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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 June 30, 2023

☐ **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.)
64 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 July 28, 2023 was 43,900,519.

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 and our proprietary antibodies;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ discovery platform and our vectorized antibody platform, and our proprietary antibodies;
- our ability to identify and optimize product candidates and proprietary AAV capsids;
- our strategic collaborations and licensing agreements with, and funding from, our collaboration partner, Neurocrine Biosciences, Inc., or Neurocrine, and our licensees Pfizer Inc., or Pfizer, and Novartis Pharma AG, or Novartis;
- our ongoing and planned preclinical development efforts, related timelines and studies;
- our ability to enter into future collaborations, strategic alliances, or option and license arrangements;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for our product candidates, including the ability to submit investigational new drug, or IND, applications for our programs;
- our estimates regarding revenue, expenses, contingent liabilities, future revenues, existing cash resources and capital requirements;
- 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;
- our need for additional funding and our plans and ability to raise additional capital, including through equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements;
- our competitive position and the success of competing products that are or might 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; and
- our ability to control costs and prioritize our product candidate pipeline and platform development objectives successfully in connection with our strategic initiatives.

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 our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 7, 2023, particularly in “Part I, Item 1A — Risk Factors,” and, if applicable, our Quarterly Reports on Form 10-Q, particularly in “Part II, Item 1A — Risk Factors,” 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, strategic collaborations, 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.

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)

	June 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 244,293	$ 98,959
Marketable securities, current	28,453	19,889
Related party collaboration receivable	3,350	257
Prepaid expenses and other current assets	6,080	5,394
Total current assets	282,176	124,499
Property and equipment, net	17,239	17,857
Deposits and other non-current assets	1,593	1,515
Operating lease, right-of-use assets	14,528	15,485
Total assets	$ 315,536	$ 159,356
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,659	$ 2,566
Accrued expenses	9,060	7,816
Other current liabilities	3,013	2,832
Deferred revenue, current	31,666	59,377
Total current liabilities	45,398	72,591
Deferred revenue, non-current	51,383	6,450
Other non-current liabilities	19,729	21,295
Total liabilities	116,510	100,336
Commitments and contingencies (see note 8)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized at June 30, 2023 and December 31, 2022; no shares issued and outstanding at June 30, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized at June 30, 2023 and December 31, 2022; 43,759,409 and 38,613,891 shares issued and outstanding at June 30, 2023 and December 31, 2022, respectively	44	38
Additional paid-in capital	490,791	452,713
Accumulated other comprehensive loss	(133)	(219)
Accumulated deficit	(291,676)	(393,512)
Total stockholders' equity	199,026	59,020
Total liabilities and stockholders' equity	$ 315,536	$ 159,356

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Collaboration revenue	$ 4,853	$ 712	$ 155,333	$ 1,371
Operating expenses:				
Research and development	21,985	12,527	40,553	26,876
General and administrative	8,294	7,552	17,322	15,211
Total operating expenses	30,279	20,079	57,875	42,087
Operating (loss) income	(25,426)	(19,367)	97,458	(40,716)
Interest income	3,274	219	5,138	271
Other income:	3	61	3	39
Total other income, net	3,277	280	5,141	310
(Loss) income before income taxes	(22,149)	(19,087)	102,599	(40,406)
Income tax provision	59	—	763	—
Net (loss) income	$ (22,208)	$ (19,087)	$ 101,836	$ (40,406)
Other comprehensive (loss) income:				
Net unrealized (loss) gain on available-for-sale securities	(1)	(141)	86	(226)
Total other comprehensive (loss) income	(1)	(141)	86	(226)
Comprehensive (loss) income	$ (22,209)	$ (19,228)	$ 101,922	$ (40,632)
Net (loss) income per share, basic	$ (0.51)	$ (0.50)	$ 2.42	$ (1.06)
Net (loss) income per share, diluted	$ (0.51)	$ (0.50)	$ 2.33	$ (1.06)
Weighted-average common shares outstanding, basic	43,520,137	38,298,426	42,102,101	38,183,192
Weighted-average common shares outstanding, diluted	43,520,137	38,298,426	43,770,999	38,183,192

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	Amount	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Stockholders' Equity
Balance at December 31, 2021	37,918,395	$ 38	$ 442,259	$ (138)	$ (347,104)	$ 95,055
Exercises of vested stock options	11,484	—	12	—	—	12
Vesting of restricted stock units	312,090	—	—	—	—	—
Stock-based compensation expense	—	—	2,268	—	—	2,268
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(85)	—	(85)
Net loss	—	—	—	—	(21,319)	(21,319)
Balance at March 31, 2022	38,241,969	$ 38	$ 444,539	$ (223)	$ (368,423)	$ 75,931
Exercises of vested stock options	63,012	—	575	—	—	575
Vesting of restricted stock units	32,165	—	—	—	—	—
Issuance of common stock under ESPP	102,105	—	313	—	—	313
Stock-based compensation expense	—	—	2,460	—	—	2,460
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(141)	—	(141)
Net loss	—	—	—	—	(19,087)	(19,087)
Balance at June 30, 2022	38,439,251	$ 38	$ 447,888	$ (364)	$ (387,510)	$ 60,052
Balance at December 31, 2022	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	59,020
Exercises of vested stock options	51,993	—	185	—	—	185
Vesting of restricted stock units	374,417	—	—	—	—	—
Issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	4,395,588	5	31,116	—	—	31,121
Stock-based compensation expense	—	—	2,504	—	—	2,504
Unrealized gain on available-for-sale securities, net of tax	—	—	—	87	—	87
Net income	—	—	—	—	124,044	124,044
Balance at March 31, 2023	43,435,889	$ 43	$ 486,518	$ (132)	$ (269,468)	$ 216,961
Exercises of vested stock options	198,348	1	1,228	—	—	1,229
Vesting of restricted stock units	62,828	—	—	—	—	—
Issuance of common stock under ESPP	62,344		418			418
Stock-based compensation expense	—	—	2,627	—	—	2,627
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(22,208)	(22,208)
Balance at June 30, 2023	43,759,409	$ 44	$ 490,791	$ (133)	$ (291,676)	$ 199,026

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)

	Six Months Ended June 30,	
	2023	**2022**
Cash flow from operating activities		
Net income (loss)	$ 101,836	$ (40,406)
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Stock-based compensation expense	5,230	4,809
Depreciation	2,161	4,016
Amortization of premiums and discounts on marketable securities	(25)	2
Loss on disposal of fixed assets	124	—
Other non-cash items	—	(2,469)
Changes in operating assets and liabilities:		
Related party collaboration receivable	(3,093)	581
Prepaid expenses and other current assets	(686)	6
Operating lease, right-of-use asset	957	2,556
Accounts payable	(907)	644
Accrued expenses	1,296	(3,652)
Operating lease liabilities	(1,385)	(2,647)
Other non-current liabilities	—	(287)
Deferred revenue	17,222	52,787
Net cash provided by operating activities	122,730	15,941
Cash flow from investing activities		
Purchases of property and equipment	(1,719)	(1,280)
Purchases of marketable securities	(28,453)	(54,848)
Proceeds from sales and maturities of marketable securities	20,000	—
Net cash used in investing activities	(10,172)	(56,128)
Cash flow from financing activities		
Proceeds from the exercise of stock options	1,414	587
Proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	31,121	
Proceeds from the purchase of common stock under ESPP	319	232
Net cash provided by financing activities	32,854	819
Net increase (decrease) in cash, cash equivalents, and restricted cash	145,412	(39,368)
Cash, cash equivalents, and restricted cash, beginning of period	100,474	119,212
Cash, cash equivalents, and restricted cash, end of period	$ 245,886	$ 79,844
Supplemental disclosure of cash and non-cash activities		
Capital expenditures incurred but not yet paid	$ 52	$ 113

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 biotechnology company dedicated to breaking through barriers in gene therapy and neurology. The Company focuses on leveraging its expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the gene therapy and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, therefore providing clinically meaningful impact to patients. The Company's gene therapy platforms enable it to engineer, optimize, manufacture and deliver its adeno-associated virus ("AAV") based gene therapies that it believes have the potential to safely provide durable efficacy. The Company's team of experts in the field of AAV gene therapy and neuroscience first identifies and selects diseases in which the Company believes an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. The Company then engineers and optimizes an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

The Company is identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. The Company's team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood-brain barrier ("BBB"). The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates. The Company believes that the capsids it discovers through its TRACER discovery platform ("TRACER Capsids") have the potential to significantly enhance the efficacy and safety of its single dose gene therapies, which the Company expects to be delivered with systemic infusions, as compared with conventional capsids. The Company has leveraged the TRACER discovery platform to generate multiple families of TRACER Capsids with robust central nervous system ("CNS") tropism following intravenous delivery. The Company has presented data at scientific conferences demonstrating strong transduction to multiple areas within the brain, and activity across multiple species. The Company has identified a receptor for one of its TRACER Capsid families as well as a ligand for that receptor. The Company is conducting experiments to evaluate the potential to leverage the receptor to shuttle non-viral genetic medicines across the BBB. The Company has also preliminarily identified two receptors for additional families of TRACER Capsids and is conducting confirmatory experiments.

In addition to leveraging TRACER Capsids in potential licensing arrangements, the Company is advancing its own proprietary pipeline of drug candidates for neurological diseases, with a focus on Alzheimer's disease. The Company's wholly-owned prioritized pipeline programs include superoxide dismutase 1 ("SOD1") gene therapy for amyotrophic lateral sclerosis ("ALS") and an anti-tau antibody for Alzheimer's disease. The Company identified a lead development candidate in its anti-tau antibody program in the first quarter of 2023 and expects to submit an investigational new drug application ("IND") to the U.S. Food and Drug Administration ("FDA") in the first half of 2024. The Company continues to evaluate the data from preclinical studies for its SOD1 program and expects to identify a lead development candidate in the second half of 2023. The Company expects to submit an IND for its SOD1 program in mid-2025. The Company's pipeline also includes four early research initiatives to develop gene therapies for the treatment of Alzheimer's disease, Huntington's disease, and brain metastases from HER2+ metastatic breast cancer.

In addition to these wholly-owned programs, the Company is actively advancing two later preclinical-stage programs in collaboration with Neurocrine Biosciences, Inc. ("Neurocrine"): a glucocerebrosidase 1 ("GBA1") gene therapy program for Parkinson's disease and other GBA1-mediated diseases ("GBA1 Program"), and a FXN gene therapy program for Friedreich's ataxia. The Company also maintains a robust early research pipeline of wholly-owned and collaborative gene therapy programs for neurological diseases.

The Company has a history of incurring annual net operating losses. As of June 30, 2023, the Company had an accumulated deficit of $291.7 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 fees, milestone payments, and cost reimbursements associated with its prior collaborations with Sanofi Genzyme Corporation ("Sanofi Genzyme") and AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, its ongoing collaborations with Neurocrine, its option and license agreement with Pfizer Inc. ("Pfizer"), and its option and license agreement with Novartis Pharma AG ("Novartis").

