

---

---

**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 March 31, 2025

**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)

**75 Hayden Avenue,  
Lexington, Massachusetts**  
(Address of principal executive offices)

**46-3003182**  
(I.R.S. Employer  
Identification No.)

**02421**  
(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	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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 May 1, 2025, was 55,336,446.

---

---

## 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 proprietary adeno-associated virus, or AAV, gene therapy, antibody, and non-viral therapeutic product candidates;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) discovery platform, our vectorized antibody platform, our non-viral therapeutics platform, and our proprietary antibody, gene therapy, vectorized antibody, and non-viral therapeutic programs;
- our ability to identify and optimize product candidates, proprietary AAV capsids, and non-viral blood-brain-barrier shuttles;
- our strategic collaborations and licensing agreements with, and funding from, our collaboration partners Neurocrine Biosciences, Inc. and Novartis Pharma AG, or Novartis, and our licensee Alexion, AstraZeneca Rare Disease (successor-in-interest to former licensee Pfizer Inc.);
- our ongoing and planned clinical trials, 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 future revenue, expenses, contingent liabilities, existing cash resources, capital requirements and cash runway;
- 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;

## [Table of Contents](#)

- 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 11, 2025, particularly in “Part I, Item 1A — Risk Factors,” and, if applicable, this Quarterly Report 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, licenses, joint ventures or investments we may enter into or 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.

We obtained the statistical and other industry and market data in this Quarterly Report on Form 10-Q and the documents we have filed as exhibits to the Quarterly Report on Form 10-Q from our own internal estimates and research, as well as from industry and general publications and research, surveys, studies and trials conducted by third parties. Some data is also based on our good faith estimates, which are derived from management’s knowledge of the industry and independent sources. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, while we believe the market opportunity information included in this Quarterly Report on Form 10-Q and the documents we have filed as exhibits to the Quarterly Report on Form 10-Q is reliable and is based upon reasonable assumptions, such data involves risks and uncertainties and are subject to change based on various factors, including those discussed under “Risk Factors” and in the documents we have filed as exhibits to the Quarterly Report on Form 10-Q. In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and investors are cautioned not to unduly rely upon these statements.

We own various U.S. federal trademark registrations and applications and unregistered trademarks, including our corporate logo. This Quarterly Report on Form 10-Q and the documents filed as exhibits to the Quarterly Report on Form 10-Q contain references to trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q and the information incorporated herein, including logos, artwork, and other visual displays, that may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks, service marks or trade names. We do not intend our use or display of other companies’ trade names, service marks or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies. All trademarks, service marks and trade names included or incorporated by reference into this Quarterly Report on Form 10-Q and the documents filed as exhibits to the Quarterly Report on Form 10-Q are the property of their respective owners.

**VOYAGER THERAPEUTICS, INC.**

**FORM 10-Q**

**TABLE OF CONTENTS**

	Page
<a href="#">PART I. FINANCIAL INFORMATION</a>	
<a href="#">ITEM 1. CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)</a>	
<a href="#">CONDENSED CONSOLIDATED BALANCE SHEETS</a>	5
<a href="#">CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS</a>	6
<a href="#">CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY</a>	7
<a href="#">CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS</a>	8
<a href="#">NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS</a>	9
<a href="#">ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</a>	20
<a href="#">ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK</a>	33
<a href="#">ITEM 4. CONTROLS AND PROCEDURES</a>	33
<a href="#">PART II. OTHER INFORMATION</a>	
<a href="#">ITEM 1. LEGAL PROCEEDINGS</a>	34
<a href="#">ITEM 1A. RISK FACTORS</a>	34
<a href="#">ITEM 5. OTHER INFORMATION</a>	35
<a href="#">ITEM 6. EXHIBITS</a>	36
<a href="#">SIGNATURES</a>	37

**PART I. FINANCIAL INFORMATION**

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

	<u>March 31,</u>	<u>December 31,</u>
	<u>2025</u>	<u>2024</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 74,755	\$ 71,367
Marketable securities	161,264	195,317
Accounts receivable	1,623	1,504
Related party collaboration receivable (see note 8)	487	676
Prepaid expenses and other current assets	7,077	7,945
Total current assets	245,206	276,809
Property and equipment, net	13,929	14,314
Restricted cash	2,874	2,874
Marketable securities, non-current	59,103	65,704
Operating lease, right-of-use assets	32,128	33,349
Total assets	<u>\$ 353,240</u>	<u>\$ 393,050</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 2,634	\$ 3,996
Accrued expenses	9,053	14,171
Operating lease liabilities	7,430	7,227
Deferred revenue (see Note 8)	21,095	24,394
Total current liabilities	40,212	49,788
Operating lease liabilities, net of current portion	34,566	36,499
Deferred revenue, net of current portion	4,760	6,003
Other non-current liabilities	1,000	1,000
Total liabilities	80,538	93,290
Commitments and contingencies (see note 7)		
Stockholders' equity:		
Preferred stock, \$0.001 par value: 5,000,000 shares authorized at March 31, 2025 and December 31, 2024; no shares issued and outstanding at March 31, 2025 and December 31, 2024	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized at March 31, 2025 and December 31, 2024; 55,209,485 and 54,731,316 shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively	55	55
Additional paid-in capital	630,051	626,296
Accumulated other comprehensive loss	(199)	(407)
Accumulated deficit	(357,205)	(326,184)
Total stockholders' equity	272,702	299,760
Total liabilities and stockholders' equity	<u>\$ 353,240</u>	<u>\$ 393,050</u>

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

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

	Three Months Ended	
	March 31,	
	2025	2024
Collaboration revenue (including from related parties, see note 12)	\$ 6,473	\$ 19,516
Operating expenses:		
Research and development	31,526	27,092
General and administrative	9,640	8,607
Total operating expenses	<u>41,166</u>	<u>35,699</u>
Operating loss	(34,693)	(16,183)
Other income, net:		
Interest income	3,291	4,867
Other income	418	—
Total other income, net	<u>3,709</u>	<u>4,867</u>
Loss before income taxes	(30,984)	(11,316)
Income tax provision	37	14
Net loss	<u>\$ (31,021)</u>	<u>\$ (11,330)</u>
Other comprehensive gain (loss):		
Net unrealized gain (loss) on available-for-sale securities	208	(458)
Total other comprehensive gain (loss)	<u>208</u>	<u>(458)</u>
Comprehensive loss	<u>\$ (30,813)</u>	<u>\$ (11,788)</u>
Net loss per share, basic	<u>\$ (0.53)</u>	<u>\$ (0.20)</u>
Net loss per share, diluted	<u>\$ (0.53)</u>	<u>\$ (0.20)</u>
Weighted-average common shares outstanding, basic	<u>58,349,769</u>	<u>57,117,046</u>
Weighted-average common shares outstanding, diluted	<u>58,349,769</u>	<u>57,117,046</u>

