. Although we resolved the clinical hold, we have now decided not to initiate our planned VYTAL Phase 1/2 clinical trial. This delay and our subsequent decision to refocus our program for the treatment of Huntington's disease through the development of a second-generation product candidate could increase our expenses and adversely impact our ability to commercialize a product candidate for the treatment of Huntington's disease in a timely manner, if at all.

We also have very limited experience with clinical trials. The transfer of sponsorship of the VY-AADC Program to Neurocrine required Neurocrine to become the sponsor of the RESTORE-1 Phase 2 clinical trial in many sites. The sponsorship transition required additional regulatory filings with and review by regulatory officials, and led to additional costs and delays in the enrollment of patients in the RESTORE-1 Phase 2 clinical trial. Following Neurocrine's termination of the Neurocrine Collaboration Agreement with respect to the VY-AADC Program, Neurocrine remains the sponsor of the RESTORE-1 Phase 2 clinical trial and the holder of the applicable IND. If sponsorship of the RESTORE-1 Phase 2 clinical trial is transferred back to us, such transition would require additional regulatory filings with and review by the FDA, and would likely lead to additional costs and delays in the enrollment of patients in any clinical trials we or a collaboration partner would pursue in connection with the VY-AADC Program.

We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial failure can occur at any stage of testing. Similarly, there may be delays or difficulties in our initiation of future clinical trials. Due to the additional regulatory uncertainties associated with gene therapy products, we did not initiate the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817) as a treatment for Parkinson's disease until we met with OTAT to discuss our proposed trial design and overall development plan. While we have received OTAT's feedback and incorporated it as appropriate in our plans, the clinical trial as designed may not achieve the prospectively defined primary clinical endpoints or provide a favorable benefit to risk ratio to support a biologics license application, or BLA, filing or approval. In November 2020, the sponsor medical monitor and surgical core requested that the data safety and monitoring board, or DSMB, for the RESTORE-1 Phase 2 clinical trial review certain patient MRI abnormalities observed in some clinical trial participants in the ongoing clinical trial. Following this review, the DSMB requested additional patient-level data and recommended a pause in the dosing of patients in the RESTORE-1 Phase 2 clinical trial pending its review of these additional data. In response to the DSMB's recommendation, we and Neurocrine

decided to delay the planned resumption of patient screening in the RESTORE-1 Phase 2 clinical trial until Neurocrine had submitted the required expedited IND safety reports related to these matters and the DSMB was able to complete its evaluation. In December 2020, the FDA notified Neurocrine that it had placed a clinical hold on the RESTORE-1 Phase 2 clinical trial. In January 2021, the FDA informed Neurocrine of the information required to provide a complete response to the FDA in connection with the clinical hold. Information required by the FDA includes an assessment of how the investigational product may have given rise to the adverse findings, a mitigation plan to manage the adverse findings, and supportive data to justify that the product candidate continues to have a favorable benefit/risk profile. The DSMB met to review additional patient data in January 2021 and characterized the MRI abnormalities as having uncertain clinical significance and requested that Neurocrine provide additional information. The clinical implications of these observations are currently unknown and are being evaluated. We intend to support Neurocrine, the clinical trial sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the DSMB and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial. We have decided that, if Neurocrine were to transfer sponsorship of the RESTORE-1 Phase 2 clinical trial back to us, we would not seek to advance the clinical trial without a partner. Our ability to establish such a collaboration regarding the VY-AADC Program will be subject to a number of factors, including, among other things, the additional information collected by Neurocrine in response to the DSMB requests.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner or at all pursuant to the requirements of the FDA, EMA, or other regulatory authorities. Patient enrollment and trial completion are affected by many factors including:

- perceived risks and benefits of AAV gene therapy approaches for the treatment of diseases;
- perceived risks of the delivery procedure, such as intracranial infusion for VY-AADC (NBIb-1817) and VY-HTT01;
- formulation changes to our product candidates, which may require us to conduct additional clinical studies to bridge our modified product candidates to earlier versions;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- patients with preexisting antibodies to the gene therapy vector that preclude their participation in the trial;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- lack of adequate compensation of patients;
- ability to obtain and maintain patient consent;

- risk that enrolled patients will drop out before completion of the trial;
- our ability to locate appropriately trained physicians to conduct such clinical trials, particularly for clinical trials requiring lengthy and highly complex surgical protocols, the performance of which may only be possible at major academic medical centers or specialized surgical centers;
- willingness of patients to participate in a placebo-controlled trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Further, we plan to seek marketing approvals in the United States, the European Union and other jurisdictions, which may require that we conduct clinical trials in foreign countries. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with clinical research organizations, or CROs, and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials in some or all localities, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities or collaborators on trial design, implementation, management, or other aspects of the clinical trial;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;
- imposition of a clinical hold by regulatory authorities, such as the clinical hold that the FDA placed on the IND application for the RESTORE-1 Phase 2 clinical trial of VY-AADC (NBIb-1817) in Parkinson’s disease in December 2020;
- as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites or the decision by us or our collaborators, such as the pause in screening and enrollment of patients in the RESTORE-1 Phase 2 clinical trial in Parkinson’s disease, or the requirement of regulators or IRBs to

suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;

- failure by us, our collaboration partners, any CROs we engage, or any other third parties to adhere to clinical trial protocols or regulatory requirements;
- failure by us, our collaboration partners, any CROs we engage, or any other third parties to perform in accordance with the FDA's good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- insufficient or inadequate supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial at a rate higher than we anticipate;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- receipt of negative or inconclusive clinical trial results;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- the cost of clinical trials of our product candidates may be greater than we anticipate.

Any inability to successfully initiate or complete preclinical studies and clinical trials could result in additional costs and potential delays to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. For example, our decision to refocus our Huntington's Disease program means we must conduct new preclinical studies, prepare a new IND, submit it to the FDA, and resolve any potential FDA objections before enrolling our first patient in a new clinical trial. In addition, if we make manufacturing or formulation changes to our product candidates, such as our previous transition from an HEK 293-based production system to a baculovirus/Sf9 AAV production system or as a result of unanticipated clinical trial results, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or SAEs associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if we are able to do so at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a Risk Evaluation and Mitigation Strategy, or REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued or otherwise become party to dispute proceedings; or
- experience damage to our reputation.

Our product candidates or the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

In past clinical trials that were conducted by others with non-AAV gene therapy vectors, several significant side effects were caused by gene therapy treatments, including reported cases of leukemia and death. Other potential side effects could include an immunologic reaction and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If our vectors demonstrate a similar adverse effect, or other adverse effects, we may be required to halt or delay further clinical development of our product candidates or withdraw the product from the market post-approval. For example, in a recently published review of patients with hepatocellular carcinomas, it was shown that a small subset contained an integrated genome sequence of wild-type AAV2 and it was suggested that AAV2 may be associated with insertional oncogenesis.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause side effects. VY-AADC (NBIb-1817) and VY-HTT01 are designed to be administered directly to the targeted areas and cells in the brain, requiring the patient to undergo brain surgery. There are risks associated with direct delivery of AAV gene therapy into the brain. In a previous Phase 1 clinical trial conducted by UCSF, three patients experienced hemorrhages caused by the surgical procedure for administering VY-AADC (NBIb-1817). Investigators in the PD-1101 Phase 1b clinical trial, the separate PD-1102 Phase 1 posterior trajectory trial, and the RESTORE-1 Phase 2 clinical trial of VY-AADC (NBIb-1817), have used the ClearPoint System to provide accurate placement of the cannula in the putamen and allow for real-time, intra-operative MRI to assist the physician in visualizing the delivery of VY-AADC (NBIb-1817) to the putamen and to avoid specific blood vessels during the duration of the surgical procedure, with the goal of reducing the risk of hemorrhages. One patient in the Phase 1b clinical trial experienced two SAEs, a pulmonary embolism, or blood clot in the lungs, and related heart arrhythmia, or irregular heartbeat, which were determined to be related to the surgical procedure and prolonged immobility, not VY-AADC (NBIb-1817). Limited clinical data are available for this route of administration. If other side effects were to occur in connection with this route of administration including the related surgical procedures or related devices, any clinical trials which we conduct utilizing intraparenchymal delivery could be suspended or terminated. While we intend to transition our product candidates away

from direct delivery into the brain, our experience outside of direct delivery is limited, and we may not see the levels of results we hope for using alternative delivery methods such as intravenous delivery.

If in the future we are unable to demonstrate that such side effects were caused by the administration process or related procedures or are unable to modify the trial protocol adequately to address such side effects, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. For products that "knock down" or reduce the expression of a gene or the production of its encoded protein, their effects on other parts of the body, or "off target" effects, could result in unforeseen toxicity. Even if we are able to demonstrate that any future SAEs are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits outweigh its risks. We believe that the likelihood of the FDA requiring a REMS may be higher for treatments with more invasive routes of administration such as direct delivery through brain surgery. Such REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners or the limitation of the use of the product to specifically trained neurosurgeons and/or certain centers. Furthermore, adverse events which were initially considered unrelated to the study treatment of the clinical trial may later be found to be caused by the study treatment. If we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations.

We may be unable to obtain orphan drug designation or exclusivity for any of our product candidates for which we seek such designation. If our competitors are able to obtain orphan drug exclusivity for products that constitute the "same drug" and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. For products for which we may obtain orphan drug designation or exclusivity, we may be unable to prevent the approval or marketing authorization of other similar products based upon regulator decisions regarding product "sameness".

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, or the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug or biological product if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug or biological product will be recovered from sales in the

United States. We have received feedback from the FDA that VY-AADC (NBIb-1817) for the treatment of Parkinson's disease does not qualify for orphan disease designation because the potential for its use in earlier stages of Parkinson's disease exceeds the 200,000 patient population criterion in the United States. In the European Union, EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product. We have received feedback from the Committee for Orphan Medicinal Products that orphan designation likely would not be granted for VY-AADC (NBIb-1817) in Parkinson's disease since the Committee does not grant such status for products targeting more severe stages of a disease.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for a product that constitutes the "same drug" treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we may be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the BLA sponsor submits pediatric data that adequately respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

We believe that many of our current programs may qualify for orphan drug designation except for VY-AADC (NBIb-1817) for Parkinson's disease. On March 15, 2019, we received notification from the FDA that VY-HTT01, an AAV gene therapy containing a transgene that encodes a microRNA targeting huntingtin messenger RNA, had been granted orphan drug designation for the treatment of Huntington's disease. This designation, however, does not mean that we will be able to obtain orphan drug designation for any future or related product candidates for the treatment of Huntington's disease, including our planned second-generation product candidate for Huntington's disease. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs or biological products can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug or biological product for the same condition if the FDA concludes that the other drug or biological product is not the "same drug" or biological product or is clinically superior in that it is shown to be safer or more effective or makes a major contribution to patient care. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or vectors. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

Even if we seek orphan drug designation from the FDA, the European Commission or other regulatory agencies for a product candidate, there can be no assurances that the regulatory agency or agencies will grant such designation. Additionally, the designation of any of our product candidates as an orphan drug does not guarantee that any regulatory agency will ultimately approve that product candidate or prevent other products from receiving marketing authorization due to decisions of the applicable regulatory agency regarding "sameness" of the products.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have sought and may in the future seek a breakthrough therapy designation for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the drugs or biological products no longer meet the conditions for qualification.

A potential regenerative medicine advanced therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have sought and may in the future seek a regenerative medicine advanced therapy, or RMAT, designation for some of our product candidates. Under the 21st Century Cures Act, or the Cures Act, to be eligible to receive RMAT designation from the FDA, a product candidate must be (i) considered a "regenerative medicine therapy" as defined in the Cures Act; (ii) intended to treat, modify, reverse, or cure one or more serious or life-threatening diseases or conditions; and (iii) indicated, in preliminary clinical evidence, to have the potential to address unmet medical needs for such diseases or conditions. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition in the Cures Act of a regenerative medicine therapy.

The RMAT program is intended to facilitate efficient development and expedite review of such therapies. A new drug application or a BLA for a product candidate that has received an RMAT designation may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-

term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate that has received an RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

In June 2018, the FDA granted RMAT designation for the VY-AADC (NBIb-1817) gene therapy treatment for advanced Parkinson's disease with motor fluctuations that are refractory to medical management. The designation was based on data from the PD-1101 Phase 1b clinical trial.

RMAT designation is within the discretion of the FDA. Accordingly, even if we believe one of our other product candidates meets the criteria for RMAT designation, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of RMAT designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that a product candidate that received RMAT designation no longer meets the conditions for designation. Alternatively, we or our collaborative partners may decide not to proceed with the clinical development of a product candidate that has previously received RMAT designation or decide to pursue such product candidate for an indication for which it has not received RMAT designation.

Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of our product candidate.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical need for this condition, the drug sponsor may apply for FDA fast track designation. VY-AADC (NBIb-1817) has been granted fast track designation by the FDA. We may seek such a designation for our other product candidates. A fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. Thus, fast track products may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from a product candidate's clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidate.

If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the FDA's goal to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we or any current or future collaborators are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we or they may not be able to commercialize our products, and our ability to generate revenue may be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, export and import, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA and comparable regulatory authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process.

Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive; may take many years if additional clinical trials are required, if approval is obtained at all and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. In the United States, for example, the submission fee to obtain U.S. marketing approval is more than $2.0 million, and may be higher in the future. Changes in marketing approval policies during the development period, in or the enactment of additional statutes or regulations, or in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any current or future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Disruptions at the FDA and other agencies may also prolong the time necessary for new products to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. The Trump Administration also took several executive actions that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities.

Additionally, in July 2018, we received 510(k) regulatory clearance of V-TAG, our potential delivery device, from the Center for Devices and Radiological Health of the FDA, or CDRH. There are additional steps needed in making this device available for use including the manufacture of the product and compliance with state and federal laws and regulations for medical devices. We have relied on, and may in the future rely on, third parties in the development and manufacture of our potential delivery devices. In May 2018, for example, we entered into a master services and supply agreement with CLPT which provides for ClearPoint Neuro, Inc., or CLPT, to perform certain manufacturing, supply, development, and services as requested by us, including the supply of the ClearPoint System and cannula devices as well as to collaborate on V-TAG. In March 2019, we transferred our premarket notification (510(k)) clearance for the V-TAG device to CLPT. CLPT has sole responsibility for regulatory compliance related to V-TAG.

Accordingly, if we or any current or future collaborators experience delays in obtaining approval or if we or they fail to obtain or retain approval of our product candidates and devices, the commercial prospects for our product candidates may be harmed and our ability to generate revenues could be materially impaired.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storing, advertising, promoting, sampling, record-keeping and submitting safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practice, or cGMP, requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or such regulatory authority disagrees with the promotion, marketing or labeling of that product, the regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application, or any supplements thereto, submitted by us or our collaboration partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above

may inhibit our ability to commercialize our product candidates and adversely affect our business, financial condition, results of operations and prospects.

In addition, FDA policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would harm our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our gene therapy platform, product programs, product candidates and scientific expertise in the fields of gene therapy and neuroscience provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

We are aware of several companies focused on developing AAV gene therapies in various indications, including AAVANTIBio, Inc., Abeona Therapeutics, Inc., Adverum Biotechnologies, Inc., Aevitas Therapeutics, Inc., Alcyone Therapeutics, Inc., Amicus Therapeutics, Inc., Apic Bio, Inc., Applied Genetic Technologies Corporation, Asklepios BioPharmaceutical, Inc., or AskBio (acquired by Bayer), Audentes Therapeutics, Inc. (acquired by Astellas Pharma Inc.), Biogen, Inc., or Biogen, Brain Neurotherapy Bio, Inc. (merged with AskBio), Encoded Therapeutics, Inc., GenSight Biologics SA, Homology Medicines, Inc., LEXEO Therapeutics, Inc., LogicBio Therapeutics, Inc., Lysogene SA, MeiraGTx Ltd., or MeiraGTx, Neurogene, Inc., Novartis Gene Therapies, Inc. (formerly AveXis, Inc.), Passage Bio, Inc., Pfizer, Prevail Therapeutics, Inc. (acquired by Eli Lilly), PTC Therapeutics, Inc., REGENXBio Inc., Sarepta Therapeutics, Inc., Sio Gene Therapies, Inc., Solid Biosciences, Inc., Spark Therapeutics, Inc. (acquired by Roche), StrideBio, Inc., Taysha Gene Therapies, Inc. and uniQure, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

We expect that VY-AADC (NBIb-1817) will potentially compete with a variety of therapies currently marketed and in development for Parkinson’s disease, including DBS marketed by Medtronic plc, Abbott Laboratories (acquired from St. Jude Medical in 2017), and other medical device companies, DUOPA/Duodopa marketed by AbbVie, as well as other novel, non-oral forms of levodopa, including Mitsubishi Tanabe Pharma’s ND0612 (acquired from NeuroDerm in 2017), Acorda Therapeutics’ inhaled levodopa, INBRIJA, and Sunovion Pharmaceuticals’, or Sunovion’s, sublingual apomorphine, KYNMOBI. Gene therapy competition for Parkinson’s disease includes AAV2-GDNF being developed by Brain Neurotherapy Bio, Inc. and AAV-GAD being developed by MeiraGTx. Sio Gene Therapies, Inc. is developing a second generation LentiVector gene therapy, AXO-Lenti-PD (previously OXB-102, licensed from Oxford Biomedica in 2018).

We expect that our preclinical programs will compete with a variety of therapies in development, including:

- Our program for Huntington’s disease will potentially compete with TAK-686 being developed by Sangamo Therapeutics, Inc. in collaboration with Takeda, and AMT-130, an AAV gene therapy being developed by uniQure and a gene therapy being developed by Spark;

- Our program for a monogenic form of ALS will potentially compete with BIIB067 (IONIS-SOD1Rx) being developed by Biogen, in collaboration with Ionis, and gene therapies being developed by Novartis Gene Therapies, Inc. and Apic Bio, Inc.;

- Our program for Friedreich's ataxia will potentially compete with AAV gene therapies being developed by Pfizer, PTC Therapeutics, Inc., StrideBio, Inc. in collaboration with Takeda, AAVANTIBio, Inc., Novartis Gene Therapies, and LEXEO Therapeutics, Inc.;

- Our program for tauopathies including Alzheimer's disease, progressive supranuclear palsy, and frontotemporal dementia will potentially compete with tau antibodies being developed by Roche Genentech Inc. in collaboration with AC Immune SA, Eli Lilly & Co., AbbVie, Biogen, and several other companies, as well as an antisense oligonucleotide program being developed by Ionis in collaboration with Biogen;

- Our program for spinal muscular atrophy, or SMA, will potentially compete with Zolgensma being marketed by Novartis Gene Therapies, Spinraza being marked by Biogen, and Evrysdi being marked by Roche; and

- Our program for diseases linked to GBA1 mutations will potentially compete with AAV gene therapies being developed by Prevail Therapeutics Inc., Freeline Therapeutics Holdings plc, Pfizer, Biogen, Lysogene SA, and Coave Therapeutics;

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries, including recent transactions involving a number of gene therapy companies, may result in even more resources being concentrated among a smaller number of competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative agreements with large and established companies. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than us or may obtain orphan drug or other marketing exclusivity, which could result in our competitors establishing a strong market position before we are able to enter the market or reducing the number of available subjects for enrollment in our clinical trials to support regulatory submissions and approvals of our product. Additionally, technologies developed or acquired by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors. These third parties also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites, and registering patients for clinical trials.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. If we are not able to compete effectively against potential competitors, our business will not grow and our financial condition and operations will be harmed.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory

authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials or manufacturing control requirements. In many countries outside the United States, a product candidate must be separately approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates in the European Union but obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed.

Additionally, we could face heightened risks with respect to seeking marketing approval in the United Kingdom as a result of the recent withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the United Kingdom and the European Union, the United Kingdom withdrew from the European Union, effective December 31, 2020. On December 24, 2020, the United Kingdom and European Union entered into a Trade and Cooperation Agreement. The agreement sets out certain procedures for approval and recognition of medical products in each jurisdiction. Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of the Trade and Cooperation Agreement would prevent us from commercializing any product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for any product candidates, which could significantly and materially harm our business.

We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

Risks Related to Third Parties

To date, all of our revenue has been derived from our prior collaborations with Sanofi Genzyme and AbbVie, our ongoing collaboration with Neurocrine, and the Pfizer Agreement. If any ongoing or future collaboration or licensing agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.

In February 2015, we entered into the Sanofi Genzyme Collaboration Agreement to leverage our combined expertise and assets in gene therapy for neurological diseases. Under the Sanofi Genzyme Collaboration Agreement, we received an upfront commitment of approximately $100.0 million. Pursuant to the agreement, we granted Sanofi Genzyme an exclusive option to license, develop and commercialize (i) ex-U.S. rights to the VY-AADC Program, Friedreich's ataxia program, or FA Program, and the Huntington's disease program, or Huntington's Program, and a future program, collectively, the Split Territory Programs, with an incremental option to co-commercialize the product candidate from our Huntington's Program in the United States and (ii) worldwide rights to our spinal muscular atrophy program. If Sanofi Genzyme would have exercised an option for a Split Territory Program, except for the VY-AADC Program, it would have been required to make an option exercise payment to us. At the inception of the agreement, we were eligible to receive up to $745.0 million in the aggregate upon the achievement of specified regulatory and commercial milestones, as well as tiered royalty payments based on a percentage of net sales of product candidates from the programs for which Sanofi Genzyme exercised its option.

In June 2019, we and Sanofi Genzyme executed a termination agreement to terminate the Sanofi Genzyme Collaboration Agreement, or the Sanofi Genzyme Termination Agreement. Under the terms of the Sanofi Genzyme Termination Agreement, Sanofi Genzyme relinquished its rights to its exclusive license options to the Huntington's Program, FA Program and the unnamed future program described above, and we were relieved of our obligations to perform the research and development services under those programs under the Sanofi Genzyme Collaboration Agreement. As a result, we gained worldwide rights to the Huntington's Program and ex-U.S. rights to the FA Program. Our ex-U.S. rights to the FA Program were, in turn, transferred from us to Neurocrine Biosciences pursuant to the Neurocrine Collaboration Agreement. In connection with the Sanofi Genzyme Termination Agreement, we also relinquished our rights to the spinal muscular atrophy program. As of the termination date, we also waived our right to approximately $0.4 million in unused in-kind services, and we no longer had the right to receive any option payments, regulatory or commercial milestone payments or royalties from Sanofi Genzyme under the Sanofi Genzyme Collaboration Agreement.

In February 2018, we entered into an exclusive collaboration and option agreement with AbbVie, which we refer to as the AbbVie Tau Collaboration Agreement, for the research, development, and commercialization of AAV gene therapy products for the treatment of diseases of the central nervous system and other neurodegenerative diseases related to defective or excess aggregation of tau protein in the human brain, including Alzheimer's disease. Under the terms of the AbbVie Tau Collaboration Agreement, we received an upfront payment of $69.0 million and were eligible to receive option exercise payments, future development and regulatory milestone payments and royalties prior to the termination of the AbbVie Tau Collaboration Agreement, effective August 3, 2020. We continue to advance the research and development efforts related to vectorized antibodies, including vectorized antibody compounds comprised of an AAV or other virus vector genome that encodes one of more antibodies that target and bind to a tau protein. We are currently evaluating our options for advancing these efforts individually or with potential collaborators. If we seek another collaboration partner for the program, we may be unable to find a suitable collaborator on a timely basis, on terms acceptable to us, or at all. If we opt to progress this program ourselves, our expenditures would increase, and we might lack the resources or expertise that an appropriate collaboration partner could provide. If we are unable to find a suitable collaboration partner or unable or unwilling to increase our financial commitment to the tau program to undertake its development, we may have to delay or curtail the program.

In January 2019, we entered into the Neurocrine Collaboration Agreement for the research, development and commercialization of four programs including the VY-AADC Program, our FA Program, and two programs to be determined by us and Neurocrine at a later date, or the Discovery Programs. Under the terms of the agreement, we received an upfront payment of $115.0 million and may receive future development and regulatory milestones and royalties. In connection with the Neurocrine Collaboration Agreement, Neurocrine also paid us $50.0 million as

consideration for an equity purchase of 4,179,728 shares of our common stock. In June 2019, in conjunction with the termination of the Sanofi Genzyme Collaboration Agreement, we and Neurocrine amended the Neurocrine Collaboration Agreement to facilitate the transfer of the ex-U.S. rights to the FA Program which we acquired from Sanofi Genzyme to Neurocrine. In connection with the amendment, we received a $5.0 million payment from Neurocrine.

Under the terms of the Neurocrine Collaboration Agreement, subject to the rights retained by us thereunder, we have agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of our intellectual property rights for all human and veterinary diagnostic, prophylactic, and therapeutic uses for the research, development, and commercialization of gene therapy products, which we refer to as the Collaboration Products, under (i) the VY-AADC Program, on a worldwide basis; (ii) the FA Program, on a worldwide basis; and (iii) each Discovery Program, on a worldwide basis. We refer to each of these programs as a Neurocrine Program and, collectively, as the Neurocrine Programs.

Pursuant to development plans to be agreed by the parties, which will be overseen by a joint steering committee, we have operational responsibility, subject to certain exceptions, for the conduct of each Neurocrine Program (prior to specified transition events for each program), and are required to use commercially reasonable efforts to develop the Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by us in conducting these activities for each Neurocrine Program in accordance with an agreed budget. If we breach our development responsibilities or in certain circumstances upon a change in control of us, Neurocrine has the right but not the obligation to assume the activities under such Neurocrine Program.

Upon the occurrence of specified events for each program, Neurocrine agreed to assume responsibility for development, manufacturing and commercialization activities for such program and to pay us milestones and royalties on future net sales. For each of the VY-AADC Program and the FA Program, we have the option to co-develop and co-commercialize such program upon the occurrence of a specified event. Should we elect to exercise our co-development and co-commercialization option, we and Neurocrine have agreed to enter into a cost- and profit-sharing arrangement whereby we and Neurocrine agree to jointly develop and commercialize Collaboration Products for such program and share in its costs, profits and losses, and we forfeit certain milestones and royalties on net sales in the United States during the effective period of the applicable co-development and co-commercialization agreement. As described above, our research and development activities in connection with a collaboration might not be successful. Neurocrine may terminate the Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least 180-day advance notice if such notice is provided prior to the first commercial sale of the Collaboration Product to which the termination applies or one-year advance notice if such notice is provided after the first commercial sale of the Collaboration Product to which the termination applies. If Neurocrine were to terminate the agreement, we would become responsible for all research and development expenses relating to the Neurocrine Programs, and would not receive any future milestone payments or royalty payments under the Neurocrine Collaboration Agreement.

Neurocrine might not be successful in obtaining approvals for the product candidates arising from our collaboration, or commercializing or manufacturing the resulting products. Further, Neurocrine’s objectives in connection with the collaboration may not be consistent with our best interests. With respect to the rights granted to Neurocrine by us, Neurocrine could take actions that may be adverse to us, or it could halt, slow, or deprioritize its development and commercialization efforts under the collaboration. In any such instances, our business, financial condition, results of operations and prospects could be materially harmed.

On February 2, 2021, Neurocrine notified us that it had elected to terminate the Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program. This termination became effective August 2, 2021, which we refer to as the Neurocrine VY-AADC Program Termination Effective Date. The Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. Upon the termination of the VY-AADC Program, the license granted by us to Neurocrine expired, and we regained worldwide intellectual property rights to the VY-AADC Program in accordance with the collaboration agreement, and the restrictions on us to develop, manufacture or commercialize a gene therapy product directed to the targets specified in the VY-AADC Program terminated. We have determined that we will not advance the VY-AADC Program on our own following the Neurocrine VY-AADC Program

Termination Effective Date. Instead, we are evaluating potential options for partnering the future development and commercialization of the VY-AADC program.

In February 2019, we entered into a collaboration agreement, which we refer to as the AbbVie Alpha-Synuclein Collaboration Agreement, for the research, development, and commercialization of AAV gene therapy products directed against alpha-synuclein for indications including Parkinson's disease and other synucleinopathies. Under the terms of the AbbVie Alpha-Synuclein Collaboration Agreement, we received an upfront payment of $65.0 million and were eligible to receive option exercise payments, future development and regulatory milestone payments and royalties prior to the termination of the AbbVie Alpha-Synuclein Collaboration Agreement, effective August 3, 2020. We are currently evaluating our options for potentially advancing our alpha-synuclein program in the future. If we seek another collaboration partner for the program, we may be unable to find a suitable collaborator on a timely basis, on terms acceptable to us, or at all. If we opt to progress this program ourselves, our expenditures would increase, and we might lack the resources or expertise that an appropriate collaboration partner could provide. If we are unable to find a suitable collaboration partner or unable or unwilling to increase our financial commitment to the alpha-synuclein program to undertake its development, we may have to delay or curtail the program.

On October 1, 2021, we entered into the Pfizer Agreement, pursuant to which we granted Pfizer options to receive an exclusive license to novel capsids generated from our TRACER screening technology to develop and commercialize certain AAV gene therapy candidates comprised of a novel capsid and specified transgenes to help treat respective central nervous system and cardiovascular diseases. Under the terms of the Pfizer Agreement, we received an upfront payment of $30.0 million in October 2021 and are eligible to receive future option exercise payments of $10.0 million upon each of up to two option exercises; specified development, regulatory, and commercialization milestone payments following each option exercise of up to an aggregate of $115.0 million for the first licensed product to achieve such milestones; specified sales milestone payments of up to an aggregate of $175.0 million per licensed product; and tiered, escalating royalties in the mid- to high-single digit percentages of annual net sales of each licensed product. The results of Pfizer's evaluation of our capsids under the Pfizer Agreement might be unfavorable, or Pfizer may otherwise elect not to exercise either or both license options. If Pfizer did exercise one or more of its license options, it might subsequently choose not to develop product candidates based on our capsids or might be unable to obtaining approvals for any product candidates containing our capsids. Further, Pfizer's objectives in connection with the Pfizer Agreement may not be consistent with our best interests. Pfizer could use intellectual property it has licensed under the Pfizer Agreement in a manner adverse to us, or it could halt, slow, or deprioritize its development and commercialization efforts under the Pfizer Agreement. In any such instances, our business, financial condition, results of operations and prospects could be materially harmed.

We have only used the ClearPoint System to deliver our product candidates to date. While other devices for delivery may be used in the future, any issues with the ClearPoint System or the manufacturer of the ClearPoint System may result in delays in the development and commercialization of certain of our product candidates, which could have an adverse impact on our business.

The surgical approach that we are using for VY-AADC (NBIb-1817) is similar, in some respects, to the stereotactic approach used for DBS. One primary difference with our approach is the ability to assist the physician in visualizing the delivery of VY-AADC (NBIb-1817) to the putamen using real-time, intra-operative, magnetic resonance imaging, or MRI, scans to avoid specific blood vessels to potentially reduce the risk of hemorrhages during the surgical procedure and to maximize the coverage of the putamen.

Investigators in the PD-1101 Phase 1b clinical trial, the separate PD-1102 Phase 1 posterior trajectory trial, and the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817) have used and may continue to use the real-time, intra-operative, MRI imaging system known as the ClearPoint System. We also considered using the ClearPoint System in our planned VYTAL Phase 1/2 clinical trial before we decided to refocus the Huntington's disease program and withdraw our existing IND. The ClearPoint System is manufactured by CLPT. Not all neurosurgical units within the United States utilize the ClearPoint system and may employ other neuro-navigational systems that are not compatible with real-time MRI imaging. Investigators have used the ClearPoint System at certain sites in the RESTORE-1 Phase 2 clinical trial and may continue to use it in future clinical trials of VY-AADC (NBIb-1817) and any other of our product candidates that are injected directly into the brain. Therefore, any issues with the ClearPoint System, such as a finding that use of the ClearPoint System causes adverse events or a product recall, or issues with CLPT, the manufacturer of the

ClearPoint System, such as bankruptcy or a decision to stop production of the system due to lack of profitability, could delay the development or commercialization of certain of our product candidates, including VY-AADC (NBIb-1817) and VY-HTT01, as there currently is no other manufacturer of the ClearPoint System. Outside the United States, the ClearPoint System is not widely available or utilized in neurosurgical units.

We have developed V-TAG as our real-time, intra-operative device that is compatible with MRI imaging and can be used with other neuro-navigational systems to dose VY-AADC (NBIb-1817) and for other surgical procedures. We believe that the experience we have gained from delivering VY-AADC (NBIb-1817) in our clinical trials to date and our work to develop V-TAG may inform AAV gene therapy delivery for our Huntington's Program and other projects. In July 2018, we received 510(k) regulatory clearance of V-TAG from the CDRH. There are additional steps needed in making this device available for use, including the manufacture of the product and compliance with state and federal laws and regulations for medical devices.

We have relied on, and may in the future rely on, third parties in the development and manufacture of our potential delivery devices. In May 2018, we entered into a master services and supply agreement with CLPT for the development and manufacture of devices, including V-TAG. This agreement provides for CLPT to perform certain manufacturing, supply, development and other services, including the supply of the ClearPoint System and cannula devices. In March 2019, we transferred our premarket notification (510(k)) clearance for the V-TAG device to CLPT, and have worked with CLPT on the manufacturing and clinical supply of the device for relevant clinical trials.

We may seek to enter into collaborations, and out-licensing transactions in the future with other third parties. If we are unable to enter into such collaborations or out-licensing transactions, or if these collaborations or out-licensing transactions are not successful, our business could be adversely affected.

We may seek to enter into additional collaborations in the future, including sales, marketing, distribution, development, licensing, and/or broader collaboration agreements. For example, we entered into the Pfizer Agreement with Pfizer in October 2021. We believe there is significant opportunity for out-licensing transactions related to the novel capsids identified by our TRACER system. To maximize the potential of novel capsids identified by our TRACER system for our own programs and out-licensing transactions, we have retained to date, and expect to retain in the future, all rights associated with such novel capsids other than the rights specific to their use in combination with a particular licensee's transgenes. Our likely collaborators and licensees include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, biotechnology companies, and medical device manufacturers. However, we may not be able to enter into additional collaborations or out-licensing transactions on favorable terms or at all. Our ability to generate revenues from our collaborations and out-licensing transactions will depend on our and our collaborators and licensees' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our collaborators and licensees might have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our collaborator or licensee is responsible could be harmful to the public perception and prospects of our gene therapy platform.

Our relationship with any current or future collaborators or licensees may pose several risks, including the following:

- Collaborators and licensees have significant discretion in determining the amount and timing of the efforts and resources that they will apply to these collaborations and out-licensing transactions;
- Collaborators or licensees may not perform their obligations as expected or desired;
- the preclinical studies and clinical trials conducted as part of these collaborations or by our licensees may not be successful;
- collaborators or licensees may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on preclinical study or clinical trial results, changes in the collaborators or licensees'

strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;

- collaborators or licensees may delay preclinical studies and clinical trials, provide insufficient funding for preclinical studies and clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new preclinical studies or clinical trials or require a new formulation of a product candidate for preclinical studies or clinical trials;

- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration or by a licensee and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;

- collaborators or licensees could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators or licensees believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- product candidates developed in collaboration with us or by a licensee may be viewed by our collaborators or licensees as competitive with their own product candidates or products, which may cause collaborators or licensees to cease to devote resources to the commercialization of our product candidates;

- a collaborator or licensee with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;

- disagreements with collaborators or licensees, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities or expenses for us with respect to such product candidates (in the case of collaborations) or may result in litigation or arbitration, any of which would be time-consuming and expensive;

- collaborators or licensees may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- disputes may arise with respect to the ownership or inventorship of intellectual property developed pursuant to our collaborations or out-licensing transactions;

- collaborators or licensees may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

- the terms of our collaboration or license agreement may restrict us from entering into certain relationships with other third parties, thereby limiting our options; and

- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration and license agreements may not lead to the development or commercialization of product candidates in the most efficient manner, or at all. If our collaborations or out-licensing transactions do not result in the

successful development and commercialization of products, or if one of our collaborators or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration or out-licensing transactions. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed, and we may need additional resources to develop our product candidates. In the event we are unable to achieve milestones necessary to demonstrate progress on those programs, Neurocrine may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Additionally, subject to its contractual obligations to us, if a collaborator or licensee of ours were to be involved in a business combination, it might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us. If one of our collaborators or licensees terminates its agreement with us, we may find it more difficult to attract new collaborators or licensees, and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this periodic report also apply to the activities of our collaborators and licensees.