As of June 30, 2023, the Company had cash, cash equivalents, and marketable securities of $272.7 million. Based upon its current operating plan, the Company expects that its existing cash, cash equivalents, and marketable securities at June 30, 2023, along with amounts expected to be received as reimbursement for development costs under the Company's collaboration and license agreements with Neurocrine, will be sufficient to meet the Company's planned operating expenses and capital expenditure requirements into 2025.

There can be no assurance that the Company will be able to obtain additional debt or equity financing on terms acceptable to the Company or generate product revenue or revenue from collaboration partners, 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. 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, 2022 as filed with the Securities and Exchange Commission ("SEC") on March 7, 2023. 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 heading "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, 2022. Intercompany balances 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.

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 and Basis of Presentation" within the "Notes to Consolidated Financial Statements" accompanying the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of June 30, 2023 and December 31, 2022 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)
June 30, 2023		*(in thousands)*		
Money market funds included in cash and cash equivalents	$ 238,434	$ 238,434	$ —	$ —
Marketable securities:				
U.S. Treasury notes	12,754	12,754	—	—
U.S. Government agency securities	4,317	4,317	—	—
Corporate bonds	1,975	—	1,975	—
Commercial paper	9,407	—	9,407	—
Total	$ 266,887	$ 255,505	$ 11,382	$ —
December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	$ 91,724	$ —	$ —
Marketable securities:				
U.S. Treasury notes	19,889	19,889	—	—
Total	$ 111,613	$ 111,613	$ —	$ —

The Company measures the fair value of money market funds, U.S. Treasury notes, and U.S. Government agency securities based on quoted prices in active markets for identical securities. The Company measures the fair value of the Level 2 securities, commercial bonds and commercial paper, based on recent trades of securities in inactive markets or based on quoted market prices of similar instruments and other significant inputs derived from or corroborated by observable market data.

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

Cash, cash equivalents, and marketable securities included the following at June 30, 2023 and December 31, 2022:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
		(in thousands)		
As of June 30, 2023				
Money market funds included in cash and cash equivalents	$ 238,434	$ —	$ —	$ 238,434
Marketable securities:				
U.S. Treasury notes	12,754	1	(1)	12,754
U.S. Government agency securities	4,321	—	(4)	4,317
Corporate bonds	1,976	—	(1)	1,975
Commercial paper	9,407	—	—	9,407
Total money market funds and marketable securities	$ 266,892	$ 1	$ (6)	$ 266,887
As of December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	—	—	$ 91,724
Marketable securities:				
U.S. Treasury notes	19,980	—	(91)	19,889
Total money market funds and marketable securities	$ 111,704	$ —	$ (91)	$ 111,613

All of the Company's marketable securities as of June 30, 2023 have a contractual maturity of one year or less.

The Company reviews investments whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. In connection with these investments, the Company evaluates whether the decline in fair value has resulted from credit losses or other factors, considering the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security, among other factors. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security is compared to the amortized cost basis of the security. If the present value of cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss on the condensed consolidated balance sheet, limited by the amount that the fair value is less than the amortized cost basis. Any impairment that is not related to credit is recognized in other comprehensive (loss) income. Changes in the allowance for credit losses are recorded as a provision for (or reversal of) credit loss expense in general and administrative expenses within the condensed consolidated statement of operations. Losses are charged against the allowance when the Company believes the uncollectability of an available-for-sale security is confirmed or when either of the criteria regarding intent or requirement to sell is met.

The Company held $11.2 million and $19.9 million in marketable securities that were in an unrealized loss position as of June 30, 2023 and December 31, 2022, respectively. The Company held $7.9 million in marketable securities that were in an unrealized gain position as of June 30, 2023. No marketable securities were in an unrealized gain position at December 31, 2022. The unrealized losses at June 30, 2023 and December 31, 2022 were attributable to changes in interest rates and the unrealized losses do not represent credit losses. The Company does not intend to sell these securities and it is not more likely than not that it will be required to sell them before recovery of their amortized cost basis.

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 June 30, 2023	As of December 31, 2022
	(in thousands)	
Cash and cash equivalents	$ 244,293	$ 98,959
Restricted cash included in deposits and other non-current assets	1,593	1,515
Total cash, cash equivalents, and restricted cash	$ 245,886	$ 100,474

5. Accrued expenses

Accrued expenses as of June 30, 2023 and December 31, 2022 consist of the following:

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Employee compensation costs	$ 3,731	$ 4,559
Research and development costs	3,335	1,895
Professional services	829	726
Accrued goods and services	1,165	636
Total	$ 9,060	$ 7,816

6. Lease obligation

Operating Leases

As of June 30, 2023, the Company has a lease for office and laboratory space at 64 Sidney Street in Cambridge, Massachusetts through November 30, 2026 and a lease for additional laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031.

In September 2021, the Company entered into an agreement with BioNTech US, Inc. (“BioNTech US”) to sublease part of the office and laboratory space leased by the Company at 75 Sidney Street in Cambridge, Massachusetts (the “Sublease Agreement”) at that time. The sublease term was for approximately 3.3 years. 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 because of the sublease and accounted for the sublease as a separate lease.

On June 22, 2022, the Company entered into a Lease Termination Agreement (the “Lease Termination Agreement”) and terminated the lease for office and laboratory space at 75 Sidney Street, effective immediately. In connection with the Lease Termination Agreement, the Company also entered into a Sublease Termination Agreement (the “Sublease Termination Agreement”) and terminated the Sublease Agreement with BioNTech US. The Company did not incur any termination penalties in connection with the Lease Termination Agreement or Sublease Termination Agreement. The Company derecognized the related right-of-use asset of approximately $14.5 million and the operating lease liabilities of $17.0 million, accordingly, resulting in a gain of $2.5 million in the three-month period ended June 30, 2022.

The Company’s lease agreements require the Company to maintain a cash deposit or irrevocable letter of credit in the aggregate amount of $1.5 million payable to its landlords as security for the performance of its obligations under the leases. These amounts are recorded as restricted cash and are included in deposits and other non-current assets in the accompanying condensed consolidated balance sheets.

During each of the three and six months ended June 30, 2023, the Company incurred lease expenses of $0.9 million and $1.8 million, respectively, for operating leases. During each of the three and six months ended

June 30, 2022, the Company incurred lease expenses of $1.4 million and $2.8 million, respectively, for operating leases. As of June 30, 2023, the weighted average remaining lease term was 5.5 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 7.4%.

The following table summarizes the operating sublease income generated under the Sublease Agreement for the three and six months ended June 30, 2023 and 2022:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating sublease income	*(in thousands)*			
	$ —	$ 690	$ —	$ 1,380

7. Other liabilities

As of June 30, 2023 and December 31, 2022, other current and non-current liabilities consisted of the following:

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Other current liabilities		
Lease liability	3,013	2,832
Total other current liabilities	$ 3,013	$ 2,832
Other non-current liabilities		
Lease liability	$ 18,729	$ 20,294
Other	1,000	1,001
Total other non-current liabilities	$ 19,729	$ 21,295

8. Commitments and contingencies

Significant Agreements

The Company's significant agreements are described in Note 9 of the December 31, 2022 consolidated financial statements included in its Annual Report on Form 10-K for the year ended December 31, 2022. During the six months ended June 30, 2023 and 2022, there were no material changes to the Company's collaboration agreement with Neurocrine executed in March 2019 (the "2019 Neurocrine Collaboration Agreement") and the option and license agreement executed with Pfizer in October 2021 (the "Pfizer Agreement"). Accordingly, there were no changes to the Company's accounting treatment for these agreements through June 30, 2023. The Company recorded revenue of $1.6 million and $0.7 million under the 2019 Neurocrine Collaboration Agreement during the three months ended June 30, 2023 and 2022, respectively. The Company recorded revenue of $3.6 million and $1.4 million under the 2019 Neurocrine Collaboration Agreement during the six months ended June 30, 2023 and 2022, respectively. The Company did not recognize any collaboration revenue related to the Pfizer Agreement during the three or six months ended June 30, 2023 or 2022.

2023 Neurocrine Collaboration Agreement

Summary of Agreement

On January 8, 2023, the Company entered into a collaboration and license agreement with Neurocrine (the "2023 Neurocrine Collaboration Agreement") for the research, development, manufacture and commercialization of gene therapy products directed to the GBA1 Program, and three early research programs focused on the research, development, manufacture and commercialization of gene therapies designed to address CNS diseases or conditions

associated with rare genetic targets (the "2023 Discovery Programs" and, collectively with the GBA1 Program, the "2023 Neurocrine Programs"). The 2023 Neurocrine Collaboration Agreement became effective upon the expiration of all applicable waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, which occurred on February 21, 2023 (the "Neurocrine Effective Date").

Collaboration and License

Under the 2023 Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to collaborate on the conduct of the 2023 Neurocrine Programs. Under the terms of the 2023 Neurocrine Collaboration Agreement, subject to the rights retained by the Company thereunder, the Company granted to Neurocrine, as of the Neurocrine Effective Date, an exclusive, royalty-bearing, sublicensable, worldwide license, under certain of the Company's intellectual property rights, to research, develop, manufacture and commercialize gene therapy products (the "2023 Collaboration Products"), arising under the 2023 Neurocrine Programs.

Pursuant to mutually-agreed workplans, during the period beginning on the Neurocrine Effective Date and ending on the third anniversary of the Neurocrine Effective Date, which period may be extended upon mutual written agreement of the Company and Neurocrine, (the "2023 Discovery Period"), and as overseen by the Joint Steering Committee ("JSC") for the ongoing collaboration with Neurocrine, the Company is responsible for identifying capsids meeting target criteria, producing development candidates, and conducting other pre-clinical activities regarding the 2023 Collaboration Products. Neurocrine has agreed to be responsible for all costs the Company incurs in conducting pre-clinical development activities for each 2023 Neurocrine Program, in accordance with JSC agreed upon workplans and budgets. If the Company breaches its responsibilities during this time or, in certain circumstances, upon a change of control, Neurocrine has the right, but not the obligation, to assume the conduct of the Company's activities under such 2023 Neurocrine Program.

The Company has been granted the option ("2023 Co-Co Option") to co-develop and co-commercialize 2023 Collaboration Products in the GBA1 Program in the United States upon the occurrence of the Company receiving topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program. Should the Company elect to exercise its 2023 Co-Co Option, the Company and Neurocrine agree to enter into a cost and profit-sharing arrangement (a "2023 Co-Co Agreement"), whereby the Company and Neurocrine agree to jointly develop and commercialize 2023 Collaboration Products in the GBA1 Program ("2023 Co-Co Products") in the United States and share equally in the GBA1 Program's costs, profits and losses in the United States, with each party entitled to or responsible for 50% of profits and losses with respect to each 2023 Co-Co Product in the United States, subject to specified exceptions. The parties have agreed that the 2023 Co-Co Agreement will provide the Company the right to terminate the 2023 Co-Co Agreement for any reason upon prior written notice to Neurocrine and provide Neurocrine the right to terminate or amend the 2023 Co-Co Agreement upon a change of control under certain circumstances. In the event the Company exercises its 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50% share of profits) 50% of the Company's share of profits until the Company's obligation to repay 50% of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50% of the Company's share of profits.