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

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

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Comprehensive (Loss) Income	Accumulated Deficit	Stockholders' Equity
<b>Balance at December 31, 2023</b>	44,038,333	\$ 44	\$ 497,506	\$ (48)	\$ (261,182)	\$ 236,320
Exercises of vested stock options	32,500	—	78	—	—	78
Vesting of restricted stock units	324,520	—	—	—	—	—
Issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement	2,145,002	2	19,303	—	—	19,305
Issuance of common stock and pre-funded warrants in connection with underwritten public offering	7,777,778	8	93,465	—	—	93,473
Stock-based compensation expense	—	—	3,498	—	—	3,498
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(458)	—	(458)
Net loss	—	—	—	—	(11,330)	(11,330)
<b>Balance at March 31, 2024</b>	<u>54,318,133</u>	<u>\$ 54</u>	<u>\$ 613,850</u>	<u>\$ (506)</u>	<u>\$ (272,512)</u>	<u>\$ 340,886</u>
<b>Balance at December 31, 2024</b>	<u>54,731,316</u>	<u>55</u>	<u>626,296</u>	<u>(407)</u>	<u>(326,184)</u>	<u>299,760</u>
Exercises of vested stock options	27,613	—	82	—	—	82
Vesting of restricted stock units	450,556	—	—	—	—	—
Stock-based compensation expense	—	—	3,673	—	—	3,673
Unrealized gain on available-for-sale securities, net of tax	—	—	—	208	—	208
Net loss	—	—	—	—	(31,021)	(31,021)
<b>Balance at March 31, 2025</b>	<u>55,209,485</u>	<u>\$ 55</u>	<u>\$ 630,051</u>	<u>\$ (199)</u>	<u>\$ (357,205)</u>	<u>\$ 272,702</u>

*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)*

	Three Months Ended	
	March 31,	
	2025	2024
<b>Cash flow from operating activities</b>		
Net loss	\$ (31,021)	\$ (11,330)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:		
Stock-based compensation expense	3,673	3,573
Depreciation	1,014	1,196
Amortization of premiums and discounts on marketable securities	(998)	(1,931)
Loss on disposal of fixed assets	30	59
Non-cash lease expense	1,221	1,057
Changes in operating assets and liabilities:		
Accounts receivable	(119)	79,313
Related party collaboration receivable	189	721
Prepaid expenses and other current assets	868	(794)
Other non-current assets	—	(15)
Accounts payable	(1,362)	5,975
Accrued expenses	(5,118)	(9,305)
Operating lease liabilities	(1,730)	891
Deferred revenue	(4,542)	(10,643)
Net cash (used in) provided by operating activities	(37,895)	58,767
<b>Cash flow from investing activities</b>		
Purchases of property and equipment	(658)	(2,141)
Purchases of marketable securities	(76,924)	(203,852)
Proceeds from sales and maturities of marketable securities	118,783	109,928
Net cash provided by (used in) investing activities	41,201	(96,065)
<b>Cash flow from financing activities</b>		
Proceeds from the exercise of stock options	82	78
Proceeds from the issuance of common stock and pre-funded warrants in connection with the underwritten public offering	—	93,473
Proceeds from the issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement	—	19,305
Net cash provided by financing activities	82	112,856
Net increase in cash, cash equivalents, and restricted cash	3,388	75,558
Cash, cash equivalents, and restricted cash, beginning of period	74,241	70,395
Cash, cash equivalents, and restricted cash, end of period	<u>\$ 77,629</u>	<u>\$ 145,953</u>
<b>Supplemental disclosure of cash and non-cash activities</b>		
Operating lease right-of-use asset obtained in exchange for operating lease liability	\$ —	\$ 26,751

*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 whose mission is to leverage the power of human genetics to modify the course of and ultimately cure neurological diseases. Its pipeline includes programs for Alzheimer’s disease (“AD”); Friedreich’s ataxia (“FA”); Parkinson’s disease; and multiple other diseases of the central nervous system (“CNS”). Its pipeline also includes other programs it wholly owns and programs which it is advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease (“Alexion”); Novartis Pharma AG (“Novartis”); and Neurocrine Biosciences, Inc. (“Neurocrine”) for the treatment of multiple other diseases of CNS.

Many of its programs are derived from the Company’s TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) adeno-associated virus (“AAV”) capsid discovery platform, which it has used to generate novel capsids (“TRACER Capsids”) and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous (“IV”) dosing. TRACER is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier (“BBB”) and enhanced CNS tropism in multiple species, including non-human primates. The Company is also developing a second, non-viral therapeutics platform focused on non-viral receptor-mediated transport across the BBB.

The Company has a history of incurring annual net operating losses. As of March 31, 2025, the Company had an accumulated deficit of \$357.2 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, option exercise payments, milestone payments, and cost reimbursements associated with its prior and ongoing collaborations and license agreements.

As of March 31, 2025, the Company had cash, cash equivalents, and marketable securities of \$295.1 million. Based upon the Company’s current operating plans, the Company expects that its existing cash, cash equivalents, and marketable securities at March 31, 2025 to be sufficient to meet the Company’s planned operating expenses and capital expenditure requirements for at least twelve months from the issuance of these consolidated financial statements.

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, 2024, as filed with the Securities and Exchange Commission (“SEC”) on March 11, 2025. 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 and Accounting Standards Updates of the Financial Accounting Standards Board.

*Principles of Consolidation*

The unaudited interim condensed 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, 2024. 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, incremental borrowing rate for leases, 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 fiscal year ended December 31, 2024.

### 3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of March 31, 2025 and December 31, 2024, are as follows:

<u>Assets</u>	<u>Total</u>	<u>Quoted Prices in Active Markets for Identical Assets (Level 1)</u>	<u>Significant Other Observable Inputs (Level 2)</u>	<u>Significant Unobservable Inputs (Level 3)</u>
<i>(in thousands)</i>				
<b>March 31, 2025</b>				
Money market funds included in cash and cash equivalents	\$ 68,727	\$ 68,727	\$ —	\$ —
Marketable securities:				
U.S. Treasury notes	130,578	130,578	—	—
U.S. Government agency securities	16,404	16,404	—	—
Certificates of deposit	3,320	—	3,320	—
Corporate bonds	61,394	—	61,394	—
Commercial paper	8,671	—	8,671	—
Total money market funds and marketable securities	<u>\$ 289,094</u>	<u>\$ 215,709</u>	<u>\$ 73,385</u>	<u>\$ —</u>
<b>December 31, 2024</b>				
Money market funds included in cash and cash equivalents	\$ 59,658	\$ 59,658	\$ —	\$ —
Marketable securities:				
U.S. Treasury notes	125,783	125,783	—	—
U.S. Government agency securities	27,518	27,518	—	—
Certificates of deposit	4,286	—	4,286	—
Corporate bonds	94,976	—	94,976	—
Commercial paper	8,458	—	8,458	—
Total money market funds and marketable securities	<u>\$ 320,679</u>	<u>\$ 212,959</u>	<u>\$ 107,720</u>	<u>\$ —</u>

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, including certificates of deposit, corporate 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 equivalents and available-for-sale marketable securities

Cash equivalents and available-for-sale marketable securities included the following as of March 31, 2025 and December 31, 2024:

	Amortized Cost	Unrealized Gains	Unrealized Losses		Fair Value
			Less than 12 months	Greater than 12 months	
<i>(in thousands)</i>					
<b>As of March 31, 2025</b>					
Money market funds included in cash and cash equivalents	\$ 68,731	\$ —	\$ (4)	\$ —	\$ 68,727
Marketable securities:					
U.S. Treasury notes	130,594	96	(55)	(57)	130,578
U.S. Government agency securities	16,412	1	(3)	(6)	16,404
Certificates of deposit	3,320	—	—	—	3,320
Corporate bonds	61,438	8	(50)	(2)	61,394
Commercial paper	8,671	—	—	—	8,671
Total money market funds and marketable securities	<u>\$ 289,166</u>	<u>\$ 105</u>	<u>\$ (112)</u>	<u>\$ (65)</u>	<u>\$ 289,094</u>
<b>As of December 31, 2024</b>					
Money market funds included in cash and cash equivalents	\$ 59,658	—	—	—	\$ 59,658
Marketable securities:					
U.S. Treasury notes	125,996	32	(45)	(200)	125,783
U.S. Government agency securities	27,552	6	(5)	(35)	27,518
Certificates of deposit	4,280	6	—	—	4,286
Corporate bonds	95,016	18	(58)	—	94,976
Commercial paper	8,456	2	—	—	8,458
Total money market funds and marketable securities	<u>\$ 320,958</u>	<u>\$ 64</u>	<u>\$ (108)</u>	<u>\$ (235)</u>	<u>\$ 320,679</u>

All of the Company's marketable securities at March 31, 2025 and December 31, 2024, have a contractual maturity of two years 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 income (loss). Changes in the allowance for credit losses are recorded as a provision for (or reversal of) credit loss expense in other income, net 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.