We will face significant competition in seeking appropriate collaborators and licensees, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration or license agreement with any future collaborators and licensees will depend, among other things, upon our assessment of the collaborator or licensee's resources and expertise, the terms and conditions of the proposed collaboration or out-licensing transactions and the proposed collaborator or licensee's evaluation of several factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator or licensee may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration or out-licensing transaction could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators or licensees. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators and licensees.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations or out-licensing transactions and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our gene therapy platform. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

We have relied, and we expect to continue to rely, on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if these third parties perform in an unsatisfactory manner, our business could be harmed.

We expect to rely on CROs, clinical trial sites, and other vendors to ensure our preclinical studies and clinical trials are conducted properly and on time. We may also engage third parties such as clinical data management organizations, medical institutions and clinical investigators to conduct or assist in our clinical trials or other preclinical and clinical research and development work. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our third-party service providers' activities. Nevertheless, we will be responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal, quality, regulatory and scientific standards. Our reliance on these third parties does not relieve us of our regulatory responsibilities. For example, the PD-1101 Phase 1b clinical trial

of VY-AADC (NBIb-1817) and the separate PD-1102 Phase 1 clinical trial exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory were conducted at several locations. The protocol for the RESTORE-1 Phase 2 clinical trial states that the clinical trial is intended to be conducted at over twenty clinical trial sites, including neurosurgical and neurology patient referral sites. Additionally, we had expected to initiate the planned VYTAL Phase 1/2 clinical trial for VY-HTT01 at multiple sites in the U.S. before our decision to refocus the Huntington's disease program. If any locations terminate a particular clinical trial, we or our collaborators would be required to find other parties or locations to conduct such clinical trial. We may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials. If we elect to internalize some or all activities related to the conduct of our preclinical studies or clinical trials that are currently performed by our third-party service providers, or if we are required to do so due to a service provider's termination of our relationship, then we may be required to source additional technology and personnel in order to perform the relevant activities. We may be unsuccessful in our efforts to internalize some or all relevant activities, either on the desired timeline or at all.

We and our third-party service providers are required to comply with the FDA's good laboratory practices, or GLPs, and GCPs for conducting, recording and reporting the results of IND-enabling preclinical studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. We are also required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. The FDA enforces these GLPs and GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites, and laboratories at which the FDA may determine that our preclinical studies and clinical trials did not comply with GLPs or GCPs. If we, our collaborators, or our third-party service providers fail to comply with applicable GLPs or GCPs, the preclinical or clinical data generated in our future preclinical studies or clinical trials may be deemed unreliable and the FDA may require us to perform additional preclinical studies or clinical trials before approving the relevant INDs or marketing applications. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if we, our collaborators, or our third-party service providers fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such preclinical studies or clinical trials, which would delay the regulatory approval process. Failure to comply can also result in fines, adverse publicity, and civil and criminal sanctions.

Our third-party service providers are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time, attention, expertise and resources to our clinical and nonclinical programs. These third-party service providers may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our third-party service providers do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our preclinical studies or clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

Risks Related to Manufacturing

Gene therapies and their companion diagnostics are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. To meet the requirements of our current and planned future trials we have developed a proprietary manufacturing platform that provides a robust and scalable process for AAV production. We are using the baculovirus/Sf9 AAV production system, a technology for producing AAV gene therapy vectors at scale in insect-

derived cells. The process has been successfully transferred to our contract manufacturing organizations where it is used in manufacturing clinical materials in accordance with the FDA's cGMPs. We have also built an onsite, state-of-the-art process research and development facility to enable the manufacturing of clinical quality AAV gene therapy vectors at laboratory scale.

We presently contract with third parties for the manufacturing of our program materials. We are currently assessing our manufacturing capabilities and although we do not currently have our own clinical or commercial scale manufacturing, we may choose to build those capabilities. The use of contracted manufacturing and reliance on collaboration partners is relatively cost efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements for our program materials. We expect third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. To meet our projected needs for commercial manufacturing, third parties with whom we currently work might need to increase their scale of production or we will need to secure alternate suppliers. We believe that there are alternate sources of supply for our program materials that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

To date, our third-party manufacturers have met our quality standards for our program materials. The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical study. If we or our manufacturers were to fail to comply with the FDA, EMA, or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Several of these raw materials, cells, and reagents are provided by a limited number of suppliers. Even though we aim to have backup supplies and suppliers of raw materials, cells, and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects on our manufacturing processes, including delays.

Companion diagnostic devices may be required to diagnose a genetic disease or to determine patient antibody levels to certain components in a product, and could also require a sophisticated, technically complex manufacturing processes. If we or our contract manufacturing organizations fail to manufacture such diagnostics or comply with relevant regulatory requirements or approvals, we might seek to transition such manufacturing processes to another contract manufacturing organization. We might not be able to transition such processes in a timely manner or at all, and our commercialization and development efforts could be delayed.

Delays in obtaining regulatory approval of our, or our collaborators', manufacturing processes and facilities, or disruptions in such manufacturing processes, may delay or disrupt our commercialization efforts. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product.

Before we can begin to commercially manufacture a product candidate in our own facility, or the facility of a collaborator, we must obtain regulatory approval from the FDA for our manufacturing process and our collaborator's facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product and, therefore, the timeframe required for us to obtain such approval is uncertain. In addition, we must pass a pre-approval inspection of our or our collaborator's manufacturing facility by the FDA and other relevant regulatory authorities before any of our product candidates can obtain marketing approval. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

The regulatory authorities may, at any time, following approval of a product for sale, audit the manufacturing facilities for such product or institute biennial inspections. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon our third-party manufacturers, our collaborators, or us could harm our business, financial condition, results of operations and prospects.

If our third-party manufacturers, our collaborators, or we fail to comply with applicable cGMP regulations, FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be harmed.

Additionally, if supply from any third-party manufacturers is delayed or interrupted, there could be a significant disruption in the supply of our clinical or commercial material. We have agreements in place with our contract manufacturers pursuant to which we are collaborating on cGMP manufacturing processes and analytical methods for the manufacture of our AAV product candidates. Therefore, if we are unable to enter into an agreement with our contract manufacturers to manufacture clinical or commercial material for our product programs, or if our agreement with our contract manufacturers were terminated, we would have to find suitable alternative manufacturers. This could delay our or our collaborators' ability to conduct clinical trials or commercialize our current and future product candidates. The regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

Any contamination in the manufacturing process for our products or product candidates, shortages of raw materials, cells or reagents, or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Interruptions in the supply of product candidates or inventory loss may harm our operating results and financial condition.

Our product candidates and our product delivery devices are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict government standards for the manufacture and storage of our product candidates and delivery devices, subjects us to manufacturing risks. While product candidate batches released for use in clinical trials or for commercialization undergo sample testing, some defects may only be identified following product release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products not complying with stability requirements or specifications. Our product candidates and delivery devices must be stored and transported at temperatures within a certain range and in sterile environments. If these temperature and environmental conditions deviate, the remaining shelf-life of a product candidate or utility of a device could be impaired or its efficacy and safety could be negatively impacted, making it no longer suitable for use.

The occurrence, or suspected occurrence, of manufacturing and distribution difficulties can lead to lost inventories and, in some cases, product recalls, with consequential reputational damage and the risk of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays of new product launches. Any interruption in the supply of finished products or the loss thereof could hinder our ability to timely distribute our products and satisfy customer demand. Any unforeseen failure in the storage of the product or loss in supply could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect our business, financial condition, results of operations and prospects.

Failure to obtain access to or to protect intellectual property related to the manufacturing of our products or product candidates may result in changes, delays and/or inability to manufacture such products or product candidates.

The intellectual property related to the manufacture of biological products is complex. If we are unable to maintain control of manufacturing technology such as our trade secrets, or we are unable to protect ongoing improvements comprehensively and in a sufficient number of jurisdictions, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

We presently manufacture our products using either an insect cell AAV production system or a mammalian cell system. We are aware of third parties which also use these systems in the manufacture of their products and who hold intellectual property on their AAV manufacturing systems. If we determine that access to certain third-party intellectual property is necessary for the manufacturing of our products and product candidates and are unable to license or otherwise access this intellectual property, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

Risks Related to Our Business Operations

We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity, or for which there is a greater likelihood of success.

The success of our business depends upon our ability to identify, develop and commercialize product candidates generated through our gene therapy platform. Research programs to identify new product candidates require substantial technical, financial and human resources. Our only active clinical trial, the RESTORE-1 Phase 2 clinical trial, is currently on clinical hold. It is uncertain when, or if, the FDA might release the hold. Although our collaboration partner Neurocrine has terminated our collaboration regarding VY-AADC, Neurocrine remains the sponsor of the RESTORE-1 Phase 2 clinical trial. Our other product candidates are in preclinical development, we may fail to identify other potential product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. Several of our current preclinical programs have previously been part of collaborations with third parties. While we have invested significant resources in these programs, we may decide in the future to cease development activities on one or more of them.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could harm our business, financial condition, results of operations and prospects.

Our future success depends on our ability to retain key members of our management team, and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, technical, and scientific expertise of principal members of our management, scientific, and clinical teams. While we have entered into employment agreements or offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We currently do not have “key person” insurance on any of our employees. The loss of the services of one or more of our current employees might impede the achievement of our research, development and commercialization objectives. Following the departures of our prior Chief Executive Officer and Chief Medical Officer and Head of Research & Development in May and June 2021, our current Chief Executive Officer and Chief Scientific Officer, each of whom is also a director of the Company, have been engaged by us on an interim basis pending the hiring of individuals to permanently fill these positions. The inability of either or both individuals to continue in these interim positions and to dedicate the time necessary to perform these roles could also impede our achievement of these objectives.

We are recruiting individuals to serve as our Chief Executive Officer and as our Chief Scientific Officer, in each case on a full-time basis. Recruiting for these two positions, and recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, is critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals

with similar skill sets. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval, the termination of relationships with collaborators, and the reduction of our workforce in connection with the development of a new portfolio and platform strategy may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and could harm our business, financial condition, results of operations and prospects.

We have recently reduced the size of our organization, and we may encounter difficulties in managing our business as a result of this reduction, or the attrition that may occur following this reduction, which could disrupt our operations. In addition, we may not achieve anticipated benefits and savings from the reduction.

On August 6, 2021, our board of directors approved a strategic restructuring plan to eliminate a portion of our workforce as part of an initiative to reduce expenses and enhance operations. The strategic restructuring plan was approved in connection with our portfolio reevaluation efforts and strategic shift to invest additional resources in our novel capsid development efforts.

During the three months ended September 30, 2021, we incurred restructuring costs of approximately $2.0 million, which primarily consists of severance related costs.

The restructuring and additional measures we might take to reduce costs could divert management attention, yield attrition beyond our intended reduction if force, reduce employee morale, or cause us to delay, limit, reduce or eliminate certain product development plans.

If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

Although we are undergoing a strategic restructuring, if we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. We can provide no assurances that we will have sufficient resources in the future to manage all of our planned programs. Future growth would impose significant added responsibilities on members of management, may lead to significant added costs, and may divert our management and business development resources. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants, collaborators, and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or

other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Current and future legislation may increase the difficulty and cost for us and any collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any collaborators, to profitably sell any products for which we obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any collaborators, may receive for any approved products. If reimbursement of our products is unavailable or limited in scope, our business could be materially harmed.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the ACA. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2030 under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit affirmed the lower court's ruling that the individual mandate portion of the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On June 17, 2021, the U.S. Supreme Court dismissed this case after finding that plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine: policies that undermine

protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our drug products, if and when approved.

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States and other jurisdictions. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for products. To those ends, President Trump issued several Executive Orders intended to lower the costs of prescription drug products. Certain of these Executive Orders are reflected in recently promulgated regulations, including an interim final rule implementing President Trump's most favored nation model, but such final rule is currently subject to a nationwide preliminary injunction. It remains to be seen whether these Executive Orders and resulting regulations will remain in force during the Biden Administration. Further, on September 24, 2020, the Trump Administration finalized a rulemaking allowing states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants are required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. The FDA has issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products). At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, health care organizations and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

In other countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain reimbursement or pricing approval in some countries, we, or our collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our products or product candidates to other available therapies. If reimbursement of our products or product candidates is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

We may be subject, directly or indirectly, to federal, state, and foreign healthcare laws and regulations, including fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims act, and the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to data privacy laws by both the federal government and the states in which we conduct our business. Such laws that may

constrain the business or financial arrangements and relationships through which we conduct our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for either the referral of an individual for, or the purchase, recommendation, leasing or furnishing of, an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. Further, the ACA amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;

- the federal civil and criminal false claims laws and civil monetary penalty laws, including the civil False Claims Act, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. The ACA provided and recent government cases against pharmaceutical and medical device manufacturers support the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the civil False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing or attempting to execute a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

- federal transparency laws, including the federal Physician Payments Sunshine Act, which is part of the ACA, that requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members, by the 90th day of each subsequent calendar year, and disclosure of such information is made by CMS on a publicly available website; and

- state and/or foreign law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws that may apply to arrangements and claims involving health care items or services reimbursed by non-governmental third-party payors; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; and state and foreign laws governing the privacy and security of health information in

certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the European Union General Data Protection Regulation, or the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR has been and will continue to be a rigorous and time-intensive process that has increased and will continue to increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we or our collaborators may be subject to fines and penalties, litigation, and reputational harm in connection with any European activities, which could adversely affect our business, prospects, financial condition and results of operations.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;

- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage in the amount of $1.0 million per occurrence and $2.0 million in the aggregate, and clinical testing liability insurance in the amount of $10.0 million per occurrence and $10.0 million in the aggregate, this insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or from any other work-related injuries, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance and workers' compensation insurance for certain costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could harm our business, financial condition, results of operations and prospects.

Further, with respect to the operations of any current or future collaborators or third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products.

A widespread outbreak of an illness or other health issue could significantly disrupt our operations. The current coronavirus disease 2019 (COVID-19) pandemic and the response to it have had, and we expect they will continue to have, an adverse effect on our business, operations, and future results.

Health issues such as epidemics or other medical emergencies outside of our control could significantly disrupt our operations and negatively impact our business.

In December 2019, a novel strain of coronavirus called severe acute respiratory syndrome coronavirus 2, also referred to as SARS-CoV-2, which causes the coronavirus disease 2019, also referred to as COVID-19, began to be reported in China and other countries. The World Health Organization has declared the outbreak a pandemic and a global public health emergency. In addition to those who have been directly affected, millions more have been affected by local and national government efforts in the United States, the European Union and around the world to slow the spread of the pandemic through quarantines, travel restrictions, heightened border scrutiny and other measures.

The COVID-19 pandemic continues to evolve rapidly. Our corporate headquarters is in Massachusetts, a state particularly hard hit by the initial wave of the pandemic. We have and will continue to adhere to applicable guidelines and safety measures including stay-at-home policies and the reporting of only essential personnel for business continuity to ensure the safety of our employees, consultants, contractors, and staff. Certain of our clinical trial sites and collaboration partners have experienced facility closures or been subject to quarantines, travel restrictions and other governmental restrictions and have appropriately diverted attention and resources to respond to the impacts of COVID-19 on their own operations and personnel. Some have even become involved in research and development efforts related to COVID-19. Additionally, we have experienced, and may continue to experience, delays in services provided to us by CROs and third-party manufacturers, including delays and disruptions due to the limited availability of materials such as glass that are also utilized in the manufacturing of COVID-19 vaccines.

The current workplace safety measures that we have enacted in response to COVID-19 have required a reduction in on-site activity at our facilities in Massachusetts, including in our laboratories in which preclinical experiments are conducted. As a result, we have had to prioritize our preclinical experiments and terminate or delay some non-critical experiments in order to maintain critical experiments for our preclinical programs. If these measures must be maintained for an extended period of time, or if more restrictive workplace safety measures are recommended by federal and state authorities, we may need to delay or terminate other preclinical experiments, including critical experiments for our preclinical programs, which we expect could have a material adverse impact on our development and regulatory plans and timelines for our preclinical programs. To the extent that any preclinical experiments impacted in this manner relate to a collaboration program, our reimbursement revenues from collaborators for the relevant activities may decrease or be delayed.

Additionally, prior to the FDA's imposition of a clinical hold on the RESTORE-1 Phase 2 clinical trial, we experienced a slower pace of enrollment in the clinical trial than we had expected because we and our collaboration partner Neurocrine paused patient screening in April 2020 to evaluate, among other things, the safety of trial participants due to the COVID-19 pandemic.

The extent to which COVID-19 ultimately impacts our business, financial condition, and results of operations will depend on future developments such as the duration and scope of the pandemic and the response of policymakers, businesses and individuals that are highly uncertain and cannot be accurately predicted. In the future, there may be other material adverse impacts on our business and operations during the pandemic and once it subsides. Employees and other key personnel could become ill, quarantined, or otherwise unable to work and/or travel due to health reasons or governmental restrictions. Disruptions in the supply chain for personal protective equipment and other supplies critical for laboratory operations and/or the maintenance of current or future workplace safety measures could limit our ability to maintain business continuity. Regulators could be delayed in inspections, reviews, and approvals of product candidates including INDs and BLAs. Quarantines and travel restrictions could impact the ability of our third-party manufacturers and other suppliers to deliver clinical supplies or raw materials to us in a timely manner. Restrictions imposed on the construction industry could cause delays in completing our current and contemplated construction projects, resulting in program delays, cost increases and disruption to our current laboratory activities and general operations. Prolonged stay-at-home policies and a distributed workforce could inhibit our ability to restore operations to pre-COVID-19 pandemic

norms and to attract, retain, and motivate qualified personnel, and consequently, to allow our operations to develop as anticipated and to make our expected organizational growth more difficult. We are dedicating financial resources towards mitigating operational adjustments arising from the COVID-19 pandemic. If we need to access the capital markets to address requirements arising from the impacts of COVID-19 pandemic, there is no assurance that financing will be available on attractive terms, if at all.

We will continue to monitor the issues raised by the global spread of COVID-19 and have put in place and will continue to put in place measures as appropriate and necessary for, or that we believe to be in the best interest of, our business, employees, collaborators, stockholders, and the community. However, there is no assurance that the pandemic will not have a material adverse impact on our business, operations, and future results.

Risks Related to the Commercialization of Our Product Candidates

The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated prevalence range for the indications we are targeting has involved collating limited data from multiple sources. While we believe these sources are reliable, we have not independently verified the data. Accordingly, the prevalence estimates included in our periodic reports and other reports filed with or furnished to the Securities and Exchange Commission, or SEC, should be viewed with caution. Further, the data and statistical information used in such reports, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties, and such data is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business. Additionally, because some patients with the diseases we are targeting in the United States, the European Union, and elsewhere may have increased susceptibility to COVID-19, the COVID-19 pandemic could limit the number of patients willing to participate in clinical trials related to our products or amenable to treatment with our products, which would harm our results of operations and our business.

If we are unable to establish sales, medical affairs and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any product revenue.

To successfully commercialize any products that may result from our clinical development programs, we will need to further develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

Under the Neurocrine Collaboration Agreement, Neurocrine agreed to fund the clinical development through the readout of the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817). If Neurocrine had not terminated the Neurocrine Collaboration Agreement with respect to VY-AADC (NBIb-1817), after the data readout of the RESTORE-1 Phase 2 clinical trial, we would have had the option to either: (1) co-commercialize VY-AADC (NBIb-1817) with Neurocrine in the United States under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties

based on ex-U.S. sales, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine. Under the terms of the Neurocrine Collaboration Agreement for the FA Program, Neurocrine has agreed to fund the development through the Phase 1 clinical trial of VY-FXN01. After the data readout of the Phase 1 clinical trial, we have the option to either: (1) co-commercialize VY-FXN01 with Neurocrine in the United States under a 60/40 cost- and profit-sharing arrangement, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine.

In the future, we may seek to enter into collaborations regarding other of our product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. We might face unforeseen costs and expenses associated with creating an independent sales and marketing organization. Our sales personnel might also face difficulties obtaining access to physicians or being able to persuade adequate numbers of physicians to use or prescribe our products or selling our products if we lack complementary products, which could disadvantage us compared to companies with more extensive product lines. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients, or third-party payors, we will not be able to generate significant revenues from such product, which could harm our business, financial condition, results of operations and prospects.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they receive regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor’s determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient and the indication;
- convenient and easy-to-administer compared to alternative treatments;
- cost-effective compared to alternative treatments; and
- neither experimental nor investigational.

No uniform policy requirement for coverage and reimbursement for biopharmaceutical products exists among third-party payors. Therefore, coverage and reimbursement for such products can differ significantly from payor to payor. As a result, obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to each different payor supporting scientific, clinical and cost-effectiveness data, and to receive the support of medical associations and technology assessment committees. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment including our research, development, manufacture, sales, and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement are critical to new product acceptance. Additionally, there may be significant delays in obtaining coverage and reimbursement for newly approved drugs and biologics, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products.

The CMS is responsible for determining whether a product should be approved for coverage and reimbursement under the Medicare program. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for novel products such as ours, as there is no body of established practices and precedents for these types of products. Currently, no gene therapy product has been approved for coverage and reimbursement by the CMS. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates, especially given that the cost of our product candidates is likely to be very high and pricing of such products is highly uncertain.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors

increasingly are considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug and device products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Therefore, it is difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from the FDA in the United States, EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the support and acceptance of medical associations and technology assessment committees, physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission, or other regulatory authorities;
- patient awareness of, and willingness to seek, genotyping;
- the willingness of physicians to prescribe new therapies;

- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling or restrictions on the use of our products together with other medications;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Our gene therapy approach utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology, with few gene therapy products approved to date in the United States and the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. Medical events such as the COVID-19 pandemic that emphasize harmful effects of certain viruses could also indirectly foster negative public perception of virus-based therapies. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop.

For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using non-AAV gene therapy vectors. Adverse events and SAEs in our clinical trials such as the MRI abnormalities detected in some patients dosed in the RESTORE-1 Phase 2 clinical trial, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

If we obtain approval to commercialize our product candidates outside of the United States, in particular in the United Kingdom or European Union, a variety of risks associated with international operations could harm our business.

We expect that we will be subject to additional risks in commercializing our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced or loss of protection under our intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism; from natural disasters including earthquakes, typhoons, floods and fires; or from social, economic or political instability; and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

We must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In many foreign countries, it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the FCPA. In addition, the FCPA presents particular challenges in the pharmaceutical industry because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from

developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Although we expect to implement policies and procedures designed to comply with these laws and policies, there can be no assurance that our employees, contractors and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Risks Related to Our Intellectual Property

Our rights to develop and commercialize our product candidates are subject to, in part, the terms and conditions of licenses granted to us by others.

We are reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. These licenses may also require us to grant back certain rights to licensors and to pay certain amounts relating to sublicensing patent and other rights under the agreement.

In some circumstances, particularly in-licenses with academic institutions, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce or defend the patents, covering technology that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. In certain circumstances, we have or may license technology from third parties on a non-exclusive basis. In such instances, other licensees may have the right to enforce our licensed patents in their respective fields, without our oversight or control. Those other licensees may choose to enforce our licensed patents in a way that harms our interest, for example, by advocating for claim interpretations or agreeing on invalidity positions that conflict with our positions or our interest. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we own or may own in the future.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly diligence obligations in developing and commercializing the product. Certain of our license agreements contain "no challenge" clauses which preclude and prevent us from taking any action to limit or narrow the intellectual property of a licensor. In some cases, these limitations extend to any intellectual property of our licensor and not just that which is licensed to us. Such constraints may limit our ability to develop or commercialize products or to expand such efforts beyond the scope of any license. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;

- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

If we fail to comply with our obligations under these license agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to develop, manufacture, or market products covered by the license or may face other penalties under the agreements. Termination of any of our agreements involving intellectual property or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Termination may also result in unfavorable terms associated with such termination or may result in obligations on our part to license or grant back intellectual property rights to prior licensors.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive, royalty-free license authorizing the U.S. government, or a third party on its behalf, to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The U.S. government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government, or a third party on its behalf, of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not of sufficient breadth, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

Our success depends, in large part, on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates and manufacturing technology. We and our licensors have sought, and we intend to seek in the future, to protect our proprietary position by filing patent applications in the United States and abroad related to many of our technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not have and may not in the future be able to file, prosecute, maintain, enforce, defend or license all necessary or desirable patent applications in some or all relevant jurisdictions at a reasonable cost or in a timely manner. For example, in some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we may not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. In some cases, we may be able to obtain patent protection, but such protections may expire before we commercialize the product protected by those rights,

leaving us no meaningful protection for our products. In other cases, where our intellectual property is being managed by a third-party collaborator, licensee or partner, that third party may fail to act diligently in prosecuting, maintaining, defending or enforcing our patents. Such conduct may result in the failure to maintain or obtain protections, loss of rights, loss of patent term or, in cases where a third party has acted negligently or inequitably, patents being found unenforceable.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value, narrow the scope, or eliminate the enforceability of our and our licensors' patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, only upon issuance or not at all. Therefore, we cannot be certain that we, or a licensor, were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, respectively, or which entity was the first to file for patent protection until such patent application publishes or issues as a patent. Databases for patents and publications, and methods for searching them, are inherently limited, so it is not practical to review and know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensed patent rights are uncertain.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

In spite of a legal presumption of validity, the issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity, or enforceability which may be challenged in the courts and patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology, resulting in termination of our access to such intellectual property, or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, result in loss of access, or increase what we believe to be our financial or other obligations under the relevant agreement, any of which could harm our business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to certain intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical or technical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We currently co-own certain intellectual property rights with one or more third parties. We may not be able to obtain a license to the third parties' interest such that we have exclusive access and control of such co-owned assets. In this case, and depending on the jurisdiction of the patent filing, we may not be able to license, enforce, or exploit the co-owned rights without the consent from, or an accounting to, the other co-owners.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to develop our program. We may also decide not to exercise an option to such institutional rights.

If we decide not to obtain, or are unable to successfully obtain, rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office, or USPTO, and various government patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own in the future. We rely on our outside counsel or our licensing partners to pay these fees due to patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction, and may compromise the strength of other intellectual property in our portfolio. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

On February 1, 2019 the government of Venezuela, in response to certain U.S. sanctions, began to require that foreign entities pay all official fees, including patent fees (either for pending matters or new petitions), in PETRO, a

"cryptocurrency" created by the Nicolás Maduro administration in February 2018 as a way to collect U.S. dollars while avoiding American financial sanctions issued under an Executive Order of President Trump on March 19, 2018. The Executive Order banned transactions involving "any digital currency, digital coin, or digital token, that was issued by, for, or on behalf of the Government of Venezuela on or after January 9, 2018." The prohibition is applicable to any U.S. entity unless exempted by license. We do not hold such a license and therefore may not be able to secure patents in Venezuela.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive. Our intellectual property rights may vary from country to country and foreign protections could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products or methods of treatment, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. For example, an April 2014 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. With Brexit, there is uncertainty associated with obtaining, defending, and enforcing intellectual property rights in the United Kingdom. International treaties and regulations promulgated as a result of this transition could impede or eliminate our ability to obtain or maintain meaningful intellectual property rights in the United Kingdom. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Issued patents covering our technology or product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent covering our technology or one of our product candidates, the defendant could counterclaim that the patent covering such technology or product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that an individual connected with prosecution of the patent, including an inventor, an employee of the company, a collaborator or advisor, withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include pre-issuance submissions, *ex parte* re-examination, post-grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Some of these mechanisms may even be exploited anonymously by third parties. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our technology or product candidates. The outcome following

legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose part or, all of the patent protection on one or more of our product candidates or our supporting technology. Such a loss of patent protection could harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection, nondisclosure, and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, contractors, and other third parties. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or threatened with, infringement litigation claims regarding our products and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including *ex parte* re-examination, post-grant review and *inter partes* review before the USPTO or foreign patent offices. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of the merit of the claim. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such asserted third-party U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similar challenges exist in other jurisdictions. If we are found to infringe a third-party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals, and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, reputation, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may infringe our intellectual property rights or the intellectual property rights of our licensing partners, or we may be required to defend against claims of infringement. To counter infringement or unauthorized use claims or to defend against claims of infringement can be expensive and time consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our directors, employees, consultants, and advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that these individuals do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees, consultants, advisors and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a "first-to-invent" system to a "first-inventor-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-inventor-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the

invention earlier. The USPTO has promulgated regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-inventor-to-file provisions, became effective on March 16, 2013. The Leahy-Smith Act has resulted in an increased investment in filing applications earlier, and consequently has increased the uncertainties and costs surrounding the prosecution of our patent applications, and may increase the enforcement or defense of our issued patents, all of which could harm our business, financial condition, results of operations and prospects.

The administrative tribunal created by the Leahy-Smith Act, known as the Patent Trial and Appeals Board, or PTAB, may have an impact on the operation of our business in the future. For example, the initial results of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many U.S. patent claims. The availability of the PTAB as a lower-cost, faster and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own licensed patents will be challenged, thereby increasing the uncertainties and costs of maintaining and enforcing them. Moreover, if such challenges occur, we may not have the right to control the defense. In certain situations, we may be required to rely on our licensor to consider our suggestions and to defend such challenges, with the possibility that it may not do so in a way that best protects our interests.

We also may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in other contested proceedings such as opposition, derivation, reexamination, *inter partes* review, or post-grant review proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future products.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain as the courts address issues such as patenting genes or gene products. The most recent guidance provided under *Berkheimer v HP, Inc*. (April 19, 2018) and *Vanda Pharmaceuticals, Inc. v West-Ward Pharmaceuticals* (June 7, 2018) instruct USPTO examiners on the ramifications of the court rulings as applied to natural products and principles including all naturally occurring nucleic acids. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, the recent USPTO guidance could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the court decisions referenced above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact decisions from the U.S. Supreme Court's decisions in *Mayo Collaborative Services v. Prometheus Laboratories* and *Molecular Pathology v. Myriad Genetics, Inc.* or other applicable court decisions may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have an adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Moreover, although the U.S. Supreme Court has held that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

Outside the United States, other courts have also begun to address the patenting of genetic material. In August 2015, the Australian High Court ruled that isolated genes cannot be patented in Australia. The decision did not address

methods of using genetic material. Any ruling of a similar scope in other countries could affect the scope of our intellectual property rights. The ambiguities and changing law in all countries as to patenting genetic material may directly affect our ability to secure and/or maintain patent protection for our products.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be harmed.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents, which may cover non-gene therapy compounds, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended per FDA-approved product, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Further, certain of our licenses currently or in the future may not provide us with the right to control decisions the licensor or its other licensees on Orange Book listings or patent term extension decisions under the Hatch-Waxman Act. Thus, if one of our important licensed patents is eligible for a patent term extension under the Hatch-Waxman Act, and it covers a product of another licensee in addition to our own product candidate, we may not be able to obtain that extension if the other licensee seeks and obtains that extension first. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements.

The Biologics Price Competition and Innovation Act of 2009 provides up to 12 years of market exclusivity for a reference biological product. We may not be able to obtain such exclusivity for our products. Moreover, the applicable time-period or the scope of patent protection afforded during any such extension could be less than we request. If we are unable to obtain patent term extension or the scope of term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be materially reduced.

If our trademarks and trade name are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We own service mark registrations in the USPTO for the marks “VOYAGER THERAPEUTICS” and “VOYAGER THERAPEUTICS Logo” and European Community trademark registrations for the marks “V-TAG” and “VOYAGER TRAJECTORY ARRAY GUIDE.” Our trademarks or our trade name may be challenged, infringed, circumvented or declared generic or found to infringe prior third-party marks. We may not be able to protect our rights in our trademarks or in our trade name, which we need in order to build name recognition among potential partners or customers in our markets of interest. It is possible that competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of prior registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade name. Over the long term, if we are unable to establish name recognition based on our trademarks and trade name, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce and protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights and other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and such rights may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we own, license or may access in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain inventions, trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

We may not be able to maintain sufficient control over our proprietary know-how or trade secrets when employees, consultants, advisors or persons with access to our proprietary information terminate their relationship with us.

Despite our efforts to protect our proprietary know-how and trade secrets, our competitors may discover this information, or obtain the benefit of this information, through a breach of confidentiality and/or non-competition obligations by persons who were formerly associated with us but who have established relationships as employees, contractors, consultants or advisors with other companies, including our competitors. The recent departures of certain executives, key employees, consultants or advisors, and the restructuring of our organization, may make it more difficult to enforce our rights in protecting this information. Further, if discovered in a timely manner, our efforts to enforce rights to protect against these types of breaches may not be possible under law, or may not be successful if commenced.

It is also possible that, as we grow and establish ourselves in multiple geographic areas, alignment and/or compliance with company polices may not be consistently maintained. In any such cases, the risk of loss of control or proper management of our proprietary information could jeopardize our intellectual property.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our product candidates and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our gene therapy platform and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

Changes to national patent laws and diminished or limited access to United States and/or foreign patent counsel and the courts in response to the ongoing COVID-19 pandemic may compromise our ability to pursue, obtain, enforce or defend our intellectual property patent protections throughout the world.

In response to the ongoing COVID-19 pandemic, many national patent offices promulgated emergency measures and alternative procedures for filing, prosecuting and adjudicating disputes regarding intellectual property. While some of these new rules involve the provision of extensions for certain filing deadlines, none of these emergency-situation rules have been tested in a litigation setting or for their harmonization with the laws of other countries.

Access to the USPTO and other patent offices were, and may again in the future, be restricted by government mandated shelter-in-place or stay-home orders thereby limiting our ability to appear before any tribunal in support of our intellectual property. Should direct or electronic access to these tribunals be interrupted or become non-existent again in the future, we may not be able to secure, defend or enforce patent protections in all jurisdictions.

We also rely on United States and foreign patent counsel in the management of our intellectual property. Should our access to counsel be diminished or lost due to effects of COVID-19 on these service providers and their organizations, we may not be able to manage, maintain or secure our intellectual property position.

Risks Related to Ownership of Our Common Stock

Our executive officers, directors, principal stockholders and their affiliates exercise significant influence over our company.

The holdings of our executive officers, directors, principal stockholders and their affiliates, including investment funds affiliated with Third Rock Ventures, LLC, Armistice Capital LLC, and Neurocrine represent beneficial ownership, in the aggregate, of approximately 44% of our outstanding common stock as of September 30, 2021. As a result, these stockholders, if they act together, will be able to influence our management and affairs and the outcome of matters submitted to our stockholders for approval, including the election of directors and any sale, merger, consolidation, or sale of all or substantially all of our assets. In addition, this concentration of ownership might adversely affect the market price of our common stock by:

- delaying, deferring or preventing a change of control of us;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

An active trading market for our common stock may not be sustained.

Our shares of common stock began trading on the Nasdaq Global Select Market on November 11, 2015. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Persons who were our stockholders prior to our initial public offering continue to hold a substantial number of shares of our common stock. If such persons sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline.

In addition, shares of common stock that are either subject to outstanding options or restricted stock units, or RSUs, or reserved for future issuance under our stock incentive plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended. We have also filed registration statements on Form S-8 permitting shares of common stock issued on exercise of options or the settlement of RSUs to be freely sold in the public market. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. We also have an effective registration statement on Form S-3 for the sale of up to $300.0 million in aggregate of an indeterminate number of shares of common stock and preferred stock, an indeterminate principal amount of debt securities, and an indeterminate number of warrants, of which we have reserved $100.0 million for the offering, issuance, and sale of common stock through at-the-market offerings or negotiated transactions under a sales agreement we entered into with Cowen and Company, LLC, on November 6, 2019.

Certain holders of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.