Candidate Selection

Either party may notify the JSC of any gene therapy product candidate that includes a Company capsid and a payload that is being developed under a 2023 Neurocrine Program (a "Collaboration Candidate"), that it desires to nominate as a development candidate. In such event, the JSC shall determine whether such nominated Collaboration Candidate meets certain development criteria. There will be a maximum of four potential development candidates for which development is being performed under any 2023 Neurocrine Program at any given time during the 2023 Discovery Period. If a Collaboration Candidate fails to meet criteria established by the JSC and is removed from consideration to become a development candidate or is named a development candidate, then a new Collaboration Candidate may be nominated to be a potential development candidate to replace the Collaboration Candidate that has failed or succeeded such that not more than four potential development candidates per program are under consideration at any one time during the 2023 Discovery Period.

Manufacturing

The parties have agreed that the applicable development plans shall specify the allocation between the Company and Neurocrine of responsibilities for the manufacturing of Collaboration Candidates associated with the applicable 2023 Neurocrine Program during the 2023 Discovery Period. In accordance with the 2023 Collaboration Agreement, the parties have also agreed that, if the Company conducts any portion of the manufacturing of a Collaboration Candidate, the applicable development plan shall include an obligation for the Company to assist with the technology transfer of such manufacturing responsibilities to Neurocrine or a third-party contract manufacturing organization, as reasonably requested by Neurocrine, on terms to be mutually-agreed by the Company and Neurocrine. Following the end of the 2023 Discovery Period, Neurocrine shall be responsible for the manufacturing of all Collaboration Candidates and products.

Financial Terms

Under the terms of the 2023 Neurocrine Collaboration Agreement, in February 2023 Neurocrine paid the Company an upfront payment of approximately $136.0 million and approximately $39.0 million for the purchase of 4,395,588 shares of common stock of the Company at a price of $8.88 per share. The 2023 Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to the Company for 2023 Collaboration Products under (a) the GBA1 Program of up to $985.0 million; and (b) each of the three 2023 Discovery Programs of up to $175.0 million for each 2023 Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $950.0 million per 2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $275.0 million per 2023 Discovery Program.

Neurocrine has also agreed to pay the Company tiered royalties, based on future net sales of the 2023 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, range from (a) for the GBA1 Program, the low double-digits to twenty and the high single-digits to mid-teens, respectively, and (b) for each 2023 Discovery Program, high single-digits to mid-teens and mid-single digits to low double-digits, respectively. On a country-by-country and 2023 Neurocrine Program-by-2023 Neurocrine Program basis, the parties have agreed royalty payments would commence on the first commercial sale of a 2023 Collaboration Product in such country and terminate upon the latest of (a) the expiration, invalidation or the abandonment of the last patent covering the composition of the 2023 Collaboration Product or its approved method of use in such country, (b) ten years from the first commercial sale of the 2023 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country, (the “2023 Royalty Term”). Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patent rights related to a 2023 Collaboration Product, approval of biosimilar products in each country, or required payment of licensing fees to third parties related to the development and commercialization of any 2023 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and 2023 Collaboration Product-by-2023 Collaboration Product basis upon the expiration of the 2023 Royalty Term applicable to the 2023 Collaboration Product in such country.

Termination

Unless earlier terminated, the 2023 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2023 Royalty Term with respect to all 2023 Collaboration Products worldwide or (b) the expiration or termination of any 2023 Co-Co Agreement. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement in its entirety or on a 2023 Neurocrine Program-by-2023 Neurocrine Program and/or country-by-country basis by providing at least (a) 180-day advance notice if such notice is provided prior to the first commercial sale of any 2023 Collaboration Product to which the termination applies or (b) one-year advance notice if such notice is provided after the first commercial sale of any product to which the termination applies. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement with respect to a given 2023 Collaboration Product by providing written notice of termination to the Company within thirty days after complete readout of any clinical trial if the results of such clinical trial fail to meet the pre-specified primary endpoint(s) set forth in the applicable protocol or if there is a safety finding during the clinical trial relating to such 2023 Collaboration Product that either (a) is substantially irreversible or not

monitorable in patients or (b) results in Neurocrine's decision to designate such 2023 Collaboration Product as a terminated product under the 2023 Collaboration Agreement.

The Company may terminate the 2023 Neurocrine Collaboration Agreement with respect to a particular patent right of the Company's, if Neurocrine challenges the validity or enforceability of such patent right. Subject to a cure period, either party may terminate the 2023 Neurocrine Collaboration Agreement in the event of a material breach in whole or in part, subject to specified conditions.

2023 Neurocrine Stock Purchase Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, Neurocrine and the Company also entered into a stock purchase agreement on January 8, 2023, for the sale and issuance of 4,395,588 shares of common stock to Neurocrine at a price of $8.88 per share, for an aggregate purchase price of approximately $39.0 million. In accordance with the terms and conditions of the stock purchase agreement, the Company issued and sold these shares to Neurocrine on February 23, 2023.

Accounting Analysis

At inception, the Company determined the 2023 Neurocrine Collaboration Agreement was a contract with a customer under Accounting Standards Codification Topic 606 ("ASC 606") and that modification accounting was not required given that the 2023 Neurocrine Collaboration Agreement did not represent a legally enforceable change in the scope or price of the 2019 Neurocrine Collaboration Agreement. The Company therefore determined that the 2023 Neurocrine Agreement should be accounted for separately. The 2023 Neurocrine Collaboration Agreement includes the following performance obligations: (i) the development and commercialization license for the GBA1 Program, (ii) the research and development services for the GBA1 Program, and (iii) the research and development services for each of the 2023 Discovery Programs combined with a development and commercialization license for each program. The license for the GBA1 Program is distinct as Neurocrine can benefit from such license on its own or from other resources commonly available in the industry given the stage of development of the product candidates subject to the license. Similarly, the research and development services for the GBA1 Program provide a distinct benefit to Neurocrine within the context of the contract, separate from the license. The research and development services for the 2023 Discovery Programs are not distinct as Neurocrine cannot benefit from such licenses 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 GBA1 license, GBA1 research and development services and the combined licenses and research and development services for the 2023 Discovery Programs are distinct from one another as Neurocrine can benefit from each program separately.

The Company identified $143.9 million of fixed transaction price consisting of the $136.0 million upfront fee, and a premium of $7.9 million related to the $39.0 million equity investment of 4,395,588 shares when measured at fair value on the date of issuance. The Company is also entitled to reimbursement of costs incurred by the Company during the 2023 Discovery Period associated with each Program.

These amounts are determinable based on development plans, and the Company has a contractual right to the payment of costs incurred under the agreed upon program development plans.

The Company utilizes the most likely amount approach to estimate the cost reimbursement and has concluded that these amounts do not require a constraint. As of June 30, 2023, the estimate of the expected reimbursement was $5.9 million of costs incurred based on expectations as of such date. The additional consideration to be paid to the Company upon reaching certain milestones is excluded from the transaction price at inception due to the uncertainty of the payment and the uncertainty of achieving the development and regulatory milestones.

The Company has allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation. The estimated standalone selling prices for performance obligations were developed using the estimated selling price of the license for the GBA1 Program and each of the three 2023 Discovery Programs, using primarily adjusted market assessment approaches that considered discounted, probability-weighted cash flow analyses and entity-specific and market factors. The Company did not allocate any of the

fixed transaction price to the GBA1 research and development services performance obligation as the consideration for such services reflects a market rate.

The Company 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 2023 Neurocrine Collaboration Agreement. The reimbursement of research services is 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. Based on the initial development plans, the total variable consideration allocated to each program related to the expected cost reimbursement was as follows as of June 30, 2023:

Performance Obligation	Amount
	(in thousands)
Variable Consideration	
GBA1 Program	$ 5,361
2023 Discovery Program 1	166
2023 Discovery Program 2	166
2023 Discovery Program 3	166
Total	$ 5,859

Based on the relative standalone selling price allocation, the allocation of the fixed transaction price to the separate performance obligations was as follows:

Performance Obligation	Amount
	(in thousands)
Fixed Consideration	
GBA1 Program	$ 69,459
2023 Discovery Program 1	24,807
2023 Discovery Program 2	24,807
2023 Discovery Program 3	24,807
Total	$ 143,880

The Company recognized the fixed transaction price allocated to the development and commercialization license for the GBA1 Program as collaboration revenue in the first quarter of 2023, upon delivery of the development and commercialization license for the GBA1 Program to Neurocrine. The Company is recognizing the consideration allocated to each of the three 2023 Discovery Program performance obligations 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. Proportional performance is determined based on the workplan cost and timeline estimates.

During the three months ended March 31, 2023, the Company recognized $69.5 million of revenue associated with the 2023 Neurocrine Collaboration Agreement related to the delivery of the development and commercialization license for the GBA1 Program. During the three months ended June 30, 2023, the Company recognized $2.2 million of collaboration revenue associated with research and development services performed during the period and the corresponding cost reimbursement receivable for the GBA1 Program. During the three months ended June 30, 2023, the Company recognized $0.7 million of revenue associated with the fixed transaction price allocated to the three 2023 Discovery Programs, and with research and development services performed during the period and the corresponding cost reimbursement receivable for the three 2023 Discovery Programs. As of June 30, 2023, there was $73.7 million of deferred revenue related to the 2023 Neurocrine Collaboration Agreement, of which $26.1 million was classified as current and $47.6 million was classified as non-current in the accompanying balance sheets based on the period the services are expected to be delivered.

The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities under the 2023 Neurocrine Collaboration Agreement during the six months ended June 30, 2023:

	Balance at December 31, 2022	Additions	Deductions	Balance at June 30, 2023
		(in thousands)		
Related party collaboration receivable	$ -	2,239	$ -	$ 2,239
Contract liabilities:				
Deferred revenue	$ -	74,420	$ (689)	$ 73,731

The Company incurred approximately $0.4 million of costs to obtain the 2023 Neurocrine Collaboration Agreement which were payable only upon the close of the transaction and therefore considered incremental costs of obtaining a contract with a customer and capitalized. The costs are recorded in prepaid expenses and are being amortized to operating expenses consistent with the manner in which the consideration allocated to the performance obligations is recognized. In conjunction with the recognition of collaboration revenue during the six months ended June 30, 2023, approximately $0.2 million of costs to obtain the 2023 Neurocrine Collaboration Agreement were expensed.

Novartis Option and License Agreement

Summary of Agreement

On March 4, 2022 (the "Novartis Effective Date"), the Company entered into an option and license agreement with Novartis (the "Novartis Agreement"). Pursuant to the Novartis Agreement, the Company has granted Novartis options (the "Novartis License Options") to license TRACER Capsids ("Novartis Licensed Capsids") for exclusive use with certain targets to develop and commercialize adeno-associated virus gene therapy candidates comprised of Novartis Licensed Capsids and payloads directed to such targets (the "Novartis Payloads").