As of March 31, 2025 and December 31, 2024, the Company held 70 and 97 marketable securities that were in an unrealized loss position, representing \$128.3 million and \$156.3 million in fair value, respectively. The unrealized losses as of March 31, 2025 and December 31, 2024, were attributable to changes in interest rates and 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 amounts shown in the condensed consolidated statements of cash flows:

	<u>As of</u> <u>March 31,</u> <u>2025</u>	<u>As of</u> <u>December 31,</u> <u>2024</u>
	<i>(in thousands)</i>	
Cash and cash equivalents	\$ 74,755	\$ 71,367
Restricted cash included in deposits and other non-current assets	2,874	2,874
Total cash, cash equivalents, and restricted cash	<u>\$ 77,629</u>	<u>\$ 74,241</u>

## 5. Accrued expenses

Accrued expenses as of March 31, 2025 and December 31, 2024, consist of the following:

	<u>As of March 31,</u> <u>2025</u>	<u>As of December 31,</u> <u>2024</u>
	<i>(in thousands)</i>	
Employee compensation costs	\$ 2,294	\$ 6,752
Research and development costs	4,709	5,246
Accrued goods and services	1,230	1,201
Professional services	820	972
Total	<u>\$ 9,053</u>	<u>\$ 14,171</u>

## 6. Lease obligation

### *Operating Leases*

As of March 31, 2025, the Company has a lease for laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031, and a lease for additional office and laboratory space at 64 Sidney Street in Cambridge, Massachusetts (the “Cambridge Facility”) through November 30, 2026.

In August 2023, the Company entered into a first amendment (the “First Amendment”) to its existing lease for laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts, pursuant to which the Company agreed to lease approximately 61,307 square feet of additional office and laboratory space through January 31, 2031. The commencement date for the First Amendment occurred on February 1, 2024. The expected contractual obligation under the First Amendment to the Company’s existing lease is approximately \$35.4 million, to be paid over the remaining seven-year term of the lease.

The Company’s lease agreements require the Company to maintain a cash deposit or irrevocable letter of credit in the aggregate amount of \$2.9 million payable to its landlords as security for the performance of its obligations under the leases. These amounts are recorded as restricted cash in the accompanying condensed consolidated balance sheets.

In June 2024, the Company vacated the Cambridge Facility. The Company recorded an impairment charge of \$2.8 million to operating expenses during the second quarter of 2024 as a result of the carrying value of the leased office and laboratory space asset group exceeding the undiscounted cash flows projected from a planned sublease of the facility. The impairment charge reduced the carrying value of the leased office and laboratory space asset group by \$2.8 million. In August 2024, the Company executed the sublease of the Cambridge Facility (the “Sublease Agreement”). Payments received under the Sublease Agreement are included in other income in the condensed consolidated statements of operations and comprehensive loss. Subject to certain conditions set forth therein, the Sublease Agreement terminates simultaneously with the Company’s lease for the Cambridge Facility.

Total lease cost for operating leases of approximately \$1.9 million and \$1.7 million was incurred during the three months ended March 31, 2025 and 2024, respectively.

The following table summarizes the operating sublease income generated under the Sublease Agreement for the three months ended March 31, 2025 and 2024:

	Three Months Ended	
	March 31,	
	2025	2024
	<i>(in thousands)</i>	
Operating sublease income	\$ 440	\$ —

Future minimum lease payments due under operating leases are as follows:

Year Ending December 31,	Total Minimum Lease Payments <i>(in thousands)</i>
2025	\$ 7,460
2026	9,983
2027	7,763
2028	7,996
2029	8,235
Thereafter	8,710
Total future minimum lease payments	\$ 50,147
Less: imputed interest	(8,151)
Total operating lease liabilities	\$ 41,996
Reported as:	
Operating lease liabilities	\$ 7,430
Operating lease liabilities, net of current portion	34,566
Total operating lease liabilities	\$ 41,996

## 7. Commitments, contingencies and other liabilities

### *Other Agreements*

In 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, as amended in 2022, provides that the Company is obligated to 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. In 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 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 March 31, 2025 or December 31, 2024. The Company did not have contingency reserves established for any litigation liabilities as of March 31, 2025 or December 31, 2024.

## 8. Significant agreements

The Company's significant agreements are described in Note 9 of the December 31, 2024, consolidated financial statements included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2024. During the three months ended March 31, 2025, there were no material changes to the Company's collaboration agreements or option and license agreements and the Company did not enter into any new collaboration or license agreements. The Company recorded collaboration revenue of \$6.5 million and \$19.5 million during the three months ended March 31, 2025 and 2024, respectively.

### *Related Party Collaboration Receivable and Deferred Revenue*

The following table presents changes in the balances of the Company's related party collaboration receivable and contract liabilities (reflected as deferred revenue in the condensed consolidated balance sheets) for the collaboration and license agreement the Company entered into with Neurocrine in January 2023 (the "2023 Neurocrine Collaboration Agreement") and the collaboration and license agreement the Company entered into with Neurocrine in January 2019 (the "2019 Neurocrine Collaboration Agreement") during the three months ended March 31, 2025:

	Balance at December 31, 2024		Additions	Deductions		Balance at March 31, 2025
					<i>(in thousands)</i>	
Related party collaboration receivables	\$ 676	\$	385	\$ (574)	\$	487
Contract liabilities:						
Deferred revenue	\$ 30,397	\$	—	\$ (4,542)	\$	25,855

The change in the related party collaboration receivable balance for the three months ended March 31, 2025 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected during the period, under the 2023 and 2019 Neurocrine Collaboration Agreements. Deferred revenue activity for the period includes the recording of \$4.5 million of collaboration revenue recognized on the proportional performance model during the period for the 2023 and 2019 Neurocrine Collaboration Agreements, which is classified as either current or non-current in the accompanying condensed consolidated balance sheet based on the period the services are expected to be delivered.