The price of our common stock is likely to be volatile and may fluctuate substantially. From July 1, 2021 through September 30, 2021, the sales price of our common stock ranged from a high of $4.22 to a low of $2.61 on the Nasdaq Global Select Market. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- our success in commercializing any product candidates for which we obtain marketing approval;
- regulatory action and results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- the results of clinical trials of our product candidates;
- the results of clinical trials of product candidates of our competitors;
- the commencement, termination, and success of our collaborations, including the ability or willingness of our collaboration partners to fulfill their obligations to us;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel, including in connection with our ongoing strategic restructuring;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or technologies, the cost of commercializing such product candidates, and the cost of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- the ability to secure third-party reimbursement for our product candidates;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section and elsewhere in this Quarterly Report on Form 10-Q.

If our operating results fall below the expectations of investors or securities analysts for a given period, the price of our common stock could decline substantially. Furthermore, any fluctuations in our operating results from period to period may, in turn, cause the price of our stock to fluctuate substantially. We believe that such comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. We also may face securities class-action litigation if we cannot obtain regulatory approvals for or if we otherwise fail to commercialize our product candidates. As described in "Part II, Item 1—Legal Proceedings," we and certain of our current and former officers and directors were named as defendants in a purported class action lawsuit. This proceeding and other similar litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

We have broad discretion in how we apply our available funds, and we may not use these funds effectively, which could affect our results of operations and cause our stock price to decline.

Our management will have broad discretion in the application of our existing cash, cash equivalents and marketable securities and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply our available funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates and preclinical programs. Pending their use, we may invest our available funds in a manner that does not produce income or that loses value.

We have recently been, and could in the future be, subject to legal actions and proceedings related to the decline in our stock price, which could distract our management and could result in substantial costs or large judgments against us.

The market prices of securities of companies in the biotechnology and pharmaceutical industry, including the market price of our common stock, have been extremely volatile and have experienced fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. On January 22, 2021, a putative class action lawsuit was filed in the U.S. District Court for the Eastern District of New York (later transferred to the U.S. District Court for the District of Massachusetts) against us and certain of our current and former officers and directors. The complaint sought, among other things, unspecified compensatory damages, interest, attorneys' and expert fees and costs. On July 2, 2021, the lead plaintiff voluntarily dismissed the action without prejudice against all defendants and as to all claims, and this action is no longer pending. Nonetheless, due to the volatility in, or the unfulfilled expectations for stockholders for, our stock price, we may be the target of similar litigation in the future.

In connection with such legal proceedings, we could incur substantial costs and such costs and any related settlements or judgments may not be covered by insurance. We could also suffer an adverse impact on our reputation and a diversion of management's attention and resources, which could cause serious harm to our business, operating results and financial condition.

We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, and rules subsequently implemented by the SEC and The Nasdaq Stock Market have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs and have made some activities more time-consuming and costly. For example, these rules and regulations have made it more difficult and more expensive for us to obtain director and officer liability insurance, and we have been required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar

coverage. As a result, it may be more difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. To remain compliant with Section 404, we must continue to engage in the process of documenting and evaluating our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Provisions in our amended and restated certificate of incorporation and bylaws and Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of members of the board is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our amended and restated certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision is inapplicable to actions arising under the Securities Exchange Act of 1934, as amended, and we likewise do not intend to apply this choice of forum provision to actions arising under the Securities Act of 1933, as amended.

This choice of forum provision may limit a stockholder's ability to bring a claim that is not arising under the Securities Exchange Act of 1934, as amended, or the Securities Act of 1933, as amended, in a judicial forum that he, she or it finds favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs and business interruption that could have a material adverse effect on our business, financial condition or results of operations.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

General Risk Factors

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A domestic or global financial crisis can cause extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the most recent global financial crisis associated with the COVID-19 pandemic, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or

declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payors or our collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

Changes in tax law may adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted the TCJA, which significantly revised the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contained significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for net interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses, or NOLs, to 80% of current year taxable income and elimination of NOL carrybacks, in each case, for losses arising in taxable years beginning after December 31, 2017 (though any such NOLs may be carried forward indefinitely), imposition of a one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

As part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, the CARES Act was enacted on March 27, 2020 and COVID-19 relief provisions were included in the Consolidated Appropriations Act, 2021 or CAA, which was enacted on December 27, 2020. All contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of NOLs, which was enacted as part of the TCJA. It also provides that NOLs arising in any taxable year beginning after December 31, 2017, and before January 1, 2021, are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30 to 50% of adjusted taxable income.

Regulatory guidance under the TCJA, the FFCR Act, the CARES Act and the CAA is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also possible that Congress will enact additional legislation in connection with the COVID-19 pandemic, and as a result of the changes in the U.S. presidential administration and control of the U.S. Senate, additional tax legislation may also be enacted; any such additional legislation could have an impact on us. In addition, it is uncertain if and to what extent various states will conform to the TCJA, the FFCR Act, the CARES Act or the CAA.

We might not be able to utilize a significant portion of our net operating loss carryforwards.

As of December 31, 2020, we had both federal and state NOL carryforwards of $153.4 million and $140.9 million, respectively, which expire beginning in 2033. These NOL carryforwards could expire unused and be unavailable to offset our future income tax liabilities. As described above under the heading "Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition," the TCJA, as amended by the CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. Nor is it clear how various states will respond to the TCJA, the FFCR Act or the CARES Act. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. Furthermore, the use of NOL carryforwards may become subject to an annual limitation under Section 382 of the Code and similar state provisions in the event of certain cumulative changes in the ownership interest of significant shareholders in excess of 50 percent over a three-year period. This could limit the amount of NOL carryforwards that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of a company immediately prior to the ownership change. Our company has completed several transactions since its inception which resulted in an ownership change under Section 382 of the Code. In addition, future changes in our stock ownership, some of which are outside of our

control, could result in ownership changes in the future. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, ransom requests, sabotage, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations or the operations of those third parties with which we contract, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions, and could require a substantial expenditure of resources to remedy. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. We could also be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in our information systems and networks, including personal information of our employees. Outside parties may attempt to penetrate our systems or those of the third parties with which we contract or to fraudulently induce our employees or employees of such third parties to disclose sensitive information to gain access to our data or to use such access to request cash compensation in the form of a ransom for the return of such data.

The number and complexity of these threats continue to increase over time. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Despite our efforts, the possibility of these events occurring cannot be eliminated entirely. Although we maintain cyber risk insurance for certain costs we may incur due to a cyber-related event, this insurance may not provide adequate coverage against potential liabilities. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, or a loss of cash in response to ransom threats, we could incur liability, our competitive and financial position and the market perception of the effectiveness of our security measures could be harmed, our credibility could be damaged, and the further development and commercialization of our product candidates could be delayed.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. If no or few analysts maintain coverage of us, the trading price of our stock would likely decrease. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

ITEM 5. OTHER INFORMATION

On September 3, 2021, we entered into a sublease agreement, or the Sublease Agreement, with BioNTech US Inc., or the Subtenant, pursuant to which we have agreed to sublease to the Subtenant approximately 17,931 square feet of office and laboratory space, or the Subleased Premises, in the property located at 75 Sidney Street, Cambridge, Massachusetts that we have leased from 45/75 Sidney Street, LLC, or the Prime Landlord, pursuant to our lease agreement with the Prime Landlord dated April 1, 2014, as amended to date, or the Prime Lease Agreement. The Sublease Agreement is subject and subordinate in all respects to the Prime Lease Agreement.

The Sublease Agreement became effective on September 3, 2021 and expires on December 31, 2024, unless sooner terminated or extended in accordance with the terms of the Sublease Agreement.

Beginning on September 7, 2021, the Subtenant is obligated to pay us an annual base rent of approximately $2.1 million, subject to an annual increase of approximately three percent of the prior year annual base rent, and its proportionate share of property taxes and operating expenses. In accordance with the terms of the Prime Lease Agreement, after deducting reasonable and ordinary sublease transaction expenses and rent abatement, we are obligated to pay to the Prime Landlord 50% of any amounts we receive from the Subtenant as rent, additional rent, or other forms of compensation or reimbursement other than those which are equal to or less than the rent, additional rent, and other monies that we are obligated to pay to the Prime Landlord for the Sublease Premises under the Prime Lease Agreement. Upon the execution of the Sublease Agreement, the Subtenant was required to deliver to us a letter of credit in the amount of approximately $0.7 million as partial security for the Subtenant's obligations under the Sublease Agreement.

We have the right to terminate the Sublease Agreement upon specified events of default including the Subtenant's failure to pay rent in a timely manner. Upon an event of default, the Subtenant has agreed to pay us damages in an amount equal to the excess, if any, of the discounted present value of the total rent reserved for the remainder of the term over the then discounted present fair value of the Subleased Premises for the remainder of the term, payable in accordance with the Sublease Agreement, less any rent we receive upon the re-subletting of the Subleased Premises, net of expenses we incur to terminate the Sublease Agreement and re-sublet the Subleased Premises.

The foregoing summary of the Sublease Agreement is qualified in its entirety by the text of the Sublease Agreement, a copy of which is attached hereto as Exhibit 10.1 to this Quarterly Report on Form 10-Q and is incorporated herein by reference.

ITEM 6. EXHIBITS

The exhibits filed or furnished as part of this Quarterly Report are set forth on the Exhibit Index, which is incorporated herein by reference.

INDEX TO EXHIBITS

		Incorporated by Reference to:				
Exhibit No.	Description	Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number	Filed Herewith
10.1	Sublease Agreement, by and between Registrant and BioNTech US Inc., dated September 3, 2021					X
10.2*	Option and License Agreement, by and between the Registrant and Pfizer Inc., dated October 1, 2021.					X
31.1	Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.					X
31.2	Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.					X
32.1+	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act Rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.					X

101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.	X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.	X
104	Cover Page Interactive Data File – The cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document	

* Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

\+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Quarterly Report on Form 10-Q and will not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: November 2, 2021

VOYAGER THERAPEUTICS, INC.

By: */s/ Michael Higgins*
Michael Higgins
Interim Chief Executive Officer, President, and Director
(Principal Executive Officer)

By: */s/ Allison Dorval*
Allison Dorval
Chief Financial Officer
(Principal Financial and Accounting Officer)

SUBLEASE AGREEMENT

THIS SUBLEASE AGREEMENT (this "Sublease") is made and entered into as of September 3 , 2021 (the "Effective Date"), by and between **VOYAGER THERAPEUTICS, INC.**, a Delaware corporation ("Sublandlord"), and **BIONTECH US INC,** a Delaware corporation ("Subtenant").

RECITALS

A. UP 45/75 Sidney Street, LLC ("UP 47/75 Sidney" and together with its successors and assigns, "Prime Landlord") and Sublandlord entered into that certain Lease dated April 1, 2014, including Exhibits, as amended by that certain First Amendment to Lease Agreement (the "First Amendment") dated as of December 23, 2015, as further amended by that certain Second Amendment to Lease Agreement (the "Second Amendment") dated as of February 5, 2018 and as further amended by that certain Third Amendment to Lease Agreement (the "Third Amendment") dated as of June 1, 2018 (as it may be further amended from time to time, the "Prime Lease"), pursuant to which Prime Landlord has leased to Sublandlord certain premises (the "Premises") in the building (the "Building") located at 75 Sidney Street, Cambridge, Massachusetts with total rentable floor area of 137,958 ("Total Rentable Floor Area of Building"). Pursuant to that certain Assignment and Assumption Agreement (Ground Lease) effective March 12, 2021, UP 45/75 Sidney assigned its interest as tenant under that certain Amended and Restated Construction and Lease Agreement 45 Sidney, 75 Sidney and 101 Pacific Street, dated as of December 15, 1997, by and between Massachusetts Institute of Technology and UP 45/75 Sidney (as the same may be amended, restated, modified or supplemented from time to time, the "Ground Lease") to BRE-BMR Pilgrim & Sidney LLC . A copy of the Prime Lease is attached hereto as Schedule A and made a part hereof to the extent set forth in Sections 7 and 8 of this Sublease. Capitalized terms not otherwise defined herein shall have the respective meanings ascribed to them in the Prime Lease.

B. Sublandlord has agreed to sublet to Subtenant, and Subtenant has agreed to sublet from Sublandlord, the Subleased Premises (defined below) upon and subject to the terms and conditions of this Sublease.

NOW, THEREFORE, for and in consideration of the rents herein provided and of the terms, covenants, conditions and agreements herein contained, and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged by each of the parties hereto, and intending to be legally bound hereby, Sublandlord and Subtenant hereby covenant and agree as follows:

1. Demise. Subject to all of the provisions of this Sublease, Sublandlord hereby demises and subleases to Subtenant, and Subtenant hereby subleases, takes and hires from Sublandlord: (i) a portion of the Premises, which consist of approximately 17,931 rentable square feet located on the fifth (5th) floor of the Building and shown on attached Schedule B; (ii) all non-exclusive appurtenant rights set forth in Section 2.2 of the Prime Lease; and (iii) the exclusive use of the acid neutralization system serving the fifth (5th) floor of the Building (collectively, the

"Subleased Premises"). The square footage set forth herein is deemed conclusive. Subject to the provisions of Section 2.4 of the Prime Lease, Subtenant shall lease 20 parking spaces in the 30 Pilgrim Street Garage (the "Parking Spaces"), such Parking Spaces shall be available to Subtenant during the Term in accordance with the terms and conditions of the Prime Lease, including, without limitation, the payment for such Parking Spaces shall constitute Additional Rent. Subject to Section 2.2 of the Prime Lease, from and after the Term Commencement Date, Subtenant shall have 24 hour, seven day per week access to the Subleased Premises and the Parking Spaces.

2. Term of this Sublease. The term of this Sublease (as the same may be earlier terminated in accordance with this Sublease, the "Term") shall commence on the later to occur of (i) September 1, 2021, (ii) the date on which Prime Landlord provides the Prime Landlord Consent, or (iii) the date on which Sublandlord tenders possession of the Subleased Premises to Subtenant in conformity with the terms of this Sublease (such date being the "Term Commencement Date") and shall end on December 31, 2024 (the "Term Expiration Date").

3. Permitted Use. Subject to Legal Requirements, Subtenant shall use and occupy the Subleased Premises for general business and administrative offices, laboratory and biotechnology research and development, and related activities thereto, subject to any and all provisions, prohibitions and restrictions set forth in the Prime Lease (the "Permitted Use").

4. Sublease Annual Fixed Rent.

(a) During the Term, Subtenant shall pay to Sublandlord Annual Fixed Rent (as defined in Section 4(b) below) in equal monthly installments, in advance and without demand, on the day that is fifth (5th) calendar day prior to first (1st) of each calender month for which such payment applies (the "Rent Due Date"). The payment of Annual Fixed Rent and Additional Rent and any other charges reserved and covenanted to be paid under this Sublease with respect to the Subleased Premises (collectively, the "Rent") shall commence on the Term Commencement Date, and shall be prorated for any partial months.

Sublandlord shall invoice Subtenant thirty (30) days prior to the Rent Due Date for all monthly installments of Annual Fixed Rent, Subtenant's Share of Operating Expenses and Subtenant's Share of Taxes but no delay or failure by Sublandlord in providing such a bill shall relieve Subtenant from the obligation to pay the Rent as provided herein. All payments shall be by electronic funds transfer as directed by Sublandlord, unless otherwise directed by Sublandlord by written notice given to Subtenant at least sixty (60) days' prior to the next Rent Due Date.

(b) The annual fixed rent ("Annual Fixed Rent") for the Term of this Sublease is payable as follows:

Months of Term	Annual Fixed Rent	Monthly Installment	Per Square Foot
1 – 12	$2,062,065.00	$171,838.75	$115.00
13 – 24	$2,123,926.95	$176,993.91	$118.45
25 – 36	$2,187,582.00	$182,298.50	$122.00
37 – Term Expiration Date	$2,253,209.46	$187,767.46	$125.66

(c) Rent shall be paid without notice or demand, and without setoff, counterclaim, defense, abatement, suspension, deferment, reduction, or deduction, except as expressly provided herein. It is understood that this Sublease is a net Sublease and that Annual Fixed Rent is absolutely net to Sublandlord. Subtenant hereby acknowledges and agrees that the obligations of Subtenant hereunder shall be separate and independent covenants and agreements, that Rent shall continue to be payable in all events, and that the obligations of Subtenant hereunder shall continue unaffected, unless the requirement to pay Rent or perform the same shall have been abated, reduced or terminated pursuant to an express provision of this Sublease. Sublandlord and Subtenant each acknowledges and agrees that the independent nature of the obligations of Subtenant hereunder represents fair, reasonable, and accepted commercial practice with respect to the type of property subject to this Sublease, and that this agreement is the product of free and informed negotiation during which both Sublandlord and Subtenant were represented by counsel skilled in negotiating and drafting commercial subleases in Massachusetts. Such acknowledgements and agreements by Subtenant are a material inducement to Sublandlord entering into this Sublease.

5. Additional Rent.

(a) In addition to Annual Fixed Rent, Subtenant shall pay to Sublandlord, as Additional Rent (defined below), an amount equal to Subtenant's Share (as defined below) of the Operating Expenses for the Property, as set forth in Section 3.3 of the Prime Lease ("Subtenant's Share of Operating Expenses"). Subtenant shall pay to Sublandlord, on the Term Commencement Date and on each Rent Due Date thereafter, an amount equal to Subtenant's Share of Operating Expenses for such fiscal year and/or part thereof divided by the number of months therein. "Subtenant's Share" is 13.00% and is the product of the total rentable floor area of the Subleased Premises divided by the Total Rentable Floor Area of the Building.

(b) All provisions of Section 3.3 of the Prime Lease relating to the payment of Tenant's Operating Expenses Allocable to the Premises are hereby expressly incorporated into this Sublease with respect to the Subtenant's Share of Operating Expenses as if the Subtenant was the Tenant thereunder with respect to Subtenant's Share of Operating Expenses, provided that Prime Landlord shall be responsible for providing any statement of Operating Expenses set forth in Section 3.3 of the Prime Lease.

(c) In addition to Annual Fixed Rent, Subtenant shall pay to Sublandlord, as Additional Rent, an amount equal to Subtenant's Share of Prime Landlord's Tax Expenses for a Tax Year (Subtenant's Share being 13.00%) pursuant to Section 3.2 of the Prime Lease ("Subtenant's Share of Taxes"). Subtenant shall pay to Sublandlord, on the Term Commencement

Date and on each Rent Due Date thereafter, an amount equal to Subtenant's Share of Taxes for such Tax Period or part thereof divided by the number of months therein.

(d) All provisions of Section 3.2 of the Prime Lease relating to the payment of Tenant's Tax Expenses Allocable to the Premises are hereby expressly incorporated into this Sublease with respect to Subtenant's Share of Taxes as if the Subtenant was the Tenant thereunder with respect to Subtenant's Share of Taxes.

(e) If any installment or other payment of Rent is not paid by the Rent Due Date, then such unpaid amount shall bear interest, from the due date thereof until paid in full, at the Default Interest Rate. The parties agree that the late charge represents a fair and reasonable estimate of the costs that Sublandlord will incur by reason of such late payment by Subtenant. All interest and late charges accrued under this paragraph shall be deemed to be Additional Rent payable hereunder.

(f) All amounts due under this Section 5, Section 6 below and any other amounts that Subtenant assumes or agrees to pay under this Sublease that are owed to Sublandlord, including any and all other sums that may become due by reason of any default of Subtenant or failure of Subtenant's part to comply with the agreements, terms, covenants and conditions of this Sublease to be performed by Subtenant, shall be referred to herein as "Additional Rent".

6. Utilities and Services. Sublandlord and Subtenant acknowledge that none of the utilities serving the Premises (and therefore the Subleased Premises) are separately metered as of the Effective Date and that the Prime Landlord pays for the utilities in the Premises (and therefore the Subleased Premises) and invoices Sublandlord for the same (such invoices being referred to herein as the "Utility Invoices"). Sublandlord agrees to promptly share such Utility Invoices, prorated to adjust between the Premises retained by Sublandlord and the Subleased Premises, to Subtenant for payment with respect to the Subleased Premises and Subtenant agrees to pay Sublandlord for amounts shown on the Utility Invoices with respect to the Subleased Premises within thirty (30) days of receipt thereof. Sublandlord reserves the right to adjust the Subtenant's share of the Utility Invoices in the event that the Sublandlord has reasonable belief, based on industry accepted methodology and supported by documentation, that Subtenant's use of utilities in the Subleased Premises exceeds its proportionate share of the Premises. Subtenant has the right to request an adjustment to the amount due under the Utility Invoices in the event that Subtenant has reasonable belief, based on industry accepted methodology and supported by documentation, that Sublandlord's use of utilities in the Premises (excluding the Subleased Premises) exceeds its proportionate share of the Premises The cost of such utilities shall constitute Additional Rent. If requested by Subtenant and actually in the possession of Sublandlord, Sublandlord shall provide Subtenant with reasonable documentation to substantiate the charges for utilities invoiced to Subtenant. If received by Sublandlord from Prime Landlord, Sublandlord shall provide Subtenant with a final accounting and reconciliation of amounts charged to Subtenant for utilities at the endof each Operating Fiscal Year in the same manner as set forth in Section 3.4 of the Prime Lease. If, in the future during the Term, any utilities are separately metered, Subtenant shall, thereafter, pay directly to the provider of the service all separately metered charges for steam, heat, gas, electricity, fuel and other services and utilities furnished to the Subleased Premises. Subtenant further acknowledges that Prime Landlord has no obligation to provide utilities and services to or

for the Subleased Premises, except to the extent provided, and subject to the terms and conditions set forth, in Section 3.4 of the Prime Lease. Subtenant further acknowledges and agrees that it shall bear all costs and expenses for or relating to the provision of any additional utilities or services required by it, including, without limitation, all costs and expenses relating to the acquisition, installation and maintenance of any equipment required in connection therewith. Subtenant further acknowledges that it shall be responsible for all janitorial services for the Subleased Premises and all matters relating to its phone and other telecommunications and information technology services in the Building. Subtenant shall have the right to a proportionate share of emergency power that is provided to Sublandlord pursuant to Section 5.1 of the Lease.

7. Incorporation of Terms of Prime Lease.

(a) Except as specifically provided in subparagraph (b) below of this Sublease and elsewhere in this Sublease, all of the terms, covenants, conditions and obligations contained in the Prime Lease, are by this reference incorporated herein and made a part of this Sublease with the same force and effect as if fully set forth herein, provided, however, that for purposes of such incorporation, (i) the term "Lease" as used in the Prime Lease shall refer to this Sublease, (ii) except as expressly set forth in this Sublease, the term "Landlord" as used in the Prime Lease shall include Sublandlord in addition to Prime Landlord, (iii) the term "Tenant" as used in the Prime Lease shall refer to Subtenant, (iv) the term "Permitted Use" as used in the Prime Lease shall refer to the Permitted Use, as defined in Section 3 above, (v) the terms "Term Commencement Date" and "Expiration Date" as used in the Prime Lease shall refer, respectively, to the Term Commencement Date as defined in this Sublease and Term Expiration Date defined in this Sublease, and (vi) the term "Premises" as used in the Prime Lease shall refer to the refer to the term Subleased Premises as defined in this Sublease. Accordingly, except as otherwise specifically set forth herein, Subtenant shall have, with respect to the Subleased Premises, all of the obligations imposed upon Sublandlord as Tenant under the Prime Lease and Sublandlord hereby assigns to Subtenant benefits granted to Sublandlord as Tenant under the Prime Lease except those excluded pursuant to paragraph (b) and Section 8 below. In the event of any inconsistency between the provisions of this Sublease and the provisions of the Prime Lease, as incorporated herein, the provisions of this Sublease shall control, provided, however, that the foregoing shall not abrogate the provisions of Section 8 hereof in any way.

(b) Notwithstanding anything to the contrary in this Sublease, the following provisions of the Prime Lease shall **NOT** be incorporated into or made part of this Sublease:

Section 2.5, Commencement Date; Rent Commencement Date;
Section 2.6, Extension Option;
Section 3.1, Annual Fixed Rent;
Section 4.5, Leasehold Improvements Allowance and Space Plan Allowance;
Sections 9(a)-(c), Tenant Default;
Section 11.1, Notice of Lease;
Section 11.9, Brokerage;
Section 11.12, Security Deposit; and
Section 11.16, Temporary Space.

Additionally, the following provisions are not incorporated into this Sublease: Sections 2,4 and 6 of the First Amendment; Sections 3, 4, 5, 6, 11 and 12 of the Second Amendment; and Sections 3, 4, 5, 6, 11 and 12 of the Third Amendment. Exhibits A, B, E, F, H, and I to the Prime Lease are not incorporated herein.

8. Subject to the Prime Lease and Ground Lease.

(a) This Sublease shall be subject and subordinate in all respects to the Prime Lease and the Ground Lease and to all of its terms, covenants and conditions other than those specifically excluded pursuant to Section 7(b) of this Sublease. Subtenant shall not do, or permit to suffer to be done, any act or omission by Subtenant, its agents, employees, contractors or invitees which is prohibited by the Prime Lease or the Ground Lease, or which would constitute a violation or default thereunder. Subtenant hereby assumes and agrees to perform all obligations of Sublandlord as Tenant under the Prime Lease applicable to the Subleased Premises, and Subtenant agrees to abide by and comply with all of the provisions of the Prime Lease applicable to the Subleased Premises during the Term of this Sublease, except to the extent otherwise expressly provided in this Sublease. Notwithstanding any provision contained in this Sublease or the Prime Lease, Subtenant shall have no option to renew or extend the Term hereof, to expand the Subleased Premises, or to receive tenant improvement allowances or other tenant incentives. Subtenant agrees that any time the consent of Prime Landlord is required under the Prime Lease, the consent of Sublandlord will also be required hereunder using the same standards for such consent placed upon Prime Landlord under the Prime Lease and Sublandlord shall have no obligation to give such consent unless Prime Landlord has consented pursuant to the terms of the Prime Lease. Subtenant hereby acknowledges that Subtenant has read and is familiar with the terms and conditions of the Prime Lease, and Subtenant further hereby acknowledges and agrees that, except as otherwise specifically set forth in this Sublease, Sublandlord expressly retains and reserves all rights and benefits applicable to Sublandlord as Tenant under the Prime Lease.

(b) Without limiting Sublandlord's covenants in Section 8(d) below, should the Prime Lease expire or terminate during the term of this Sublease for any reason, this Sublease shall automatically terminate on the date of expiration or termination of the Prime Lease, with the same force and effect as if such expiration or termination date had been specified in this Sublease as the Term Expiration Date hereof.

(c) Sublandlord represents to Subtenant that the Prime Lease is in full force and effect, that the Prime Lease constitutes the entire agreement between Prime Landlord and Sublandlord concerning the Subleased Premises, and that, to the best of Sublandlord's knowledge, as of the Effective Date, no Event of Default under the Prime Lease on the part of Sublandlord or Prime Landlord has occurred and is continuing, and Sublandlord has neither delivered nor received a notice of any event which, with the passage of time, would constitute an Event of Default under the Prime Lease. Sublandlord agrees to promptly provide Subtenant with (i) copies of any notices of default that Sublandlord may receive from or send to Prime Landlord; and (ii) notice of the occurrence of any of the following to the extent Sublandlord receives written notice of the same: the taking of the estate created in the Prime Lease on execution or other process of law; a judicial declaration that the Sublandlord is bankrupt or insolvent according to law; any assignment of the property of the Sublandlord for the benefit of creditors; the appointment of a receiver, guardian,

conservator, trustee in bankruptcy or other similar office to take charge of all or any substantial part of the Sublandlord's property by a court of competent jurisdiction; or the filing of an involuntary petition against the Sublandlord under any provisions of the bankruptcy act now or hereafter enacted if the same is not dismissed within ninety (90) days; or the filing by the Sublandlord of any voluntary petition for relief under provisions of any bankruptcy law now or hereafter enacted.

(d) For so long as Subtenant is not in default of this Sublease beyond applicable notice and cure periods, Sublandlord shall (a) not enter into any modification, amendment, replacement or other revision to the Prime Lease, or any provision thereof, which has an adverse effect on Subtenant's rights or increases Subtenant's obligations or liabilities under this Sublease, without, in each instance, first obtaining Subtenant's prior written consent thereto (which consent shall be granted or withheld in Subtenant's reasonable discretion, and not unreasonably conditioned or delayed), and (b) not intentionally breach or default under the Prime Lease.

9. Prime Landlord's Obligations. Notwithstanding anything to the contrary in this Sublease or the Prime Lease including, without limitation, subsections (i) through (iii) of Section 7(a) above, Sublandlord shall have no obligation to perform (and Sublandlord shall not be deemed to guarantee the performance by Prime Landlord of) any of the terms, covenants or conditions contained in the Prime Lease to be performed by Prime Landlord nor to provide any services or make any repairs except as expressly set forth in this Sublease. Without limiting the foregoing, Sublandlord shall have no obligation to maintain the insurance required to be maintained by Prime Landlord pursuant to Section 7.4 of the Prime Lease or to provide any or all of the services, utilities, repairs, maintenance, or restoration work to be provided by Prime Landlord pursuant to Sections 3.4, 5.1 and Article VIII of the Prime Lease, and Sublandlord shall in no way be liable to Subtenant for any failure of Prime Landlord to maintain such insurance or provide such services, utilities, repairs, maintenance, or restoration. Sublandlord have no obligation to indemnify Subtenant for actions of the Prime Landlord. No default of Prime Landlord under the Prime Lease shall affect this Sublease or waive or defer the payment or performance of any of Subtenant's obligations hereunder. Notwithstanding the foregoing, if Prime Landlord fails to provide any services, utilities, repairs, maintenance, or restoration required under the Prime Lease, Sublandlord shall, upon the written request of Subtenant and at the direction of Subtenant, give Prime Landlord notice of such failure and use commercially reasonable efforts to exercise such rights and remedies as are available to Sublandlord under the Prime Lease to effect such performance; provided, however, that Subtenant shall have no right to cause Sublandlord to exercise any right of Sublandlord to terminate the Prime Lease, whether pursuant to Section 8.3 of the Prime Lease or otherwise. Subtenant shall pay all costs and expenses, including reasonable attorneys' fees, that may be incurred by Sublandlord in enforcing the provisions of this Sublease or in enforcing Prime Landlord's obligations under the Prime Lease if requested to do so by Subtenant. Any amounts recoved from Prime Landlord by Sublandlord (including, but not limited to, abatement of Annual Fixed Rent under the Prime Lease) that results from Sublandlord's enforcement of Prime Landlord's obligations under the Prime Lease shall: (i) if related to more than the Subleased Premises, Subtenant shall only be required to contribute to related costs and expenses on a pro rata basies and a pro rata amount of amounts recovered shall be allocated to Subtenant; or (ii) if related exclusively to the Subleased Premises, all costs and expenses and any amounts recovered shall be allocated entirely to Subtenant.

10. Insurance. Subtenant shall procure, pay for, and keep in force throughout the Term, all insurance required under the Prime Lease to be carried by Tenant thereunder with respect to the Subleased Premises in accordance with the terms of Section 7.2 of the Prime Lease. Such insurance shall name Sublandlord and Prime Landlord, and others as set forth in the Prime Lease, as additional insureds with respect to liability insurance. Subtenant shall furnish Sublandlord and Prime Landlord with certificates of insurance evidencing compliance with the foregoing insurance requirements simultaneously with the execution of this Sublease.

11. Additional Covenants of Subtenant. Subtenant covenants as follows during the Term and such further time as Subtenant occupies any part of the Subleased Premises:

(a) At the expiration or termination of this Sublease, Subtenant shall peaceably quit and surrender to Sublandlord the Subleased Premises in accordance with all requirements of Section 11.10 of the Prime Lease and will have the Subleased Premises decontaminated and decommissioned by a certified industrial hygienist at Subtenant's sole cost and expense, such certified industrial hygienist and decommissioning plan to be first approved by Sublandlord.

(b) Subtenant shall abide by all requirements of the Prime Lease, including without limitation, Section 6.1 of the Prime Lease with regard to the Permitted Uses of the Subleased Premises, Section 5.2 with regard to the maintenance and repair of the Subleased Premises, and Section 6.6 with regard to floor load in the Subleased Premises.

(c) Subtenant will faithfully observe and comply with all rules and regulations promulgated in writing by Prime Landlord pursuant to Section 6.3 of the Prime Lease (Subtenant hereby acknowledging receipt and review of the Rules and Regulations attached as Exhibit D to the Prime Lease, which Exhibit, as the same may be modified from time to time over the Term hereof, Subtenant acknowledges, is incorporated into this Sublease as if set forth at length herein).

(d) Subtenant shall be responsible at its sole cost and expense for complying with, and keeping the Subleased Premises in compliance with, all applicable Legal Requirements and for obtaining all required permits (the "Required Permits"), including, without limitation, the MWRA permit. Subtenant shall provide Sublandlord with a copy of all Required Permits, evidence of its own pH neutralization system, and a list of all chemical inventory and/or Hazardous Materials and associated quantities on the Effective Date and each anniversary of the Term Commencement Date.

(e) Subtenant covenants and agrees, to the maximum extent permitted by Legal Requirements, that all of Subtenant's Property shall be at the sole risk and hazard of Subtenant, as set forth in Section 7.3 of the Prime Lease.

(f) Sublandlord acknowledges that (i) Subtenant is a wholly-owned subsidiary of BioNTech SE ("Parent") which, as of the Effective Date is a public company traded on the NASDAQ exchange and subject to the public reporting requirements of the Securities Exchange Act of 1934 (the "'34 Act"), and (ii) Subtenant's financial statements are consolidated into Parent's financial statements. Subtenant shall deliver to Sublandlord, within

thirty (30) days after Sublandlord's reasonable request, the most recent audited balance sheet and related statements of income, shareholders' equity and cash flow statements of Parent which shall be prepared by an independent certified public accountant. Notwithstanding the foregoing, Subtenant's obligation to provide financial statements to Sublandlord under this Section 11(f) shall be suspended for all periods of time during which Parent continues to be subject to the public reporting requirements of the '34 Act.

(g) Subtenant does hereby waive and surrender all rights and privileges which it might have under or by reason of any present or future Legal Requirements and hereby waives any statutory notice and grace periods provided by law.

12. Indemnity.

(a) Except to the extent caused by the negligence or willful misconduct of any of Sublandlord or the Prime Landlord, Subtenant shall defend, indemnify and save Sublandlord and the Prime Landlord harmless from and against any and all Claims asserted by or on behalf of any person, firm, corporation or public authority arising from:

(i) Subtenant's breach of any covenant or obligation under this Sublease;

(ii) Any injury to or death of any person, or loss of or damage to property, sustained or occurring in, upon, at or about the Subleased Premises;

(iii) Any injury to or death of any person, or loss of or damage to property arising out of the use or occupancy of the Subleased Premises by or the negligence or willful misconduct of Subtenant or its agents, servants, employees, consultants, contractors, subcontractors, licensees and/or subtenants (collectively with Subtenant, the "Subtenant Parties"); and

(iv) On account of or based upon any work or thing whatsoever done (other than by Sublandlord or the Prime Landlord) at the Subleased Premises during the Term and during the period of time, if any, prior to the Term Commencement Date that any of the Subtenant Parties may have been given access to the Subleased Premises.

(b) Except to the extent caused by the negligence or willful misconduct of any of the Subtenant Parties, Sublandlord shall defend, indemnify and save Subtenant harmless from and against any and all Claims asserted by or on behalf of any person, firm, corporation or public authority arising solely from (i) Sublandlord's breach of any covenant or obligation under this Sublease or (ii) Sublandlord's occupancy and use of the Subleased Premises prior to the Term Commencement Date.

13. Notices.

(a) The addresses to which notices are to be sent under this Sublease and Section 24 of the Prime Lease are as follows:

To Sublandlord:

Voyager Therapeutics, Inc.
75 Sidney Street
Cambridge, MA 02139
Attention: Allison Dorval

With a copy (which shall not constitute notice) to:
Voyager Therapeutics, Inc.
75 Sidney Street
Cambridge, MA 02139
Attention: Robert Hesslein

And

To Subtenant:

BioNTech
40 Erie Street
Cambridge, MA 02139
Attention: Seth Harmon
Email: seth.harmon@biontech.us
With copies (which shall not constitute notice) to:

BioNTech
40 Erie Street
Cambridge, MA 02139
Attn: Brian Kickham
Email: brian.kickham@biontech.us

BioNTech
40 Erie Street
Cambridge, MA 02139
Attn: Fran Lacombe
Email: fran.lacombe@biontech.us

Either party may inform the other in the manner provided for the giving of notices of any changein address.