During the period commencing on the Novartis Effective Date and ending on the first anniversary thereof or, in the event Novartis exercises a Novartis License Option, the third anniversary thereof, on a target-by-target basis (the "Novartis Research Term"), the Company has granted Novartis a non-exclusive research license to evaluate the Company's TRACER Capsids for potential use, in combination with Novartis Payloads, in programs targeting three specified genes (the "Initial Novartis Targets"). Upon the payment of additional fees, Novartis may also assess the Company's TRACER Capsids for use with up to two other targets (the "Additional Novartis Targets"), subject to certain conditions including that such target is not part of, or reasonably competitive with, the Company's current development programs (the Initial Novartis Targets and the Additional Novartis Targets collectively, the "Novartis Targets"). During the Novartis Research Term, as applicable, the Company may, at its sole discretion and expense, conduct further research activities to identify additional TRACER Capsids. If the Company elects to do so, the Company has agreed to disclose performance characteristics of such new TRACER Capsids to Novartis on a rolling basis.

During the applicable Novartis Research Term, Novartis may exercise up to three Novartis License Options—or up to five Novartis License Options if Novartis is evaluating the Additional Novartis Targets—in the aggregate, provided that Novartis may only exercise one Novartis License Option for each Novartis Target. Upon the exercise of any Novartis License Option, the Company has agreed to grant Novartis a target-exclusive, worldwide license, with the right to sublicense, under certain of the Company's intellectual property, the rights to develop and commercialize the applicable Novartis Licensed Capsid as incorporated into products containing the corresponding Novartis Payload (the "Novartis Licensed Products"). Upon the exercise of a Novartis License Option, the Company has agreed to provide certain additional know-how to enable Novartis to exploit the Novartis Licensed Capsid and the corresponding Novartis Payload for use in a Novartis Licensed Product. Novartis may, during the applicable Novartis Research Term but following the exercise of a Novartis License Option, conduct additional evaluation of the Company's capsid candidates and has the right to substitute any other TRACER Capsid for a Novartis Licensed Capsid.

Subject to the Company's disclosure obligations described above, the Company and Novartis have agreed to conduct their respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of the Company and Novartis.

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

During the applicable Novartis Research Term, the Company has agreed to provide plasmids to Novartis for the production of TRACER Capsids for evaluation upon request. The Company has also granted Novartis a non-exclusive license, effective upon an exercise of a Novartis License Option and in addition to its options for target-exclusive licenses under certain of the Company's intellectual property described above, on a Novartis Licensed Capsid-by-Novartis Licensed Capsid basis, under certain of the Company's know-how to exploit the applicable Novartis Licensed Capsid as incorporated into Novartis Licensed Products containing the corresponding Novartis Payload.

Under the terms of the Novartis Agreement, Novartis paid the Company an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from the Company's TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. With Novartis' option exercise on two Initial Novartis Targets, the Company received a $25.0 million option exercise payment in April 2023, and is eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in the Company's internal and partnered pipeline. In addition, during the research term, Novartis retains the right to expand the agreement to include options to license capsids for up to two Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, the Company would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and tiered mid- to high-single digit royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids.

Novartis elected not to license a capsid for one Initial Novartis Target under the Novartis Agreement prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that the Company granted to Novartis in connection with this Initial Novartis Target has terminated, the Novartis Research Term for this Initial Novartis Target has expired, and the Company is no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this Initial Novartis Target. All capsid rights with respect to that Initial Novartis Target have returned to the Company.

Under the terms of the Novartis Agreement, each party 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 Novartis Effective Date, or invented, developed, created, generated or acquired solely by or on behalf of such party after the Novartis 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 Novartis Agreement and in the course of the parties' activities under the Novartis Agreement will follow inventorship under U.S. patent law.

Subject to certain limitations and exceptions, the Company has agreed (a) during the Novartis Research Term, not to conduct any internal program or program on behalf of a third party that is directed to the development or commercialization of any Company's capsids, or grant any third party or affiliate any right or license under the Company's rights in such capsids, to exploit any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on any of the Novartis Targets; and (b) after Novartis' exercise of Novartis License Options, not to grant any third party or affiliate any right or license under the Company's patents to exploit any Novartis Licensed Capsid for the applicable Novartis Target.

Unless earlier terminated, the Novartis Agreement expires on the expiration of the last-to-expire royalty term with respect to all Novartis Licensed Products in all countries. Subject to a cure period, either party may terminate the Novartis Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the Novartis Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, the occurrence of a violation of global trade control laws, or for the Company's non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the Novartis Agreement, in whole or in part, for any or no reason upon ninety days' written notice to the Company.

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

Accounting Analysis

At inception, the Company determined the Novartis Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services and determined that the Novartis Agreement contains three performance obligations consisting of three material rights, one for each of the Novartis License Options. The Company concluded that each Novartis License Option provides a material right as consideration for each option is less than the amount that the Company would otherwise have expected to receive outside the context of the contract. The promises at inception do not include the underlying goods or services that would be delivered upon exercise of the option, but rather represent the value to the customer of having the right to exercise the Novartis License Option at the specified exercise fee. Upon the exercise of a Novartis License Option, until March 4, 2025, while the Company is not obligated to conduct additional research activities upon any option exercise to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases, it has agreed to continue to disclose to Novartis, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Novartis may conduct additional evaluation of such capsid candidates and has the right to substitute any other capsid candidate for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option. The Company determined that this promise to provide Novartis the ability to evaluate and potentially substitute other capsid candidates for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option, if and when available, is an additional performance obligation in the arrangement (the "Novartis Substitution Right Performance Obligation"). The Company concluded the options for Additional Novartis Targets are not material rights as the price reflects the standalone selling price of the options. The Company will therefore account for the options for Additional Novartis Targets separately, if and when exercised.

The Company received a nonrefundable, upfront payment of $54.0 million as consideration under the Novartis Agreement, which represents the transaction price at inception. Additional consideration to be paid to the Company upon exercise of the Novartis License Options or upon reaching certain milestones are excluded from the transaction price as they relate to option fees and milestones that could only be achieved subsequent to an option exercise.

The Company allocated the transaction price to the three material rights based on their relative standalone selling prices. The estimated standalone selling price for each material right was based on an adjusted market assessment approach. The Company concluded that the market would be willing to pay an equal amount for each Novartis License Option on a standalone basis. The Company reached this conclusion after considering (i) the downstream economics including option fees, milestones and royalties related to each Novartis License Option being identical and (ii) comparable market data. The Company determined the standalone selling price for the Novartis Substitution Right Performance Obligation was insignificant to the allocation of the transaction price using the relative standalone selling price model and did not allocate any transaction price to the Novartis Substitution Right Performance Obligation, accordingly. This determination was supported by qualitative and quantitative assessments of the standalone selling price that considered the cost of identifying other potential capsid candidates and the likelihood of license substitution. As such, based on the relative standalone selling price for each of the three material rights, the allocation of the transaction price to the separate performance obligations is $18.0 million for each material right.

The amount allocated to each material right was recorded as deferred revenue.

During the three months ended March 31, 2023, the Company recognized $79.0 million in collaboration revenue related to the Novartis Agreement. Of this $79.0 million, $54.0 million is attributable to the exercise of the two material rights for Novartis License Options and the expiration of the third material right and was previously deferred as of December 31, 2022. The remaining $25.0 million represents the option exercise fee of $25.0 million. This amount was received by the Company during the second quarter of 2023.

License Agreement with Touchlight IP Limited

On November 3, 2022, the Company and Touchlight IP Limited ("Touchlight") entered into a license agreement (the "Touchlight License Agreement") to authorize historical use by the Company of a certain DNA preparation process ("Subject DNA Preparation Process"), and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process.

The terms of the Touchlight License Agreement include a one-time, non-refundable technology access fee of $5.0 million, which was paid during the fourth quarter of 2022. The Company recorded the $5.0 million to research and development expense in the year ended December 31, 2022, accordingly.

The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight if the Company or its program collaborators or licensees choose to utilize in a therapeutic product TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, the Company is obligated to pay low single-digit royalties to Touchlight on future payments the Company receives in connection with licensing of TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any Company therapeutic programs.

During the three months ended March 31, 2023, the Company recorded a $1.0 million fee to research and development expense per the terms of the Touchlight License Agreement in conjunction with Novartis' exercise of its Novartis License Options to license novel capsids generated from the Company's TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. This amount was paid to Touchlight during the second quarter of 2023.

Other Licensing Agreements

On June 28, 2023, the Company and Sangamo Therapeutics, Inc. ("Sangamo") entered into a definitive license agreement for a potential treatment of prion disease. Using its proprietary epigenetic regulation platform, Sangamo has developed zinc finger transcriptional regulators which it believes can specifically and potently block expression of the prion protein, the pathogenic driver of prion disease. The Company is eligible to earn certain license fees, royalties on potential commercial sales of any products using the Company's capsid, and, in the event the prion program is out licensed by Sangamo, a portion of all licensing revenues received with respect to this program. The Company has evaluated this license agreement under ASC 606 and determined that this agreement is not material to the financial statements, and all variable consideration is fully constrained.

Other Agreements

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 evaluated the arrangement and 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 for the year ended December 31, 2017 is recorded as a non-current liability in the condensed consolidated balance sheet.

Litigation

The Company was not a party to any material legal matters or claims as of June 30, 2023, or December 31, 2022. The Company did not have contingency reserves established for any litigation liabilities as of June 30, 2023, or December 31, 2022.

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 (loss) income was as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Research and development	$ 690	$ 781	$ 1,553	$ 1,582
General and administrative	1,982	1,709	3,677	3,227
Total stock-based compensation expense	$ 2,672	$ 2,490	$ 5,230	$ 4,809

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Stock options	$ 1,903	$ 1,618	$ 3,566	$ 3,095
Restricted stock awards and units	724	842	1,565	1,634
Employee stock purchase plan awards	45	30	99	81
Total stock-based compensation expense	$ 2,672	$ 2,490	5,230	4,809

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 six months ended June 30, 2023 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2022	1,112,563	$ 5.27
Granted	684,950	$ 7.28
Vested	(437,245)	$ 5.62
Forfeited	(46,768)	$ 4.43
Unvested restricted stock units as of June 30, 2023	1,313,500	$ 6.23

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. All of the restricted stock units granted in the six months ended June 30, 2023 vest in equal amounts, annually over three years. The stock-based compensation expense related to restricted

stock units was $0.7 million and $1.6 million for the three and six months ended June 30, 2023, respectively. The stock-based compensation expense related to restricted stock units was $0.8 million and $1.6 million for the three and six months ended June 30, 2022, respectively.

As of June 30, 2023, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $6.4 million, which is expected to be recognized over the remaining average vesting period of 2.4 years.

Stock Options

The following is a summary of stock option activity for the six months ended June 30, 2023:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value *(in thousands)*
Outstanding at December 31, 2022	6,199,571	$ 8.12	7.9	$ 6,095
Granted	1,663,200	$ 8.32		
Exercised	(250,341)	$ 5.97		
Cancelled or forfeited	(277,659)	$ 9.71		
Outstanding at June 30, 2023	7,334,771	$ 8.18	8.0	$ 29,875
Exercisable at June 30, 2023	3,300,800	$ 9.77	6.7	$ 11,634

As of June 30, 2023, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $17.2 million which is expected to be recognized over the remaining weighted-average vesting period of 2.8 years.