## 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 was as follows:

	Three Months Ended	
	March 31,	
	2025	2024
	<i>(in thousands)</i>	
Research and development	\$ 1,485	\$ 1,280
General and administrative	2,188	2,293
Total stock-based compensation expense	<u>\$ 3,673</u>	<u>\$ 3,573</u>

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

	Three Months Ended	
	March 31,	
	2025	2024
	<i>(in thousands)</i>	
Stock options	\$ 2,391	\$ 2,408
Restricted stock awards and units	1,205	1,090
Employee stock purchase plan awards	77	75
Total stock-based compensation expense	<u>\$ 3,673</u>	<u>\$ 3,573</u>

### *Restricted Stock Units and Performance Restricted Stock Units*

A summary of the status of and changes in restricted stock unit, or RSU, and performance-based restricted stock unit, or PRSU, activity under the Company's equity award plans for the three months ended March 31, 2025 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested as of December 31, 2024	1,649,943	\$ 7.44
Granted	1,717,311	4.26
Vested	(450,556)	6.47
Forfeited	(67,420)	7.22
Outstanding as of March 31, 2025	<u>2,849,278</u>	<u>\$ 5.68</u>

During the three months ended March 31, 2025, the Company granted 940,311 RSUs to employees. All RSUs granted in the three months ended March 31, 2025 vest in equal amounts, annually over three years. Stock-based compensation for RSUs is based on the fair value of the Company's common stock on the date of grant and is recognized over the vesting period. The stock-based compensation expense related to RSUs was \$1.2 million and \$1.1 million for the three months ended March 31, 2025 and 2024, respectively.

As of March 31, 2025, the Company had unrecognized stock-based compensation expense related to its unvested RSUs of \$10.9 million, which is expected to be recognized over the remaining average vesting period of 2.2 years.

During the three months ended March 31, 2025 in connection with the Company’s annual compensation cycle, the Company also granted PRSUs to members of the Company’s senior leadership team to more closely align with long-term performance incentives. The awards vest as to a certain number of PRSUs upon the achievement of specified clinical performance milestones over multiple years. Stock-based compensation for PRSUs is based on the fair value of the Company’s common stock on the date of grant and will be recognized at the time the achievement of the specified performance milestones becomes probable. The Company did not recognize any stock-based compensation expense in connection with the PRSUs during the three months ended March 31, 2025.

As of March 31, 2025, the Company had unrecognized stock-based compensation expense related to its unvested PRSUs of \$3.3 million, which will be recognized at the time the achievement of the specified performance milestones becomes probable.

**Stock Options**

The following is a summary of stock option activity for the three months ended March 31, 2025:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2024	8,800,464	\$ 8.10	7.3	3,462
Granted	1,911,670	4.27		
Exercised	(27,613)	2.96		
Cancelled or forfeited	(162,438)	7.41		
Outstanding as of March 31, 2025	<u>10,522,083</u>	\$ 7.43	7.4	\$ 499
Exercisable as of March 31, 2025	<u>5,065,944</u>	\$ 8.41	6.1	\$ 406

As of March 31, 2025, the Company had unrecognized stock-based compensation expense related to its unvested stock options of \$20.5 million which is expected to be recognized over the remaining weighted-average vesting period of 2.7 years.

**10. Net loss per share**

For purposes of the diluted net loss per share, unvested restricted common stock awards, unvested RSUs and PRSUs, and outstanding stock options are considered to be potentially dilutive securities. Unvested restricted common stock awards, unvested RSUs and PRSUs, and outstanding stock options were excluded from the calculation of diluted net loss per share in the three months ended March 31, 2025 and 2024, because their effect would be anti-dilutive and therefore, basic and diluted net loss per share were the same for the three months ended March 31, 2025 and 2024.

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

	As of March 31,	
	2025	2024
Unvested restricted common stock awards	—	22,500
Unvested RSUs and PRSUs	2,849,278	1,719,338
Outstanding stock options	10,522,083	8,909,596
Total	<u>13,371,361</u>	<u>10,651,434</u>

## **11. Underwritten public offering**

On January 4, 2024, the Company entered into an underwriting agreement (the “Underwriting Agreement”) with the representatives of several underwriters named therein (the “Underwriters”), relating to an underwritten public offering of 7,777,778 shares of the Company’s common stock, par value \$0.001 per share, and, in lieu of common stock to certain investors, pre-funded warrants (the “Pre-Funded Warrants”) to purchase up to 3,333,333 shares of common stock. The Underwriters agreed to purchase the Company’s stock from the Company pursuant to the Underwriting Agreement at a price of \$8.46 per share and the Pre-Funded Warrants from the Company pursuant to the Underwriting Agreement at a price of \$8.459 per share underlying each Pre-Funded Warrant.

On January 9, 2024, the Company issued 7,777,778 shares of common stock and 3,333,333 Pre-Funded Warrants for net proceeds of approximately \$93.5 million after deducting underwriting discounts and commissions and offering expenses pursuant to the underwritten public offering. The Pre-Funded Warrants met the equity classification guidance and therefore are classified as stockholders’ equity.

The Pre-Funded Warrants issued in connection with the underwritten public offering are included in basic and diluted weighted average shares outstanding for the three months ended March 31, 2025 and 2024 disclosed in the condensed consolidated statements of operations and comprehensive loss.

## **12. Related-party transactions**

During the three months ended March 31, 2025, the Company received scientific advisory board and other scientific advisory services from one of its prior executives, Dinah Sah, Ph.D., the Company’s former Chief Scientific Officer. The total amount of fees paid to Dr. Sah for services provided during each of the three months ended March 31, 2025 and 2024, was \$150,000.

Under both the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to conduct research, development and commercialization activities for certain of the Company’s AAV gene therapy product candidates. Amounts due from Neurocrine are reflected as related party collaboration receivable. Amounts received from Neurocrine that have not yet been recognized as revenue are reflected as deferred revenue. Refer to Note 8 for further description of these account balances.

For the three months ended March 31, 2025 and 2024, the Company recognized collaboration revenue of \$5.0 million and \$18.0 million related to the 2023 and 2019 Neurocrine Agreements.

## **13. Segment Information**

The Company’s Chief Operating Decision Maker (“CODM”), the Company’s Chief Executive Officer, views the Company’s operations and manages its business as a single operating segment, which is the business of developing and eventually commercializing neurogenetic medicines. The Company therefore has one reportable segment.

The CODM reviews the segment’s profit or loss based on net loss reported on the condensed consolidated statement of operations and comprehensive loss and considers forecast-to-actual variances monthly for expenses that are deemed significant in making resource allocation decisions and assessing performance. Further, the CODM reviews the segment’s assets based on total assets reported on the condensed consolidated balance sheets. All long-lived assets are held in the United States.

The CODM views specific categories within research and development expenses and general and administrative expenses in totality as significant. Further, the CODM reviews the external research and development expenses of specific programs as significant given the impact on achieving the Company’s corporate goals. The following table reconciles reported collaboration revenue and segment expenses to net loss under the significant expense principle for the three months ended March 31, 2025 and 2024:

[Table of Contents](#)

	Three months ended March 31, 2025	Three months ended March 31, 2024
Collaboration revenue	\$ 6,473	\$ 19,516
External research and development (1):		
Anti-tau antibody program (VY7523)	4,249	2,946
SOD1 silencing gene therapy program (VY9323)	1,886	1,964
Tau silencing gene therapy program (VY1706)	3,174	996
Partnered programs (2)	991	2,040
Other programs and platforms (3)	3,181	2,760
Internal research and development (4)	9,958	9,480
Facilities and other research and development (5)	8,087	6,906
General and administrative, total	9,640	8,607
Interest income	3,291	4,867
Other income	418	-
Income tax provision	37	14
Net loss	<u>\$ (31,021)</u>	<u>\$ (11,330)</u>

- 1) External research and development is allocated to the Company's programs and platforms and includes contract development and manufacturing organization expenses, laboratory supplies, the costs of non-employee consultants and contractors, and all other expenses paid to external vendors.
- 2) Partnered programs include programs in which the Company is collaborating with partners to develop AAV gene therapy products and product candidates under the Company's 2019 and 2023 Neurocrine Collaboration Agreements, and the License and Collaboration Agreement the Company entered into with Novartis on December 28, 2023.
- 3) Other programs and platforms consist of expenses related to other early research programs and platforms which are not considered quantitatively and qualitatively significant, including capsid discovery, non-viral delivery, and early research programs.
- 4) Internal research and development consists of employee-related expenses including salaries, benefits, and stock-based compensation expense.
- 5) Depreciation and amortization are included within facilities and other research and development, which are the same as the amounts in the accompanying condensed consolidated statements of operations as the Company operates as a single operating segment.