(b) Whenever notice is required to be given to Prime Landlord under the Prime Lease, or notice is required to be given to Sublandlord under this Sublease, Subtenant shall provide Sublandlord with such notices sufficiently in advance of the time that Sublandlord is required to transmit notice to Prime Landlord. All notices required hereunder shall be delivered in the manner set forth in Section 11.2 of the Prime Lease.

14. Assignment and Subletting. Subtenant shall have the right to sublease or assign this Sublease in its entirety, subject to Prime Landlord's and Sublandlord's prior written consent.

Sublandlord's consent shall not be unreasonably withheld, conditioned or delayed; provided in all instances such right to assign or sublease shall be subject to all of the terms and conditions of Section 6.8 of the Prime Lease and that the Prime Landlord has granted its consent pursuant thereto. Notwithstanding anything set forth in this Sublease to the contrary, Subtenant may assign this Sublease without Sublandlord's or Prime Landlord's prior consent in connection with any transaction that is a Permitted Transfer pursuant to the provisions of Section 6.8 of the Prime Lease; provided, that with respect to any Permitted Transfer proposed by Subtenant under clause (b)(i) of the second paragraph of Section 6.8, the net assets of the Acquiring Company at the timeof the transfer or merger shall not be less than One Hundred Million and 00/100 Dollars ($100,000,000.00).

15. Condition, Acceptance and Use of Subleased Premises; Disclaimer of Warranty.

(a) Subtenant shall and does hereby accept the Subleased Premises, in their "AS-IS, WHERE-IS" condition, and with all faults, existing as of the Term Commencement Date; *provided however*; Sublandlord shall deliver the Subleased Premises to Subtenant on the Term Commencement Date (i) in substantially the same condition as the Subleased Premises are in on the Effective Date, reasonable wear and tear excepted; (ii) in broom clean condition; (iii) free of personal property (other than the furniture and equipment set forth in Section 15(b) below) and occupants; and (iv) decontaminated and decommissioned by a certified industrial hygienist at Sublandlord's sole cost and expense. Without limiting the foregoing, Subtenant acknowledges that no representations have been made to Subtenant with respect to the condition of the Subleased Premises and that Subtenant has relied upon its own examination of the Subleased Premises in entering into this Sublease. Furthermore, Subtenant further acknowledges and agrees that, (a) Subtenant has had full opportunity to examine the Subleased Premises and is fully informed, independently of Sublandlord or any employee, agent, representative, shareholder, officer or director of Sublandlord, as to the character, construction and structure of the Subleased Premises; and (b) neither Sublandlord nor any of Sublandlord's employees, agents, representatives, shareholders, officers or directors, nor Prime Landlord nor any of Prime Landlord's employees, agents, representatives, shareholders, officers or directors, has made any representations, warranties or promises with respect to the Subleased Premises, including, without limitation, any representation or warranty as to fitness thereof for any purpose.

(b) Sublandlord hereby agrees that, during the Term hereof, Subtenant shall have the use of the existing furniture and equipment listed on Schedule C which is located in the Subleased Premises. All furniture and equipment shall be tendered to Subtenant on the Term Commencement Date in its then "AS-IS, WHERE-IS" condition and shall be left on the Subleased Premises by Subtenant at the end of the Lease Term subject to normal wear and tear. Sublandlord makes no representations about the condition of any furniture or equipment or its fitness for any purpose.

16. Alterations. Subtenant shall not make any alterations, decorations, installations, removals, additions, improvements (collectively herein and in the Prime Lease referred to as "Alterations") in or to the Subleased Premises, except as provided in Article IV of the Prime Lease and this Section 16. To the extent Subtenant desires to make any Alterations to the Subleased Premises, Subtenant shall submit to Sublandlord written plans and specifications and the proposed

time schedule and contractors for the performance of such work and, provided Sublandlord deems the proposed Alterations, time schedule and contractors reasonably satisfactory and determines that the performance of such work will not expose Sublandlord to any liability, Sublandlord shallsubmit a request to Prime Landlord pursuant to Article IV of the Prime Lease. Notwithstanding the foregoing, subject to Prime Landlord's consent, Sublandlord hereby (i) consents to Subtenant installing a door connecting the two lab suites within the Subleased Premises in the location indicated on Exhibit B, and (ii) agrees that Subtenant shall not be required to remove such door before yielding up the Subleased Premises on the Term Expiration Date.

17. Signs. Subtenant shall, at Subtenant's sole cost and expense, be permitted, accordance with Section 6.3 of the Prime Lease, to install building standard signage identifying Subtenant's business at the entrance of the Subleased Premises, which signage shall be subject to the Prime Landlord's prior written consent. Subtenant shall have no other rights to place or causeto be placed signage on the interior or exterior of the Building.

18. Events of Default. The occurrence of any one or more of the following events shall constitute an "Event of Default" hereunder by Subtenant:

(a) If Subtenant fails to make any payment of Rent or any other payment required hereunder on the Rent Due Date if such condition continues for five (5) business days after written notice that the same are due; provided, however if Subtenant shall fail to pay any of the Rent (after receipt by Subtenant of written notice from Sublandlord) when due two (2) times in any period of twelve (12) consecutive months, then Sublandlord shall not be required to give notice to Subtenant of any future failure to pay Rent during the remainder of the Term, and such failure shall thereafter constitute an Event of Default if not cured within five (5) business days after the Rent Due Date;

(b) If Subtenant fails to perform or observe any other term or condition contained in this Sublease and such failure is not cured within fifteen (15) days after written notice from the Sublandlord, so long as the Subtenant commences such cure within the fifteen (15) days, such breach remains susceptible to cure, and the Subtenant diligently pursues such cure, such breach shall not be deemed an Event of Default;

(c) The taking of the estate hereby created on execution or other process of law;or a judicial declaration that the Subtenant is bankrupt or insolvent according to law; or any assignment of the property of the Subtenant for the benefit of creditors; or the appointment of a receiver, guardian, conservator, trustee in bankruptcy or other similar office to take charge of all or any substantial part of the Subtenant's property by a court of competent jurisdiction; or the filing of an involuntary petition against the Subtenant under any provisions of the bankruptcy act now or hereafter enacted if the same is not dismissed within ninety (90) days; the filing by the Subtenantof any voluntary petition for relief under provisions of any bankruptcy law now or hereafter enacted.

Sublandlord shall have all of the remedies set forth in the Prime Lease upon any Event of Default by Subtenant.

19. Brokers. Sublandlord and Subtenant each warrants and represents to the other that it had no dealing with any broker or finder concerning the subletting of the Subleased Premises, except CBRE (the "Broker") who will be paid a leasing fee by Sublandlord pursuant to a separate written agreement. Each party hereto agrees to indemnify, defend and hold the other party harmless from any and all liabilities and expenses, including, without limitation, commissions, brokerage fees and reasonable attorneys' fees, arising out of claims against the other party by any broker, consultant, finder or like agent, other than the Broker, claiming to have brought about this Sublease based upon the alleged acts of the indemnifying party. This Section 19 shall survive the expiration or earlier termination of this Sublease.

20. Limitation of Liability. Neither Prime Landlord nor Sublandlord shall be liable to Subtenant, or any of Subtenant's agents, employees, contractors, servants, customers, guests or invitees, for any damage to persons, animals, or property due to the condition, design, or any defect in the Subleased Premises or its mechanical systems that may exist on the Term Commencement Date or resulting from fire, explosion, falling plaster, steam, gas, air contaminants or emissions, electricity, electrical or electronic emanations or disturbance, water, rain or snow or leaks from any part of the Building or from the pipes, appliances, equipment or plumbing works or from the roof, street, or subsurface or from any other place or caused by dampness, vandalism, malicious mischief or by any other cause of whatever nature, except to the extent caused by or arising out of the negligence or willful misconduct of Prime Landlord or Sublandlord. Subtenant, with respect to itself and its agents, employees, contractors, servants, customers, guests or invitees, hereby expressly assumes all risks of damage to persons and property, either proximate or remote, by reason of the present or future condition of the Subleased Premises. Neither Sublandlord nor Subtenant, nor any of their respective trustees, members, managers, partners, officers, directors, shareholders, or employees, shall have any personal liability under this Sublease, it being hereby agreed that the liability of Sublandlord and Sublandlord's trustees, managers, partners, officers, directors, shareholders, and employees for any default by Sublandlord under the terms of this Sublease shall be recoverable solely from the leasehold interest of Sublandlord in and to the Subleased Premises, and the rents, insurance and condemnation proceeds arising therefrom. Neither party to this Sublease shall be liable to the other for any indirect or consequential damages, except that this sentence shall not apply to: (i) holdover damages to the extent provided in Section 22 of this Sublease, (ii) any costs, expenses, or damages claimed by or through Prime Landlord, or (iii) any costs, expenses or damages to which either party is expressly entitled pursuant to the Prime Lease or this Sublease.

21. No Waiver. No waiver of any of the terms of this Sublease shall be binding upon Sublandlord or Subtenant unless reduced to writing and signed by such party. The failure of Sublandlord or Subtenant to insist in any one or more instances upon the strict performance of any of the covenants, agreements, terms, provisions or conditions of this Sublease, or to exercise any election or option contained herein, shall not be construed as a waiver or relinquishment, in the future or in any other instance, of such covenant, agreement, term, provisions, condition, election or option.

22. Holdover. If Subtenant shall unlawfully holdover after the end of the Term of this Sublease, Subtenant shall be considered a tenant-at-sufferance and shall pay to Sublandlord monthly holdover rent equal to 200% of the monthly Annual Fixed Rent payable in the last month

of the Term, together with all Additional Rent and any other direct damages actually incurred by Sublandlord as a result of such holdover by Subtenant. In furtherance and not in limitation of the foregoing, Subtenant shall save Sublandlord, its trustees, officers, agents and employees, from and against any and all damages which Sublandlord may suffer on account of Subtenant's hold-over in the Subleased Premises after the expiration or prior termination of this Sublease, including, without limitation, any damages for which Sublandlord may be liable pursuant to Section 11.10 of the Prime Lease.

23. Mutual Warranties and Representations.

(a) Subtenant warrants and represents that (i) Subtenant is a corporation, duly organized, validly existing and in good standing under the laws of the State of Delaware and qualified to do business in Massachusetts; (ii) Subtenant has the authority to own its property and to carry on its business in Massachusetts as contemplated under this Sublease; (iii) Subtenant is in compliance with all laws and orders of public authorities applicable to Subtenant that would impact Subtenant's ability fully to perform its obligations under this Sublease; (iv) Subtenant has duly executed and delivered this Sublease; (v) the execution, delivery and performance by Subtenant of this Sublease (x) are within the powers of Subtenant, (y) have been duly authorized by all requisite action, (z) will not violate any provision of law or any order of any court or agency of government, or any agreement or other instrument to which Subtenant is a party or by which it or any of its property is bound, and (vi) this Sublease is a valid and binding obligation of Subtenant in accordance with its terms.

(b) Sublandlord warrants and represents that (i) Sublandlord is a corporation, duly organized, validly existing and in good standing under the laws of the State of Delaware and qualified to do business in Massachusetts; (ii) Sublandlord has duly executed and delivered this Sublease; (iii) the execution, delivery and performance by Sublandlord of this Sublease (x) are within the powers of Sublandlord, (y) have been duly authorized by all requisite action, (z) will not violate any provision of law or any order of any court or agency of government, or any agreement or other instrument to which Sublandlord is a party or by which it or any of its property is bound, and (iv) this Sublease is a valid and binding obligation of Sublandlord in accordance with its terms.

24. Waiver of Jury Trial. IT IS AGREED BY AND BETWEEN SUBLANDLORD AND SUBTENANT THAT THE RESPECTIVE PARTIES HERETO SHALL AND THEY HEREBY DO WAIVE TRIAL BY JURY IN ANY ACTION, PROCEEDING OR COUNTERCLAIM BROUGHT BY EITHER OF THE PARTIES HERETO AGAINST THE OTHER ON ANY MATTERS ARISING OUT OF OR IN ANY WAY CONNECTED WITH THIS SUBLEASE, THE RELATIONSHIP OF SUBLANDLORD AND SUBTENANT, OR SUBTENANT'S USE OR OCCUPANCY OF THE SUBLEASED PREMISES. SUBTENANT FURTHER AGREES THAT IT SHALL NOT IMPOSE ANY COUNTERCLAIM IN A SUMMARY PROCEEDING OR IN ANY ACTION BASED ON NON-PAYMENT OF RENT OR ANY OTHER PAYMENT REQUIRED BY SUBTENANT HEREUNDER.

25. Quiet Enjoyment. Subject to the terms and provisions of this Sublease, the Prime Lease (including, without limitation, the rights of Prime Landlord) and the Ground Lease and

subject to the payment by Subtenant of all items constituting Rent pursuant to this Sublease, and to the observance, keeping and performance of all of the other terms and provisions of this Sublease on Subtenant's part to be observed, kept and performed, Subtenant shall lawfully, peaceably and quietly have, hold occupy and enjoy the Subleased Premises during the term hereof, without hindrance or ejection by Sublandlord or any persons claiming title that is paramount or adverse to that of Sublandlord. The foregoing covenant of quiet enjoyment is in lieu of any other covenant, express or implied.

26. Lease Security. Upon execution of this Sublease, Subtenant shall deliver to Sublandlord an amount equal to $687,355.00 (as may be reduced as set forth herein, the "Security Deposit"). Sublandlord shall retain the Security Deposit as security for the performance by Subtenant of each of its obligations hereunder. If at any time Subtenant fails to perform any of its obligations under this Sublease (beyond any applicable notice and cure period), including the payment of Rent, Sublandlord may, at its option, draw down the Security Deposit (or any portion thereof) to cure Subtenant's default or to pay for damages caused by Subtenant's default. In the event of any assignment of this Sublease by the Sublandlord, Sublandlord shall transfer the Security Deposit to the assignee and shall provide Subtenant with evidence of such assignment. Subtenant may provide the Security Deposit to Sublandlord either in the form of cash (via electronic funds transfer) or by letter of credit in a form attached hereto as Schedule D (the "Letter of Credit"). Subtenant shall be responsible for the payment of any fee to the issuing bank for the transfer of the Letter of Credit. Sublandlord shall return the Security Deposit, net of any amounts withheld by Sublandord to satisfy obligations under this Sublease, to Subtenant no later than sixty (60) days following the Term Expiration Date.

27. Prime Landlord's Consent. This Sublease shall be of no force or effect whatsoever, or be binding in any way, unless and until Prime Landlord has given its written consent to this Sublease (the "Prime Landlord Consent"). If Prime Landlord has not granted consent to this Sublease by the date that is sixty (60) days after the Effective Date (the "Prime Landlord Consent Date"), then after the Prime Landlord Consent Date, either party to this Sublease may terminate this Sublease upon fifteen (15) days' prior written notice to the other, provided, however, that such notice shall be of no force and effect if the Prime Landlord Consent is delivered within such 15-day period; provided, further, that if the Prime Landlord denies approval of this Sublease prior to the Prime Landlord Consent Date that Sublandlord may immediately terminate this Sublease upon written notice to Subtenant.

28. Estoppel Certificate. Subtenant agrees at any time and from time to time upon not less than seven (7) days' prior written request by the Sublandlord, to execute, acknowledge and deliver to the Sublandlord a statement in writing made certifying that this Sublease is unmodified and in full force and effect (or if there have been modifications, that the same is in full force and effect as modified and stating the modifications), and the dates to which Rent has been paid in advance, if any, stating whether or not the Sublandlord is, to the knowledge of Subtenant, in default in performance of any covenant, agreement, term, provision or condition contained in this Lease and, if so, specifying each such default and such other facts as may be reasonably requested, and any such other statements as may be required by Sublandlord. Any such statement delivered pursuant to this Section 28 may be relied upon by Prime Landlord or any actual or prospective

purchaser or mortgagee of the Building or of any interest of Prime Landlord therein or any prospective assignee of Sublandlord or any ground lessor.

29. Miscellaneous.

(a) This Sublease together with the Prime Lease, the Exhibits thereto and the Ground Lease (i) contains the entire agreement of the parties with respect to the subject matter which it covers; (ii) supersedes all prior or other negotiations, representations, understandings and agreements of, by or between the parties, which shall be deemed fully merged herein; and (iii) may not be changed or terminated orally.

(b) THIS SUBLEASE SHALL BE GOVERNED BY AND CONSTRUED UNDER AND IN ACCORDANCE WITH THE LAWS OF THE COMMONWEALTH OF MASSACHUSETTS AND THE OBLIGATIONS OF THE PARTIES HERETO ARE AND SHALL BE PERFORMABLE IN MIDDLESEX COUNTY, MASSACHUSETTS. BY EXECUTING THIS SUBLEASE, EACH PARTY HERETO EXPRESSLY (a) CONSENTS AND SUBMITS TO PERSONAL JURISDICTION CONSISTENT WITH THE PREVIOUS SENTENCE, (b) WAIVES TO THE FULLEST EXTENT PERMITTED BY LAW, ANY CLAIM OR DEFENSE THAT SUCH VENUE IS NOT PROPER OR CONVENIENT AND (c) CONSENTS TO SERVICE OF PROCESS IN ANY MANNER AUTHORIZED BY MASSACHUSETTS LAW. ANY FINAL JUDGMENT ENTERED IN AN ACTION BROUGHT HEREUNDER SHALL BE CONCLUSIVE AND BINDING UPON THE PARTIES HERETO.

(c) This Sublease may be executed in any number of counterparts, each of which shall be deemed to be an original and all of which shall constitute one and the same instrument.

(d) The captions and headings herein are inserted only as a matter of convenience and for reference and in no way define, limit, construe or describe the scope of this Sublease or the meaning or intent of any provision hereof.

(e) This Sublease shall be binding upon and inure to the benefit of the parties hereto and their respective permitted successors and assigns. Notwithstanding anything to the contrary set forth herein, it is hereby agreed that Sublandlord has the right to (upon consent of Prime Landlord) assign, transfer, pledge or otherwise convey any interest of Sublandlord in the Subleased Premises and/or this Sublease, and Subtenant agrees that in the event of any such transfer, Sublandlord shall automatically be released from all liability under this Sublease accruing from and after the date of such transfer of interest by Sublandlord, and Subtenant agrees to thereupon look solely to the transferee for the performance of Sublandlord's obligations hereunder accruing from and after the date of such transfer of interest by Sublandlord.

(f) In case any one or more of the provisions contained in this Sublease shall for

any reason be held to be invalid, illegal, or unenforceable in any respect, such invalidity, illegality, or unenforceability shall not affect any other provisions hereof and this Sublease shall be construed as if such invalid, illegal, or unenforceable provisions had never been included herein.

(g) If any action at law or in equity, including an action for declaratory relief, is brought by other party hereto to enforce or interpret the provisions of this Sublease, the prevailing party in such action shall be entitled to recover reasonable attorneys' fees from the non-prevailing party, which fees may be set by the court in the trial of such action or may be enforced in a separate action for that purpose, and which fees shall be in addition to any other relief which may be awarded in such action.

(h) Without limiting any other obligation which may survive the expiration or prior termination of the Term, all obligations on the part of each party to indemnify, defend, or hold the other harmless, and obligations arising during the Term which have not been fulfilled as of the expiration or prior termination, as set forth herein, shall survive the expiration or prior termination of the Term.

(i) Time is of the essence in this Sublease.

(j) In the event that the date upon which any of the duties or obligations hereunder to be performed shall occur upon a Saturday, Sunday or legal holiday, then, in such event, the due date for performance of any duty or obligation shall thereupon be automatically extended to the next succeeding business day.

(k) Neither party shall record this Sublease or a notice of lease.

[***SIGNATURE PAGE FOLLOWS***]

IN WITNESS WHEREOF, the parties hereto have caused this Sublease to be duly executedas of the day and year first above written.

SUBLANDLORD:

VOYAGER THERAPEUTICS, INC.

By: */s/ Allison Dorval*

Name: Allison Dorval
Title: Chief Financial Officer

SUBTENANT:

BIONTECH US INC.

By: */s/ Richard Gaynor*

Name: Richard Gaynor
Title: President

SCHEDULE A

[Copy of Prime Lease]

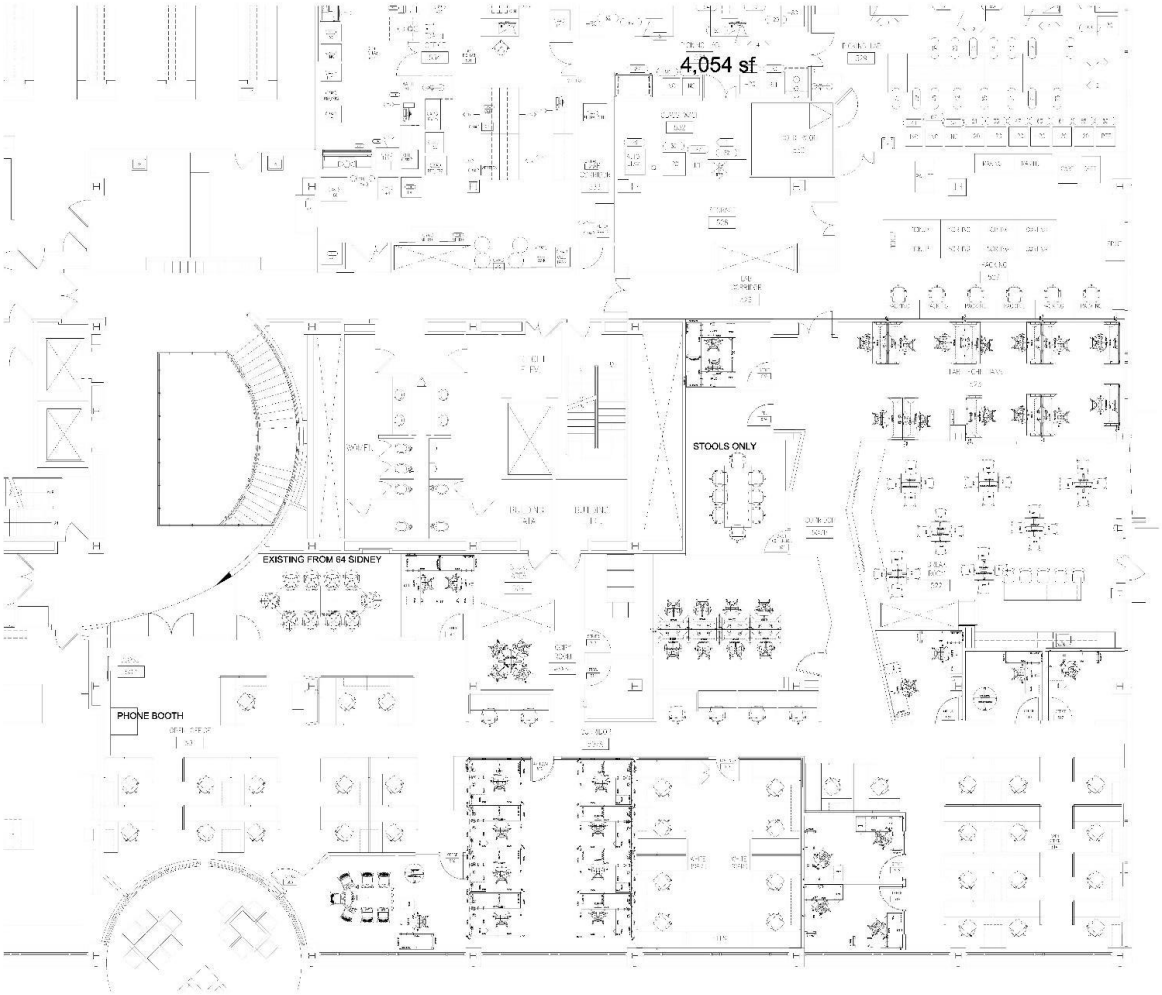
[*to be attached*]

SCHEDULE B

[Floorplan] [*see following page*]

PLAN VIEW

SCALE: 1/4" = 1'



KEY PLAN:

PROJECT NOTES:

NO.	REVISION ISSUE	DATE
1	FIRST ISSUE	----
2	REVISION	----
3	REVISION	----

PROJECT NAME & ADDRESS:
Voyager
75 Sidney St.
Cambridge MA

PROJECT DESCRIPTION:
Updated Floor Plan

Sales Representative:
Matt Ferdi

DRAWN BY:
AP

DATE:
04.24.18

SCALE:
1/4" = 1'

SHEET
1 1

SCHEDULE C

[Furniture and Equipment List]

- Autoclave (Amsco 20X20 Stage 3 serial number 41329)
- Four (4) foot fume hood (Supreme Air)

[*continued on following page*]

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www.strategicspaces.com

QUOTATION

Page: 1

Quote #	Date	Customer Order #	Customer #	Account Representative	Project #
23047	04/24/19		1736	Matt Feroli	27-20

To: Voyager Therapeutics
75 Sidney Street

Cambridge, MA 02139

Attn:
P. 857-259-5341
F. invoices to both

Terms: 50%Dep/BalN10

Ship To: Voyager Therapeutics
75 Sidney Street
5th Floor
Cambridge, MA 02139

Attn:

Line #	Quantity	Catalog #	Description
1	8	E-MW8W4DO1	Duplex Outlet - Line 1 Tag(s): 1. Team Room 512
2	8	E-MW8W4DO2	Duplex Outlet - Line 2 Tag(s): 1. Team Room 512
3	8	E-MX8W4PTPC 18	18 in. Electrical Jumper (Panel To Panel) Tag(s): 1. Team Room 512
4	6	E-MX8W4PTPC 21	21 in. Electrical Jumper (Extended Panel To Panel) Tag(s): 1. Team Room 512
5	1	E-UN8W4BIFL	LH Receptacle Mounted Base Infeed - 72L - 8 Wire, 4 Circuit Tag(s): 1. Team Room 512
6	1	E-UN8W4BIFR	RH Receptacle Mounted Base Infeed - 72L - 8 Wire, 4 Circuit Tag(s): 1. Team Room 512
7	24	WPS-FP	Flat Plate Tag(s): 1. Team Room 512
8	8	E-UNWC	Duplex Hole Cover SW - Satin White Tag(s): 1. Team Room 512

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Attn:

Line #	Quantity	Catalog #	Description
9	10	P-DVSEOR	End of Run Base Raceway Shroud SW - Satin White Tag(s): 1. Team Room 512
10	12	WPS-UNCT24L	Regular Cantilever - Left Hand - 24D BK - Black Tag(s): 1. Team Room 512
11	12	WPS-UNCT24R	Regular Cantilever - Right Hand - 24D BK - Black Tag(s): 1. Team Room 512
12	8	S-PEDBBFJ24	Pedestal - B/B/F - 24D Paint Grade A SW - White Tag(s): 1. Team Room 512
13	6	P-DVBS3W	3-Way 90 Degree Base Raceway Shroud No SW - Satin White Tag(s): 1. Team Room 512
14	12	P-DVBS90	2-Way 90 Degree Base Raceway Shroud No SW - Satin White Tag(s): 1. Team Room 512
15	8	W-WS2442	Rectangular - 2mm Edge - 24D x 42W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 1. Team Room 512

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Cambridge, MA 02139

Attn:

Line #	Quantity	Catalog #	Description
16	8	W-WS2478	Rectangular - 2mm Edge - 24D x 78W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 1. Team Room 512
17	8	P-DVFPPM5024	Hard Surface Panel - Powered - 50H x 24W SW - Satin White No Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Panel Trim Paint Grade A SW Satin White Tag(s): 1. Team Room 512
18	8	P-DVFPPM5042	Hard Surface Panel - Powered - 50H x 42W SW - Satin White No Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Panel Trim Paint Grade A SW Satin White Tag(s): 1. Team Room 512
19	8	P-DVFRPM5030	Hard Surface Panel - Non Powered - 50H x 30W No SW - Satin White Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Panel Trim Paint Grade A SW Satin White CONTINUED...

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Attn:

Line #	Quantity	Catalog #	Description
			Tag(s): 1. Team Room 512
20	10	P-DVFRPM5036	Hard Surface Panel - Non Powered - 50H x 36W No SW - Satin White Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Panel Trim Paint Grade A SW Satin White Tag(s): 1. Team Room 512
21	10	P-DVFRPM5042	Hard Surface Panel - Non Powered - 50H x 42W No BK - Black Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Panel Trim Paint Grade A SW Satin White Tag(s): 1. Team Room 512
22	25	WPS-FP	Flat Plate Tag(s): 2. Private Offices
23	25	O-PL2525	Desking 2.5 x 2.5 in. Post Leg Painted Oxygen Grade A Paint Selection SW - White Tag(s): 2. Private Offices

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Line #	Quantity	Catalog #	Description
24	13	S-PEDBBFJ24	Pedestal - B/B/F - 24D Paint Grade A SW - White Tag(s): 2. Private Offices
25	7	WFS-AL22718	A-Leg - 2.5 x 2.5 Post - 27H - 18 Depth Paint Grade A SW - White Tag(s): 2. Private Offices
26	4	WFS-AL22724	A-Leg - 2.5 x 2.5 Post - 27H - 24 Depth Paint Grade A SW - White Tag(s): 2. Private Offices
27	2	A-UNSUF1624	Universal Up-Mount Privacy Screen - Tackable Fabric 16 X 24 Fabric Grade A Directional Fabric - Grade A New England Concord Fabric Grade A Directional Fabric - Grade A New England Concord Paint Grade A SW - White Tag(s): 2. Private Offices
28	7	WFS-FLSPF1E 18	FLSP F - Female 1 End - 18D 2mm Grade A 2MM Edge - Grey Value Skipped Option Tag(s): 2. Private Offices
29	2	WFS-FLSPMEF 18	FLSP A - Male 1 End Flush - 18D 2mm Grade A 2MM Edge - Grey Value Skipped Option Tag(s): 2. Private Offices
30	6	WFS-FLSPMEF 24	FLSP A - Male 1 End Flush - 24D 2mm Grade A 2MM Edge - Grey Value Skipped Option CONTINUED...

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Cambridge, MA 02139

Attn:

Line #	Quantity	Catalog #	Description
			Tag(s): 2. Private Offices
31	6	W-WS1854	Worksurface Rect 18"D X 54"W X 1 1/8"Th 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
32	1	W-WS1866	Worksurface Rect 18"D X 66"W X 1 1/8"Th 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
33	2	W-WS2448	Rectangular - 2mm Edge - 24D x 48W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
34	11	W-WS2454	Rectangular - 2mm Edge - 24D x 54W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet CONTINUED...

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Line #	Quantity	Catalog #	Description
			Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
35	3	W-WS2460	Rectangular - 2mm Edge - 24D x 60W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
36	1	W-WS2484	Rectangular - 2mm Edge - 24D x 84W 2mm Grade A 2MM Edge - GV1/Summer Drops E - Locations 2 & 3 Grommet Cover Color Selection Grommet Cover - Grey AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Tag(s): 2. Private Offices
37	1	T-RDR3029SX G	Tbl, Rnd, 2mm, 30dx29h, Stlx30, Gld 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops TABLE GRADE A LAMINATES AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Grade A Paint For Steel Bases SW - White Tag(s): 2. Private Offices

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Line #	Quantity	Catalog #	Description
38	1	T-RDR3629SXG	Tbl, Rnd, 2mm, 36dx29h, Sttx36, Gld 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops TABLE GRADE A LAMINATES AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Grade A Paint For Steel Bases SW - White Tag(s): 2. Private Offices
39	5	BA-DBRPS4866	Aloft V2 Double Bench Powered Fixed 48 X 66 2mm Grade A EDGE DETAIL 2MM GV1/SUMMER DROPS RIGID SMOOTH 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) PAINT v2 ALOFT FIXED KITS DOUBLE BENCHES GRADE A ALOFT FIXED BASE KIT DOUBLE BENCH SATIN WHITE Oxygen Grade A Paint Selection SW - White Tag(s): 3. Lab Tech 523
40	3	BA-SBRPS2466	Aloft V2 Single Bench Powered Fixed 24 X 66 2mm Grade A EDGE DETAIL 2MM GV1/SUMMER DROPS RIGID SMOOTH 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) PAINT v2 ALOFT FIXED KITS SINGLE BENCHES GRADE A ALOFT v2 FIXED BASE KIT SINGLE CONTINUED...

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Attn:

Line #	Quantity	Catalog #	Description
			BENCH SATIN WHITE Oxygen Grade A Paint Selection SW - White Tag(s): 3. Lab Tech 523
41	12	E-MW8W4DO1	Duplex Outlet - Line 1 Tag(s): 3. Lab Tech 523
42	14	E-MW8W4DO2	Duplex Outlet - Line 2 Tag(s): 3. Lab Tech 523
43	8	E-UN8W4BIFS	Side Mounted Base Infeed - 72L - 8 Wire, 4 Circuit Tag(s): 3. Lab Tech 523
44	8	O-SSH1666	Spine Screen 46H Horizon 16H 66W Skipped Option Skipped Option Oxygen Grade A Paint Selection SW - White Tag(s): 3. Lab Tech 523
45	10	RZ2460A	Ritz Table (Complete), 3mm PVC Edge, Black Casters, 24"D x 60"W No Cable Management Arctic White FIN: Wilsonart Portico Teak (8210K-28) Tag(s): 4. Breakroom
46	7	CR3030	Rectangle Top, Sq. Corners 30x30 Std Laminate 3mm PVC Edge (all sides) FIN: Wilsonart Portico Teak (8210K-28) No Cable Management CONTINUED...

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Attn:

Line #	Quantity	Catalog #	Description
			Tag(s): 4. Breakroom
47	7	FT5226	Duracast Flip Top X Base 27 3/4 High Powder Coat White Tag(s): 4. Breakroom
48	49	6753	BAKHITA, Molded Plastic Back & Frame w/ Upholstered Seat, Armless, Std 4 Legged Base, Stacks 12 High on Dolly 6756, Stacks 8 High on Floor, GLOBAL SE Bakita Back/FramePlastic Finishes 1-Milk [MLK] Grade 02 Release (Morbern) 1-Cool Grey Tag(s): 4. Breakroom GUS - Seating U
49	8	6413	NOVELLO, Upholstered Back, Upholstered Seat, Drafting Stool w/ Footring, Armless, Std Molded Black Base, Std 2" Dual Wheel Carpet Casters, GLOBAL SEA Grade 01 Hudson (Global) 1-Grey F-Ivory Clouds Shell / Ivory Clouds Arm Color M-(STD) Black Nylon Base P-(STD) 10" Soft Descent Cylinder M-Polished Aluminum, Adjustable Footring C-Fog Dual Wheel Carpet Casters M-(STD) Standard Seat Foam M-(STD) RTA Code in Pricebook per Model Tag(s): 5. Conf Room 521 GUS - Seating

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Line #	Quantity	Catalog #	Description
50	4	FBWSWM	FOOTPRINT,WORKSURFACE BRACKET,WALL MOUNT Tag(s): 6. Huddle Rooms
51	1	71K2728MLGBLY	TEEM,MEDIA LEGS,Y,BLADE,15 INCH FOOT,METAL DESIGNER WHITE DESIGNER WHITE Tag(s): 6. Huddle Rooms
52	1	71K4284MWSUL	TEEM,MEDIA WORKSURFACE,U SHAPE,LAM 1/8" MOLDED PVC C4,4X8,CENTER,CUTOUT ONLY STANDARD GROUP 1 FOG FOG Tag(s): 6. Huddle Rooms
53	1	T-RDR4229SXG	Tbl, Rnd, 2mm, 42dx29h, Stlx36, Gld 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops TABLE GRADE A LAMINATES AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) Grade A Paint For Steel Bases SW - White Tag(s): 6. Huddle Rooms
54	4	BA-DBRPS4848	Aloft V2 Double Bench Powered Fixed 48 X 48 2mm Grade A EDGE DETAIL 2MM GV1/SUMMER DROPS RIGID SMOOTH 2mm T-Mold Edge - Grade A - Curved Edging 2MM T-Mold - GV1/Summer Drops AIS Grade A Laminates Laminate - Summer Drops - (A-T-S-W-L202) PAINT v2 ALOFT FIXED KITS DOUBLE BENCHES GRADE A CONTINUED...