10. Net (loss) income per share

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

	Three Months Ended June 30,		Six Months Ended June 30	
	2023	2022	2023	2022
Unvested restricted common stock awards	45,000	137,255	45,000	137,255
Unvested restricted common stock units	1,313,500	1,031,365	725,876	1,031,365
Outstanding stock options	7,334,771	6,629,600	6,253,497	6,629,600
Total	8,693,271	7,798,220	7,024,373	7,798,220

Basic net (loss) income and diluted weighted-average shares outstanding are as follows for the three and six months ended June 30, 2023 and 2022:

	Three Months Ended June 30		Six Months Ended June 30	
	2023	2022	2023	2022
Numerator:				
Net (loss) income *(in thousands)*	$ (22,208)	$ (19,087)	$ 101,836	$ (40,406)
Denominator for basic net (loss) income per share:				
Weighted average shares outstanding-basic	43,520,137	38,298,426	42,102,101	38,183,192
Denominator for diluted net (loss) income per share:				
Weighted average shares outstanding-basic	43,520,137	38,298,426	42,102,101	38,183,192
Common stock options and restricted stock units	—	—	1,668,898	—
Weighted average shares outstanding-diluted	43,520,137	38,298,426	43,770,999	38,183,192

11. Related-party transactions

During the six months ended June 30, 2023, the Company received scientific advisory board and other scientific advisory services from 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 six months ended June 30, 2023, was $184,000 and $383,800, respectively. The total amount of fees paid to Dr. Sah for services provided during the three and six months ended June 30, 2022 was $82,425 and $92,325, respectively. During the second quarter of 2023, the Company and Dr. Sah agreed to a fee of $50,000 per month for advisory services from Dr. Sah. This agreement became effective during June 2023.

The Company received advisory services related to strategic planning, operations, and management from Alfred Sandrock, M.D., Ph.D., the Company's current President and Chief Executive Officer and a member of the Company's Board of Directors, before he commenced service in the capacity of President and Chief Executive Officer in March 2022. The total amount of fees paid to Dr. Sandrock for services provided was $60,000 for the six months ended June 30, 2022.

Under each of the Company's collaboration agreements with Neurocrine, the Company and Neurocrine have agreed to conduct research, development, and commercialization activities for certain of the Company's AAV gene therapy product candidates. Amounts due from Neurocrine are reflected as related party collaboration receivables. As of June 30, 2023, the Company had approximately $0.8 million in related party collaboration receivables associated with the 2019 Neurocrine Collaboration Agreement. As of June 30, 2023, the Company had approximately $2.2 million in related party collaboration receivables associated with the 2023 Neurocrine Collaboration Agreement.

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, 2022, which was filed with the Securities and Exchange Commission, or the SEC, on March 7, 2023.

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 in Part I, Item 1A,"Risk Factor" of our Annual Report on Form 10-K for the year ended December 31, 2022, and, if applicable, those included under Part II, Item 1A of our Quarterly Reports on Form 10-Q., that could cause actual future results or events to differ materially from the forward-looking statements that we make. Additional risk factors may be identified from time to time in our future filings with the SEC.

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 biotechnology company dedicated to breaking through barriers in gene therapy and neurology. We believe the potential of both disciplines has been constrained by delivery challenges; we are leveraging expertise in capsid discovery and neuropharmacology to address these constraints. Our gene therapy platforms enable us to engineer, optimize, manufacture and deliver adeno-associated virus, or AAV, based gene therapies that we believe have the potential to safely provide durable efficacy. Our team of experts in the field of AAV gene therapy and neuroscience first identifies and selects diseases in which we believe an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. We then engineer and optimize an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

We are identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. Our team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood-brain barrier, or BBB. The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates. We believe that capsids we discover through our TRACER discovery platform, which we refer to as TRACER Capsids, have the potential to significantly enhance the efficacy and safety of our single dose gene therapies, which we expect to be delivered with systemic infusions, as compared with conventional capsids. We have leveraged the TRACER discovery platform to generate multiple families of TRACER Capsids with robust central nervous system, or CNS, tropism following intravenous delivery. We have also identified a receptor for one of our TRACER Capsid families as well as a ligand for that receptor. We are conducting experiments to evaluate the potential to leverage the receptor to shuttle non-viral genetic medicines across the BBB. We have also preliminarily identified two receptors for additional families of TRACER Capsids and are conducting confirmatory experiments.

In addition to leveraging TRACER Capsids in potential licensing arrangements, we are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on Alzheimer's disease, or AD. Our wholly-owned prioritized pipeline programs include superoxide dismutase 1, or SOD1, gene therapy for amyotrophic lateral sclerosis, or ALS, and an anti-tau antibody for AD. We have identified a lead development candidate for our anti-tau antibody program in the first quarter of 2023 and we expect to submit an investigational new drug application, or IND, to the U.S. Food and Drug Administration for this program in the first half of 2024. We continue to evaluate the data from preclinical studies for our SOD1 program and expect to identify a lead development candidate in the second half of 2023. We expect to submit the IND for our SOD1 program in mid-2025. Our pipeline also includes four early research initiatives to develop gene therapies for the treatment of AD, Huntington's disease, and brain metastases from HER2+ metastatic breast cancer. In addition to these wholly-owned programs, we are actively advancing two later preclinical stage programs in collaboration with Neurocrine: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a FXN gene therapy program for Friedreich's ataxia.

AAV Gene Therapy

Gene therapy is an approach whereby gene expression is directly altered in patients to address the underlying cause or predominant manifestations of disease. We believe that the targeted nature of gene therapy may enable powerful treatment options and provide these patients with meaningful and durable benefits.

While AAV gene therapy can potentially be harnessed for multiple treatment methods, we are currently focused on gene replacement, gene knockdown and vectorized antibody approaches. Gene replacement is intended to restore the expression of a protein that is not expressed, expressed at abnormally low levels or functionally mutated with loss of function. Gene knockdown, or gene silencing, is intended to reduce the expression of a pathologically mutated RNA or protein that has detrimental effects. Vectorizing an antibody for delivery using AAV has the ability to increase exposure of large antibodies in brain parenchyma and interstitial fluid that otherwise show minimal penetration across the BBB

when administered passively. Our gene therapy approach uses AAV vectors which we believe are ideal vectors for gene therapy for several reasons:

- ***Broad Applicability.*** AAV is able to transduce, or transfer a therapeutic gene, into numerous cell types including target cells in the CNS, cardiac, and other tissues.
- ***Safety.*** We do not believe AAV is known to cause any disease in humans.
- ***Does Not Readily Integrate.*** AAV does not readily integrate into the genome of the target cell, an attribute which we believe reduces the potential for oncogenesis, or the induction of cancer.
- ***Scalability.*** AAV is able to be manufactured at commercial quality and scale.

We believe that neurological diseases are well-suited for treatment with AAV gene therapy for the following reasons:

- ***Validated Targets.*** Many neurological, cardiac, and other diseases are caused by well-defined mutations in genes and these genes represent genetically validated drug targets for AAV gene therapy.
- ***Targeted Delivery.*** We believe our TRACER Capsids may allow for significantly enhanced gene therapy delivery to specific types of cells and tissues at lower doses.
- ***Durable Expression.*** Long-term gene expression may be achievable in the CNS and other tissues following one-time dosing and transfer of the therapeutic gene with an AAV vector. Because repeated or continual dosing with direct injection of drugs into the CNS and other tissues is complex, a one-time AAV gene therapy has significant advantages.

The Voyager Gene Therapy Platform

We have built a gene therapy platform that we believe positions us to be the leading company at the intersection of AAV gene therapy and neurological diseases. Our team of experts in the field of AAV gene therapy first identifies and selects diseases that are well-suited for treatment using AAV gene therapy. We then engineer and optimize AAV vectors, identifying a capsid for delivery of a payload, comprising a therapeutic gene or transgene, and a promoter to drive expression of the transgene, to the targeted tissue or cells. Finally, we leverage established routes of administration and advances in dosing techniques to optimize delivery of our AAV vectors to target cells that are critical to the disease of interest. We believe that optimizing each of these parameters is a key factor for overall program success. We expect that our current and future pipeline programs will make use of technological advances generated with our gene therapy platform.

Disease Selection

Following an internal review process, we have prioritized pipeline programs for our development. This review evaluated the opportunity presented by each prioritized program based on the following criteria: high unmet medical need, target validation, efficient path to human proof of biology, robust preclinical pharmacology, and strong commercial potential.

Vector Engineering and Optimization

The key components of an AAV vector include: (a) the capsid; (b) the therapeutic gene, or transgene; and (c) payload control elements, including the promoter or other DNA sequences that modulate the expression of the transgene. We have advanced or intend to advance our multiple preclinical programs towards selection of lead clinical candidates using AAV vectors that we believe are best suited for each of our programs either through use of our existing capsids, through exercising a non-exclusive worldwide commercial license to capsid sequences covered by third parties, or by engineering or optimizing TRACER Capsids. We have also built, or intend to build, capabilities to design, screen, and

advance genetic sequences within our AAV vectors, including transgenes and payload control elements, to create optimized therapeutic candidates for each of our preclinical programs.

TRACER Capsid Discovery

Our scientists have developed TRACER, a proprietary AAV capsid discovery platform to facilitate the selection of TRACER Capsids for particular therapeutic applications based on BBB-crossing and cell-specific transduction properties in multiple species, including non-human primates, or NHPs. We believe these TRACER Capsids may allow for significantly enhanced gene delivery to specific types of cells in the brain at lower doses and, potentially, with fewer safety and tolerability issues than first-generation therapies. These TRACER Capsids are now in advanced stages of characterization for deployment in our gene therapy development programs. We continue to perform screening campaigns with our TRACER discovery platform to identify additional proprietary AAV9- and AAV5-derived TRACER Capsids and to refine previously-identified TRACER Capsids to target or de-target multiple tissue and cell types. At the American Society of Gene & Cell Therapy 26th Annual Meeting in May 2023, or the ASGCT 2023 Meeting, we presented data demonstrating greater than 50% cell transduction in multiple areas of the brain at a dose of 2E12 vg/kg following intravenous administration of our VCAP-102 TRACER Capsid in marmosets.

We are actively engaged in discussions to make TRACER Capsids available to third parties for use in their drug development programs through potential option and license and other arrangements. We believe there is significant opportunity for option and license transactions related to our TRACER Capsids. To maximize the potential of our TRACER Capsids for both our own programs and option and license transactions, we have retained to date, and expect to retain in the future, all rights associated with such TRACER Capsids other than the rights specific to their use in combination with the optionee's or licensee's transgenes or collaborators' programs.