## 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 fiscal year ended December 31, 2024, which was filed with the Securities and Exchange Commission, or the SEC, on March 11, 2025.*

*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. The following information and any forward-looking statements should be considered in light of factors discussed in Part I, Item 1A, "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, 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.*

### Overview

We are a biotechnology company whose mission is to leverage the power of human genetics to modify the course of and ultimately cure neurological diseases. Our pipeline includes programs for Alzheimer's disease, or AD; Friedreich's ataxia, or FA; Parkinson's disease; and multiple other diseases of the central nervous system, or CNS. Many of our programs are derived from our TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) adeno-associated virus, or AAV, capsid discovery platform, which we have used to generate novel capsids, or TRACER Capsids, and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous, or IV, dosing. Some of our programs are wholly-owned, and some are advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease, or Alexion; Novartis Pharma AG, or Novartis; and Neurocrine Biosciences, Inc., or Neurocrine.

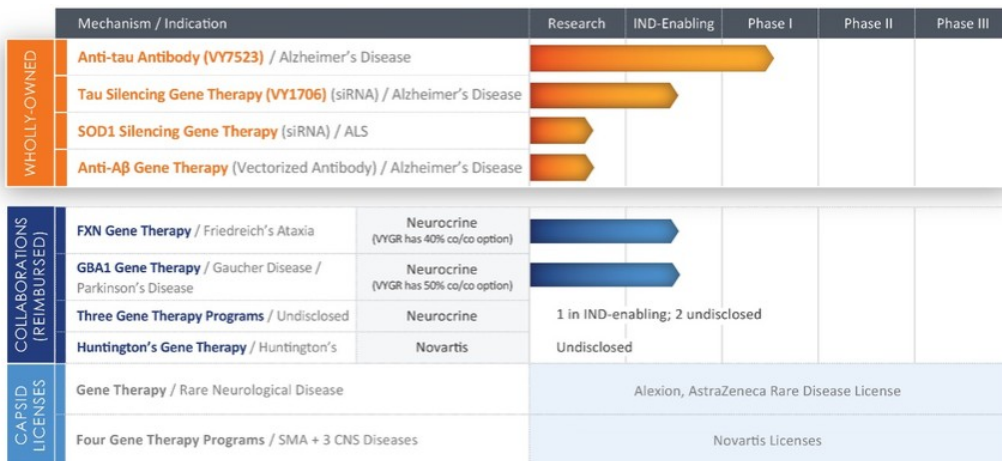
We are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on AD, and particularly on tau, which we view as a critically important AD target. Our wholly-owned prioritized pipeline includes two tau targeting programs: VY7523 (which we formerly referred to as VY-TAU01), an anti-tau antibody for AD, and VY1706, a tau silencing gene therapy for AD. VY7523 is an IV-administered, recombinant, humanized IgG4 monoclonal antibody developed to inhibit the spread of pathological tau, which is closely correlated with disease progression and cognitive decline in AD. The murine version of VY7523 reduced tau spread by approximately 70% in preclinical studies. VY7523 demonstrated an acceptable safety, tolerability, and immunogenicity profile as well as expected pharmacokinetic results in a Phase 1, single ascending dose, or SAD, clinical trial in healthy volunteers. In February 2025, we initiated a Phase 1 multiple ascending dose, or MAD, clinical trial of VY7523 in early AD patients. We believe this trial has the potential to generate initial data in the second half of 2026. VY1706 is a gene therapy that leverages an intravenously delivered TRACER Capsid containing a vectorized small interfering RNA specifically targeting tau mRNA. VY1706 was selected as the development candidate for the tau silencing gene therapy program in November 2024. In a non-human primate, or NHP, study, a single 1.3E13 vector genomes per kilogram dose of VY1706 delivered intravenously resulted in reductions in tau mRNA levels of 50% to 73% across the cerebral cortex, including in areas of the brain where tau accumulates during progression of AD. We anticipate the submission of an investigational new drug, or IND, application for the VY1706 program in 2026. Our proprietary pipeline also includes early research initiatives to develop a vectorized anti-amyloid antibody for AD and a superoxide dismutase 1, or SOD1, gene therapy for SOD1 amyotrophic lateral sclerosis.

We are also working with our collaboration partners on multiple programs. We are advancing five gene therapy programs with Neurocrine. Development candidates were selected for three of these programs in 2024, resulting in milestone payments to us. We expect that Neurocrine will submit IND filings in 2025 for the two most advanced of these programs, a glucosylceramidase beta 1, or GBA1, gene therapy program, and a frataxin, or FXN, gene therapy program for FA, or the FA Program, provided the ongoing GLP toxicology studies support clinical development. These two programs are particularly significant to us for two reasons: the subsequent clinical trials of these programs have the potential to establish human proof-of-concept for the TRACER Capsids, and we have the option to opt-in on co-development and co-commercialization in the United States for both products. Initiation of the first clinical trials for the FA and GBA1 programs are expected in 2026, and the associated regulatory and clinical milestones that we could potentially realize related to these indications in 2025-2026 total up to \$35 million.

The GBA1 program, or the GBA1 Program, will focus on both Gaucher and Parkinson’s disease. Gaucher disease is a rare genetic disorder passed down from parents to children. People who have Gaucher disease are missing an enzyme that breaks down fatty substances called lipids. Lipids start to build up in certain organs such as the spleen and liver. In April 2025, Neurocrine deprioritized two discovery-stage programs against undisclosed targets which we had been developing under a collaboration and license agreement with Neurocrine for the research, development and commercialization of certain of our AAV gene therapy products, or the 2019 Neurocrine Collaboration Agreement. On April 30, 2025, the joint steering committee, or JSC, with Neurocrine agreed to discontinue these programs, so rights to these targets returned to us. The discontinuations were not due to any safety concerns. In addition to the Neurocrine collaborations, we have partnered with Novartis on TRACER Capsid-based gene therapies for spinal muscular atrophy and Huntington’s disease. We have also licensed capsids to Novartis for three undisclosed CNS targets and to Alexion for one undisclosed rare neurological disease target. In total, these partnerships have delivered more than \$500.0 million in non-dilutive funding to us to date, including upfront fees, development milestone payments, option exercise fees, license fees, and research and development expense reimbursement. Looking forward, we have the potential to earn up to \$7.4 billion in milestone payments across the partnered portfolio, including \$2.6 billion in potential development milestone payments, as well as royalties.

All of the gene therapies in our wholly-owned and collaborative pipeline leverage TRACER. TRACER is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier, or BBB, and enhanced CNS tropism in multiple species, including NHPs. We are also developing a second, non-viral therapeutics platform focused on non-viral receptor-mediated transport across the BBB.