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Line #	Quantity	Catalog #	Description
			ALOFT FIXED BASE KIT DOUBLE BENCH SATIN WHITE Paint - Oxygen - Grade C ($10) Oxygen Grade C Paint Selection SW - White Tag(s): 7. Workstations 520
55	8	E-MW8W4DO1	Duplex Outlet - Line 1 Tag(s): 7. Workstations 520
56	8	E-MW8W4DO2	Duplex Outlet - Line 2 Tag(s): 7. Workstations 520
57	1	E-UN8W4BIFS	Side Mounted Base Infeed - 72L - 8 Wire, 4 Circuit Tag(s): 7. Workstations 520
58	3	E-UN8W4J30	30 in. Electrical Jumper Tag(s): 7. Workstations 520
59	4	O-SSH1648	Spine Screen 46H Horizon 16H 48W SPINE SCREEN FABRIC INSERT A 46H HORIZON 16H 48W Fabric Grade A Directional Fabric - Grade A New England Concord SPINE SCREEN FABRIC INSERT B 46H HORIZON 16H 48W Fabric Grade A Directional Fabric - Grade A New England Concord Oxygen Grade A Paint Selection SW - White Tag(s): 7. Workstations 520

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Attn:

Line #	Quantity	Catalog #	Description
60	1	NB1LC	Nevins PhoneBoothw/ClearGlass Tag(s): 8. Lobby
61	1		Freight Tag(s): 8. Lobby
62	1	INSTALLATIO N	Receive, Deliver and Install per approved plans. Normal Business Hours, Non-Union Labor Voyager - 75 Sidney St., 5th Floor Tag(s): 9. All

Accepted By_____ Title_____ Date_____

SCHEDULE D

[Form of Letter of Credit]

L/C DRAFT LANGUAGE

IRREVOCABLE STANDBY LETTER OF CREDIT NUMBER _____

ISSUE DATE: _____

ISSUING BANK:
SILICON VALLEY BANK
3003 TASMAN DRIVE
2ND FLOOR, MAIL SORT HF210
SANTA CLARA, CALIFORNIA 95054

BENEFICIARY:
VOYAGER THERAPEUTICS, INC.75
SIDNEY STREET 5TH FLOOR
CAMBRIDGE, MA 02139

APPLICANT:
BIONTECH US INC.
60 HAMILTON STREET
CAMBRIDGE, MA 02139

AMOUNT: US$687,355.00 (SIX HUNDRED EIGHTY SEVEN THOUSAND THREE HUNDRED FIFTY FIVE AND 00/100 U.S.DOLLARS)

EXPIRATION DATE: ___ONE YEAR FROM ISSUANCE_____

PLACE OF EXPIRATION: ISSUING BANK'S COUNTERS AT ITS ABOVE ADDRESS

DEAR SIR/MADAM:

WE HEREBY ESTABLISH OUR IRREVOCABLE STANDBY LETTER OF CREDIT NO. SVBSF_____IN YOUR FAVOR AVAILABLE BY PAYMENT AGAINST YOUR PRESENTATION TO US OF THE FOLLOWING DOCUMENT:

1. 1.BENEFICIARY'S SIGNED AND DATED STATEMENT STATING AS FOLLOWS:

(A) "THE AMOUNT REPRESENTS FUNDS DUE AND OWING TO US PURSUANT TO THE TERMS OF THAT CERTAIN SUBLEASE BY AND BETWEEN VOYAGER THERAPEUTICS, INC., AS SUBLANDLORD, AND BIONTECH US, INC., AS S U B TENANT. THE AMOUNT HEREBY DRAWN UNDER THE LETTER OF CREDIT ISUS$_____, WITH PAYMENT TO BE MADE TO THE FOLLOWING ACCOUNT: [INSERT WIRE INSTRUCTIONS (TO INCLUDE NAME AND ACCOUNT NUMBER OF THE BENEFICIARY)"

OR

(B) " VOYAGER THERAPEUTICS, INC. HEREBY CERTIFIES THAT IT HAS RECEIVED NOTICE FROM SILICONVALLEY BANK THAT THE LETTER OF CREDIT NO._____ WILL NOT BE EXTENDED, AND THAT IT HAS NOT RECEIVED A REPLACEMENT OF THIS LETTER OF CREDIT FROM. BIONTECH US, INC. SATISFACTORY TO VOYAGER THERAPEUTICS, INC. THE AMOUNT HEREBY DRAWN UNDER THE LETTEROF CREDIT IS US$ _____, WITH PAYMENT TO BE MADE TO THE FOLLOWING ACCOUNT: [INSERT WIRE INSTRUCTIONS (TO INCLUDE NAME AND ACCOUNT NUMBER OF THE BENEFICIARY)."

PARTIAL DRAWS AND MULTIPLE PRESENTATIONS ARE ALLOWED.

ALL THE DETAILS SET FORTH HEREIN IN THIS LETTER OF CREDIT DRAFT IS APPROVED BY APPLICANT. IF THERE IS ANY DISCREPANCY BETWEEN THE DETAILS OF THIS LETTER OF CREDIT DRAFT AND THE LETTER OF CREDIT APPLICATION, BETWEEN APPLICANT AND SILICON VALLEY BANK, THE DETAILS HEREOF SHALL PREVAIL.

THIS LETTER OF CREDIT SHALL BE AUTOMATICALLY EXTENDED FOR ADDITIONAL PERIODS OFONE YEAR, WITHOUT AMENDMENT, FROM THE PRESENT OR EACH FUTURE EXPIRATION DATE UNLESS AT LEAST 60 DAYS PRIOR TO THE THEN CURRENT EXPIRATION DATE WE SEND YOU A NOTICE BY REGISTERED MAIL/OVERNIGHT COURIER SERVICE AT THE ABOVE ADDRESS THAT THIS LETTER OF CREDIT WILL NOT BE EXTENDED BEYOND THE THEN CURRENT EXPIRATION DATE. IN NO EVENT SHALL THIS LETTER OF CREDIT BE AUTOMATICALLY EXTENDED BEYOND JANUARY 30, 2025.

THIS LETTER OF CREDIT MAY ONLY BE TRANSFERRED IN ITS ENTIRETY BY THE ISSUING BANK, ASSUMING SUCH TRANSFER TO SUCH TRANSFEREE WOULD BE IN COMPLIANCE WITH THEN APPLICABLE LAW AND REGULATIONS, INCLUDING BUT NOT LIMITED TO THE REGULATIONS OF THE U.S. DEPARTMENT OF TREASURY AND U.S. DEPARTMENT OF COMMERCE, UPON OUR RECEIPT OF THE ATTACHED "EXHIBIT A" DULY COMPLETED AND EXECUTED BY THE BENEFICIARY AND ACCOMPANIED BY THE ORIGINAL LETTER OF CREDIT AND ALL AMENDMENT(S), IF ANY, TOGETHER WITH THE PAYMENT OF OUR TRANSFER FEE ¼ OF 1% OF THE TRANSFER AMOUNT (MINIMUM USD250.00). HOWEVER, PAYMENT OF SUCH TRANSFER FEE SHALL NOT BE A CONDITION OF SUCH TRANSFER. EACH TRANSFER SHALL BE EVIDENCED BY EITHER (1) OUR ENDORSEMENT ON THE REVERSE OF THE LETTER OF CREDIT AND WE SHALL FORWARD THE ORIGINAL OF THE LETTER OF CREDIT SO ENDORSED TO THE TRANSFEREE OR (2) OUR ISSUING A REPLACEMENT LETTER OF CREDIT TO THE TRANSFEREE ON SUBSTANTIALLY THE SAME TERMS AND CONDITIONS AS THE TRANSFERRED LETTER OF CREDIT (IN WHICH EVENT THE TRANSFERRED LETTER OF CREDIT SHALL HAVE NO FURTHER EFFECT).

ALL DEMANDS FOR PAYMENT SHALL BE MADE BY PRESENTATION OF THE REQURIED DOCUMENTS DURING REGULAR BUSINESS HOURS, ON A BUSINESS DAY AT OUR OFFICE (THE "BANK'S OFFICE") AT: SILICON VALLEY BANK, 3003 TASMAN DRIVE, MAIL SORT HF210, SANTA CLARA, CALIFORNIA 95054, ATTENTION: GLOBAL TRADE FINANCE. AS USED IN THIS LETTER OF CREDIT, "BUSINESS DAY" SHALL MEAN ANY DAY OTHER THAN A SATURDAY, SUNDAY OR A DAY ON WHICH BANKING INSTITUTIONS IN THESTATE OF CALIFORNIA ARE AUTHORIZED OR REQUIRED BY LAW TO CLOSE.

FACSIMILE PRESENTATIONS ARE ALSO PERMITTED. SHOULD BENEFICIARY WISH TO MAKE A PRESENTATION UNDER THIS LETTER OF CREDIT ENTIRELY BY FACSIMILE TRANSMISSION IT NEED NOT TRANSMIT THE ORIGINAL OF THIS LETTER OF CREDIT AND AMENDMENTS, IF ANY. EACH FACSIMILE TRANSMISSION SHALL BE MADE AT: (408) 496-2418 OR (408) 969-6510; AND UNDER CONTEMPORANEOUS TELEPHONE ADVICE TO: (408)450-5001 OR (408) 654-7176, ATTENTION: GLOBAL TRADE FINANCE. ABSENCE OF THE AFORESAID TELEPHONE ADVICE SHALL NOT AFFECT OUR OBLIGATION TO HONOR ANY DRAW REQUEST.

WE HEREBY AGREE WITH THE BENEFICIARY THAT THE DRAFTS DRAWN UNDER AND IN ACCORDANCEWITH THE TERMS AND CONDITIONS OF THIS LETTER OF CREDIT SHALL BE DULY HONORED UPON PRESENTATION TO THE DRAWEE, IF PRESENTED ON OR BEFORE THE EXPIRATION DATE OR ANY AUTOMATICALLY EXTENDED EXPIRATION DATE OF THIS LETTER OF CREDIT.

IF ANY INSTRUCTIONS ACCOMPANYING A DRAWING UNDER THIS LETTER OF CREDIT REQUEST THAT PAYMENT IS TO BE MADE BY TRANSFER TO YOUR ACCOUNT WITH ANOTHER BANK, WE WILL ONLY EFFECT SUCH PAYMENT BY FED WIRE TO A U.S. REGULATED BANK, AND WE AND/OR SUCH OTHER BANK MAY RELY ON AN ACCOUNT NUMBER SPECIFIED IN SUCH INSTRUCTIONS EVEN IF THE NUMBERIDENTIFIES A PERSON OR ENTITY DIFFERENT FROM THE INTENDED PAYEE.

THIS LETTER OF CREDIT IS SUBJECT TO THE INTERNATIONAL STANDBY PRACTICES (ISP98),INTERNATIONAL CHAMBER OF COMMERCE, PUBLICATION NO. 590.

AUTHORIZED SIGNATURE AUTHORIZED SIGNATURE

ALL THE DETAILS SET FORTH HEREIN IN THIS LETTER OF CREDIT DRAFT IS APPROVED BY APPLICANT. IF THERE IS ANY DISCREPANCY BETWEEN THE DETAILS OF THIS LETTER OF CREDIT DRAFT AND THE LETTER OF CREDIT APPLICATION, BETWEEN APPLICANT AND SILICON VALLEY BANK, THE DETAILS HEREOF SHALL PREVAIL.

IRREVOCABLE STANDBY LETTER OF CREDIT NUMBER _____

EXHIBIT A

FORM OF TRANSFER FORM

DATE: _____

TO: SILICON VALLEY BANK
3003 TASMAN DRIVE
SANTA CLARA, CA 95054
ATTN: GLOBAL TRADE FINANCE
STANDBY LETTERS OF CREDIT

RE: IRREVOCABLE STANDBY LETTER OF CREDIT
NO._____ISSUED BY
SILICON VALLEY BANK, SANTA CLARA
L/C AMOUNT: _____

GENTLEMEN:

FOR VALUE RECEIVED, THE UNDERSIGNED BENEFICIARY HEREBY IRREVOCABLY TRANSFERS TO:

(NAME OF TRANSFEREE)

_____

(ADDRESS)

ALL RIGHTS OF THE UNDERSIGNED BENEFICIARY TO DRAW UNDER THE ABOVE LETTER OF CREDIT UP TO ITS AVAILABLE AMOUNT AS SHOWN ABOVE AS OF THE DATE OF THIS TRANSFER.

BY THIS TRANSFER, ALL RIGHTS OF THE UNDERSIGNED BENEFICIARY IN SUCH LETTER OF CREDIT ARE TRANSFERRED TO THE TRANSFEREE. TRANSFEREE SHALL HAVE THE SOLE RIGHTS AS BENEFICIARY THEREOF, INCLUDING SOLE RIGHTS RELATING TO ANY AMENDMENTS, WHETHER INCREASES OR EXTENSIONS OR OTHER AMENDMENTS, AND WHETHER NOW EXISTING OR HEREAFTER MADE. ALL AMENDMENTS ARE TO BE ADVISED DIRECTLY TO THE TRANSFEREE WITHOUT NECESSITY OF ANY CONSENTOF OR NOTICE TO THE UNDERSIGNED BENEFICIARY.

THE ORIGINAL OF SUCH LETTER OF CREDIT IS RETURNED HEREWITH, AND WE ASK YOU TO EITHER (1) ENDORSE THE TRANSFER ON THE REVERSE THEREOF, AND FORWARD IT DIRECTLY TO THE TRANSFEREE WITH YOUR CUSTOMARY NOTICE OF TRANSFER, OR (2) ISSUE A REPLACEMENT LETTER OF CREDIT TO THE TRANSFEREE ON SUBSTANTIALLY THE SAME TERMS AND CONDITIONS AS THE TRANSFERRED LETTER OF CREDIT (IN WHICH EVENT THE TRANSFERRED LETTER OF CREDIT SHALL HAVE NO FURTHER EFFECT).

SINCERELY,

(BENEFICIARY'S NAME)

(SIGNATURE OF BENEFICIAR)

The name(s), title(s), and signature(s) conform to that/those on file with us for the company and the signature(s) is/are authorized to execute this instrument.

(Name of Bank)

(Address of Bank)

N, NY

(City, State, ZIP Code) (Authorized Name and Title)

APPLICANT'S SIGNATURE(S) DATE

SIGNATURE AUTHENTICATED

(Authorized Signature)

(Telephone number)

(NAME AND TITLE)

3638\0004\711990.9

ALL THE DETAILS SET FORTH HEREIN IN THIS LETTER OF CREDIT DRAFT IS APPROVED BY APPLICANT. IF THERE IS ANYDISCREPANCY BETWEEN THE DETAILS OF THIS LETTER OF CREDIT DRAFT AND THE LETTER OF CREDIT APPLICATION, BETWEEN APPLICANT AND SILICON VALLEY BANK, THE DETAILS HEREOF SHALL PREVAIL.

APPLICANT'S SIGNATURE(S) DATE

Exhibit 10.2

EXECUTION VERSION
Confidential

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

OPTION AND LICENSE AGREEMENT

By and between

VOYAGER THERAPEUTICS, INC.

AND

PFIZER INC.

October 1, 2021

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Schedule 1.18: Capsid Patents Covering Capsid Candidates as of the Effective Date

OPTION AND LICENSE AGREEMENT

This OPTION AND LICENSE AGREEMENT (the "Agreement") is entered into and made effective as of October 1, 2021 (the "Effective Date"), by and between Voyager Therapeutics, Inc., a Delaware corporation, having its principal place of business at 75 Sidney Street, Cambridge, MA 02139 ("Voyager"), and Pfizer Inc., a Delaware corporation, having its principal place of business at 235 East 42nd Street NY, NY 10017 ("Pfizer"). Voyager and Pfizer are referred to herein individually as a "Party" and collectively as the "Parties".

RECITALS

WHEREAS, Voyager Controls certain Patents, Know-How, scientific and technical information, and other proprietary rights and information relating to the generation and selection of Capsids (as defined below) for use in AAV Gene Therapy;

WHEREAS, Pfizer is engaged in the research, development and commercialization of certain AAV Gene Therapies (as defined below), and desires to access certain Capsids developed by Voyager; and

WHEREAS, in furtherance of the foregoing, Voyager and Pfizer are entering into this Agreement for Voyager to provide Pfizer with access to Capsids discovered by Voyager prior to the Effective Date or discovered by Voyager after the Effective Date in its ongoing screening campaigns, and to provide Pfizer with an option and license under Voyager's intellectual property rights to develop and commercialize Licensed Products in the Territory.

NOW, THEREFORE, in consideration of the premises and mutual covenants set forth in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

As used in this Agreement, the following terms will have the meanings set forth in this Article 1 or otherwise ascribed to them elsewhere in this Agreement:

1.1 "AAV" means an adeno-associated virus, including its recombinant forms.

1.2 "AAV Gene Therapy" means therapies and products that use a viral vector, including an AAV vector, to deliver nucleic acid(s) into a patient's cells to treat a human disease, syndrome, disorder, illness or condition.

1.3 "Accounting Standards" means United States Generally Accepted Accounting Principles, as generally and consistently applied throughout the applicable Party's organization.

1.4 "Acquiring Entity" has the meaning set forth in Section 1.27.

1.5 "Affiliate" means with respect to a Person, any other Person that (directly or indirectly) is controlled by, controls or is under common control with such Person as of any point

in time and continuing for as long as such relationship continues to exist with respect to such other Person. For the purposes of this definition, the term "control" (including, with correlative meanings, the terms "controlled by" and "under common control with") as used with respect to a Person, will mean the possession, directly or indirectly, of the power to direct, or cause the direction of, the management or policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and "control" will be presumed to exist if either of the following conditions is met: (a) in the case of a corporate entity, direct or indirect ownership of voting securities entitled to cast at least 50% (or the maximum ownership interest permitted by applicable Law) of the voting securities or other ownership or general partnership interest (whether directly or pursuant to any option, warrant or other similar arrangement) or other comparable equity interests of an entity; *provided*, *however*, that where an entity owns a majority of the voting power necessary to elect a majority of the board of directors or other governing board of another entity, but is restricted from electing such majority by contract or otherwise, such entity will not be considered to be in control of such other entity until such time as such restrictions are no longer in effect.

1.6 "Agreement" has the meaning set forth in the Preamble.

1.7 "Alliance Manager" has the meaning set forth in Section 2.1.

1.8 "Annual Net Sales" means, on a Licensed Product-by-Licensed Product basis, the total, aggregate Net Sales of such Licensed Product in the Territory in a particular Calendar Year.

1.9 "Antitrust Filings" has the meaning set forth in Section 2.4.2.

1.10 "Arbitration Request" has the meaning set forth in Section 11.3.

1.11 "Biosimilar Product" means, with respect to a particular Licensed Product in a particular country in the Territory: (a) any pharmaceutical or biological product sold by a Third Party that is not a Sublicensee of Pfizer or its Affiliates and that did not purchase such product in a chain of distribution that included Pfizer or any of its Affiliates or Sublicensees; and (b) which pharmaceutical or biological product (i) is approved by the applicable Regulatory Authority as biosimilar to, or interchangeable with, such Licensed Product (including, with respect to the United States, a product that is the subject of an application submitted under Section 351(k) of the Public Health Services Act citing the Licensed Product as the reference product) or (ii) for which the Regulatory Approval otherwise references or relies on such Licensed Product as a reference product or any corresponding foreign application in the Territory (including, with respect to the EU, a marketing authorization application for a biosimilar biological medicinal product pursuant to Article 10(4) of Directive 2001/83/EC.

1.12 "BLA" means (a) an application requesting permission from the FDA to introduce, or deliver for introduction, a biopharmaceutical product into interstate commerce, or (b) any similar application or submission for Marketing Approval of a biopharmaceutical product filed with a Regulatory Authority in a country or group of countries.

1.13 "Business Day" means a day other than a Saturday or Sunday or holiday observed by the United States federal government or the state or commonwealth in which a Party's primary office is located.

1.14 "Calendar Quarter" means a period of three (3) consecutive months ending on the last day of March, June, September, or December, respectively; provided that: (a) the first Calendar Quarter during the Term will begin on the Effective Date and end on the last day of the Calendar Quarter within which the Effective Date falls; and (b) the last Calendar Quarter during the Term will end upon the effective date of expiration or termination.

1.15 "Calendar Year" means a period of twelve (12) consecutive months beginning on January 1 and ending on December 31; provided that: (a) the first Calendar Year starts on the Effective Date and ends on December 31, 2021; and (b) the last Calendar Year starts on January 1 of such year and ends on the effective date of expiration or termination.

1.16 "Capsid" means the protein shell of an AAV, consisting of oligomeric structural subunits made of certain proteins.

1.17 "Capsid Candidate" means any Cardiology Capsid, CNS Capsid or TRACER Capsid Candidate that is not a Licensed Capsid.

1.18 "Capsid Patent" means any Patent Controlled by Voyager as of the Effective Date or at any time during the Term with claims directed to: (a) compositions of matter of any Capsid Candidate or Licensed Capsid; or (b) methods of use of any Capsid Candidate or Licensed Capsid; in each case (a) and (b), including any Patent Controlled by Voyager that contains a claim directed to a Capsid Candidate or Licensed Capsid alone or in combination with a Payload. The Capsid Patents existing as of the Effective Date are set forth in Schedule 1.18, which exhibit shall be updated annually by Voyager.

1.19 "Cardiology Capsid" means the Capsid identified as [**] protein and having the amino acid sequence set forth on Exhibit A.

1.20 "Change of Control" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock redemption, cancellation, or other recapitalization, and immediately after such acquisition or increase such Third Party is, directly or indirectly, the beneficial owner of voting securities representing more than fifty percent (50%) of the total voting power of all of the then outstanding voting securities of such Party; (b) any merger, consolidation, recapitalization, or reorganization of such Party is consummated that would result in shareholders or equity holders of such Party immediately prior to such transaction owning less than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve any plan of complete liquidation of such Party, or an agreement for the sale or disposition by such Party of all or substantially all of such Party's assets, in each case, through one or more related transactions, other than to an Affiliate or pursuant to one or more related transactions that would result in shareholders or equity holders of such Party immediately prior to such transaction owning more than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; or (d) the sale or transfer to any Third Party, in one or more related transactions, of all or substantially all of such Party's consolidated assets taken as a whole.

1.21 "Clinical Trial" means a human clinical study conducted on sufficient numbers of human subjects that is designed to: (a) establish that a biopharmaceutical product is reasonably safe for continued human testing; (b) investigate the safety and efficacy of the biopharmaceutical product for its intended use, and to define warnings, precautions and adverse reactions that may be associated with the pharmaceutical product in the dosage range to be prescribed; or (c) support Regulatory Approval of a biopharmaceutical product or label expansion of a pharmaceutical product.

1.22 "CNS Capsid" means the Capsid identified as [**] protein and having the amino acid sequence set forth on Exhibit B.

1.23 "CNS/Cardiology Campaign" means completion of at least [**] of Capsid candidates and at least [**] of Capsid candidates in a campaign directed to identification of Capsids useful for Development of AAV Gene Therapy for central nervous system and cardiology indications; excluding any such campaign conducted specifically for a Third Party. For the purposes of this definition, [**].

1.24 "Commercialization" means any and all activities directed to the marketing, promotion, distribution, offering for sale, sale, having sold, importing, having imported, exporting, having exported or other commercialization of a pharmaceutical or biologic product, but excluding activities directed to Manufacturing or Development. "Commercialize", "Commercializing", and "Commercialized" have correlating meanings.

1.25 "Commercially Reasonable Efforts" means, with respect to the efforts to be expended by a Party with respect to any objective, those reasonable, good faith efforts to accomplish such objective as such Party would normally use to accomplish a similar objective under similar circumstances. With respect to any efforts relating to the Development, Regulatory Approval, or Commercialization of a Licensed Product by Pfizer, generally or with respect to any particular country in the Territory, [**]. Further, to the extent that the performance of a Party's obligations hereunder is adversely affected by the other Party's failure to perform its obligations hereunder, the impact of such performance failure will be taken into account in determining whether such Party has used its Commercially Reasonable Efforts to perform any such affected obligations.

1.26 "Confidential Information" has the meaning set forth in Section 7.1.

1.27 "Control" means, with respect to a Person and any Know-How or Patent, the possession by such Person of the right (whether through ownership, license, or otherwise (other than by a license under this Agreement)) to grant the rights and licenses as provided herein, without violating the terms of any agreement with any Third Party. Notwithstanding the foregoing, in the event that a Third Party becomes an Affiliate or assignee of a Party after the Effective Date as a result of a Change of Control of such Party (such Third Party, together with its Affiliates immediately prior to the consummation of such Change of Control, the "Acquiring Entities"), the following will be deemed to be not Controlled by such Party or any of its Affiliates: (a) any Patent, Know-How, Regulatory Filing, or Regulatory Approval owned or otherwise controlled by such Acquiring Entity immediately prior to the consummation of such Change of Control; and (b) any Patent, Know-How, Regulatory Filing, or Regulatory Approval developed by or on behalf of such

Acquiring Entity outside the scope of activities under this Agreement or acquired by or on behalf of such Acquiring Entity after the consummation of such Change of Control.

1.28 "Cover" means with regard to a particular Licensed Product, a Valid Claim in a Patent, that in the absence of a license granted herein, the offer for sale, sale, importation, or method of use of such Licensed Product, would infringe such Valid Claim.

1.29 "Debtor" has the meaning set forth in Section 10.6.1.

1.30 "Defense Proceeding" means an opposition, reexamination request, action for declaratory judgment, nullity action, interference or post-grant proceeding or other attack upon the validity, title or enforceability of a Patent that occurs in the context of litigation; excluding any such proceeding brought as a counterclaim to or defense of, or that accompanies a defense of, any enforcement action under Section 6.3.3.

1.31 "Develop" or "Developing" means to discover, research or otherwise develop a process, compound or product, including conducting non-clinical and clinical research and development activities prior to Regulatory Approval. When used as a noun, "Development" means any and all activities involved in Developing.

1.32 "Development Milestone Event" means any Milestone Event set forth in Section 5.3.1.

1.33 "Development Milestone Event Notice" has the meaning set forth in Section 5.3.1.

1.34 "Development Milestone Payment" has the meaning set forth in Section 5.3.2.

1.35 "Diligence Issue" has the meaning set forth in Section 4.1.2(f).

1.36 "Disclosing Party" has the meaning set forth in Section 7.1.

1.37 "Dollars" or "$" means the legal tender of the U.S.

1.38 "Effective Date" has the meaning set forth in the Preamble.

1.39 "EMA" means the European Medicines Agency, and any successor entity thereto.

1.40 "Evaluate" means evaluation conducted by or on behalf of Pfizer during the Research Term, to assess any Capsid Candidate and to determine its interest in exercising an Option or substitution right for such Capsid Candidate. "Evaluation" and "Evaluating" have correlating meanings.

1.41 "Executive Officers" means: (a) with respect to Voyager, [**], or his or her designee; or (b) with respect to Pfizer, [**], or his or her designee.

1.42 "Existing Confidentiality Agreement" has the meaning set forth in Section 7.1.4.

1.43 "Exploit" means to Develop, Manufacture, Commercialize, or otherwise exploit. "Exploitation" and "Exploiting" have correlating meanings.

1.44 "FDA" means the U.S. Food and Drug Administration, and any successor entity thereto.

1.45 "FD&C Act" means the U.S. Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

1.46 "First Commercial Sale" means, with respect to a Licensed Product, the first sale for end use or consumption of such Licensed Product in such country after all Regulatory Approvals and pricing and reimbursement approvals legally required for such sale have been granted by the applicable Regulatory Authority of such country or, if Regulatory Approval is not required, after the date on which sales are permitted by applicable Law.

1.47 "Functionally Equivalent Variant" means with respect to any Licensed Capsid, any Capsid that Voyager derives from the Licensed Capsid (including any modification thereof) that meets each of the following criteria as compared to the Licensed Capsid: [**].

1.48 "Global Trade Control Laws" has the meaning set forth in Section 11.8.

1.49 "Governmental Authority" means any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

1.50 "HSR Act" has the meaning set forth in Section 2.4.2.

1.51 "IND" means an investigational new drug application (including any amendment or supplement thereto) submitted to the FDA pursuant to U.S. 21 C.F.R. Part 312, including any amendments thereto, or any comparable filing(s) outside the United States for the investigation of any product in any other country or group of countries.

1.52 "Indemnified Party" has the meaning set forth in Section 9.3.

1.53 "Indemnifying Party" has the meaning set forth in Section 9.3.

1.54 "Infringement Notice" has the meaning set forth in Section 6.3.1.

1.55 "Initiation" means, with respect to any Clinical Trial, first dosing of the first human subject in such Clinical Trial.

1.56 "Invention" or "Invented" means the result or act of invention (whether patentable or not) as determined in accordance with U.S. patent laws.

1.57 "Joint Inventions" has the meaning set forth in Section 6.1.3.

1.58 "Joint Patents" means all Patents within the Joint Inventions.

1.59 "Know-How" means all proprietary information, know-how and data, including trade secrets, Inventions (whether patentable or not), discoveries, methods, specifications, processes, procedures, formulas, expertise, technology, data (including non-clinical, pre-clinical and clinical data), documentation, materials, and results (including pharmacological, toxicological, biological, chemical, physical, safety and Manufacturing data and results), analytical and quality control data and results, Manufacturing techniques, Regulatory Filings and other technical information. "Know-How" excludes in any event any Patents.

1.60 "Law" means any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.61 "Licensed Capsid" means: (a)(i) a Capsid Candidate for which Pfizer has exercised the applicable Option in accordance with Section 2.4 and paid the applicable Option Exercise Fee; or (ii) any Substitute Capsid that Pfizer has designated as a replacement therefor in accordance with Section 2.5 and (b) any Functionally Equivalent Variant of the Capsid Candidate or Substitute Capsid described in (a)(i) or (a)(ii) as applicable.

1.62 "Licensed Capsid Patent" means, collectively, any Capsid Patent that Covers any Licensed Capsid, but excluding any Licensed Product Patent.

1.63 "Licensed Field" means all indications for therapeutic, diagnostic and prophylactic human and veterinary use.

1.64 "Licensed Product" means a product comprising both of the following: (a) a Licensed Capsid; and (b) the specific Pfizer Transgene for which Pfizer exercised its Option for such Licensed Capsid, as identified in Pfizer's Option Exercise Notice.

1.65 "Licensed Product Patent" means, collectively, any Patent Controlled by Pfizer at any time during the Term with claims directed to the combination of a Licensed Capsid and a Pfizer Transgene together or any method of use directed to such combination.

1.66 "Litigation Conditions" has the meaning set forth in Section 9.4.

1.67 "Losses" has the meaning set forth in Section 9.1.

1.68 "Major Market Country" means the United Kingdom, France, Germany, Italy, Spain and Japan.

1.69 "Manufacture" means activities directed to manufacturing, processing, packaging, labeling, filling, finishing, assembly, quality assurance, quality control, testing, and release, shipping, or storage of any pharmaceutical or biologic product (or any components or process steps involving any product or any companion diagnostic), placebo, or comparator agent, as the case may be, including process development, qualification, and validation, scale-up, pre-clinical, clinical, and commercial manufacture and analytic development, product characterization, and stability testing. "Manufacturing" has correlating meaning.

1.70 "[**] Transgene" means a [**].

1.71 Net Sales.

1.71.1 "Net Sales" means the gross amount invoiced (or if not invoiced, received) by Pfizer or any of its Affiliates or Sublicensees (other than Third Party distributors) for any Licensed Product sold to Third Parties (other than Sublicensees but including Third Party distributors), after deducting, if not previously deducted, from the amount invoiced, the following, in each case to the extent included in the gross invoice price and in accordance with GAAP:

(a) reasonable trade, quantity and cash discounts and rebates (including wholesaler inventory management fees), chargebacks, and retroactive price reductions or allowances actually allowed or granted from the billed amount;

(b) credits or allowances actually granted upon claims, rejections or returns of such sales of the Licensed Product, including recalls and amounts credited or repaid because of retroactive price reductions specifically identifiable to the Licensed Product;

(c) taxes imposed on the production, sale, import, delivery or use of the Licensed Product (including sales, use, excise or value added taxes but excluding income taxes), duties or other governmental charges (including charges for product testing required for importation) levied on or measured by the billing amount when included in billing, as adjusted for rebates and refunds;

(d) costs incurred for importing (including transportation, freight and insurance, and warehousing in the Territory);

(e) delayed ship order credits, discounts or payments related to the impact of price increases between purchase and shipping dates or retroactive price reductions; and

(f) any invoiced amounts from a prior period which are not collected and are written off by Pfizer or its Affiliates or Sublicensees, including bad debts, to the extent such amounts are deducted in accordance with Pfizer's or its Affiliates' or Sublicensee's standard policies and procedures consistently applied across its products, as applicable.

If non-monetary consideration is received for any Licensed Product, Net Sales will be calculated based on the fair market value of the Licensed Product, as determined by the Parties in good faith. Net Sales shall be determined from Pfizer's, its Affiliates, or Sublicensee's books and records maintained in accordance with GAAP consistently applied.

1.72 "Non-Disclosing Party" has the meaning set forth in Section 7.5.

1.73 "Option" has the meaning set forth in Section 2.4.1.

1.74 "Option Exercise Date" has the meaning set forth in Section 2.4.1.

1.75 "Option Exercise Fee" has the meaning set forth in Section 5.2.

1.76 "Option Exercise Notice" has the meaning set forth in Section 2.4.1.

1.77 "Option Period" has the meaning set forth in Section 2.4.1.

1.78 "Patent" means (a) any patent, patent application or utility models (including any provisional application, priority application, or international applications) in any country or multinational jurisdiction in the Territory (including any converted application, continuation, continuation-in-part, continued prosecution application or divisional of any such application, any reissue, renewal, extension, registration, confirmation, revalidation, restoration, substitution, reexamination, supplementary protection certificate, pediatric exclusivity period or the like of any such patent); (b) any foreign equivalent of any patent or patent application described in clause (a); and (c) all rights of priority in any of the foregoing.

1.79 "Parties" or "Party" has the meaning set forth in the Preamble.

1.80 "Payload" means a DNA sequence that is intended to have a therapeutic effect on a Target when packaged into a Capsid and delivered to the appropriate cells.

1.81 "Person" means any individual, partnership, limited liability company, firm, corporation, association, trust, unincorporated organization or other similar entity or organization.

1.82 "Pfizer" has the meaning set forth in the Preamble.

1.83 "Pfizer Background IP" has the meaning set forth in Section 6.1.1.

1.84 "Pfizer Evaluation Data" has the meaning set forth in Section 2.3.2.

1.85 "Pfizer Quarter" means each of the four (4), three (3) month periods: (a) with respect to the United States, commencing on January 1 of any Pfizer Year; and (b) with respect to any country in the Territory other than the United States, commencing on December 1 of any Pfizer Year.

1.86 "Pfizer Transgene" means: (a) the [**] Transgene; or (b) the [**] Transgene.

1.87 "Pfizer Year" means the twelve-month fiscal periods observed by Pfizer: (a) commencing on January 1 with respect to the United States; and (b) commencing on December 1 with respect to any country in the Territory other than the United States.

1.88 "Phase I Clinical Trial" means a Clinical Trial (or a portion of a human clinical trial) of a product in any country, the principal purpose of which is a preliminary determination of safety in healthy individuals or patients, that would satisfy the requirements of 21 C.F.R. 312.21(a), or a similar clinical study prescribed by the relevant Regulatory Authorities in a country other than the United States.

1.89 "Pivotal Clinical Trial" means a Clinical Trial of a Licensed Product that either (a) would satisfy the requirements of 21 C.F.R. 312.21(c) or corresponding foreign regulations; or (b) is intended (as of the time the Clinical Trial is Initiated) to obtain sufficient data to support the filing of a BLA for such Licensed Product. Pivotal Trial may include (i) a Clinical Trial that is designed to satisfy the requirements of both 21 C.F.R. 312.21(b) and 21 C.F.R. 312.21(c) or corresponding foreign regulations, or (ii) a Clinical Trial that is designed to satisfy the

requirements of 21 C.F.R. 312.21(b) that is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. 312.21(c) or to provide sufficient data to support the filing of a BLA for such Licensed Product, as supported by a Regulatory Authority's formal meeting minutes or comparable documents, in which case such Pivotal Trial shall be deemed to have been Initiated upon the first dosing of the first human subject under the optimized or expanded protocol for such Clinical Trial.