Collaboration Agreements

2019 Neurocrine Collaboration

In January 2019, we entered into a collaboration with Neurocrine, or the 2019 Neurocrine Collaboration Agreement, for the research, development and commercialization of certain of our AAV gene therapy products, or the 2019 Collaboration Products. Under the 2019 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2019 Neurocrine Programs: the NBIb-1817 (VY-AADC) program for the treatment of Parkinson's disease, or the VY-AADC Program, the program for the treatment of Friedreich's ataxia, or the FA Program, including the development of the VY-FXN01 product candidate, and other undisclosed programs, or the 2019 Discovery Programs. In August 2021, the collaboration was terminated with respect to the VY-AADC Program. Under the FA Program, we and Neurocrine are currently 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. Development of the two targets approved by the joint steering committee under the 2019 Discovery Program is ongoing.

Under the terms of the 2019 Neurocrine Collaboration Agreement, Neurocrine has paid us an upfront payment of $115.0 million. In connection with the 2019 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. The 2019 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for 2019 Collaboration Products under (a) the VY-AADC Program of up to $170.0 million, which we are no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) the FA Program of up to $195.0 million, and (c) each of the two 2019 Discovery Programs of up to $130.0 million per 2019 Discovery Program. We may be entitled to receive aggregate commercial milestone payments of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $1.1 billion.

Neurocrine has also agreed to pay us royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the VY-AADC Program, from the mid-teens to thirty and the low-teens to twenty, respectively, which we are no longer eligible to

receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (c) for each 2019 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 2019 Collaboration Product and terminate on the later of (x) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (y) 10 years from the first commercial sale of the 2019 Collaboration Product in such country and (z) the expiration of regulatory exclusivity in such country, or the 2019 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patents rights related to a 2019 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 2019 Collaboration Product. 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 2019 Royalty Term applicable to such 2019 Collaboration Product in such country.

2023 Neurocrine Collaboration

On January 8, 2023, we entered into a second collaboration agreement, or the 2023 Neurocrine Collaboration Agreement, with Neurocrine for the research, development, manufacture and commercialization of gene therapy products directed to the gene that encodes glucosylceramidase beta 1, or GBA1, for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program, and three new programs focused on the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets, or the 2023 Discovery Programs, and, collectively with the GBA1 Program, the 2023 Neurocrine Programs.

Under the terms of the 2023 Neurocrine Collaboration Agreement, in February 2023 Neurocrine paid us an upfront payment of approximately $136.0 million and approximately $39.0 million for the purchase of 4,395,588 shares of common stock at a price of $8.88 per share. The 2023 Collaboration Agreement also provides for aggregate development milestone payments from Neurocrine for gene therapy products arising under the 2023 Neurocrine Programs, or the 2023 Collaboration Products under (a) the GBA1 Program of up to $985.0 million and (b) each of the three 2023 Discovery Programs of up to $175.0 million for each 2023 Discovery Program. We may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $950.0 million per 2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $275.0 million per 2023 Discovery Program.

The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023, upon expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended. On February 23, 2023, we received the upfront payment, and the shares of our commons stock were issued and sold to Neurocrine pursuant to the applicable stock purchase agreement.

License Agreements

In October 2021, we entered into an option and license agreement with Pfizer, or the Pfizer Agreement, pursuant to which we granted Pfizer options to receive an exclusive license, or the Pfizer License Options, to certain TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes, or Pfizer Transgenes. Effective as of September 30, 2022, Pfizer exercised a Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. In connection with the exercise of the Pfizer License Option for a rare neurological disease, we granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene, or the Pfizer Licensed CNS Products. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to us.

Under the terms of the Pfizer Agreement, Pfizer has paid us an upfront payment of $30 million and a payment of $10 million in connection with the exercise of the Pfizer License Option for a rare neurological disease. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115 million for the first Pfizer Licensed CNS Product to achieve the applicable milestone. On a Pfizer Licensed CNS Product-by-Pfizer Licensed CNS Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175 million per Pfizer Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Pfizer Licensed CNS 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. For a further description of the Pfizer Agreement, refer to Note 9 to the consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2022.

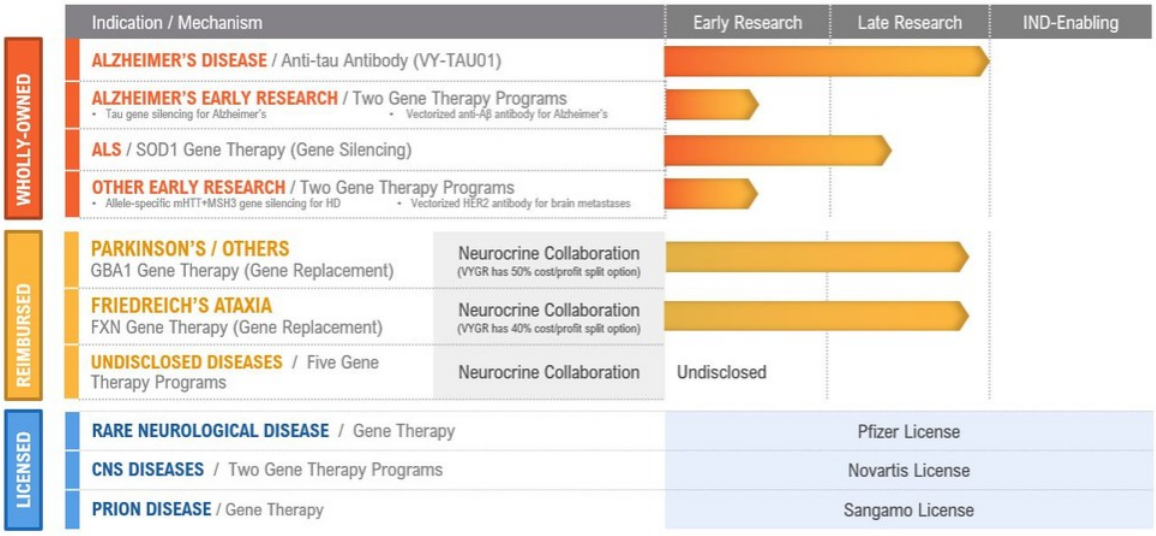
In March 2022, we entered into an option and license agreement, or the Novartis Agreement, with our collaborative partner Novartis. Pursuant to the Novartis Agreement, we have granted Novartis options, or the Novartis License Options, to license TRACER Capsids, or the Novartis Licensed Capsids, for exclusive use with certain targets to develop and commercialize certain adeno-associated virus gene therapy candidates comprised of a Novartis Licensed Capsid and a payload directed to such target, or a Novartis Payload. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from our TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed targets. Novartis elected not to license a capsid for a third target under the Novartis Agreement prior to the expiration of the applicable Novartis License Option. All capsid rights with respect to that target have reverted to us. For a further description of the Novartis Agreement, refer to Note 8, *Commitments and Contingencies*, to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q under the caption "—Novartis Option and License Agreement."

In November 2022, we and Touchlight IP Limited, or Touchlight, entered into a license agreement, or the Touchlight License Agreement, to authorize historical use by us of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process.

Overview of Our Pipeline

We have leveraged our TRACER discovery platform and other gene therapy platforms, our expertise with proprietary antibodies, and our vectorized antibody platform to assemble a pipeline of proprietary AAV gene therapies and passive and vectorized payloads for the treatment of neurological and other diseases which we believe have high unmet medical need. Depending on the disease, we are seeking to develop AAV gene therapies that will use a gene replacement or gene silencing approach, and antibodies that will use a passive administration or vectorized delivery approach. Our goal is to address the underlying causes or the predominant manifestations of specific diseases by significantly increasing or decreasing expression of the relevant proteins in targeted tissues.

Our pipeline of our programs, all of which are in preclinical development, is summarized in the table below:



Wholly-Owned Programs

Anti-Tau Antibody Program for the Treatment of AD

Disease Overview

We are developing proprietary antibodies that selectively target and reduce the spread of pathological tau for the treatment of tauopathies, and our lead indication is AD. The spread of tau pathology closely correlates with disease progression and cognitive decline in AD, which affects approximately 6 million people in the United States and is a growing health care burden to society. Recently, anti-amyloid antibodies have been approved for treatment of AD, and there is substantial remaining unmet medical need.

Our Treatment Approach

We have maintained a long-standing focus on developing proprietary and complimentary approaches to disrupt the progression of tau pathology believed to be central to AD and other tauopathies. Reduction of toxic tau aggregates may slow disease progression and cognitive decline in these diseases. We are exploring passive administration of our anti-tau antibody. Our anti-tau antibodies have differentiated properties including improved targeting of specific regions of tau protein that could offer an improved profile compared to first-generation approaches. We believe that our antibody targeting the C-terminus is highly differentiated from other approaches. Further, we believe that following the clearance of an IND application, clinical assessments utilizing positron emission tomography (PET) imaging of human tau, together with measuring plasma and cerebrospinal fluid biomarkers, have the potential to enable an efficient and accelerated demonstration of human proof-of-biology.

Preclinical Studies

At the Alzheimer's Association International Conference in August 2022, we presented data for our proprietary anti-tau antibodies, targeting the mid-domain and C-terminus with high affinity and showing favorable biophysical characteristics and strong activity in preclinical studies in mouse models. In the P301S seeding-propagation tauopathy mouse model, our C-terminal targeting anti-tau antibody blocked the seeding/propagation of filamentous tau and

demonstrated substantial reduction of induced tau pathology. In March 2023, we presented new data at the AD/PD 2023 Conference highlighting the differentiating characteristics resulting in the selection of lead candidate VY-TAU01.

Program Status

In January 2023, we selected a lead humanized anti-tau antibody candidate to advance against AD. The lead candidate, VY-TAU01, targets the C-terminal domain. VY-TAU01 was selected for its affinity, selectivity, and biophysical characteristics. In April 2023, we received pre-IND written feedback from the FDA for VY-TAU01. Process development and manufacturing at a contracted manufacturer have been initiated, and we expect to initiate a good laboratory practices, or GLP, toxicology study in the second half of 2023 to enable an IND submission in the first half of 2024.

Early Research Programs for the Treatment of AD

During the first quarter of 2023, we announced an early research initiative investigating a gene therapy targeting tau for the treatment of AD. The program combines an siRNA tau knockdown payload with an intravenously delivered TRACER Capsid.

In August 2023, we announced an early research initiative investigating a gene therapy targeting anti-amyloid for the treatment of AD. The program combines a vectorized anti-amyloid antibody with an intravenously delivered TRACER Capsid.

SOD1 Gene Silencing Program for the Treatment of ALS

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER Capsid to treat ALS caused by the SOD1 mutation via a gene silencing approach. SOD1 ALS is typically fatal within approximately three years of diagnosis and impacts approximately 800 patients in the United States, 1,000 patients in the European Union, and 500 patients in Japan. SOD1 mutations in ALS patients are thought to cause a toxic gain-of-function that leads to the degeneration of motor neurons along the entire length of the spinal cord, the brainstem, and the upper motor neurons in the cerebral cortex.