**Overview of Our Pipeline**



## **Collaboration and License Agreements**

### **2023 Novartis License and Collaboration Agreement**

On December 28, 2023, we entered into a License and Collaboration Agreement with Novartis, or the 2023 Novartis Collaboration Agreement, to (a) provide rights to Novartis with respect to certain TRACER Capsids for use in the research, development, and commercialization by Novartis of AAV gene therapy products and product candidates, comprising such TRACER Capsids and payloads intended for the treatment of spinal muscular atrophy, or the Novartis SMA Program, and (b) collaborate to develop AAV gene therapy products and product candidates intended for the treatment of Huntington's disease, or the Novartis HD Program, in each case, leveraging TRACER Capsids and other intellectual property controlled by us. We refer to products and product candidates developed under the Novartis SMA Program as Novartis SMA Program Products, and products and candidates developed under the Novartis HD Program as Novartis HD Program Products. With respect to the Novartis HD Program, the parties have agreed to conduct research and pre-clinical development of Novartis HD Program Products pursuant to a research plan, with Novartis reimbursing us for our activities thereunder in accordance with the agreed-to budget.

Under the 2023 Novartis Collaboration Agreement, Novartis paid us an upfront payment of \$80.0 million. We are eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of \$200.0 million for the Novartis SMA Program and up to an aggregate of \$225.0 million for the Novartis HD Program, in each case for the first corresponding product to achieve the corresponding milestone. We are also eligible to receive (a) specified sales milestone payments of up to an aggregate of \$400.0 million for the Novartis SMA Program and up to an aggregate of \$375.0 million for the Novartis HD Program and (b) tiered, escalating royalties in the high single-digit to low double-digit percentages of annual net sales of the Novartis SMA Program Products and the Novartis HD Program Products. The royalties are subject to potential customary reductions, including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits. For a further description of the 2023 Novartis Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, under the caption "2023 Novartis License and Collaboration Agreement."

### **2023 Novartis Stock Purchase Agreement**

We and Novartis also entered into a stock purchase agreement on December 28, 2023, or the 2023 Novartis Stock Purchase Agreement, for the sale and issuance of 2,145,002 shares of our common stock, or the Novartis Shares, to Novartis at a price of \$9.324 per share, for an aggregate purchase price of approximately \$20.0 million. In accordance with the terms and conditions of the 2023 Novartis Stock Purchase Agreement, we issued and sold the Novartis Shares to Novartis on January 3, 2024.

### **2022 Novartis Option and License Agreement**

On March 4, 2022, or the 2022 Novartis Option and License Effective Date, we entered into an option and license agreement with Novartis, or the 2022 Novartis Option and License Agreement. Pursuant to the 2022 Novartis Option and License Agreement, we 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 AAV gene therapy candidates comprised of Novartis Licensed Capsids and payloads directed to such targets, or the Novartis Initial Licensed Products. During the period commencing on the 2022 Novartis Option and License Effective Date and ending on the first anniversary thereof or, in the event Novartis has exercised a Novartis License Option, the third anniversary thereof, on a target-by-target basis, we have granted Novartis a non-exclusive research license to evaluate our TRACER Capsids for potential use in programs targeting three specified genes, or the Initial Novartis Targets. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license TRACER Capsids for use in gene therapy programs against two undisclosed programs targeting specified genes, or the Initial Novartis Targets. Novartis elected not to license a capsid for one Initial Novartis Target prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that we granted to Novartis in connection with this third Initial Novartis Target terminated, and all capsid rights with respect to that Initial Novartis Target returned to us.

On September 3, 2024, we entered into an amendment, or the Novartis Amendment, to the 2022 Novartis Option and License Agreement. Pursuant to the Novartis Amendment, we agreed to amend the 2022 Novartis Option and License Agreement to incorporate the grant to Novartis of a direct license, or the Novartis Direct License, to a TRACER Capsid, or the Novartis Direct Licensed Capsid, for exclusive use with a certain gene, or the Novartis Direct License Target, to develop and commercialize the Novartis Direct Licensed Capsid as incorporated into AAV gene therapy candidates comprised of the Novartis Direct Licensed Capsid and a payload directed to the Novartis Direct License Target, or Novartis Direct Licensed Products. We refer to the Novartis Initial Licensed Products and Novartis Direct Licensed Products collectively as Novartis Licensed Products. As a result of the Novartis Amendment, the Novartis Direct License Target is now deemed a Licensed Target under the 2022 Novartis Option and License Agreement, as such term is defined therein, and the Novartis Direct License is subject to all other terms and conditions applicable to other licenses granted to Novartis under the 2022 Novartis Option and License Agreement. Novartis agreed to pay us a one-time fee of \$15.0 million in consideration for the rights granted under the Novartis Amendment which we received in October 2024. In connection with the Novartis Amendment, the parties acknowledged that Novartis' prior rights to exercise options for any initial targets and additional targets as described in the 2022 Novartis Option and License Agreement, other than those that had previously been exercised, had expired as of the effective date of the Novartis Amendment.

We are eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of \$125.0 million for the first Novartis Initial Licensed Product for each Initial Novartis Target for which a Novartis License Option has been exercised to achieve the corresponding milestone. Additionally, we are eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of \$130.0 million for the first Novartis Direct Licensed Product to achieve the corresponding milestone. On a Novartis Licensed Product-by-Novartis Licensed Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of \$175.0 million per Novartis Licensed Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Novartis Licensed Product. For a further description of the 2022 Novartis Option and License Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, under the caption "2022 Novartis Option and License Agreement."

### **2023 Neurocrine Collaboration Agreement**

In January 2023, we entered into a collaboration agreement with Neurocrine for the research, development, manufacture and commercialization of certain of our AAV gene therapy products, or the 2023 Neurocrine Collaboration Agreement. Under the 2023 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2023 Neurocrine Programs: 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. Neurocrine has agreed to be responsible for all costs we incur in conducting preclinical development activities for each 2023 Neurocrine Program, in accordance with workplans and budgets agreed upon by a JSC.

The 2023 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for the research, development, manufacture, and commercialization of gene therapy products, 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. In September 2024, we announced that the JSC with Neurocrine selected a development candidate in a gene therapy program for the potential treatment of an undisclosed neurological disease under the 2023 Neurocrine Collaboration Agreement, which triggered a \$3.0 million milestone payment to us. We received the \$3.0 million in October 2024.

Neurocrine has also agreed to pay us 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. For a further description of the 2023 Neurocrine Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, under the caption “2023 Neurocrine Collaboration Agreement.”

### **2019 Neurocrine Collaboration Agreement**

In January 2019, we entered into the 2019 Neurocrine Collaboration Agreement. 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 FA Program; and two other undisclosed programs, which we refer to as the 2019 Discovery Programs. Neurocrine has agreed to be responsible for all costs we incur in conducting preclinical development activities for each 2019 Neurocrine Program, in accordance with JSC-agreed upon workplans and budgets. Effective August 2, 2021, Neurocrine terminated the VY-AADC Program under the 2019 Neurocrine Collaboration Agreement.

The 2019 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for the research, development, manufacture, and commercialization of gene therapy products, or the 2019 Collaboration Products, under the FA Program of up to \$195.0 million. We may be entitled to receive aggregate commercial milestone payments for each 2019 Collaboration Product of up to \$275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of \$1.1 billion. We are no longer eligible to receive milestone or royalty payments for the VY-AADC Program in light of the partial termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program. The JSC’s selection of a lead development candidate for the FA Program in February 2024 triggered a \$5.0 million milestone payment to us, which we received in March 2024. In April 2025, Neurocrine deprioritized both 2019 Discovery Programs. On April 30, 2025, the JSC agreed to discontinue these programs, so rights to these targets returned to us. The discontinuations were not due to any safety concerns. As a result, such programs are deemed terminated, and we are no longer eligible to receive the milestones for either of the two 2019 Discovery Programs of up to \$130.0 million per 2019 Discovery Program or any commercial milestones or royalties related thereto.