1.90 "Price Approval" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of such reimbursement authorization or pricing approval or determination (as the case may be).

1.91 "[**] Transgene" means a [**].

1.92 "Prosecution and Maintenance" or "Prosecute and Maintain" means, with regard to a Patent: (a) the preparation, filing, prosecution, maintenance, and requests for patent term adjustments or patent term extensions, including terminally disclaiming an application or issued patent of or for such Patent, as well all appeals therefrom; and (b) any proceeding, other than routine *ex parte* prosecution, which challenges such Patent occurring independently of litigation of the Patent, including re-examinations, nullity actions, interferences, oppositions, derivation proceedings, post-grant reviews, reissues, and other similar proceedings with respect to such Patent and any appeals therefrom.

1.93 "rAAV" means a recombinant AAV.

1.94 "Receiving Party" has the meaning set forth in Section 7.1.

1.95 "Redacted Version" has the meaning set forth in Section 7.4.2.

1.96 "Regulatory Approval" means the approval of the applicable Regulatory Authority necessary for the marketing and sale of a product in a country(ies), including any required Price Approval.

1.97 "Regulatory Approval Application" means a Regulatory Filing submitted to an applicable Regulatory Authority to obtain Regulatory Approval to market and sell a particular product in the country or countries that such Regulatory Authority is responsible for, including any amendments thereto and supplemental applications.

1.98 "Regulatory Authority" means the FDA in the United States or any Governmental Authority in another country in the Territory that is a counterpart to the FDA and holds responsibility for granting Regulatory Approval for a product in such country, including the EMA, and any successor(s) thereto.

1.99 "Regulatory Filing" means, with respect to a product, any documentation comprising or relating to or supporting any filing or application with any Regulatory Authority with respect to such product, or its use or potential use in the Field, including any document submitted to any Regulatory Authority, including any IND, any Regulatory Approval Application

and any correspondence with any Regulatory Authority with respect to such product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

1.100 "Relevant Capsid Patents" has the meaning set forth in Section 8.2.2.

1.101 "Relevant Factors" means all relevant factors that may affect the Development, Regulatory Approval or Commercialization of a Licensed Product, including (as applicable): actual and potential issues of safety, efficacy or stability; product profile (including product modality, category and mechanism of action); stage of development or life cycle status; actual and projected Development, Regulatory Approval, Manufacturing, and Commercialization costs; any issues regarding the ability to Manufacture or have Manufactured any Licensed Capsid or Licensed Product; the likelihood of obtaining Regulatory Approvals (including satisfactory or required Price Approvals); the timing of such approvals; the current guidance and requirements for Regulatory Approval for the Licensed Product and similar products and the current and projected regulatory status; labeling or anticipated labeling; the then-current competitive environment and the likely competitive environment at the time of projected entry into the market; past performance of the Licensed Product or similar products; present and future market potential; the ability to obtain adequate supply of any Licensed Capsid or Licensed Product, or any component thereof, from any Third Party as may be required to Develop, secure Regulatory Approval for or Commercialize any Licensed Capsid or Licensed Product; Patent Rights of a Third Party; existing or projected pricing, sales, reimbursement and profitability; pricing or reimbursement changes in relevant countries; proprietary position, strength and duration of patent protection and anticipated exclusivity; and other relevant scientific, technical, operational and commercial factors.

1.102 "Representatives" means: (a) with respect to Pfizer, Pfizer and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents; and (b) with respect to Voyager, Voyager and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents.

1.103 "Research Term" means the period commencing on the Effective Date and ending on the first (1st) anniversary of the Effective Date; provided that if an Option is exercised, the Research Term for the applicable Licensed Capsid for which the Option was exercised will extend until the third (3rd) anniversary of the Effective Date.

1.104 "Residual Knowledge" means knowledge, techniques, experience and Know-How that: (a) are, or are based on any Confidential Information Controlled by the Disclosing Party; and (b) are retained in the unaided memory of any authorized Representative of the Receiving Party after having access to such Confidential Information. An individual's memory will be considered to be unaided if the individual has not intentionally memorized the Confidential Information for the purpose of retaining and subsequently using or disclosing it. In no event, however, will Residual Knowledge include any knowledge, techniques, experience and Know-How to the extent (at any time, for such time) within the scope of any issued, valid, and enforceable patent claim Controlled by the Disclosing Party.

1.105 "Restricted Market" has the meaning set forth in Section 11.8.1.

1.106 "Restricted Party" has the meaning set forth in Section 11.8.2.

1.107 "Royalty Floor" has the meaning set forth in Section 5.5.4.

1.108 "Royalty Term" means, on a country-by-country and Licensed Product-by-Licensed Product basis, the period commencing on the First Commercial Sale of such Licensed Product in such country and terminating upon the latest to occur of: (a) expiration date of the last Valid Claim of a Licensed Capsid Patent Covering Licensed Product in such country; (b) termination or expiration of regulatory or data exclusivity for such Licensed Product in such country; and (c) [**] after the First Commercial Sale of such Licensed Product in such country; provided that in each country where no Valid Claim ever existed, in no event shall the Royalty Term extend beyond [**] from the earliest priority date of the earliest Valid Claim anywhere in the world of the Licensed Capsid Patent Covering such Licensed Product.

1.109 "Sublicense" has the meaning set forth in Section 3.2.

1.110 "Sublicensee" has the meaning set forth in Section 3.2.

1.111 "Substitute Capsid" has the meaning set forth in Section 2.5.

1.112 "Term" has the meaning set forth in Section 10.1.

1.113 "Territory" means worldwide.

1.114 "Third Party" means any Person that is neither a Party nor an Affiliate of a Party.

1.115 "Third Party Claims" has the meaning set forth in Section 9.1.

1.116 "Third Party License" has the meaning set forth in Section 5.5.2.

1.117 "TRACER Capsid Candidate" means any Capsid that is identified through a CNS/Cardiology Campaign and that has not been previously identified by Voyager and disclosed to Pfizer.

1.118 "United States" or "U.S." means the United States of America and all of its territories and possessions.

1.119 "Valid Claim" means, with respect to a particular country and Licensed Product: (a) a claim of an issued and unexpired Licensed Capsid Patent (i) that has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other Governmental Authority of competent jurisdiction from which no appeal can be taken or has not been appealed within the time allowed for appeal and (ii) that has not been irrevocably abandoned, disclaimed, denied, or admitted to be invalid or unenforceable through reissue, re-examination, or disclaimer or otherwise; or (b) a claim of a pending patent application within the Licensed Capsid Patent(s) that has not been cancelled, withdrawn, abandoned or finally rejected by an administrative agency action from which no appeal can be taken, provided that any claim in any patent application pending for more than [**] from the earliest date on which such claim claims priority shall not be considered a Valid Claim for purposes of the Agreement from and after such [**] date.

1.120 "VAT" has the meaning set forth in Section 5.11.2.

1.121 "Voyager" has the meaning set forth in the Preamble.

1.122 "Voyager Background IP" has the meaning set forth in Section 6.1.2.

1.123 "Voyager Know-How" means Know-How that: (a) is Controlled by Voyager or any of its Affiliates as of the Effective Date or that comes into the Control of Voyager or any of its Affiliates during the Term (other than through the grant of a license by Pfizer); (b) is disclosed or is required to be disclosed by or on behalf of Voyager to Pfizer in connection with this Agreement; and (c) relates to any Capsid Candidate or Licensed Capsid or the Exploitation of any Capsid Candidate or Licensed Capsid. "Voyager Know-How" expressly excludes any Know-How relating to Voyager's proprietary SF9 manufacturing technology.

1.124 "Voyager's Knowledge" means the actual knowledge, as of the Effective Date, of Voyager's [**].

ARTICLE 2
RESEARCH AND LICENSE OPTION

2.1 Alliance Managers. Within [**] after the Effective Date, each Party will appoint an individual to act as an alliance manager for such Party (each, an "Alliance Manager"). The Alliance Managers will be the primary point of contact for the Parties under this Agreement, including with regard to Voyager's disclosure of any Capsid Candidates and Pfizer's Evaluation of any Capsid Candidate. The name and contact information for each Party's Alliance Manager, as well as any replacement chosen by such Party, in its sole discretion, from time to time, will be promptly provided to the other Party in writing. Each Party may change its designated Alliance Manager at any time upon written notice to the other Party; provided that each Party will maintain an Alliance Manager throughout the duration of the Term. The Parties may mutually agree in writing to eliminate the requirement to maintain an Alliance Manager at any point following the expiration of the last to expire Research Term.

2.2 Capsid Candidate Supply. Immediately following the Effective Date, Voyager shall provide Pfizer with the quantities of Cardiology Capsid and the CNS Capsid for Pfizer's use in the Evaluation as described in Exhibit C.

2.3 TRACER Capsid Candidate Evaluation.

2.3.1 Campaigns and Disclosure. During the Research Term, Voyager may (but will not be obligated to), at Voyager's sole discretion and expense, conduct CNS/Cardiology Campaigns and identify proprietary Capsids that may be useful for AAV Gene Therapy for the treatment of central nervous system or cardiology disorders. Voyager will disclose to Pfizer, on a rolling basis, the performance characteristics for all TRACER Capsid Candidates arising from the CNS/Cardiology Campaigns conducted during the Research Term.

2.3.2 Evaluation of TRACER Capsid Candidates. During the Research Term, following the disclosure by Voyager to Pfizer of a TRACER Capsid Candidate, Pfizer will have the right, in its sole discretion, to select [**] such TRACER Capsid Candidates for Evaluation by written notice to Voyager, and upon receipt of such written notice, Voyager will promptly provide to Pfizer plasmids for the production of each such TRACER Capsid Candidate for such Evaluation.

Pfizer will promptly provide to Voyager all results of such Evaluation that are generated during the Research Term to the corresponding Capsid Candidate that are related to biodistribution, expression level, and toxicity ("Pfizer Evaluation Data"); provided that Pfizer, in its sole discretion, may choose to redact, mask, or not provide any information related to a Pfizer Transgene or Manufacturing. Voyager will be free to use the corresponding Evaluation data for its own internal research purposes, in support of Voyager's Patent filings, and as part of data packages shared under confidentiality in association with the applicable TRACER Capsid Candidate (without attribution of the source of such data to Pfizer); provided, however, that Voyager shall not include Pfizer Evaluation Data in any Patent filing without Pfizer's prior written consent, which consent shall not be unreasonably withheld. In the event Pfizer does not exercise its Option for a particular Capsid Candidate, Pfizer will not: (a) disclose the data from the corresponding Evaluation of such Capsid Candidate to any Third Party; or (b) include the data from the corresponding Evaluation of such Capsid Candidate in any Patent filing, except in each case of (a) or (b) where such data has become publicly available through no breach of this Agreement or with Voyager's prior written consent. Pfizer may perform such Evaluation at any time during the Research Term.

2.3.3 Reporting. During the Research Term, Voyager shall provide written reports summarizing the TRACER Capsid Candidates disclosed pursuant to Section 2.3.1, and Pfizer will provide a written reports summarizing all results of the Evaluation conducted pursuant to Section 2.3.2 with timing to be mutually agreed by the Parties. The Alliance Managers will coordinate meetings to be held within [**] following receipt of such written reports to discuss the contents of such reports, with each Party providing the appropriate personnel to address any reasonable inquiries of the other Party.

2.4 Option to License Capsid Candidates for Development and Commercialization of Licensed Products.

2.4.1 Voyager hereby grants to Pfizer an option to receive the license as set forth in Section 3.1.2 for up to two (2) Pfizer Transgenes (each, an "Option"). Pfizer may exercise the Option at any time prior to the first (1st) anniversary of the Effective Date (the "Option Period") by providing written notice to Voyager, in accordance with Section 11.7, identifying, for each Pfizer Transgene for which an Option is exercised, the specific Capsid Candidate that will become a Licensed Capsid for such Pfizer Transgene (an "Option Exercise Notice"). Pfizer may exercise each of its Options on the same or different Capsid Candidates for each Pfizer Transgene during the Research Term, but may only exercise one Option per Pfizer Transgene. Upon Voyager's receipt of each Option Exercise Notice (the "Option Exercise Date") each Capsid Candidate identified in the corresponding Option Exercise Notice will be deemed a "Licensed Capsid" for the selected Pfizer Transgene. Promptly following receipt of Pfizer's Option Exercise Notice, Voyager will issue the appropriate invoice in accordance with Section 5.3.3 and provide Pfizer with any Voyager Know-How for the corresponding Licensed Capsid that has not been previously provided to Pfizer as may be reasonably necessary or that the Parties mutually agree may be useful to enable Pfizer to Exploit such Licensed Capsid for use in Licensed Products containing such Pfizer Transgene; provided that Voyager shall not provide Pfizer with any Know-How that is not reasonably necessary for Exploiting a Licensed Capsid without Pfizer's prior written consent. In the event Voyager provides Know-How that is not reasonably necessary for Exploiting a Licensed

Capsid without Pfizer's prior written consent, then Pfizer shall have the right to use such Know-How for all research, development, and regulatory purposes, without accountability to Voyager.

2.4.2 All Option exercise notices delivered by Pfizer shall specify whether the exercise of the applicable Option, in Pfizer's good faith assessment based on advice from specialized counsel, requires filings under the Hart-Scott-Rodino Antitrust Improvement Act (as amended from time to time, the "HSR Act") or similar antitrust or competition laws of other jurisdictions (collectively, the "Antitrust Filings"). If Pfizer concludes that Antitrust Filings are required, then: (a) the Parties will (i) use reasonable efforts to make the requisite filings as promptly as possible, and in the case of filings under the HSR Act in any event no later than [**] after the exercise notice for the applicable Option, and (ii) collaborate with each other in taking appropriate steps to achieve expiration or termination of all applicable waiting periods as promptly as possible; and (b) the effectiveness of the relevant license(s) set forth in Section 3.1.2 shall be conditioned upon expiration or termination of such applicable waiting periods.

2.5 Capsid Substitution. After exercise of any Option but during the Research Term, Pfizer may conduct additional Evaluation of the TRACER Capsid Candidates, and may elect to substitute any TRACER Capsid Candidate for any Licensed Capsid by providing written notice to Voyager, in accordance with Section 11.7, identifying the TRACER Capsid Candidate (the "Substitute Capsid") and the specific Pfizer Transgene for which the Substitute Capsid will replace the previously designated Licensed Capsid. Immediately following Pfizer's exercise of such substitution notice, the Substitute Capsid will replace the previous Licensed Capsid for such Pfizer Transgene, and Voyager will provide Pfizer with any Voyager Know-How for the Substitute Capsid that has not been previously provided to Pfizer as may be reasonably necessary or that the Parties mutually agree may be useful to enable Pfizer to Exploit such Substitute Capsid for use in Licensed Products containing such Pfizer Transgene.

ARTICLE 3
GRANT OF LICENSES

3.1 Licenses to Pfizer.

3.1.1 Research License. Subject to the terms and conditions of this Agreement, with respect to each Capsid Candidate, Voyager hereby grants to Pfizer, and Pfizer hereby accepts, a non-exclusive (subject to Section 8.4.1), non-transferable (except in accordance with Section 11.3), non-sublicensable (except in the case of contractors performing services related to Evaluation for or on behalf of Pfizer), worldwide, royalty-free right and license during the Research Term, under the Capsid Patents and Voyager Know-How, to Develop each Capsid Candidate with a Pfizer Transgene solely for the purpose of performing the Evaluation.

3.1.2 Exclusive Licenses from Voyager to Pfizer.

(a) On a Licensed Capsid-by-Licensed Capsid basis and effective as of the Option Exercise Date for such Licensed Capsid, Voyager hereby grants to Pfizer an exclusive license (exclusive even as to Voyager) under the Licensed Capsid Patents and Voyager's interest in the Joint Patents, to use, have used, Develop, have Developed, Commercialize, and have

Commercialized the applicable Licensed Capsid(s) as incorporated into Licensed Products containing the corresponding Pfizer Transgene in the Territory.

(b) In the event Pfizer assigns any Licensed Product Patent to Voyager in accordance with Section 6.2.3(b), Voyager hereby grants to Pfizer an exclusive (even as to Voyager), fully paid-up, perpetual, non-revocable, royalty and fee free license under such assigned Licensed Product Patent for all purposes, including Exploitation of any Capsid, without any accountability to Voyager.

Notwithstanding anything to the contrary, the exclusive licenses in this Section 3.1.2 are exclusive solely as each relates to the Exploitation of a Licensed Product.

3.1.3 Non-Exclusive License from Voyager to Pfizer. Without limiting any other license granted under this Agreement, on a Licensed Capsid-by-Licensed Capsid basis and effective as of the Option Exercise Date, Voyager hereby grants to Pfizer a non-exclusive right and license, under the Voyager Know-How, to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, and have Commercialized the applicable Licensed Capsid(s) as incorporated into Licensed Products containing the corresponding Pfizer Transgene in the Territory.

3.1.4 Direct Licenses to Affiliates. Pfizer may, from time to time, request that Voyager grant licenses within the scope of the licenses granted in Sections 3.1.1, 3.1.2, and 3.1.3 directly to Affiliates of Pfizer by giving written notice. Upon receipt of any such notice, Voyager will negotiate in good faith and enter into a separate direct license agreement consistent with the terms of this Agreement and within the scope of the licenses granted in Section 3.1.1, 3.1.2, and 3.1.3 with such designated Affiliate of Pfizer. The Parties further agree to make any amendments to this Agreement that are necessary to conform the combined terms of such direct licenses and this Agreement to the terms of this Agreement. All costs of making such direct license agreement(s) and amendments, including Voyager's reasonable attorneys' fees, under this Section 3.1.4 shall be borne by Pfizer.

3.1.5 Right of Reference. Voyager hereby grants to Pfizer a "Right of Reference," as that term is defined in 21 C.F.R. § 314.3(b) (or any analogous Law recognized outside of the United States), to all data Controlled by Voyager or its Affiliates that relates to any Licensed Capsid or Licensed Product solely for purposes of seeking Regulatory Approval for Licensed Products, and Voyager shall provide a signed statement to this effect, if requested by Pfizer, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous Law outside of the United States).

3.2 Pfizer's Sublicensing Rights. Pfizer and its Affiliates will have the right to grant and authorize sublicenses through multiple tiers under the rights granted to it under this Agreement by Voyager, including Section 3.1.2 and Section 3.1.3 for the Exploitation of a Licensed Product (each such Third Party, a "Sublicensee"). Pfizer will use reasonable efforts to include in each Sublicense, an obligation of the Sublicensee to provide Pfizer with written notice of its achievement of a Development Milestone Event within [**] after such Sublicensee achieves the Development Milestone Event. Within [**] following execution of a sublicense with a Sublicensee (a "Sublicense"), Pfizer will provide Voyager with a fully executed copy of the corresponding Sublicense, which copy may be redacted by Pfizer to remove confidential or commercially

sensitive information and any other information that is not necessary to demonstrate compliance with the terms of this Agreement. Each sublicense will be consistent with the terms of this Agreement. During the Term, Pfizer will be responsible for any act or omission by a Sublicensee that would be a breach of this Agreement if such act or omission had been engaged in by Pfizer. Pfizer shall remain responsible for the payment to Voyager of all Development Milestone Payments, Sales Milestone Payments, and royalties that are payable with respect to the Development Milestone Event(s) achieved by, or the Net Sales of, a Licensed Product made by such Sublicensees.

3.3 Voyager Reservation of Rights. Notwithstanding anything to the contrary set forth in this Agreement: (a) the exclusive licenses and exclusivity covenants set forth in this Agreement will not prevent Voyager from internal Development activities relating to the Capsid Candidates or Licensed Capsids, including any Development activities that may result in generation of Functionally Equivalent Variants; (b) nothing in this Agreement will prevent Voyager from Exploiting (or granting rights to an Affiliate or Third Party to Exploit) (i) subject to Section 8.4.1, any Capsid that is not a Licensed Capsid or a Functionally Equivalent Variant for use in connection with the Pfizer Transgenes or (ii) any Licensed Capsid or Functionally Equivalent Variant for use with in products that do not contain a Pfizer Transgene.

3.4 No Other Rights. Except as otherwise expressly provided in this Agreement, under no circumstances will a Party, as a result of this Agreement, obtain any ownership interest, license right or other right in any Know-How, Patent, or other intellectual property rights of the other Party or any of its Affiliates, including items owned, controlled, developed, or acquired by the other Party or any of its Affiliates, or provided by the other Party to the first Party at any time pursuant to this Agreement.

ARTICLE 4
DEVELOPMENT, REGULATORY AND COMMERCIALIZATION ACTIVITIES

4.1 Pfizer Authority and Obligations.

4.1.1 As of each applicable Option Exercise Date, Pfizer will be solely responsible for, and have sole decision-making authority with respect to, at its own expense, the Exploitation of Licensed Products and Licensed Capsids as they are used to Exploit a Licensed Product. During the Term, Voyager will be responsible for maintaining any third-party agreement it has entered as of the Effective Date (if any) that is required for Pfizer to practice the rights granted by Voyager to Pfizer in the Agreement, including payment by Voyager of any amounts due under such third-party agreements.

4.1.2 Diligence.

(a) Development Diligence. Pfizer will use Commercially Reasonable Efforts to Develop and obtain Regulatory Approval for at least one (1) Licensed Product for each Pfizer Transgene for which Pfizer has exercised its Option in the United States and at least one Major Market Country. Pfizer will have no other diligence obligations with respect to the Development or Regulatory Approval of Products under this Agreement.

(b) Commercial Diligence. Pfizer will use its Commercially Reasonable Efforts to Commercialize each Licensed Product in the United States and at least one Major Market Country in the Territory where Pfizer or its designated Affiliates or Sublicensee has received Regulatory Approval for such Licensed Product. Pfizer will have no other diligence obligations with respect to the Commercialization of Products under this Agreement.

(c) Exceptions to Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, Pfizer will be relieved of its diligence obligations under this Agreement with respect to any Licensed Product to the extent that any of the following occurs with respect to such Licensed Product:

(i) Pfizer or Voyager receives, generates, or otherwise becomes aware of, any safety, tolerability, or other data reasonably indicating or signaling that a Licensed Capsid or Licensed Product has or would have an unacceptable risk-benefit profile or is otherwise not reasonably suitable for initiation or continuation of Clinical Trials; or

(ii) Pfizer or Voyager receive any notice, information or correspondence from any applicable Regulatory Authority, or any applicable Regulatory Authority takes any action, that reasonably indicates that a Licensed Product is unlikely to receive Regulatory Approval.

(d) Deemed Satisfaction of Pfizer's Diligence Obligations. Without in any way expanding Pfizer's obligations under this Agreement:

(i) Pfizer's achievement of any Development Milestone Event entitling Voyager to receive a specific Development Milestone Payment described in Section 5.3.2 will be conclusive evidence that Pfizer has satisfied all of its diligence obligations under this Agreement for the corresponding Licensed Product, up to the date that such Development Milestone Event is achieved;

(ii) Pfizer's payment, and Voyager's acceptance, of any royalties to Voyager pursuant to Section 5.4.1 will be conclusive evidence that Pfizer has satisfied all of its diligence obligations under this Agreement for the corresponding Licensed Product to the date of such payment; provided that if Voyager does not return in full a payment of royalties by Pfizer with a written rejection of such payment within [**] of receipt, Voyager shall be deemed to have accepted such royalty payment; and

(iii) Pfizer's payment of any Sales Milestone Payment as set forth in Section 5.3.4 will be conclusive evidence that Pfizer has satisfied all its diligence obligations under this Agreement for the corresponding Licensed Product to the date of such payment.

(e) For the avoidance of doubt, the provisions of Section 4.1.2(d) are intended only as examples of diligence constituting satisfaction of Pfizer's diligence obligations. Pfizer may fully satisfy its diligence obligations without achieving any of the specific diligence examples set forth in Section 4.1.2(d), *provided that* Pfizer otherwise complies with the provisions of Section 4.1.2(a) or Section 4.1.2(b), as applicable.

(f) Assertion of Pfizer Diligence Obligation Claims. If Voyager is, becomes, or reasonably should be aware of facts that might form a reasonable basis to allege that Pfizer has failed to meet any of its diligence obligations, then Voyager will promptly notify Pfizer in writing of such potential alleged performance failure (each such potential alleged performance failure, a "Diligence Issue"). Promptly upon Pfizer's receipt of any notice of a Diligence Issue pursuant to this Section 4.1.2(f), the Pfizer Alliance Manager will contact the Voyager Alliance Manager to discuss the specific nature of such Diligence Issue and seek to identify an appropriate corrective course of action. If, no later than [**] after Pfizer's receipt of such a notice, (i) the Parties have not reached consensus regarding whether Pfizer has failed to satisfy its obligations pursuant to Section 4.1.2(a) or Section 4.1.2(b) and (ii) the Parties' respective Alliance Managers have not agreed upon an appropriate corrective course of action for such Diligence Issue, then such Diligence Issue will be escalated and resolved pursuant to the dispute resolution provisions set forth in Section 11.2. If Voyager fails to notify Pfizer of a Diligence Issue pursuant to this Section 4.1.2(f) within [**] after the date that Voyager first discovers or reasonably should have discovered such Diligence Issue, then Pfizer will be deemed to have satisfied its obligations under Section 4.1.2(a) and Section 4.1.2(b) with respect to such Diligence Issue.

4.1.3 Remedies for Breach of Pfizer Diligence Obligations. If Pfizer materially breaches any of its diligence obligations under Section 4.1.2 and Pfizer fails to remedy such breach within [**] after Pfizer's receipt of notice of such breach from Voyager, then Voyager may, in its sole discretion, elect to either: (i) terminate this Agreement pursuant to the provisions of Section 10.3.1 on a Licensed Product-by-Licensed Product and country-by-country basis; or (ii) convert any exclusive license or sublicense granted to Pfizer under this Agreement into a non-exclusive license, solely in each case of (i) and (ii) with respect to a Licensed Product in the country that is the subject of the material breach.

4.1.4 Reporting. Following the exercise of each Option and prior to the First Commercial Sale of the corresponding Licensed Product in each of the jurisdictions where any of the milestone payments under Section 5.3 remain outstanding, for each Licensed Product, Pfizer will provide to Voyager a confidential [**] written report summarizing the material Development, Manufacture and Commercialization activities it has undertaken in such jurisdiction(s) during the preceding [**] period and the material Development, Manufacture and Commercialization activities it expects to take in the following [**] period, including any milestones expected to be achieved.

4.1.5 Cooperation. Upon Pfizer's request and at Pfizer's expense, Voyager will provide Pfizer with reasonable assistance in connection with Pfizer's preparation of any portion(s) of the relevant Regulatory Filings that relate to the Licensed Products, including by providing relevant data in Voyager's possession and participating in meetings between the Parties to prepare documents to be filed.

4.2 Remedy for Pfizer Deprioritizing a Licensed Capsid. Without limiting Pfizer's obligations under Section 4.1.2, on a Licensed Product-by-Licensed Product basis, if, during the period beginning on the corresponding Option Exercise Date for a Pfizer Transgene and ending on the date Pfizer first Commercializes the corresponding Licensed Product in the United States and one Major Market Country, (i) Pfizer declares a lead candidate incorporating a Pfizer Transgene for Development, and (ii) Pfizer does not include a lead candidate or a back-up candidate

incorporating a Licensed Capsid and a Pfizer Transgene in its Development efforts for any contiguous [**] period, as indicated in Pfizer's [**] report made under Section 4.1.4 and provided that such lack of inclusion is not a result of a matter set forth in Section 4.1.2(c)(i), then, within [**] after receiving such report, Voyager shall notify Pfizer in accordance with Section 11.7 of any objection it has to such lack of inclusion. If Voyager timely notifies Pfizer of its objection and Pfizer does not include a lead candidate or back-up candidate that incorporates the corresponding Licensed Candidate and Pfizer Transgene within [**] after Pfizer's receipt of such notice, then Voyager may, in its sole discretion, immediately elect to convert the corresponding exclusive license or sublicense granted to Pfizer under this Agreement into a non-exclusive license, with (a) all subsequent development obligations under Section 4.1.2 terminating for the corresponding Licensed Product and (b) all amounts for the corresponding Licensed Product that would be due hereunder after Voyager elects such non-exclusive license being reduced by [**] percent ([**]%).

4.3 Compliance. All activities to be conducted by a Party under this Agreement will be conducted in compliance with applicable Laws.

ARTICLE 5
INITIAL FEE; MILESTONES AND ROYALTIES; PAYMENTS

5.1 Upfront Fee. Pfizer will pay Voyager an initial, one-time, non-refundable, non-creditable payment of Thirty Million Dollars ($30,000,000) within [**] after the Effective Date.

5.2 Option Exercise Fees. For each Option that Pfizer exercises, Pfizer will pay to Voyager a fee of Ten Million Dollars ($10,000,000) per Pfizer Transgene (the "Option Exercise Fee") following receipt of an invoice from Voyager in accordance with Section 5.3.3.

5.3 Milestone Payments.

5.3.1 Generally. Pfizer will provide Voyager with written notice (a "Development Milestone Event Notice") of the achievement of a development milestone event specified in Section 5.3.2 for the first Licensed Product, per Pfizer Transgene, to achieve such milestone event (each, a "Development Milestone Event"). Such notice will be provided within [**] after such Development Milestone Event is achieved; provided that in the case such Development Milestone Event is achieved by a Sublicensee, Pfizer's notice shall be provided within [**] after Pfizer receives notice from the corresponding Sublicensee of achieving the Development Milestone Event.

5.3.2 Development Milestone Events and Payments. Notwithstanding anything to the contrary in this Agreement, this Section 5.3.2 shall apply only if Pfizer exercises the corresponding Option. Pfizer will pay Voyager the amounts set forth below within [**] following the first occurrence of each event described below for the first Licensed Product for each Pfizer Transgene to achieve such Development Milestone Event (each, a "Development Milestone Payment"). If for any reason Development Milestone Event (a) below does not occur prior to the occurrence of Development Milestone Event (b) below, then Development Milestone Event (a) will be deemed to occur concurrently with the occurrence of Development Milestone Event (b), and the Development Milestone Payments associated with both Development Milestone Events will be paid following the achievement of Development Milestone Event (b).

	Development Milestone Event	**Development Milestone Payment (per Pfizer Transgene)**
(a)	[**]	[**] Dollars ($[**])
(b)	[**]	[**] Dollars ($[**])
(c)	[**]	[**] Dollars ($[**])
(d)	[**]	[**] Dollars ($[**])
(e)	[**]	[**] Dollars ($[**])
(f)	**Total Per Pfizer Transgene**	One Hundred Fifteen Million ($115,000,000)

Each of the Development Milestone Payments set forth above will be payable one time only per Pfizer Transgene incorporated into a Licensed Product (regardless of the number of Licensed Products with the same Pfizer Transgene, or the number of times with respect to any Licensed Product with the same Pfizer Transgene, achieves the specified Development Event occurs). No Development Milestone Payments will be payable by Pfizer for any subsequent Licensed Product for a Pfizer Transgene regardless of the number of Licensed Products for such Pfizer Transgene are Developed. For clarification, if one Licensed Product replaces another Licensed Product in Development, then such replacement Licensed Product will only be subject to Development Milestone Payments that have not previously been triggered by one or more prior Licensed Products for the corresponding Pfizer Transgene. The maximum amount payable by Pfizer under this Agreement with respect to all Development Milestone Payments if all Development Milestone Events occur will be $230,000,000.

5.3.3 Invoicing and Payment Procedure. All fees owed to Voyager will be payable within [**] after Pfizer's receipt of an invoice from Voyager, including an appropriate Pfizer Purchase Order (PO) number, reference to this Agreement, amount owed and name and address payment is to be sent to. All invoices will be delivered to Pfizer by email to [**] with a copy to [**] and [**]. All invoice or billing related questions should be referred to Pfizer's Accounting Department at [**] or go to the Accounts Payable Inquiry Tool (APIQ) at www.pfizeraccountspayable.com.

5.3.4 Sales Milestones. On a Licensed Product-by-Licensed Product basis, Pfizer will pay to Voyager sales milestones with respect to Annual Net Sales of each Licensed Product for the first occurrence of each milestone event as follows:

	Milestone Event (per Licensed Product)	Sales Milestone Payment
(a)	First Calendar Year with Cumulative Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])
(b)	First Calendar Year with Cumulative Annual Net Sales exceeding of [**] Dollars ($[**])	[**] Dollars ($[**])
(c)	First Calendar Year with Cumulative Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])
(d)	**Total Per Licensed Product**	One Hundred Seventy-Five Million Dollars ($175,000,000)

Pfizer will pay Voyager the foregoing sales milestones within [**] after the end of the Calendar Year in which the first occurrence of each sales milestone occurs for each Licensed Product.

5.4 Royalties.

5.4.1 Royalties on Licensed Products Sold.

(a) Annual Net Sales. Subject to the adjustments under Section 5.5, Pfizer will make tiered royalty payments to Voyager in respect of Annual Net Sales, on a Licensed Product-by-Licensed Product basis, by Pfizer, its Affiliates or Sublicensees at the following royalty rates during the applicable Royalty Term:

	Annual Net Sales of each Licensed Products	Royalty Rate
(a)	Annual Net Sales less than [**] Dollars ($[**])	[**]%
(b)	Annual Net Sales greater than [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**]%
(c)	Annual Net Sales greater than [**] Dollars ($[**])	[**]%

5.4.2 Calculation of Royalties. Royalties on aggregate Annual Net Sales of Licensed Products in a Calendar Year during the Royalty Term will be paid at the rate applicable to the portion of Net Sales within each of the Annual Net Sales tiers during such Calendar Year. For example, if, during a Calendar Year during the Royalty Term, Annual Net Sales of all Licensed Products are equal to $[**], then the royalties payable by Pfizer would be calculated by [**].

5.5 Royalty Adjustments.

5.5.1 Valid Claim Expiration. If, during any Calendar Quarter during the Royalty Term, on a country-by-country and Licensed Product-by-Licensed Product basis, there is no Valid Claim within the Licensed Capsid Patents that Covers such Licensed Product in such country, then the royalty rate for such Licensed Product in such country will be reduced by [**] percent ([**]%) from the applicable rate(s) set forth in Section 5.4.1.

5.5.2 Third Party Licenses. In the event that, during the Royalty Term on a Licensed Product-by-Licensed Product basis, Pfizer, its Affiliates or Sublicensees are required to pay royalties to a Third Party in consideration for a license under Patents Controlled by such Third Party that are reasonably necessary for Exploiting a Licensed Capsid as part of a Licensed Product ("Third Party License "), then Pfizer may deduct [**] percent ([**]%) of the royalties payable to such Third Party for such Third Party License(s) from royalties owed by Pfizer to Voyager under Section 5.4.1 for Net Sales of the applicable Licensed Product(s), with such reduction continuing until all such amounts have been expended for such Calendar Quarter; provided that royalties to a Third Party in consideration for a license under Patents Controlled by a Third Party that are reasonably necessary for Exploiting the Pfizer Transgene or other component of a Licensed Product shall not be deductible against the royalties owed by Pfizer to Voyager hereunder.

5.5.3 Biosimilar Products. For any Pfizer Quarter in the applicable Royalty Term for a Licensed Product in a country in the Territory during which (1) a Biosimilar Product with respect to such Product is being sold in such country; and (2) the unit volume of such Biosimilar Product sold in such country in such Pfizer Quarter exceeds [**] percent ([**]%) of the combined unit volume of such Product and such Biosimilar Product sold in such country in such Pfizer Quarter, subject to Section 5.5.4, the royalties payable on Net Sales of such Product in such country in such Pfizer Quarter would be reduced by [**] percent ([**]%) of the amounts of royalties otherwise payable on such Net Sales pursuant to Section 5.4.1 for the remainder of the applicable Royalty Term, such reduction to be prorated appropriately in aggregate for the then-current Pfizer Quarter. The unit volume of the Licensed Product and Biosimilar Product shall be calculated using a mutually acceptable method and using market share data provided by a reputable and mutually agreed upon provider.