Our Treatment Approach

We believe that a therapeutic delivering a vectorized highly potent small interfering RNA, or siRNA, construct via intravenous administration of an AAV gene therapy with a vectorized siRNA may enable broad CNS knockdown of SOD1, potentially slowing the decline of functional ability in ALS patients with the SOD1 mutation. We believe that a Phase 1 clinical trial to demonstrate reductions in SOD1 in the cerebrospinal fluid and in neurofilament light chain in the plasma will provide evidence of target engagement and the attenuation of motor neuron loss, respectively.

Preclinical Studies

At the American Society of Gene & Cell Therapy 25th Annual Meeting in May 2022, or the ASGCT 2022 Meeting, we presented preclinical data demonstrating robust SOD1 knockdown in all levels of the spinal cord and significant improvements in motor performance, body weight, and survival in an SOD1-ALS mouse model following intravenous delivery of a vectorized siRNA using a mouse BBB-penetrant capsid.

Program Status

We have identified a potent and specific vectorized siRNA transgene that resulted in substantially extended lifespan and motor function when delivered using a BBB-penetrant capsid in a mouse model. We continue to evaluate the data from preclinical studies for this program and now expect to identify a lead development candidate in the second half of 2023. We expect to submit an IND for this program in mid-2025.

Other Early Research Programs

In January 2023, we announced the launch of an updated early research initiative for the treatment of Huntington's disease. The updated gene therapy program, which leverages the latest insights in disease biology, combines an intravenous TRACER Capsid with vectorized siRNAs to enable specific knockdown of mHTT and MSH3. Early data on the selection and vectorization of siRNAs targeting mHTT were presented at the 18th Annual Huntington's Disease Therapeutics Conference held in Dubrovnik, Croatia, in April 2023.

Our wholly-owned early research programs also include a program exploring a vectorized antibody against HER2 for the treatment of brain metastases from HER2+ metastatic breast cancer. Pre-clinical data has demonstrated that our vectorized antibody against HER2 inhibits proliferation and promote antibody-dependent cell cytotoxicity, a process that recruits natural killer cells, macrophages and/or brain-resident innate immune cells called microglia to eliminate tumor cells.

Collaboration Programs

GBA1 Gene Replacement Program for the Treatment of Parkinson's Disease (2023 Neurocrine Collaboration)

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER Capsid to treat diseases linked to GBA1 mutations via a gene replacement approach. Our lead indication for this gene therapy is Parkinson's disease with GBA1 mutations. Mutations in GBA1, the gene encoding the lysosomal glucocerebrosidase enzyme, or Gcase, are the most common genetic risk factor for synucleinopathies such as Parkinson's disease. Parkinson's disease is among the most common neurodegenerative diseases, impacting about one million patients in the United States and more than 10.0 million patients worldwide. Up to 10% of Parkinson's disease patients have a GBA1 mutation, and these mutations increase the risk of Parkinson's disease by approximately 20-fold. GBA1 mutations can decrease the activity of Gcase, leading to the accumulation of Gcase substrates which is linked to alpha-synuclein aggregates, which are thought to be toxic to neurons.

Our Treatment Approach

We believe that restoring Gcase activity may attenuate disease progression and potentially slow neurodegeneration. We anticipate delivering GBA1 via intravenous administration of an AAV gene therapy to enable widespread distribution to multiple affected brain regions and to avoid the need for more invasive approaches. We believe that the measurement of the Gcase substrates such as glucosylsphingosine as cerebrospinal fluid biomarkers may facilitate efficient clinical demonstration of proof-of-biology. Such substrates of the Gcase enzyme are elevated in the cerebrospinal fluid of Parkinson's disease patients who harbor the GBA1 mutation, and we expect that substrate levels would be normalized if our gene therapy restores Gcase enzyme expression in the brain. This gene therapy may also have potential utility in idiopathic Parkinson's disease, where there is evidence of loss of Gcase activity in the substantia nigra in Parkinson's disease patients even in the absence of GBA1 mutations as well as evidence of lysosomal dysfunction in general.

Preclinical Studies

At the ASGCT 2022 Meeting, we presented preclinical data demonstrating CNS target engagement and delivery of therapeutically relevant levels of Gcase in a GBA1 loss of function mouse model, as well as sustained expression for three or more months following intravenous administration. At the AD/PD 2023 Conference, we presented new data from additional mouse efficacy studies showing that three potential development candidates each demonstrated significant improvement in several efficacy biomarkers. We presented data at the ASGCT 2023 Meeting summarizing the mouse findings and additional data from a non-human primate study showing that the administration of a reporter transgene via a single, intravenous dose using two novel BBB-penetrant AAV capsids demonstrated substantially improved biodistribution and gene expression compared to conventional AAV9 in the putamen and substantia nigra, two areas of the brain that are affected in Parkinson's disease.

Program Status

Under the 2023 Neurocrine Collaboration Agreement, we are developing gene therapy products directed to the gene that encodes GBA1 for the treatment of Parkinson's disease and other diseases associated with GBA1. The GBA1 Program is currently in preclinical development. We and Neurocrine are in the process of identifying a lead candidate that will be comprised of a TRACER Capsid, promoter, and transgene. If we and Neurocrine successfully identify a lead development candidate for this program, we plan to complete IND enabling studies to evaluate its safety and efficacy.

Friedreich's Ataxia Program: VY-FXN01 (2019 Neurocrine Collaboration)

Disease Overview

Friedreich's ataxia is 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. The typical age of onset is 10 to 12 years, and life expectancy is severely reduced with patients generally dying of neurological and cardiac complications between the ages of 35 and 45. According to the Friedreich's ataxia Research Alliance, there are approximately 6,400 patients living with the disease in the United States. While one treatment for Friedreich's ataxia has recently been approved by the FDA, there remains a significant unmet need.

Friedreich's ataxia patients have mutations of the FXN gene that reduce production of the frataxin protein, resulting in the degeneration of sensory pathways and a variety of debilitating symptoms. Friedreich's ataxia is an autosomal recessive disorder, meaning that a person must obtain a defective copy of the FXN gene from both parents in order to develop the condition. One healthy copy of the FXN gene, or 50% of normal frataxin protein levels, is sufficient to prevent the disease phenotype. We therefore believe that restoring FXN protein levels to at least 50% of normal levels by AAV gene therapy might lead to a successful therapy.

Our Treatment Approach

We are seeking to develop an AAV gene therapy approach that we believe will deliver a functional version of the FXN gene to the sensory pathways through intravenous injection. We think this approach has the potential to improve balance, ability to walk, sensory capability, coordination, strength and functional capacity of Friedreich's ataxia patients. Most Friedreich's ataxia patients produce low levels of the frataxin protein, which although insufficient to prevent the disease, exposes the patient's immune system to frataxin. This reduces the likelihood that the FXN protein expressed by AAV gene therapy will trigger a harmful immune response.

Preclinical Studies

We initially conducted preclinical studies in non-human primates and achieved high FXN expression levels within the target sensory ganglia, or clusters of neurons, along the spinal region following intrathecal injection. More recently, we conducted preclinical studies in non-human primates with intravenous injection and achieved target FXN expression levels within sensory ganglia and the heart. The levels of FXN expression observed in the brain using an AAV vector were, on average, greater than FXN levels present in control normal human brain tissue. FXN expression was also observed in the cerebellar dentate nucleus, another area of the CNS that is often affected in Friedreich's ataxia, and that is often considered difficult to target therapeutically.

Our Program Status

As part of the 2019 Neurocrine Collaboration Agreement, we are developing VY-FXN01 for the treatment of Friedreich's ataxia. VY-FXN01 is currently in preclinical development. We and Neurocrine are in the process of identifying a lead candidate that will be comprised of a capsid, promoter, and FXN transgene and are evaluating the potential use of TRACER Capsids in the program. We are completing AAV capsid biodistribution experiments to confirm capsid serotypes that effectively transduce disease target tissues in non-human primates following intravenous

injection. If we and Neurocrine successfully identify a development lead candidate for this program, we plan to complete IND enabling studies to evaluate its safety and efficacy.

Accumulated Deficit

We have a history of incurring significant losses. As of June 30, 2023, we had an accumulated deficit of $291.7 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct preclinical development activities and initiate GLP toxicology studies and clinical trials in connection with our tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques by continuing to develop our proprietary antibodies and vectorized antibody platform;
- increase our investment in and support for our TRACER discovery platform to facilitate the selection of AAV capsids and expand our investment to discover TRACER Capsids with broad tropism in CNS and other tissues with cell-specific transduction properties for particular therapeutic applications;
- increase our investment in the identification of receptors for our TRACER Capsids and related initiatives to leverage these receptors for further novel capsid discovery and the delivery of non-viral genetic medicines;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs, including our FA Program pursuant to the 2019 Neurocrine Collaboration Agreement, and our GBA1 gene therapy program pursuant to our 2023 Neurocrine Collaboration Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other 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;
- seek marketing and regulatory approvals for any of our product candidates that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- expand 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 three months ended June 30, 2023, we recognized $2.9 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $1.6 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, and $0.3 million of other collaboration revenue. For the six months ended June 30, 2023, we recognized $79.0 million of collaboration revenue from the Novartis Agreement, $72.4 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $3.6 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, and $0.3 million of other collaboration revenue.

For additional information about our revenue recognition policy related to collaborations and a description of the key terms of the 2023 Neurocrine Collaboration Agreement and the Novartis Agreement, 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 the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement, the Pfizer Agreement, the Novartis Agreement, and any other strategic collaborations and out-licensing arrangements we may enter into in the future. If our development efforts are successful, we may also generate revenue from product sales.

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, gene therapy platform, proprietary antibodies, and vectorized antibody 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, preclinical activities, manufacturing and production design on our behalf;
- the cost of purchasing laboratory supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation and maintenance expenses;
- the cost of securing and protecting intellectual property rights associated with our research and development activities; 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.

Research and development activities are central to our business model. We are in the early stages of development of our product candidates. During the six months ended June 30, 2023, our research and development expenses have increased as compared to the amounts recorded in the same period in the prior year. 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 continue to increase. However, 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.

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. Our expenses will increase if:

- we are required by the FDA or the European Medicines Agency 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
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

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.

During the six months ended June 30, 2023, our general and administrative expenses have increased as compared to the amount recorded in the same period in prior year. As our development programs progress and we identify product candidates and initiate preclinical studies and clinical trials, we will continue to expect general and administrative expenses to increase to support these additional research and development activities.

Other Income, Net

Other income, net for the six months ended June 30, 2023, consists primarily of interest income on our marketable securities.

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 six months ended June 30, 2023, as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022. 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 March 7, 2023.