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, range from the low-teens to high-teens and high-single digits to mid-teens for the FA Program. For a further description of the 2019 Neurocrine Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 under the caption “2019 Neurocrine Collaboration Agreement.”

### **Alexion Option and License Agreement**

In October 2021, we entered into an option and license agreement with Pfizer Inc., or Pfizer, pursuant to which we granted Pfizer options to receive an exclusive license to certain TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified transgene. Effective as of September 30, 2022, Pfizer exercised one option with respect to a capsid for a specified transgene for the potential treatment of a rare neurological disease, and 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 specified transgene.

Effective upon the closing of a transaction on September 20, 2023, Alexion (via Alexion Pharma International Operations Limited, or APIO) acquired all of Pfizer's rights under the option and license agreement, which we refer to as the Alexion Agreement, and became the successor-in-interest to Pfizer thereunder. APIO subsequently assigned the Alexion Agreement to its affiliate AstraZeneca Ireland Limited, or AstraZeneca. Neither the acquisition by Alexion nor the subsequent assignment from APIO to AstraZeneca impacted the material terms of the Alexion Agreement.

Pursuant to an amendment to the Alexion Agreement effective as of September 30, 2024, we agreed to extend the research term thereunder until April 1, 2025, during which period we may, at our sole discretion and expense, conduct further research activities to identify additional TRACER Capsids for potential use in the development of AAV gene therapies under the Alexion Agreement. Effective March 15, 2025, we agreed to further extend the research term to October 1, 2025. For a further description of the Alexion Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, under the caption "Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)."

**Accumulated Deficit; Expenses**

We have a history of incurring significant losses. We reported a net loss of \$31.0 million for the three months ended March 31, 2025 and a net loss of \$65.0 million for the fiscal year ended December 31, 2024. As of March 31, 2025, we had an accumulated deficit of \$357.2 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 clinical trials in connection with VY7523, including our ongoing Phase 1 MAD clinical trial;
- continue investing in our proprietary antibody program, gene therapy and vectorized antibody platforms and programs, and other research and development initiatives;
- continue investing in and supporting TRACER, our proprietary discovery platform to facilitate the selection of AAV capsids and 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 discovery and development of modalities for receptor-mediated non-viral delivery of therapeutic payloads to the CNS;
- 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, our GBA1 Program pursuant to the 2023 Neurocrine Collaboration Agreement, and the Novartis HD Program pursuant to the 2023 Novartis 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 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;
- increase our clinical trial insurance coverage as we expand our clinical trials and increase our product liability insurance once we engage in 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 March 31, 2025, we recognized \$4.5 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, \$0.5 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, \$1.2 million of collaboration revenue in connection with the 2023 Novartis Collaboration Agreement, and \$0.3 million of other collaboration revenue. For the three months ended March 31, 2024, we recognized \$11.5 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, \$6.5 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, \$0.8 million of collaboration revenue in connection with the 2023 Novartis Collaboration Agreement, and \$0.7 million of collaboration revenue in connection with the premium on the issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement.

For additional information about our revenue recognition policy related to collaborations pursuant to the 2023 and 2019 Neurocrine Collaboration Agreements and the 2023 Novartis Collaboration Agreement, refer to Note 9 of the December 31, 2024, consolidated financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024.

For the foreseeable future, we expect substantially all of our revenue will be generated from our current strategic collaborations and out-licensing arrangements with Neurocrine, Novartis, and Alexion, 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 in the future.

### ***Expenses***

#### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred for our research activities, including our program discovery efforts, and the development of our proprietary antibody, gene therapy, vectorized antibody, and non-viral therapeutic platforms and programs 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 and clinical 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 and clinical 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 three months ended March 31, 2025, our research and development expenses have increased as compared to the amounts recorded in the same period in the prior year. As our research and development programs progress and as we identify product candidates and initiate preclinical studies and clinical trials, including our SAD and MAD clinical trials to evaluate VY7523, we expect research and development costs to continue to increase. 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 U.S. Food and Drug Administration 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, information technology, 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 three months ended March 31, 2025, our general and administrative expenses have increased as compared to the amount recorded in the same period in prior year primarily due to additional professional services used in the quarter compared to the three months ended March 31, 2024.

#### *Other Income, Net*

Other income, net consists primarily of interest income on our marketable securities and payments received under the sublease of the office and laboratory space in Cambridge, Massachusetts.

#### *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 three months ended March 31, 2025, as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024. It is important that the discussion of our operating results that follow be read in conjunction with the critical accounting policies disclosed in Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations – *Critical Accounting Policies and Estimates*” in our Annual Report on Form 10-K, as filed with the SEC on March 11, 2025.

## Results of Operations

### Comparison of the three months ended March 31, 2025 and 2024

The following table summarizes our results of operations for the three months ended March 31, 2025, and 2024, together with the changes in those items in dollars:

	Three Months Ended March 31,		Change
	2025	2024 <i>(in thousands)</i>	
Collaboration revenue	\$ 6,473	\$ 19,516	\$ (13,043)
Operating expenses:			
Research and development	31,526	27,092	4,434
General and administrative	9,640	8,607	1,033
Total operating expenses	41,166	35,699	5,467
Other income, net:			
Interest income	3,291	4,867	(1,576)
Other income	418	—	418
Total other income, net	3,709	4,867	(1,158)
Loss before income taxes	(30,984)	(11,316)	(19,668)
Income tax provision	37	14	23
Net loss	\$ (31,021)	\$ (11,330)	\$ (19,691)

#### Collaboration Revenue

Collaboration revenue was \$6.5 million and \$19.5 million for the three months ended March 31, 2025 and 2024, respectively. During the three months ended March 31, 2025, we recognized collaboration revenue in connection with the following agreements:

- \$4.5 million with the 2023 Neurocrine Collaboration Agreement;
- \$0.5 million with the 2019 Neurocrine Collaboration Agreement;
- \$1.2 million with the 2023 Novartis Collaboration Agreement; and
- \$0.3 million in other collaboration revenue.

During the three months ended March 31, 2024 we recognized collaboration revenue in connection with the following agreements:

- \$11.5 million with the 2023 Neurocrine Collaboration Agreement;
- \$6.5 million with the 2019 Neurocrine Collaboration Agreement;
- \$0.8 million with the 2023 Novartis Collaboration Agreement; and
- \$0.7 million with the premium on the issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement.

### Research and Development Expense

Research and development expense increased by \$4.4 million from \$27.1 million for the three months ended March 31, 2024 to \$31.5 million for the three months ended March 31, 2025. The following table summarizes our research and development expenses for the three months ended March 31, 2025 and 2024. All amounts for the three months ended March 31, 2024 have been reclassified to conform to the current year's presentation.

	Three Months Ended		Change
	March 31,		
	2025	2024	
	<i>(in thousands)</i>		
Internal research and development	\$ 9,958	\$ 9,480	\$ 478
External research and development	13,481	10,706	2,775
Facilities and other	8,087	6,906	1,181
Total research and development expenses	<u>\$ 31,526</u>	<u>\$ 27,092</u>	<u>\$ 4,434</u>

The increase in research and development expense for the three months ended March 31, 2025 was primarily attributable to the following:

- approximately \$2.8 million for external research and development costs related to increased program-related spending, driven by increases in VY7523 as our Phase 1 MAD clinical trial has commenced, along with increases in VY1706, offset by decreases in external research and development on our partnered programs;
- approximately \$1.2 million for facility and other costs primarily related to our lease for additional laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts, which we took occupancy of on February 1, 2024; and
- approximately \$0.5 million for increased internal research and development costs associated with higher headcount in research and development functions as compared to the same period in the prior year.