5.5.4 Limit on Deductions.

(a) On a Licensed Product-by-Licensed Product basis, in no event will the cumulative effect of the adjustments in Sections 5.5.1 through Section 5.5.3 reduce the royalties payable to Voyager under Section 5.4.1 by more than [**] percent ([**]%) of the amounts that would otherwise have been payable with respect to the applicable Licensed Product in the applicable country in the applicable Calendar Quarter (the "Royalty Floor"). In the event that a reduction would be permitted under this Section 5.5 but for the fact that such reduction would reduce the applicable royalties payable in accordance with Section 5.4.1 by more than the Royalty Floor. Pfizer may carry over such royalty reduction to payments payable hereunder with respect to any royalty payments owed in any future Calendar Quarter, in each case on a with such reduction continuing until all such amounts have been expended.

5.6 Reports; Payment of Royalty. Within [**] after the end of each Calendar Quarter, Pfizer will deliver to Voyager a report setting forth, for the most recent Pfizer Quarter ending during such Calendar Quarter, the following information, on a Licensed Product-by-Licensed

Product, country-by-country and Territory-wide basis: (a) Net Sales of each Licensed Product; (b) the basis for any adjustments to the royalty payable for the sale of any such Licensed Product; and (c) the royalty due hereunder for the sale of each such Licensed Product. No such reports will be due for any such Licensed Product: (x) before the First Commercial Sale of such Product; or (y) after the Royalty Term for such Licensed Product has expired in all countries in the Territory. The total royalty due for the sale of all such Licensed Products during such Pfizer Quarter will be remitted at the time such report is made; provided that to the extent any royalties are payable by Pfizer hereunder on Net Sales of a Product in a country solely due to a Valid Claim of a Licensed Capsid Patent Covering such Licensed Product in such country that is subject to a revocation, invalidity or unenforceability ruling that is appealable or being appealed, during the time of such appeal or appealability, [**] percent ([**]%) of such royalties payable by Pfizer shall be placed into an escrow account and either (I) returned to Pfizer upon a final, unappealable determination that such revocation, invalidity or unenforceability ruling is upheld in a final, unappealable determination or (II) released to Voyager in the event such revocation, invalidity or unenforceability ruling is not upheld in a final, unappealable determination.

5.7 Accounting; Audit.

5.7.1 Records. Pfizer agrees to keep, and to require its Affiliates and Sublicensees to keep, full, clear and accurate records for a minimum period of [**] after the relevant payment is owed pursuant to this Agreement, setting forth as applicable the sales and other disposition of Licensed Products sold or otherwise disposed of, in sufficient detail to enable royalties and compensation payable to Voyager hereunder to be determined.

5.7.2 Audits. Pfizer agrees, upon not less than [**] prior written notice, to permit, and to require its Affiliates and Sublicensees to permit, such books and records relating to such Licensed Products to be examined during regular business hours at such place or places where such records are customarily kept by an independent internationally-recognized accounting firm selected by Voyager and reasonably acceptable to Pfizer for the purpose of verifying reports provided (or required to be provided) by Pfizer under this Article 5. Any such audit will not be performed more frequently than once in any Calendar Year, and will be conducted under appropriate confidentiality provisions, for the sole purpose of verifying the accuracy and completeness of all financial, accounting and numerical information and calculations provided under this Agreement. The independent accounting firm will only share the results of the audit, not the underlying records, with the auditing party.

5.7.3 Audit Reports and Disputes. The independent accounting firm will provide its audit report and the basis for any determination to Pfizer at the time such report is provided to Voyager before such report is considered to be final. Pfizer will have the right to request a further determination by such accounting firm as to matters which Pfizer disputes within [**] following Pfizer’s receipt of such report. Pfizer will provide Voyager and the accounting firm with a reasonably detailed statement of the grounds upon which it disputes any findings in the audit report and the accounting firm will undertake to complete such further determination, at Pfizer’s expense, within [**] after the dispute notice is provided, which determination will be limited to the disputed matters.

5.7.4 Audit Expenses. Except as provided in Section 5.7.3, any audit conducted by Voyager is to be made at the expense of Voyager, except if the results of the audit reveal an underpayment of royalties, milestones or other payments to Voyager under this Agreement of [**] percent ([**]%) or more in any Calendar Quarter, in which case (a) Pfizer will promptly remit to Voyager the amount of such underpayment and (b) the reasonable fees and expenses for such audit will be paid by Pfizer.

5.8 Currency Conversion. Notwithstanding anything to the contrary in the Agreement, conversion of sales recorded in local currencies to U.S. dollars will be performed in a manner consistent with Pfizer's normal practices used to prepare its audited financial statements for external reporting purposes, provided that such practices use a widely accepted source of published exchange rates.

5.9 Books and Records. Any books and records to be maintained under this Agreement by a Party or its Affiliates or Sublicensees will be maintained in accordance with Accounting Standards.

5.10 Methods of Payments. All payments due from Pfizer to Voyager under this Agreement will be paid in Dollars by wire transfer to a bank in the United States designated in writing by Voyager.

5.11 Taxes.

5.11.1 Each Party will be solely responsible for the payment of all taxes imposed on its share of income arising directly or indirectly from the activities of the Parties under this Agreement.

5.11.2 It is understood and agreed between the Parties that any payments made under this Agreement are exclusive of any value added or similar tax ("VAT"), which shall be added thereon as applicable. Where VAT is properly added to a payment made under this Agreement, the Party making the payment will pay the amount of VAT only on receipt of a valid tax invoice issued in accordance with the laws and regulations of the country in which the VAT is chargeable. In addition, in the event any of the payments made by Pfizer pursuant to this Agreement become subject to withholding taxes under the Laws of any jurisdiction, Pfizer shall deduct and withhold the amount of such taxes for the account of Voyager, to the extent required by Law, such amounts payable to Voyager shall be reduced by the amount of taxes deducted and withheld, and Pfizer shall pay the amounts of such taxes to the proper Governmental Authority in a timely manner and promptly transmit to Voyager an official tax certificate or other evidence of such tax obligations together with proof of payment from the relevant Governmental Authority of all amounts deducted and withheld sufficient to enable Voyager to claim such payment of taxes. Any such withholding taxes required under applicable Law to be paid or withheld shall be an expense of, and borne solely by, Voyager. Pfizer will provide Voyager with reasonable assistance to enable Voyager to recover such taxes as permitted by Law.

5.11.3 Notwithstanding anything in this Agreement to the contrary, (i) if an action (including any assignment or sublicense of its rights or obligations under this Agreement, or any failure to comply with applicable Laws or filing or record retention requirements) by a Party leads

to the imposition of withholding tax liability or VAT on the other Party that would not have been imposed in the absence of such action or in an increase in such liability above the liability that would have been imposed in the absence of such action, then the sum payable by that Party (in respect of which such deduction or withholding is required to be made) shall be increased to the extent necessary to ensure that the other Party receives a sum equal to the sum which it would have received had no such action occurred, (ii) otherwise, the sum payable by that Party (in respect of which such deduction or withholding is required to be made) shall be made to the other Party after deduction of the amount required to be so deducted or withheld, which deducted or withheld amount shall be remitted in accordance with applicable law.

5.12 Late Payments. Any amount required to be paid by a Party hereunder which is not paid on the date due shall bear interest compounded daily, to the extent permitted by law, at the Federal Funds Effective Rate EFFR or any successor to such rate) for the date such payment was due, as reported by the Federal Reserve of New York (https://apps.newyorkfed.org/markets/autorates/fed%20funds).

ARTICLE 6
INTELLECTUAL PROPERTY RIGHTS

6.1 Ownership; Disclosure.

6.1.1 Pfizer Background IP. As between the Parties, Pfizer will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Pfizer and existing as of or before the Effective Date; or (b) Invented, developed, created, generated or acquired solely by or on behalf of Pfizer after the Effective Date((a) and (b), collectively, "Pfizer Background IP").

6.1.2 Voyager Background IP. As between the Parties, Voyager will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Voyager and existing as of or before the Effective Date; or (b) Invented, developed, created, generated or acquired solely by or on behalf of Voyager after the Effective Date ((a) and (b), collectively, "Voyager Background IP").

6.1.3 Joint Inventions. Subject to Section 6.2.3, ownership of any Patents and Know-How that are Invented or otherwise developed jointly by or on behalf of the Parties during Term and in the course of the Parties' activities under this Agreement ("Joint Inventions") will follow inventorship under U.S. patent law.

6.2 Patent Prosecution and Maintenance; Defense Proceedings.

6.2.1 Capsid Patents; Licensed Capsid Patents.

(a) Prior to Pfizer's exercise of an Option, Voyager will have the sole obligation, at its sole cost and expense (except as otherwise provided herein), to Prosecute and Maintain the Capsid Patents and for conducting any Defense Proceeding with respect to the Capsid Patents, and will have sole decision-making authority with respect to matters relating to the Prosecution and Maintenance or the conduct of Defense Proceedings for the Capsid Patents. Voyager will: (i) allow Pfizer a reasonable opportunity and reasonable time to review and provide

comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager before any applicable filings are submitted to any relevant patent office and (ii) reasonably consider any reasonable and timely comments offered by Pfizer in any final filings submitted by Voyager to any relevant patent office; provided that Pfizer will not have any right to review or comment on any Capsid Patent application prior to filing of such application with the relevant patent office. Voyager will not disclose in, or in connection with Prosecution of, any Capsid Patent any of Pfizer's Confidential Information without the prior written consent of Pfizer.

6.2.2 Licensed Capsid Patents. Following Pfizer's exercise of an Option:

(a) Voyager will have the sole right (but not the obligation), at its sole cost and expense (except as otherwise provided herein), (i) to Prosecute and Maintain the Licensed Capsid Patents and (ii) for conducting any Defense Proceeding with respect to the Licensed Capsid Patents, subject to Pfizer's comment rights set forth below. Upon Pfizer's request, Voyager will reasonably consider filing, Prosecuting and Maintaining the Licensed Capsid Rights in any jurisdiction reasonably requested by Pfizer including consideration of an arrangement in which Pfizer pays Voyager for all of its costs, or a pro-rata share of costs as applicable, for such activity. Following Pfizer's exercise of an Option, the Parties will coordinate to develop a patent strategy designed to maximize the value and coverage of the Licensed Capsid Patents for the associated Licensed Products.

(b) Voyager will have sole decision-making authority with respect to matters relating to the Prosecution and Maintenance or the conduct of Defense Proceedings for the Capsid Patent(s) or the Licensed Capsid Patent(s), including any decisions to terminally disclaim a Patent in which Voyager has an interest.

(c) With regard to the Licensed Capsid Patents, Voyager will: (i) allow Pfizer a reasonable opportunity and reasonable time to review and provide comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager before any applicable filings are submitted to any relevant patent office and (ii) give due consideration to any reasonable and timely comments offered by Pfizer in any final filings submitted by Voyager to any relevant patent office. Voyager will not disclose in, or in connection with prosecution of, any Licensed Capsid Patent any of Pfizer's Confidential Information without the prior written consent of Pfizer.

6.2.3 Licensed Product Patents.

(a) As between the Parties, and subject to Section 6.2.2(c)(b), Pfizer will own and Control all right, title, and interest in and to all Licensed Product Patents.

(b) Pfizer shall not file a Licensed Product Patent prior the first publication of any Capsid Patent that first discloses the Capsid that is the subject of the corresponding Licensed Product, without first receiving Voyager's written approval to make such filing. In addition to other provisions that the Parties may agree are appropriate to implement, in the event Pfizer receives Voyager's approval to file and does file a Licensed Product Patent during the Term and prior to the first publication of Voyager's first Licensed Capsid Patent for the Capsid

set forth in such Licensed Product Patent, Pfizer will assign its right, title, and interest in such Licensed Product Patent to Voyager, subject to Pfizer receiving the exclusive license set forth in Section 3.1.2(b); provided that Pfizer will retain the sole right, at its sole cost and expense, (i) to Prosecute and Maintain the Licensed Product Patents in all countries and (ii) for enforcing or defending all assigned Licensed Product Patents.

6.2.4 Joint Patents.

(a) Neither Party will file any Patent application for a Joint Invention without mutual consent. If the Parties decide to seek patent protection for any Joint Invention, the Parties will cooperate in good faith to determine, on a case-by-case basis, which Party will have the responsibility for Prosecuting and Maintaining, and conducting Defense Proceedings relating to any Joint Patents, and how the cost for such activities will be shared.

(b) Cooperation. Each Party will reasonably cooperate with and assist the other Party in connection with the activities of such Party under this Section 6.2.4 upon the reasonable request of the other Party, including by making scientists and scientific records reasonably available and the execution of all such documents and instruments and the performance of such acts as may be reasonably necessary in order to continue any Prosecution and Maintenance or conduct any Defense Proceedings of such Patents.

6.3 Enforcement.

6.3.1 Notice. Each Party will promptly notify the other Party in writing of any knowledge it acquires of any actual or potential infringement by a Third Party (the "Infringement Notice").

6.3.2 Capsid Patents. Unless and until each applicable Option Exercise Date, as between Pfizer and Voyager, Voyager will have the sole right, but not the obligation, to institute litigation or take other steps to remedy infringement in connection with the Capsid Patents in the Territory, and any such litigation or steps will be at Voyager's expense and all recoveries will be retained by Voyager.

6.3.3 Licensed Capsid Patents. Upon the applicable Option Exercise Date, as between Pfizer and Voyager, Voyager will have the first right (but not the obligation), using Commercially Reasonable Efforts, to institute litigation or take other steps to remedy such infringement in connection with the Licensed Capsid Patents in the Territory, and any such litigation or steps will be at Voyager's expense and all recoveries will be retained by Voyager. In the event that (a)(i) Voyager (A) does not institute litigation or take other steps to remedy such infringement in connection with the Licensed Capsid Patent within [**] after the corresponding infringement is first identified, or (B) does not continue its litigation to a final, unappealable decision, or (B) does not remedy the infringement through other means within such [**] period, and (ii) such infringement has (or reasonably threatens to have) a direct and material adverse impact on Pfizer's Commercialization of Licensed Products, then (b) the royalties due to Voyager pursuant to Section 5.4 and payable as of the date of the Infringement Notice shall be reduced by [**] percent ([**]%), but only in the country in which the infringing activity exists with no right of offset with regard to royalties payable for other jurisdictions.

6.3.4 Licensed Product Patents. As between Pfizer and Voyager, Pfizer will have the sole right, but no obligation, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any Licensed Product Patent in the Territory, and any such litigation or steps will be at Pfizer's expense and all recoveries will be retained by Pfizer.

6.3.5 Joint Patents. Immediately after an infringement of a Joint Patent is first identified, the Parties shall meet and cooperate in good faith to determine, on a case-by-case basis, (i) what action, if any, the Parties will take to obtain a discontinuance of such infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any Joint Patent, and (ii) how the costs for and any recoveries from such activities will be shared.

6.4 Infringement Claimed by Third Parties.

6.4.1 Notice. If a Third Party commences, or threatens to commence, any proceeding against a Party alleging infringement of such Third Party's intellectual property by the Exploitation by a Party, its Affiliates, subcontractors or Sublicensees of any Licensed Product, the Party against whom such proceeding is threatened or commenced will give prompt notice to the other Party.

6.4.2 Control of Proceeding. Unless the Party against whom such proceeding is filed seeks indemnification for such claim under Article 9, such Party will control the defense and settlement of any such proceeding described in Section 6.4.1 at its own cost and expense, using counsel of its choice, in its sole discretion. If the Party against whom such proceeding is filed does seek indemnification for such claim, then the provisions of Article 9 will govern the Parties' rights and responsibilities with respect to such claim.

ARTICLE 7
CONFIDENTIALITY

7.1 Confidentiality; Exceptions. Except to the extent expressly authorized by this Agreement, the Parties agree that the receiving Party (the "Receiving Party") will keep confidential and will not publish or otherwise disclose or use for any purpose other than to perform its obligations and exercise its rights as provided for in this Agreement any Know-How or other confidential and proprietary information and materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise) that is disclosed to it by the other Party (the "Disclosing Party"), including trade secrets, Know-How, inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the Disclosing Party's past, present or future marketing, financial, or Exploitation activities of any product or potential product or technology of the Disclosing Party or the pricing thereof (collectively, "Confidential Information"). For clarity, any data, information, or Patent filings provided by one Party to the other Party will constitute the Disclosing Party's Confidential Information. Without limiting the foregoing, the Receiving Party will treat all Confidential Information provided by the Disclosing Party with the same degree of care as the Receiving Party uses for its own similar information, but in no event less than a reasonable degree of care. Notwithstanding the foregoing, "Confidential Information" will exclude information to the extent that it can be established by the Receiving Party that such information:

7.1.1 was in the lawful knowledge or possession of the Receiving Party prior to the time it was first disclosed to the Receiving Party by the Disclosing Party, or was otherwise developed independently by the Receiving Party without reference to any of the Disclosing Party's Confidential Information, as evidenced by written records kept in the ordinary course of business, or other documentary proof knowledge or possession by the Receiving Party;

7.1.2 was generally available to the public or otherwise part of the public domain at the time of its first disclosure to the Receiving Party by the Disclosing Party;

7.1.3 became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party by the Disclosing Party and other than through any act or omission of the Receiving Party in breach of this Agreement or the Existing Confidentiality Agreement; or

7.1.4 was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

Any information disclosed by a Party to the other Party prior to the Effective Date pursuant to the Mutual Confidential Disclosure Agreement between Parties dated [**] (the "Existing Confidentiality Agreement"), that was considered Confidential Information (as defined in the Existing Confidentiality Agreement) will be Confidential Information of such Disclosing Party hereunder, subject to the provisions of Sections 7.1.1, 7.1.2, 7.1.3, and 7.1.4. The existence and terms of this Agreement will be considered the Confidential Information of both Parties. Any reports, Know-How, and other proprietary or sensitive information disclosed or shared by one Party with the other Party pursuant to the activities contemplated by this Agreement will be the Confidential Information of the Party that first shared such report, Know-How or other proprietary or sensitive information with the other Party.

7.2 Authorized Disclosure.

7.2.1 Disclosure to a Party's Representatives. Notwithstanding the foregoing provisions of Section 7.1, the Receiving Party may disclose Confidential Information belonging to the Disclosing Party to the Receiving Party's Representatives who (a) have a need to know such Confidential Information in connection with the performance of the Receiving Party's obligations or the exercise of the Receiving Party's rights under this Agreement and (b) have agreed in writing to non-disclosure and non-use provisions with respect to such Confidential Information that are at least as restrictive as those set forth in this Article 7.

7.2.2 Disclosure to Third Parties. Notwithstanding Section 7.1, each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary:

(a) to Governmental Authorities (i) to the extent desirable to obtain or maintain INDs or Regulatory Approvals for any Licensed Product within the Territory and (ii) in order to respond to inquiries, requests or investigations relating to Licensed Products or this Agreement;

(b) to existing or prospective outside consultants, contractors, advisory boards, investors, collaboration partners, professional advisors, managed care organizations, and non-clinical and clinical investigators, in each case to the extent desirable to develop, register or market any Licensed Product or otherwise as reasonably necessary to perform such Party's obligations under this Agreement; provided that the Receiving Party shall obtain the same confidentiality obligations from such Third Parties as it obtains with respect to its own similar types of confidential information;

(c) in connection with filing or prosecuting Patent rights or trademark rights as permitted by this Agreement;

(d) in connection with prosecuting or defending litigation as permitted by this Agreement;

(e) subject to the provisions of Section 7.5, in connection with or included in scientific presentations and publications relating to Compounds or Products, including abstracts, posters, journal articles and the like, and posting results of and other information about clinical trials to clincialtrials.gov or PhRMA websites;

(f) to a court or arbitrator the extent reasonably necessary in order to enforce its rights under this Agreement;

(g) in communication with existing or prospective investors, lenders, professional advisors, acquirers, merger partners, collaboration partners, subcontractors, Sublicensees, or licensees on a need to know basis, in each case under appropriate confidentiality obligations substantially equivalent to those of this Agreement; or

(h) to the extent mutually agreed to in writing by the Parties.

7.3 Residual Knowledge Exception. Notwithstanding any provision of this Agreement to the contrary, Residual Knowledge shall not be considered Confidential Information for purposes of this Article 7.

7.4 Press Release; Disclosure of Agreement.

7.4.1 Press Releases. On or promptly after the Effective Date, the Parties anticipate issuing a public announcement regarding the signing of this Agreement in a form to be agreed by the Parties. Except as may be expressly permitted under Section 7.4.2, neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party; provided that to the extent information regarding this Agreement has already been publicly disclosed, except as a result of a breach of this Agreement, each Party may subsequently disclose the same information to the public without the consent of the other Party, provided that such information remains true, accurate, and up to date. In addition, nothing in this Agreement shall prevent Pfizer from making any scientific publication or public announcement with respect to any Licensed Product under this Agreement; *provided*, *however*, that, except as permitted under Section 7.2.1, Pfizer shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so.

7.4.2 SEC Filings and other Disclosures of this Agreement. Notwithstanding Section 7.4.1, each Party will be permitted to disclose the existence and terms of this Agreement to the extent required to comply with applicable Laws including the rules or regulations of the U.S. Securities and Exchange Commission, or similar agency in any country other than the United States, or of any stock exchange, including Nasdaq, provided that (a) prior to disclosing this Agreement or any of the terms hereof as permitted under this Section 7.4.2, the Parties will coordinate in advance with each other in connection with the redaction of certain provisions of this Agreement prior to such disclosure (the "Redacted Version"), (b) to the extent permitted by applicable Laws, the Parties will use reasonable efforts to file redacted versions with such agencies and stock exchanges that are consistent with the Redacted Version, and (c) each Party will, at its own expense, use reasonable efforts to seek confidential treatment for such terms as may be reasonably requested by the other Party.

7.5 Publications. On a Capsid Candidate-by-Capsid Candidate basis, prior to exercising its Option for a particular Capsid Candidate, Pfizer will not publish or publicly disclose the scientific results of any of the Evaluation conducted by it for such Capsid Candidate, without the prior written consent of Voyager. Following the Option Exercise Date for a particular Licensed Capsid, nothing in this Agreement shall prevent Pfizer from making any scientific publication or public announcement with respect to any Licensed Product containing such Licensed Capsid; *provided*, *however*, that, except as permitted under Section 7.2, Pfizer shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so. In addition, (i) Voyager shall not publish or make any public announcement regarding a Licensed Product without Pfizer's prior written approval, and (ii) Pfizer shall provide Voyager a copy of each publication or other public disclosure relating to a Licensed Product that contains unpublished information relating to a Licensed Capsid. During the Term, each Party will provide the other Party (the "Non-Disclosing Party") for review and approval any proposed abstract, manuscript, or presentation that contains the Non-Disclosing Party's Confidential Information. Written copies of each proposed publication that are required to be submitted hereunder shall be provided to the Non-Disclosing Party no less than [**] prior to its intended submission for publication or presentation. The Non-Disclosing Party will respond in writing promptly and in no event later than [**] after receipt of the proposed publication or presentation, with one or more of the following: (a) comments on the proposed publication or presentation, which the publishing Party will consider in good faith and use reasonable efforts to incorporate, (b) a specific statement of concern, based upon the need to delay publication if the Non-Disclosing Party determines that the proposed publication or presentation contains or describes intellectual property that needs to be incorporated into a Patent application; provided that such delay shall not exceed an additional [**] unless agreed in writing by the Parties, or (c) an identification of the Non-Disclosing Party's Confidential Information that needs to be removed from the proposed publication or presentation.

7.6 Remedies. Each Party will be entitled to seek, in addition to any other right or remedy it may have, at Law or in equity, a temporary injunction, without the posting of any bond or other security, enjoining or restraining the other Party from any violation or threatened violation of this Article 7.

ARTICLE 8
REPRESENTATIONS AND WARRANTIES

8.1 Representations and Warranties of Both Parties. Each Party hereby represents and warrants to the other Party, as of the Effective Date, that:

8.1.1 such Party is duly organized, validly existing and in good standing under the Laws of the jurisdiction of its incorporation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

8.1.2 such Party has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder and does not require any action or approval by any of its shareholders or other holders of its voting securities or voting interests;

8.1.3 this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against it in accordance with the terms hereof;

8.1.4 the execution, delivery and performance of this Agreement by such Party does not conflict with any agreement or any provision thereof, or any instrument or understanding, oral or written, to which it is a party or by which it is bound, nor violate any law or regulation of any court, governmental body or administrative or other agency having jurisdiction over such Party;

8.1.5 neither such Party nor any of its Affiliates has been debarred or is subject to debarment pursuant to Section 306 of the FD&C Act, as amended, or that is the subject of a conviction described in such section; and

8.1.6 no government authorization, consent, approval, license, exemption of or filing or registration with any court or governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, under any applicable Laws currently in effect, is or will be necessary for, or in connection with, the transaction contemplated by this Agreement or any other agreement or instrument executed in connection herewith, or for the performance by it of its obligations under this Agreement.

8.2 Representations and Warranties, as applicable, of Voyager. Voyager hereby represents, warrants, and covenants to Pfizer, as of the Effective Date that:

8.2.1 Voyager has disclosed to Pfizer all material scientific and technical information and all material information that, to Voyager's Knowledge, are relevant to safety and efficacy with respect to the Capsids;

8.2.2 (a) Schedule 1.18 sets forth a true and complete list of all Capsid Patents as of the Effective Date that Cover the Capsid Candidates (the "Relevant Capsid Patents"), (b) each such Patent remains in full force and effect and (c) Voyager or its Affiliates have timely paid all filing and renewal fees payable with respect to such Patents;

8.2.3 Voyager is the sole and exclusive owner of the Relevant Capsid Patents and Voyager's Know-How, all of which is free and clear of any claims, liens, charges, or encumbrances that would conflict with the rights granted to Pfizer hereunder;

8.2.4 Voyager has and will have the right, power, and authority to grant all rights, title, and interests in the licenses granted or to be granted to Pfizer under this Agreement;

8.2.5 Voyager has not granted any right or license, to any Third Party relating to any of the Relevant Capsid Patents that would conflict with the rights or licenses granted to Pfizer hereunder as of the Effective Date;

8.2.6 no claim, demand, suit, proceeding, arbitration, inquiry, investigation, litigation, or other legal action of any nature, civil, criminal, regulatory or otherwise, is pending, has been brought, or to Voyager's Knowledge, threatened against Voyager or any Affiliate of Voyager, or, to Voyager's Knowledge, any Third Party, alleging that the Exploitation of Voyager's Background IP is infringing or, if practiced or commercialized, will infringe the rights of any Third Party;

8.2.7 there is no judgment or settlement against or owed by Voyager or any of its Affiliates, in each case in connection with the Relevant Capsid Patents or Voyager Know-How relating to the transactions contemplated by this Agreement;

8.2.8 to Voyager's Knowledge, no Third-Party has challenged or threatened to challenge the scope, validity or enforceability of any Relevant Capsid Patents (including, by way of example, through the institution or written threat of institution of interference, nullity or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority;

8.2.9 to Voyager's Knowledge: (a) the Relevant Capsid Patents, are, or, upon issuance, will be, valid and enforceable patents; and (b) as of the Effective Date no Person is infringing or threatening to infringe, or misappropriating or threatening to misappropriate, the Relevant Capsid Patents in a manner that would affect Pfizer's rights under this Agreement;

8.2.10 all of its employees, officers, and consultants have executed (a) valid and enforceable agreements assigning or (b) have existing obligations under applicable Laws requiring assignment to Voyager of all Inventions made during the course of and as the result of their association with Voyager and obligating the individual to maintain as confidential Voyager's Confidential Information as well as confidential information of other Persons (including Pfizer and its Affiliates) which such individual may receive;

8.2.11 Voyager has taken reasonable precautions to preserve the confidentiality of any Know-How that constitutes Voyager's Background IP existing as of the Effective Date and that would be licensed to Pfizer upon exercise of any Option, including requiring each Person having access to any Know-How within such Voyager's Background IP to be subject to confidentiality, non-use and non-disclosure obligations protecting such Know-How as the confidential, proprietary materials and information of Voyager;

8.2.12 to Voyager's Knowledge, Voyager has complied with all applicable Laws, including any disclosure requirements, in connection with the filing, prosecution, and maintenance of the Relevant Capsid Patents;

8.2.13 to Voyager's Knowledge, Voyager has independently developed all Voyager Know-How or otherwise has a valid right to use, and to permit Pfizer, Pfizer's Affiliates, and Pfizer's Sublicensees to use, the Voyager Know-How for all permitted purposes under this Agreement;

8.2.14 no Relevant Capsid Patent is subject to any funding agreement with any government or Governmental Authority;

8.2.15 neither Voyager nor any of its Affiliates are party to or otherwise subject to any agreement or arrangement that would conflict with Pfizer's rights or Voyager's obligations under this Agreement;

8.2.16 [**], to the best of Voyager's Knowledge, the Exploitation by Voyager or Pfizer (or their respective Affiliates or Sublicensees) of any CNS Capsid or Cardiology Capsid does not infringe any claim of an issued patent of any Third Party as of the Effective Date;

8.2.17 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied in all material respects with all applicable Law and accepted pharmaceutical industry business practices with regard to the subject matter of this Agreement, including, to the extent applicable, the FD&C Act (21 U.S.C. § 301, et seq.), the Anti-Kickback Statute (42 U.S.C. § 1320a-7b), Civil Monetary Penalty Statute (42 U.S.C. § 1320a-7a), the False Claims Act (31 U.S.C. § 3729 et seq.), comparable state statutes, the regulations promulgated under all such statutes, and the regulations issued by the FDA, consistent with the 'Compliance Program Guidance for Pharmaceutical Manufacturers' published by the Office of Inspector General, U.S. Department of Health and Human Services;

8.2.18 with respect to any Licensed Capsids, payments, or services provided under this Agreement, Voyager, its Affiliates, and to its Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have not taken and will not during the Term take any action directly or indirectly to offer, promise or pay, or authorize the offer or payment of, any money or anything of value in order to improperly or corruptly seek to influence any government official or any other person in order to gain an improper advantage, and has not accepted, and will not accept in the future such payment; and

8.2.19 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied with the laws and regulations of the countries where it operates, including anti-bribery and anti-corruption laws, including, to the extent applicable, the U.S. Foreign Corrupt Practices Act of 1977 and the U.K. Bribery Act 2010, accounting and record keeping laws, and laws relating to interactions with healthcare professionals or healthcare providers and government officials.

8.3 Mutual Covenants. Each Party hereby covenants to the other Party that, from the Effective Date until expiration or termination of this Agreement:

8.3.1 it will perform its obligations under this Agreement in compliance with applicable Laws;

8.3.2 All individuals who are employees or independent contractors of such Party or any of its Affiliates working under this Agreement will be under the obligation to assign or exclusively license all right, title and interest in and to their Know-How, and all intellectual property rights therein, to such Party or its Affiliate as the sole owner or exclusive licensee thereof;

8.3.3 such Party will not knowingly (a) employ, or use any contractor or consultant that employs or uses, any Person debarred or disqualified by the FDA (or subject to a similar sanction of EMA or any other Governmental Authority) or, (b) employ any Person that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA or any other Governmental Authority), in each of clauses (a) and (b) in the conduct of its activities under this Agreement; and

8.3.4 in performing its obligations or exercising its rights under this Agreement, such Party, its Affiliates, and, with respect to Pfizer, its Sublicensees, will comply with all applicable Law, including all anti-corruption Laws.

8.4 Voyager Covenants. In addition to the covenants made by Voyager elsewhere in this Agreement, Voyager hereby covenants to Pfizer that:

8.4.1 during the Research Term: (a) other than the conduct of the CNS/Cardiology Campaigns, Voyager will not conduct any internal program or program on behalf of a Third Party that is directed to Development or Commercialization of any Capsid Candidates for use in any therapeutic product comprising a Capsid Candidate or a Licensed Capsid in combination with any Pfizer Transgene in the Licensed Field; and (b) Voyager will not grant any Third Party or Affiliate any right or license under Voyager's rights in any Capsid Candidate or Licensed Capsid to Exploit any therapeutic product comprising a Capsid Candidate in combination with any Pfizer Transgene in the Licensed Field;

8.4.2 from and after the applicable Option Exercise Date, during the Term, Voyager shall not, and shall cause its Affiliates not to: (a) license, sell, assign or otherwise transfer to any Person (other than Pfizer or its Affiliates or Sublicensees pursuant to the terms of this Agreement) any Licensed Capsid Patents for use with the corresponding a Pfizer Transgene (or agree to do any of the foregoing) or (b) incur or permit to exist, with respect to any Licensed Capsid Patents, any lien, encumbrance, charge, security interest, mortgage, liability, assignment, grant of license or other binding obligation that is or would be inconsistent with the licenses and other rights granted to Pfizer or its Affiliates under this Agreement;

8.4.3 during the Term, Voyager will: (a) not enter into any agreement with a Third-Party that conflicts with (i) the rights granted to Pfizer under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; (b) not amend or otherwise modify any agreements with a Third-Party or consent or waive rights with respect thereto in any manner that conflicts with (i) the rights granted to Pfizer under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; and

8.4.4 during the Term, Voyager will maintain valid and enforceable agreements with all Persons acting by or on behalf of Voyager or its Affiliates under this Agreement which

require such Persons to assign to Voyager their entire right, title and interest in and to all Licensed Capsid Patents and Voyager's Know-How.

8.5 Representation by Legal Counsel. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

8.6 Disclaimer. Except as otherwise expressly set forth in this Agreement, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, ALL OF WHICH ARE HEREBY SPECIFICALLY EXCLUDED AND DISCLAIMED.

ARTICLE 9
INDEMNIFICATION; INSURANCE

9.1 Indemnification by Pfizer. Pfizer will indemnify, hold harmless and defend Voyager and its Affiliates, and its or their respective directors, officers, employees, agents, consultants and Representatives (each a "Voyager Indemnified Party"), from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys (collectively, "Losses") that the Voyager Indemnified Party may be required to pay to one or more Third Parties to the extent arising out of or resulting from any Third Party suits, claims, actions, proceedings, hearings, investigations, judgments, orders, decrees, stipulations, or injunctions or demands ("Third Party Claims") arising out of or resulting from:

9.1.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Pfizer or any of its Affiliates or Sublicensees, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Pfizer or exercise of Pfizer's rights under this Agreement;

9.1.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Pfizer; or

9.1.3 the Exploitation of any Licensed Product conducted by or on behalf of Pfizer, any of its Affiliates or any Sublicensee hereunder, including: (a) any product liability, personal injury, property damage or other damage; and (b) infringement of any Patent or other intellectual property right of any Third Party;

except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Voyager Indemnified Party, or (y) any Third Party Claim for which Voyager is responsible for indemnifying Pfizer pursuant to Section 9.2.

9.2 Indemnification by Voyager. Voyager will indemnify, hold harmless and defend, Pfizer and its Affiliates, and its or their respective directors, officers, employees, consultants, agents, and Representatives (each a "Pfizer Indemnified Party"), from and against any and all

Losses that the Pfizer Indemnified Party may be required to pay to one or more Third Parties, to the extent arising out of or resulting from any Third Party Claims arising out of or resulting from:

9.2.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Voyager or any of its Affiliates or subcontractors, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Voyager or exercise of Voyager's rights under this Agreement; or

9.2.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Voyager;

except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Pfizer Indemnified Party or (y) any Third Party Claim for which Pfizer is responsible for indemnifying Voyager pursuant to Section 9.1.

9.3 Notice. Each Party will notify the other Party in writing in the event it becomes aware of a claim for which indemnification may be sought hereunder. In the event that any Third Party asserts a Claim or other proceeding (including any governmental investigation) with respect to any Third Party Claim for which a Party (the "Indemnified Party") is entitled to indemnification hereunder, then the Indemnified Party shall promptly notify the Party obligated to indemnify the Indemnified Party (the "Indemnifying Party") thereof; *provided, however,* that no delay on the part of the Indemnified Party in notifying the Indemnifying Party shall relieve the Indemnifying Party from any obligation hereunder unless (and then only to the extent that) the Indemnifying Party is prejudiced thereby.