Results of Operations

Comparison of the three months ended June 30, 2023 and 2022

The following table summarizes our results of operations for the three months ended June 30, 2023 and 2022, together with the changes in those items in dollars:

	Three Months Ended June 30,		
	2023	2022	Change
	(in thousands)		
Collaboration revenue	$ 4,853	$ 712	$ 4,141
Operating expenses:			
Research and development	21,985	12,527	9,458
General and administrative	8,294	7,552	742
Total operating expenses	30,279	20,079	10,200
Other income, net:			
Interest income	3,274	219	3,055
Other income	3	61	(58)
Total other income, net	3,277	280	2,997
Net loss before income taxes	$ (22,149)	$ (19,087)	$ (3,062)

Collaboration Revenue

Collaboration revenue was $4.9 million and $0.7 million for the three months ended June 30, 2023 and 2022, respectively. The increase in collaboration revenue was a result of, during the three months ended June 30, 2023, we recognized $2.9 million of revenue associated with the 2023 Neurocrine Collaboration Agreement, $1.6 million of revenue associated with the 2019 Neurocrine Collaboration Agreement, and $0.3 million of other collaboration revenue. During the three months ended June 30, 2022, collaboration revenue was entirely related to research services and cost reimbursement from the 2019 Neurocrine Collaboration Agreement.

Research and Development Expense

Research and development expense increased by $9.5 million from $12.5 million for the three months ended June 30, 2022, to $22.0 million for the three months ended June 30, 2023. The following table summarizes our research and development expenses for the three months ended June 30, 2023 and 2022, together with the changes in those items in dollars:

	Three Months Ended June 30,		
	2023	2022	Change
	(in thousands)		
Employee and consultant	$ 9,888	$ 6,924	$ 2,964
External research and development	8,555	2,794	5,761
Facilities and other	1,608	1,022	586
Professional fees	1,934	1,787	147
Total research and development expenses	$ 21,985	$ 12,527	$ 9,458

The increase in research and development expense for the three months ended June 30, 2023 was primarily attributable to the following:

- approximately $3.0 million for increased employee and consultant related costs associated with higher headcount in research and development functions as compared to the three months ended June 30, 2022;

- approximately $5.8 million for external research and development costs related to increased program-related spend primarily on anti-tau antibody for AD and ALS; and

- approximately $0.5 million for increased facility and other costs primarily related to the gain recorded in conjunction with the terminated lease at 75 Sidney Street during the three months ended June 30, 2022.

General and Administrative Expense

General and administrative expense increased by $0.7 million from $7.6 million for the three months ended June 30, 2022, to $8.3 million for the three months ended June 30, 2023. The increase in general and administrative expense was primarily attributable to the following:

- approximately $0.2 million for increased compensation costs and stock-based compensation associated with higher headcount in general and administrative functions as compared to the three months ended June 30, 2022;

- approximately $0.4 million for increased facility and other costs primarily related to the gain recorded in conjunction with the terminated lease at 75 Sidney Street during the second quarter of 2022; and

- approximately $0.1 million for increased legal and patent expenses.

Other Income, net

Other income, net of approximately $3.3 million was recognized during the three months ended June 30, 2023, as compared to $0.3 million during the three months ended June 30, 2022. Other income, net during both the three months ended June 30, 2023 and 2022 was related to interest income on marketable securities balances. The increase was due to higher cash balances and increased interest rates during the three months ended June 30, 2023, as compared to the three months ended June 30, 2022.

Comparison of the six months ended June 30, 2023 and 2022

The following table summarizes our results of operations for the six months ended June 30, 2023 and 2022, together with the changes in those items in dollars:

	Six Months Ended June 30,		
	2023	2022	Change
	(in thousands)		
Collaboration revenue	$ 155,333	$ 1,371	$ 153,962
Operating expenses:			
Research and development	40,553	26,876	13,677
General and administrative	17,322	15,211	2,111
Total operating expenses	57,875	42,087	15,788
Other income, net:			
Interest income	5,138	271	4,867
Other income	3	39	(36)
Total other income, net	5,141	310	4,831
Net income (loss) before income taxes	$ 102,599	$ (40,406)	$ 143,005

Collaboration Revenue

Collaboration revenue was $155.3 million and $1.4 million for the six months ended June 30, 2023 and 2022, respectively. The increase in collaboration revenue was the result of $79.0 million in revenue recognized during the six months ended June 30, 2023, in connection with Novartis' decision to exercise two Novartis License Options, along with the expiration of a third Novartis License Option. In addition, during the six months ended June 30, 2023, we recognized $72.4 million of revenue associated with the 2023 Neurocrine Collaboration Agreement, $3.6 million of revenue associated with the 2019 Neurocrine Collaboration Agreement, and $0.3 million of other collaboration revenue. During the six months ended June 30, 2022, collaboration revenue was entirely related to research services and cost reimbursement from the 2019 Neurocrine Collaboration Agreement.

Research and Development Expense

Research and development expense increased by $13.7 million from $26.9 million for the six months ended June 30, 2022, to $40.6 million for the six months ended June 30, 2023. The following table summarizes our research and development expenses for the six months ended June 30, 2023 and 2022, together with the changes in those items in dollars:

	Six Months Ended June 30,		
	2023	2022	Change
	(in thousands)		
Employee and consultant	$ 20,016	$ 13,567	$ 6,449
External research and development	13,388	4,595	8,793
Facilities and other	3,203	4,626	(1,423)
Professional fees	3,946	4,088	(142)
Total research and development expenses	$ 40,553	$ 26,876	$ 13,677

The increase in research and development expense for the six months ended June 30, 2023 was primarily attributable to the following:

- approximately $6.4 million for increased employee and consultant related costs associated with higher headcount in research and development functions as compared to the same period in the prior year;
- approximately $8.8 million for external research and development costs related to increased program-related spend primarily on anti-tau antibody for AD and ALS, and the fee due to Touchlight in conjunction with Novartis' exercise of two Novartis License Options; and
- partially offset by approximately $1.5 million for decreased facility and other costs primarily related to the termination of the lease for office and laboratory space at 75 Sidney Street during the second quarter of 2022.

General and Administrative Expense

General and administrative expense increased by $2.1 million from $15.2 million for the six months ended June 30, 2023, to $17.3 million for the six months ended June 30, 2023. The increase in general and administrative expense was primarily attributable to the following:

- approximately $1.6 million for increased compensation costs and stock-based compensation associated with higher headcount in general and administrative functions as compared to the same period in the prior year;
- approximately $0.7 million for increased legal and patent expenses; and

- offset by approximately $0.2 million in decreased facility and other costs primarily related to the termination of the lease for office and laboratory space at 75 Sidney Street during the second quarter of 2022.

Other Income, net

Other income, net of approximately $5.1 million was recognized during the six months ended June 30, 2023, as compared to $0.3 million during the six months ended June 30, 2022. Other income, net during both the six months ended June 30, 2023, and 2022 was primarily related to interest income on marketable securities balances. The increase is due to higher cash balances and increased interest rates during the six months ended June 30, 2023, as compared to the six months ended June 30, 2022.

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, strategic collaborations and option and license arrangements, including our 2019 Neurocrine Collaboration Agreement and 2023 Neurocrine Collaboration Agreement, our ongoing option and license arrangements with Pfizer and Novartis under the Pfizer Agreement and the Novartis Agreement, respectively, and with our prior collaboration agreements.

As of June 30, 2023, we had cash, cash equivalents, and marketable securities of $272.7 million. Based upon our current operating plans, we expect that our existing cash, cash equivalents, and marketable securities at June 30, 2023, along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine, will enable us to meet our planned operating expenses and capital expenditure requirements into 2025.

Cash Flows

The following table provides information regarding our cash flows for the six months ended June 30, 2023 and 2022:

	Six Months Ended June 30,	
	2023	2022
	(in thousands)	
Net cash provided (used in) by:		
Operating activities	$ 122,730	$ 15,941
Investing activities	(10,172)	(56,128)
Financing activities	32,854	819
Net increase (decrease) in cash, cash equivalents, and restricted cash	$ 145,412	$ (39,368)

Net Cash Provided by Operating Activities

Net cash provided by operating activities was $122.7 million during the six months ended June 30, 2023, compared to $15.9 million of net cash provided by operating activities during the six months ended June 30, 2022. The increase was primarily due to our net income for the six months ended June 30, 2023 of $101.6 million as compared to our net loss for the six months ended June 30, 2022 of $40.4 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $10.2 million during the six months ended June 30, 2023, compared to $56.1 million during the six months ended June 30, 2022. The change was primarily due to increased purchases of marketable securities during the six months ended June 30, 2022 as compared to the six month ended June 30, 2023.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $32.9 million during the six months ended June 30, 2023, driven by proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement.

Funding Requirements

Our expenses increased during the six months ended June 30, 2023, as compared with the six months ended June 30, 2022, as our development programs progressed and we increased headcount. We expect our expenses to continue to increase as we continue the research and development of, conduct clinical trials of, and seek marketing approval for, our product candidates and as we continue to enter into or conduct activities in connection with 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, executing financial statement controls, satisfying regulatory and quality standards, fulfilling healthcare compliance requirements, and maintaining product, clinical trial and directors' and officers' liability insurance coverage. We also anticipate the cost of goods and services and the levels of compensation paid to employee will increase due to inflationary conditions existing in the general economy. 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 plans, we expect that our existing cash, cash equivalents, and marketable securities at June 30, 2023, along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine, will enable us to meet our planned operating expenses and capital expenditure requirements into 2025. 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;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and option and license agreements and any similar arrangement we may enter into in the future, including any research and development costs for which we are responsible, and our receipt of any future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty payments, under any collaboration or license agreements to which we might become a party, such as the Touchlight License Agreement;
- 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 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 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 consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partners and licensors for reimbursement of certain research and development expenses, potential option exercises, the achievement of specified regulatory and commercial milestones, and royalty payments under our collaboration, and option and license agreements, as applicable. 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, and 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 option and license 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 generally are cancelable at any time by us, 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. In certain instances, we are also obligated to pay our licensors royalties based on sales of products, if approved, using the intellectual property licensed under 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. For more information, refer to Note 6 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

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 costs of labor, goods, and services. We do not believe that inflation had a material effect on our business, financial condition, or results of operations during the six months ended June 30, 2023.

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 June 30, 2023. 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 June 30, 2023, 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 six months ended June 30, 2023, 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

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of any such matters cannot be predicted with certainty, as of June 30, 2023, we were not party to any material pending proceedings. No material governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

ITEM 1A. RISK FACTORS

We are subject to a number of risks that could adversely affect our business, results of operations financial condition and future prospects including those identified in our Annual Report on Form 10-K for the year ended December 31, 2022, filed with the SEC on March 7, 2023.

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	Employment Agreement by and between the Registrant and Jacquelyn Fahey Sandell, effective as of July 5, 2023.	8-K	10.1	07/10/2023	001-37625	
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	

+ 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: August 3, 2023

VOYAGER THERAPEUTICS, INC.

By: */s/ Alfred Sandrock, M.D., Ph.D.*
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

By: */s/ Peter P. Pfreundschuh*
Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended June 30, 2023 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: August 3, 2023

/s/ Alfred Sandrock, M.D., Ph.D.

Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Exhibit 31.2

Certification

I, Peter P. Pfreundschuh, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended June 30, 2023 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: August 3, 2023

/s/ Peter P. Pfreundschuh

Peter P. Pfreundschuh
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 June 30, 2023, 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 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: August 3, 2023

/s/ Alfred Sandrock, M.D., Ph.D.

Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Date: August 3, 2023

/s/ Peter P. Pfreundschuh

Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)