9.4 Control. Subject to each Party's right to control certain actions described in Sections 6.3 and 6.4 (even where such Party is the Indemnifying Party), the Indemnifying Party shall have the right, exercisable by notice to the Indemnified Party within [**] after receipt of notice from the Indemnified Party of the commencement of or assertion of any Third Party Claim, to assume direction and control of the defense, litigation, settlement, appeal, or other disposition of the Third Party Claim (including the right to settle the claim solely for monetary consideration) with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party; provided that (a) the Indemnifying Party has sufficient financial resources, in the reasonable judgment of the Indemnified Party, to satisfy the amount of any adverse monetary judgment that is sought, (b) the Third Party Claim seeks solely monetary damages, and (c) the Indemnifying Party expressly agrees in writing that as between the Indemnifying Party and the Indemnified Party, the Indemnifying Party shall be solely obligated to satisfy and discharge the Third Party Claim in full (the conditions set forth in clauses (a), (b), and (c) above are collectively referred to as the "Litigation Conditions"). Within [**] after the Indemnifying Party has given notice to the Indemnified Party of its exercise of its right to defend a Third Party Claim, the Indemnified Party shall give notice to the Indemnifying Party of any objection thereto based upon the Litigation Conditions. If the Indemnified Party reasonably so objects, the Indemnified Party shall continue to defend the Third Party Claim, at the expense of the Indemnifying Party, until such time as such objection is withdrawn. If no such notice is given, or if any such objection is withdrawn, the Indemnifying Party shall be entitled, at its sole cost and expense, to assume direction and control of such defense, with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party. During such time as the Indemnifying Party is controlling the defense of such

Third Party Claim, the Indemnified Party shall cooperate, and shall cause its Affiliates and agents to cooperate upon request of the Indemnifying Party, in the defense or prosecution of the Third Party Claim, including by furnishing such records, information, and testimony and attending such conferences, discovery proceedings, hearings, trials, or appeals as may reasonably be requested by the Indemnifying Party. In the event that the Indemnifying Party does not satisfy the Litigation Conditions or does not notify the Indemnified Party of the Indemnifying Party's intent to defend any Third Party Claim within [**] after notice thereof, the Indemnified Party may (without further notice to the Indemnifying Party) undertake the defense thereof with counsel of its choice and at the Indemnifying Party's expense (including reasonable, out-of-pocket attorneys' fees and costs and expenses of enforcement or defense). The Indemnifying Party or the Indemnified Party, as the case may be, shall have the right to join in (including the right to conduct discovery, interview, and examine witnesses and participate in all settlement conferences), but not control, at its own expense, the defense of any Third Party Claim that the other Party is defending as provided in this Agreement.

9.5 Settlement. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party (not to be unreasonably withheld, conditioned or delayed), enter into any compromise or settlement that commits the Indemnified Party to take, or to forbear to take, any action (other than the payment of money which will be fully satisfied by the Indemnifying Party). The Indemnified Party shall have the sole and exclusive right to settle any Third Party Claim, on such terms and conditions as it deems reasonably appropriate, to the extent such Third Party Claim involves equitable or other non-monetary relief, but shall not have the right to settle such Third Party Claim to the extent such Third Party Claim involves monetary damages for which the Indemnifying Party would be responsible without the prior written consent of the Indemnifying Party (not to be unreasonably withheld). Each of the Indemnifying Party and the Indemnified Party shall not make any admission of liability in respect of any Third Party Claim without the prior written consent of the other Party, and the Indemnified Party shall use reasonable efforts to mitigate Liabilities arising from such Third Party Claim.

9.6 Insurance. Each Party agrees to obtain and maintain, during the Term, commercial general liability insurance, including products liability insurance (or clinical trials insurance, if applicable), with minimum "A-" A.M. Best rated insurance carriers to cover its indemnification obligations under Section 9.1 or Section 9.2, as applicable, in each case with limits of not less than $[**] U.S. dollars) per occurrence and in the aggregate. All deductibles and retentions will be the responsibility of the named insured. Pfizer and its Affiliates will be an additional insured on Voyager's commercial general liability and products liability policies (or clinical trials insurance, if applicable), and be provided with a waiver of subrogation. For U.S. exposures, additional insured status on Voyager's commercial general liability and products liability policies shall be via form CG20101185 or its equivalent. Licensed Products liability coverage shall be maintained for [**] following termination of this Agreement. To the extent of its culpability or negligence, all coverages of Voyager will be primary and non-contributing with any similar insurance, carried by Pfizer. Notwithstanding any provision of this Section 9.6 to the contrary, Pfizer may meet its obligations under this Section 9.6 through any combination of insurance and self-insurance. Neither Party's insurance will be construed to create a limit of liability with respect to its indemnification obligations under this Article 9. Each Party will furnish the other Party with a certificate of such insurance promptly following request.

9.7 LIMITATION OF LIABILITY. EXCEPT FOR A BREACH OF ARTICLE 6, ARTICLE 7 OR FOR CLAIMS OF A THIRD PARTY THAT ARE SUBJECT TO INDEMNIFICATION UNDER THIS ARTICLE 9, NEITHER VOYAGER NOR PFIZER, NOR ANY OF THEIR RESPECTIVE AFFILIATES, LICENSORS, LICENSEES OR SUBLICENSEES, WILL BE LIABLE UNDER THIS AGREEMENT TO THE OTHER PARTY, ITS AFFILIATES OR REPRESENTATIVES FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL OR PUNITIVE DAMAGES OR LOST PROFITS OR ROYALTIES, LOST DATA OR COST OF PROCUREMENT OF SUBSTITUTE GOODS OR SERVICES, WHETHER LIABILITY IS ASSERTED IN CONTRACT, TORT (INCLUDING NEGLIGENCE AND STRICT LIABILITY), INDEMNITY OR CONTRIBUTION, AND IRRESPECTIVE OF WHETHER THAT PARTY OR ANY REPRESENTATIVE OF THAT PARTY HAS BEEN ADVISED OF, OR OTHERWISE MIGHT HAVE ANTICIPATED THE POSSIBILITY OF, ANY SUCH LOSS OR DAMAGE. Without limiting the generality of the foregoing, "consequential damages" will be deemed to include, and neither Party will be liable to the other Party or any of such other Party's Representatives or stockholders for any damages based on or measured by loss of projected or speculative future sales of the Licensed Products, any payment due upon any unachieved Development Milestone Event, any Sales Milestone Payment due upon any unachieved total annual Net Sales level, any unearned royalties or any other unearned, speculative or otherwise contingent payments provided for in this Agreement.

ARTICLE 10
TERM AND TERMINATION

10.1 Term. This Agreement will commence as of the Effective Date and, unless terminated earlier, this Agreement will continue in full force and effect until (a) if no Option is exercised, the first (1st) anniversary of the Effective Date, or (b) if at least one Option is exercised, with respect to any Licensed Product(s) for which the Option is exercised, on a country-by-country basis, the expiration of the last to expire Royalty Term with respect to such Licensed Product in such country in the Territory (the "Term"). Upon expiration of the Royalty Term for any Licensed Product in any country, the licenses granted with respect to the applicable Licensed Product in such country will become fully paid-up and irrevocable.

10.2 Automatic Termination Upon End of Option Exercise Period**.** If Pfizer does not exercise an Option by delivering the Option Exercise Notice to Voyager pursuant to Section 2.4.1 and pay the Option Exercise Fee(s) as set forth in Section 5.2, then, effective automatically upon the later of (a) the first day following the Option Exercise Period and the (b) the [**] following the due date of the Option Exercise Fee, and without further notice on the part of either Party, this Agreement will automatically terminate with regard to the corresponding Capsid Candidate(s).

10.3 Termination for Breach.

10.3.1 Subject to Section 4.1.3, 4.2, and the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), this Agreement may be terminated by Voyager (a) on a Licensed Product-by-Licensed Product basis, if Pfizer is in material breach of its obligations under this Agreement with respect to such Licensed Product, by providing written notice that includes the particulars of the alleged material breach, or (b) in its entirety, if Pfizer is in material breach of is obligations under this Agreement with respect to all Licensed Products, by

providing written notice that includes the particulars of the alleged material breach, and in either case ((a) or (b)), the material breach remains uncured for [**] in the case of nonpayment), measured from the date written notice of such material breach is given to Pfizer; provided, however, that if any breach is not reasonably curable within [**] in the case of a nonpayment) and if Pfizer is making a bona fide effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Pfizer a reasonable period of time to cure such breach. If the alleged material breach relates to non-payment of any amount due under this Agreement other than the upfront fee payable under Section 5.1 and Option Exercise Fees, the cure period shall be tolled pending resolution of any bona fide, good faith dispute between the Parties as to whether such payment is due.

10.3.2 Subject to the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), Pfizer may terminate this Agreement for cause with respect to one or more Licensed Products in one or more countries in the Territory or may terminate this Agreement in its entirety, at any time during the Term, by giving written notice to Voyager in the event that Voyager commits a material breach of its obligations under this Agreement and such material breach remains uncured for [**], measured from the date written notice of such material breach is given to Voyager; *provided*, *however*, that if any breach is not reasonably curable within [**] and if Voyager is making a bona fide effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Voyager a reasonable period of time to cure such breach.

10.3.3 Notwithstanding anything to the contrary in this Agreement and subject to the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), Pfizer may terminate this Agreement in whole or relevant part, immediately and without regard to any cure period, if, in Pfizer's reasonable opinion, a violation of Global Trade Control Laws has occurred. Any such termination will be deemed for cause under this Section 10.3.3, under which Pfizer will not be responsible for any related payments due, even if activities have already occurred. Voyager will be responsible for reimbursing Pfizer for any payments due to Pfizer under this Agreement that are blocked due to violation of Global Trade Control Laws

10.4 Termination for Compliance with the Law-related Breach. Subject to the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), Pfizer may terminate this Agreement if Voyager breaches any of the representations or warranties set forth in Sections 8.2.17 through 8.2.19 or if Pfizer learns that improper payments are being or have been made to government officials by Voyager with respect to services performed in connection with this Agreement. Further, in the event of such termination, Voyager shall not be entitled to any further payment, regardless of any activities undertaken or agreements with additional Third Parties entered into prior to termination, and Voyager shall be liable for damages or remedies as provided by law.

10.5 Termination for Convenience. Pfizer may terminate this Agreement in its entirety or on a Licensed Product-by-Licensed Product and country-by-country basis for any or no reason upon ninety (90) days' written notice to Voyager.

10.6 Provisions for Insolvency.

10.6.1 Termination Right. Voyager will be deemed a "Debtor" under this Agreement if, at any time during the Term (a) a case is commenced by or against Voyager under the Bankruptcy Code, (b) Voyager files for or is subject to the institution of bankruptcy, reorganization, liquidation or receivership proceedings (other than a case under the Bankruptcy Code), (c) Voyager assigns all or a substantial portion of its assets for the benefit of creditors, (d) a receiver or custodian is appointed for Voyager's business or (e) a substantial portion of Voyager's business is subject to attachment or similar process; *provided, however*, that in the case of any involuntary case under the Bankruptcy Code, Voyager will not be deemed a Debtor if the case is dismissed within [**] after the commencement thereof. If Voyager is deemed a Debtor, then Pfizer may terminate this Agreement by providing written notice to Voyager. If Pfizer terminates this Agreement pursuant to this Section 10.6.1, then, in addition to all other rights that it may have at law, Pfizer will have the right to offset, against any payment owing to Voyager hereunder, any damages found or agreed by the Parties to be owed by Voyager to Pfizer.

10.6.2 Rights to Intellectual Property. All rights and licenses now or hereafter granted by Voyager to Pfizer under or pursuant to any Section of this Agreement, including Article 2 and Article 3 hereof, are rights to "intellectual property" (as defined in the Bankruptcy Code). The Parties acknowledge and agree that the payments provided for under Sections 5.1, 5.2, and 5.3 and all other payments by Pfizer to Voyager hereunder, other than royalty payments pursuant to Section 5.4, do not constitute royalties within the meaning of Section 365(n) of the Bankruptcy Code or relate to licenses of intellectual property hereunder. If: (a) (i) a case under the Bankruptcy Code is commenced by or against Voyager, (ii) this Agreement is rejected as provided in the Bankruptcy Code and (iii) Pfizer elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code; then (b) Voyager (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) will provide to Pfizer all intellectual property licensed hereunder, and agrees to grant and hereby grants to Pfizer and its Affiliates a right to access and to obtain possession of and to benefit from and, in the case of any chemical or biological material or other tangible item of which there is a fixed or limited quantity, to obtain a pro rata portion of, any "embodiments" of intellectual property pursuant to Section 365(n) of the Bankruptcy Code (which will be deemed to include the Licensed Know-How), and all other embodiments of the intellectual property licensed hereunder. Voyager will not interfere with the exercise by Pfizer or its Affiliates of rights and licenses to intellectual property licensed hereunder and embodiments thereof in accordance with this Agreement and agrees to use Commercially Reasonable Efforts to assist Pfizer and its Affiliates to obtain such intellectual property and embodiments thereof in the possession or control of Third Parties as reasonably necessary or desirable for Pfizer or its Affiliates or Sublicensees to exercise such rights and licenses in accordance with this Agreement.

10.6.3 No Limitation of Rights. All rights, powers and remedies of Pfizer provided in this Section 10.6 are in addition to and not in substitution for any and all other rights, powers and remedies now or hereafter existing at Law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code involving Voyager.

10.7 Effects of Termination.

10.7.1 Effect of Termination.

(a) Automatic Termination Upon End of Option Period or Termination Prior to Option Exercise Date. On a Capsid Candidate-by-Capsid Candidate basis, in the event this Agreement is terminated pursuant to Section 10.2 or pursuant to any other provision of this Agreement prior to the Option Exercise Date, the following will apply upon the effective date of termination:

(i) Except as otherwise expressly provided herein, all rights and obligations of each Party hereunder will cease with regard to the corresponding Capsid Candidate or in its entirety if the Agreement is terminated for all Capsid Candidate, including, for the avoidance of doubt, all rights under the Option, all rights to conduct the Evaluation, any and all rights and licenses and sublicenses granted by either Party to the other Party hereunder.

(ii) The Parties shall discuss and determine, based on mutual consent, whether to seek or continue to seek patent protection with respect to any data generated from the Evaluation and, if applicable, each Party's rights and obligations with respect to such activities. If the Parties cannot reach mutual written agreement on the course of action to take with respect to the filing, prosecution or maintenance such Patent, neither Party will have any responsibility to file, prosecute or maintain such Patent or share in the costs thereof.

(b) Termination for Cause by Voyager; Termination for Convenience by Pfizer After the Option Exercise Date. On a Licensed Product-by-Licensed Product basis, in the event that, following the Option Exercise Date, Voyager terminates this Agreement for cause pursuant to Section 10.3.1 or Pfizer terminates this Agreement for convenience pursuant to Section 10.5, except as otherwise expressly provided herein, all rights and obligations of each Party hereunder that correspond with such termination will cease (including all rights and licenses and sublicenses granted by either Party to the other Party hereunder corresponding to the terminated Licensed Product(s)); provided that Pfizer will have the right, in its sole discretion, to sell any inventory of any Licensed Product affected by such termination that remains on hand as of the effective date of the termination, so long as Pfizer pays to Voyager the royalties and other amounts payable hereunder (including milestones) that are applicable to such subsequent sales in the applicable Territory in accordance with the terms of this Agreement.

(c) Termination for Cause by Pfizer.

(i) Partial Termination. In the event that, on or following the Option Exercise Date, (a) Pfizer terminates this Agreement pursuant to Section 10.3.2 or 10.3.3 with respect to a Licensed Product in any country in the Territory, and the event that gave rise to the right of termination materially impairs the ability to Exploit the applicable Licensed Product in the applicable terminated country, then (b) all licenses granted to Pfizer under this Agreement with respect to such Licensed Product in such country will become irrevocable and perpetual, and Pfizer will have no further obligations to Voyager under this Agreement with respect to such Licensed Product in such country, other than (i) those obligations that expressly survive termination in accordance with Section 10.7.3, (ii) an obligation to pay all milestones and royalties under Sections 5.3 and 5.4 with respect to such Licensed Products in such terminated country in an amount equal to [**] percent ([**]%) of the amount that would otherwise have been payable under this Agreement, such amount to be paid in accordance with and subject to the other terms of this Agreement governing such payments, and (iii) in addition to all other rights that it may have

at law, Pfizer will have the right to offset, against any payment owing to Voyager hereunder, any damages awarded to Pfizer in a proceeding under Section 11.3 or agreed by the Parties to be owed by Voyager to Pfizer. The foregoing will not be construed to limit Voyager's right to receive the full amount of any payments that accrued before the effective date of such termination.

(ii) Complete Termination. In the event that, on or following the Option Exercise Date, (a) Pfizer terminates this Agreement in its entirety pursuant to Section 10.3.2 or 10.3.3, and the event that gave rise to the right of termination materially impairs the ability to Exploit the Licensed Products in the United States, then (b) all licenses granted to Pfizer under this Agreement with respect to all Licensed Products will become irrevocable and perpetual, and Pfizer will have no further obligations to Voyager under this Agreement with respect to such Licensed Products, other than (i) those obligations that expressly survive termination in accordance with Section 10.7.3, (ii) an obligation to pay all milestones and royalties under Sections 5.3 and 5.4 with respect to such Licensed Products in an amount equal to [**] percent ([**]%) of the amount that would otherwise have been payable under this Agreement, such amount to be paid in accordance with and subject to the other terms of this Agreement governing such payments, and (iii) in addition to all other rights that it may have at law, Pfizer will have the right to offset, against any payment owing to Voyager hereunder, any damages awarded to Pfizer in a proceeding under Section 11.3 or agreed by the Parties to be owed by Voyager to Pfizer. The foregoing will not be construed to limit Voyager's right to receive the full amount of any payments that accrued before the effective date of such termination.

10.7.2 Accrued Rights. Termination or expiration of this Agreement for any reason will be without prejudice to any payments that accrued before the effective date of such termination or expiration or rights that will have accrued to the benefit of any Party prior to such termination or expiration, and any and all damages or remedies arising from any breach hereunder. Such termination or expiration will not relieve any Party from obligations which are expressly indicated to survive expiration or termination of this Agreement.

10.7.3 Survival. The provisions of Article 1 (for purposes of interpreting any other surviving provision of this Agreement), Article 5 (with respect to payment obligations accruing prior to expiration or termination and for purposes of calculation and payment of any payment obligations that survive termination under Section 10.7.1(c)), Section 6.1, Section 6.2.4, Section 7.1 through Section 7.4, Section 7.6, Section 9.1 through 9.5, Section 9.7, Section 10.6 (solely in the event the termination is triggered pursuant to Section 10.6.1(a)), Section 10.7, and Article 11, together with any sections that expressly survive (including any perpetual licenses and sublicenses granted hereunder) and remedies for breach of this Agreement, will survive the termination of this Agreement in its entirety or expiration of this Agreement for any reason, in accordance with their respective terms and conditions, and for the duration stated, and where no duration is stated, will survive indefinitely.

ARTICLE 11
MISCELLANEOUS

11.1 Governing Law. This Agreement and any dispute arising from the performance or breach hereof will be governed by and construed and enforced in accordance with the Laws of the state of Delaware without reference to conflicts of laws principles; provided that with respect to

matters involving the enforcement of intellectual property rights, the Laws of the applicable country will apply. The provisions of the United Nations Convention on Contracts for the International Sale of Goods will not apply to this Agreement or any subject matter hereof.

11.2 Dispute Resolution. If a dispute between the Parties arises under this Agreement, either Party will have the right to refer such dispute in writing to the respective Executive Officers, and such Executive Officers will attempt in good faith to resolve such dispute. If the Parties are unable to resolve a given dispute pursuant to this Section 11.2 within [**] after referring such dispute to the Executive Officers, either Party may have the given dispute settled by binding arbitration pursuant to Section 11.3 (subject to the exceptions specified therein).

11.3 Arbitration Request. If a Party intends to begin an arbitration to resolve a dispute arising under this Agreement, such Party will provide written notice (the "Arbitration Request") to the other Party of such intention and a statement of the issues for resolution. From the date of the Arbitration Request and until such time as the dispute has become finally settled, the running of the time periods as to which Party must cure a breach of this Agreement becomes suspended as to any breach that is the subject matter of the dispute.

11.3.1 Additional Issues. Within [**] after the receipt of the Arbitration Request, the other Party may, by written notice, add additional issues for resolution in a statement of counter-issues.

11.3.2 No Arbitration of Patent Issues. Notwithstanding anything to the contrary in this Agreement, any disputes, claims or controversies arising out of, or for which resolution depends in whole or in part on a determination of the ownership, inventorship, interpretation, validity, enforceability, or infringement of United States Patent rights will not be subject to arbitration under this Agreement, but instead may be brought by either Party in the United States District Court for the District of Delaware, before the United States Patent & Trademark Office, before United States appellate courts as applicable.

11.3.3 Arbitration Procedure. Any arbitration pursuant to this Section 11.3 will be held in Boston, Massachusetts unless another location is mutually agreed by the Parties. The arbitration will be governed under the rules of the International Chamber of Commerce, to the exclusion of any inconsistent state Law. The Parties will mutually agree on the rules to govern discovery and the rules of evidence for the arbitration within [**] after the Arbitration Request. If the Parties fail to timely agree to such rules, the United States Federal Rules of Civil Procedure will govern discovery and the United States Federal Rules of Evidence will govern evidence for the arbitration. The arbitration will be conducted by three (3) arbitrators, of which each Party will appoint one, and the arbitrators so appointed will select the third and final arbitrator. The arbitrators will have experience of biotechnology and therapeutics licensing disputes. The arbitrators may proceed to an award, notwithstanding the failure of either Party to participate in the proceedings. The arbitrators will, within [**] after the conclusion of the arbitration hearing, issue a written award and statement of decision describing the essential findings and conclusions on which the award is based, including the calculation of any damages awarded. The arbitrators will be limited in the scope of their authority to resolving only the specific matters which the Parties have referred to arbitration for resolution and will not have authority to render any decision or award on any other issues. Subject to Section 9.7, the arbitrators will be authorized to award compensatory

damages, but will not be authorized to award punitive, special, consequential, or any other similar form of damages, or to reform, modify, or materially change this Agreement. The arbitrators also will be authorized to grant any temporary, preliminary or permanent equitable remedy or relief the arbitrators deem just and equitable and within the scope of this Agreement, including an injunction or order for specific performance. The award of the arbitrators will be the sole and exclusive remedy of the Parties, except for those remedies that are set forth in this Agreement or which apply to a Party by operation of the applicable provisions of this Agreement, and the Parties hereby expressly agree to waive the right to appeal from the decisions of the arbitrators, and there will be no appeal to any court or other authority (government or private) from the decision of the arbitrators. Judgment on the award rendered by the arbitrator may be enforced in any court having competent jurisdiction thereof, subject only to revocation of the award on grounds set forth in the United Nations Convention on the Recognition and Enforcement of Foreign Arbitral Awards.

11.3.4 Arbitration Expenses. Each Party will bear its own attorneys' fees, costs, and disbursements arising out of the arbitration, and will pay an equal share of the fees and costs of the arbitrator; provided, however, that the arbitrators, in their award, will be authorized to determine whether a Party is the prevailing Party, and if so, to award to that prevailing Party reimbursement for its reasonable attorneys' fees, costs and disbursements (including, for example, expert witness fees and expenses, transcripts, photocopy charges and travel expenses).

11.3.5 Preliminary Injunctions. Notwithstanding anything in this Agreement to the contrary, a Party may seek any remedy at law or in equity, including the issuance of a temporary restraining order or a preliminary, temporary, or permanent injunction from any court of competent jurisdiction in order to preserve or enforce its rights under this Agreement or to prevent immediate and irreparable injury, loss, or damage on a provisional basis, pending the award of the arbitrator on the ultimate merits of any dispute.

11.3.6 Confidentiality. All proceedings and decisions of the arbitrator will be deemed Confidential Information of each of the Parties and will be subject to Article 7. For clarity, no information concerning an arbitration, beyond the names of the Parties and the relief requested, may be unilaterally disclosed to a third party by any party unless required by law. In addition, any documentary or other evidence given by a Party or witness in the arbitration shall be treated as Confidential Information by any Party whose access to such evidence arises exclusively as a result of its participation in the arbitration, and shall not be disclosed to any Third Party (other than a witness or expert), except as may be required by applicable Law.

11.4 Assignment. This Agreement may not be assigned or otherwise transferred, nor may any right, interest, or obligation hereunder be assigned or transferred, by either Party without the written consent of the other Party; provided, however, that either Party may assign this Agreement or its rights and obligations under this Agreement to (a) a Third Party that acquires all or substantially all of the business or assets of such Party to which this Agreement relates (whether by merger, reorganization, acquisition, sale or otherwise), and agrees in writing to be bound by the terms of this Agreement, or (b) an Affiliate; provided that in each case of (a) and (b) the assignee will expressly agree to be bound by such Party's obligations under this Agreement and that such Party will remain liable for all of its rights and obligations under this Agreement. Any purported assignment in violation of this Section 11.3 will be void. All terms of this Agreement will remain in full force and effect in the event of a Change of Control of either Party and will be binding upon

any Acquiring Entity of either Party. In addition, Pfizer may assign its rights and obligations under this Agreement to a Third Party where Pfizer or its Affiliate is required or makes a good-faith determination based on advice of legal counsel, to divest a Product in order to comply with Law or the order of any Governmental Authority as a result of a merger or acquisition, provided that the assignee will expressly agree to be bound by Pfizer's obligations under this Agreement. Each Party will promptly notify the other Party of any assignment or transfer under the provisions of this Section 11.4.

11.5 Performance by Affiliates and Sublicensees. Each Party may perform some or all of its obligations or exercise some or all of its rights under this Agreement through Affiliates; provided that each Party hereby acknowledges and agrees that it will be responsible for the full and timely performance and observance of all the covenants, terms, conditions and agreements set forth in this Agreement by its Affiliate(s) and Sublicensees.

11.6 Force Majeure. No Party will be held liable or responsible to the other Party nor be deemed to be in default under, or in breach of any provision of, this Agreement for failure or delay in fulfilling or performing any obligation (other than a payment obligation) of this Agreement when such failure or delay is due to force majeure, whether or not foreseeable as of the Effective Date or thereafter. For purposes of this Agreement, force majeure is defined as any cause beyond the control of the affected Party and without the fault or negligence of such Party, which may include acts of God; material changes in Law; war; civil commotion; destruction of production facilities or materials by fire, flood, earthquake, explosion or storm; labor disturbances; epidemics, pandemics, and the spread of infectious diseases, including COVID-19 (as defined by the World Health Organization and any of the strains, variants, or mutations thereof); quarantines; and failure of public utilities or common carriers. In such event the Party affected by such force majeure will immediately notify the other Party of such inability and a good faith estimate of the period for which such inability is expected to continue based on currently available information. The Party giving such notice will thereupon be excused from such of its obligations under this Agreement as it is thereby disabled from performing for so long as the condition constituting force majeure continues and the non-performing Party takes Commercially Reasonable Efforts to remove the condition, after which time the Parties will promptly meet to discuss in good faith how to best proceed in a manner consistent with this Agreement. To the extent possible, each Party will use reasonable efforts to minimize the duration of any force majeure.

11.7 Notices. Any notice or request required or permitted to be given under or in connection with this Agreement will be deemed to have been sufficiently given if in writing and personally delivered or sent by certified mail (return receipt requested), or overnight express courier service (signature required), prepaid, to the Party for which such notice is intended, at the address set forth for such Party below:

If to Voyager,

addressed to: Voyager Therapeutics, Inc.
75 Sidney Street, Cambridge, MA 02139
Attention: Chief Executive Officer
Telephone: 857-259-5340

with a copy to (which will not constitute notice):

Voyager Therapeutics, Inc.
75 Sidney Street, Cambridge, MA 02139
Attention: General Counsel
Telephone: 857-259-5340

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Brian A. Johnson, Esq.
Telephone: 617-526-6706
Email: brian.johnson@wilmerhale.com

and an email copy to (which will not constitute notice): Voyager's Alliance Manager, to the contact information provided in accordance with Section 2.1

If to Pfizer,

addressed to: Pfizer Inc.
WRD Business Development
235 East 42nd Street
New York, NY 10017
ATTN: WRDM BD Contract Notice
And an email copy to: [**]

with a copy to (which will not constitute notice):

Pfizer Inc.
Pfizer Legal Division
235 East 42nd Street
New York, NY 10017
ATTN: Chief Counsel, R&D

and an email copy to (which will not constitute notice): Pfizer's Alliance Manager, to the contact information provided in accordance with Section 2.1

Copies of notices may be provided to Pfizer by email but must be supplemented by one of the following methods: (a) personal delivery, (b) first class certified mail with return receipt requested, or (c) next-day delivery by major international courier, with confirmation of delivery. Electronic copies may be sent via email to [**] or to such other address for such Party as it will have specified by like notice to the other Party, provided that notices of a change of address will be effective only upon receipt thereof. If delivered personally, the date of delivery will be deemed to be the date on

which such notice or request was given. If sent by overnight express courier service, the date of delivery will be deemed to be the next Business Day after such notice or request was deposited with such service. If sent by certified mail, the date of delivery will be deemed to be the third (3rd) Business Day after such notice or request was deposited with the U.S. Postal Service.

11.8 Global Trade Control Laws. The Parties acknowledge that certain activities covered by or performed under this Agreement may be subject to laws, regulations, or orders regarding economic sanctions, import controls, or export controls ("Global Trade Control Laws"). Each of the Parties will perform all activities under this Agreement in compliance with all applicable Global Trade Control Laws. Furthermore, with respect to the activities performed under this Agreement, each of the Parties represents, warrants, and covenants that:

11.8.1 It will not, for activities under this Agreement, (i) engage in any such activities in a Restricted Market; (ii) involve individuals ordinarily resident in a Restricted Market; or (iii) include companies, organizations or Governmental Entities from or located in a Restricted Market. "Restricted Market" for purposes of this Agreement means the Crimean Peninsula, Cuba, the Donbass Region, Iran, North Korea, Sudan, and Syria, or any other country or region sanctioned by the United States or European Union.

11.8.2 It is not a Restricted Party and is not owned or controlled by a Restricted Party. With respect to activities performed under this Agreement, neither Party will engage or delegate to any Restricted Party for any activities under this Agreement. Each Party will screen all relevant third parties involved by such Party in the activities under this Agreement under the relevant Restricted Party Lists. "Restricted Party" for purposes of this Agreement means any individual or entity on any of the following "Restricted Party Lists": the list of sanctioned entities maintained by the United Nations; the Specially Designated Nationals List and the Sectoral Sanctions Identifications List of the U.S. Treasury Department's Office of Foreign Assets Control; the U.S. Denied Persons List, the U.S. Entity List, and the U.S. Unverified List of the U.S. Department of Commerce; entities subject to restrictive measures and the Consolidated List of Persons, Groups and Entities Subject to E.U. Financial Sanctions, as implemented by the E.U. Common Foreign & Security Policy; the List of Excluded Individuals / Entities published by the U.S. Health and Human Services' Office of Inspector General; any lists of prohibited or debarred parties established under the U.S. Federal Food Drug and Cosmetic Act; the list of parties suspended or debarred from contracting with the U.S. government; and similar lists of restricted parties maintained by the Governmental Authorities of the countries that have jurisdiction over the activities conducted under this Agreement.

11.8.3 It will not knowingly transfer to the other Party any goods, software, technology or services that are (i) controlled under the U.S. International Traffic in Arms Regulations or at a level other than EAR99 under the U.S. Export Administration Regulations; or (ii) specifically identified as an E.U. Dual Use Item or on an applicable export control list of another country.

11.9 Waiver. No provision of this Agreement will be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The failure of either Party to assert a right hereunder or to insist upon compliance with any term of this

Agreement will not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either Party of any condition or term in any one or more instances will be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

11.10 Severability. If any provision hereof should be invalid, illegal or unenforceable in any jurisdiction, the Parties will negotiate in good faith a valid, legal and enforceable substitute provision that most nearly reflects the original intent of the Parties and all other provisions hereof will remain in full force and effect in such jurisdiction and will be construed in order to maintain this Agreement's existence, validity and enforceability to the greatest extent possible and to carry out the intentions of the Parties as nearly as may be possible. Such invalidity, illegality or unenforceability will not affect the validity, legality or enforceability of such provision in any other jurisdiction.

11.11 Entire Agreement. This Agreement, together with the Schedules and Exhibits hereto, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and sets forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the Parties and supersede and terminate all prior agreements negotiations, correspondence, agreements, and understanding, whether oral or written, between the Parties. In particular, and without limiting the foregoing, this Agreement supersedes and replaces the Existing Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties prior to the Effective Date. No subsequent alteration, amendment, change or addition to this Agreement will be valid or effective unless reduced to writing and signed by the respective authorized officers of each Party.

11.12 Independent Contractors. Nothing herein will be construed to create any relationship of employer and employee, agent and principal, partnership or joint venture or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party between the Parties. Each Party is an independent contractor under this Agreement. Neither Party will have the authority to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind or obligate the other Party and neither Party will represent that it has such authority.

11.13 Headings; Construction; Interpretation. Headings and any table of contents used herein are for convenience only and will not in any way affect the construction of or be taken into consideration in interpreting this Agreement. The terms of this Agreement represent the results of negotiations between the Parties and their Representatives, each of which has been represented by counsel of its own choosing, and neither of which has acted under duress or compulsion, whether legal, economic or otherwise. Accordingly, the terms of this Agreement will be interpreted and construed in accordance with their usual and customary meanings, and each of the Parties hereby waives the application in connection with the interpretation and construction of this Agreement of any rule of Law to the effect that ambiguous or conflicting terms or provisions contained in this Agreement will be interpreted or construed against the Party whose attorney prepared the executed draft or any earlier draft of this Agreement. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause, Exhibit or Schedule will be deemed to be a reference to any Article, Section, subsection, paragraph, clause, Exhibit or Schedule, of or to, as the case may be, this

Agreement. Except where the context otherwise requires, (a) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (b) any reference to any Law refers to such Law includes all rules and regulations thereunder and any successor Law, in each case as from time to time enacted, repealed or amended, (c) the words "herein," "hereof" and "hereunder," and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof, (d) the words "include," "includes," "including," "exclude," "excludes," and "excluding," will be deemed to be followed by the phrase "but not limited to," "without limitation" or words of similar import, (e) the word "or" is used in the inclusive sense (and/or), (f) words in the singular or plural form include the plural and singular form, respectively, (g) references to any gender refer to each other gender, (h) references to a particular Person include such Person's successors and assigns to the extent not prohibited by this Agreement, and (i) a capitalized term not defined herein but reflecting a different part of speech than a capitalized term which is defined herein will be interpreted in a correlative manner.

11.14 Further Actions. Each Party will execute, acknowledge and deliver such further instruments, and do all such other acts, as may be necessary or appropriate in order to carry out the expressly stated purposes and the clear intent of this Agreement.

11.15 Parties in Interest. All of the terms and provisions of this Agreement will be binding upon, and will inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

11.16 Counterparts. This Agreement may be signed in counterparts, each and every one of which will be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF will be treated as original signatures.

[Signature page follows.]

IN WITNESS WHEREOF, and intending to be legally bound hereby, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

Voyager Therapeutics, Inc.

By: /s/ Michael Higgins
Name: Michael Higgins
Title: Interim CEO

Pfizer Inc.

By: /s/ Mikael Dolsten
Name: Mikael Dolsten
Title: Chief Scientific Officer, President,
WW Research, Dev & Med

List of Exhibits:

Exhibit A: Cardiology Capsid

Exhibit B: CNS Capsid

Exhibit C: Capsid Supply Quantities

List of Schedules:

Schedule 1.18: Capsid Patents Covering Capsid Candidates as of the Effective Date

Exhibit 31.1

Certification

I, Michael Higgins, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2021 of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 2, 2021

/s/ Michael Higgins

Michael Higgins
Interim Chief Executive Officer, President, and Director
(Principal Executive Officer)

Exhibit 31.2

Certification

I, Allison Dorval, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2021 of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 2, 2021

/s/ Allison Dorval

Allison Dorval
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Voyager Therapeutics, Inc. (the "Company") for the period ended September 30, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 that to his or her knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 2, 2021

/s/ Michael Higgins
Michael Higgins
Interim Chief Executive Officer, President, and Director
(Principal Executive Officer)

Date: November 2, 2021

/s/ Allison Dorval
Allison Dorval
Chief Financial Officer
(Principal Financial and Accounting Officer)