### General and Administrative Expense

General and administrative expense increased by \$1.0 million from \$8.6 million for the three months ended March 31, 2024 to \$9.6 million for the three months ended March 31, 2025. The increase in general and administrative expense was primarily attributable to increased employee-related costs, along with increased professional services fees compared to the three months ended March 31, 2024.

### Other Income, Net

Other income, net of approximately \$3.7 million was recognized during the three months ended March 31, 2025, as compared to \$4.9 million during the three months ended March 31, 2024. Other income during both periods was primarily related to interest income on marketable securities. The decrease is primarily due to decreased interest rates and also decreased marketable securities during the three months ended March 31, 2025 as compared to the three months ended March 31, 2024.

## Liquidity and Capital Resources

### Sources of Liquidity

We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings and private placements of our common stock, strategic collaborations and option and license arrangements, including our 2019 Neurocrine Collaboration Agreement, 2023 Neurocrine Collaboration Agreement, 2022 Novartis Option and License Agreement, 2023 Novartis Collaboration Agreement, Alexion Agreement, and with our prior collaboration agreements.

During the three months ended March 31, 2024, we completed an underwritten public offering which resulted in net proceeds to us of approximately \$93.5 million after deducting underwriting discounts and commissions and offering expenses.

We and Novartis entered into the 2023 Novartis Stock Purchase Agreement, on December 28, 2023, for the sale and issuance of the Novartis Shares to Novartis at a price of \$9.324 per share, for an aggregate purchase price of approximately \$20.0 million. In accordance with the terms and conditions of the 2023 Novartis Stock Purchase Agreement, we issued and sold these shares to Novartis on January 3, 2024.

### Cash Flows

The following table provides information regarding our cash flows for the three months ended March 31, 2025, and 2024:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<i>(in thousands)</i>	
Net cash (used in) provided by:		
Operating activities	\$ (37,895)	\$ 58,767
Investing activities	41,201	(96,065)
Financing activities	82	112,856
Net increase in cash, cash equivalents, and restricted cash	<u>\$ 3,388</u>	<u>\$ 75,558</u>

### Net Cash (Used in) Provided by Operating Activities

Net cash used in operating activities was \$37.9 million during the three months ended March 31, 2025 compared to \$58.8 million in net cash provided by operating activities during the three months ended March 31, 2024. Net cash used in operating activities during the three months ended March 31, 2025 was primarily due to our net loss of \$31.0 million, combined with changes in our working capital accounts, including a decrease in accrued expenses and deferred revenue during the three months ended March 31, 2025.

The net cash provided by operating activities during the three months ended March 31, 2024 was primarily due to a decrease in accounts receivable from the cash receipt under the 2023 Novartis Collaboration Agreement during the first quarter of 2024, offset by the net loss of \$11.3 million, combined with changes in our working capital accounts, including a decrease in deferred revenue and a decrease in accrued expenses during the three months ended March 31, 2024.

### Net Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities was \$41.2 million during the three months ended March 31, 2025 compared to \$96.1 million of net cash used in investing activities during the three months ended March 31, 2024. The change was primarily due to a decrease of \$126.9 million of purchases of marketable securities during the three months ended March 31, 2025 as compared to March 31, 2024. Further, proceeds from sales and maturities of marketable securities increased by \$8.9 million during the three months ended March 31, 2025 compared to March 31, 2024.

### *Net Cash Provided by Financing Activities*

Net cash provided by financing activities was \$0.1 million during the three months ended March 31, 2025 compared to \$112.9 million during the three months ended March 31, 2024. The decrease was primarily due to \$93.5 million in net proceeds from the issuance of common stock and pre-funded warrants in connection with the public offering in 2024, and \$19.3 million in proceeds from the issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement, during the three months ended March 31, 2024.

### *Funding Requirements*

Our expenses during the three months ended March 31, 2025, increased as compared with the three months ended March 31, 2024, as we progressed our research and development programs, advanced VY7523 into clinical development, and 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, including in our ongoing Phase 1 MAD clinical trial to evaluate VY7523 in 2025, 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 employees will increase due to the current 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.

As of March 31, 2025 we had cash, cash equivalents, and marketable securities of \$295.1 million. Based upon our current operating plans, we expect that our existing cash, cash equivalents, and marketable securities at March 31, 2025 along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine and Novartis and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into mid-2027. 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, including our ongoing clinical trials to evaluate VY7523;
- 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 arrangements we may enter into in the future, including any research and development costs for which we are responsible, future additional obligations that we may be committed to in connection with these agreements, and our receipt of any expense reimbursements, 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 license agreement we entered into with Touchlight IP Limited in November 2022;
- 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 licensees 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 products or product candidates that we would otherwise prefer to develop and market ourselves.

### ***Contractual Obligations***

We enter into agreements in the normal course of business with clinical research organizations, contract manufacturing organizations, and institutions to license intellectual property. These contracts are generally 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, we believe 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 three months ended March 31, 2025.

### **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 of 1934, or 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 Securities and Exchange Commission'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 March 31, 2025. 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 March 31, 2025, our disclosure controls and procedures were effective at the reasonable assurance level.

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

***Changes in Internal Control over Financial Reporting***

During the three months ended March 31, 2025, 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 March 31, 2025, 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 fiscal year ended December 31, 2024, filed with the Securities and Exchange Commission on March 11, 2025. Any of the risks and uncertainties described in our Annual Report on Form 10-K could materially and adversely affect our business, financial condition, results of operations and future growth prospects, and such risks and uncertainties are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see the discussion under the caption “Forward-Looking Statements” in this Quarterly Report on Form 10-Q for a discussion of some of the forward-looking statements that are qualified by these risk factors.

**ITEM 5. OTHER INFORMATION**

**Director and Officer Trading Arrangements**

A portion of the compensation of our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended (the “Exchange Act”)) is in the form of equity awards and, from time to time, directors and officers engage in open-market transactions with respect to the securities acquired pursuant to such equity awards or our other securities, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in our securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in our securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

During the quarterly period covered by this report, no new trading arrangement for the sale or purchase of our securities was adopted or terminated by our directors and officers that is either (1) a contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) (a “Rule 10b5-1 trading arrangement”) or (2) a “non-Rule 10b5-1 trading arrangement” (as defined in Item 408(c) of Regulation S-K) other than the adoption by Toby Ferguson, M.D., Ph.D., our Chief Medical Officer, of a durable sale instruction constituting a Rule 10b5-1 trading arrangement on March 27, 2025, related to sell-to-cover transactions for restricted stock units, or RSUs, granted on or after April 1, 2024, which superseded a durable sale instruction Dr. Ferguson had previously adopted on March 22, 2024, with regard to the same transactions that constituted a non-Rule 10b5-1 trading arrangement. The superseding Rule 10b5-1 trading arrangement did not modify or change the amount, price, or timing of the sale of securities subject to a durable sale instruction by Dr. Ferguson.

**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**

Exhibit No.	Description	Incorporated by Reference to:				
		Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number	Filed Herewith
10.1	<a href="#">Form of Performance Restricted Stock Unit Award Agreement.</a>					X
10.2	<a href="#">Second Amendment to Option and License Agreement, by and between the Company and AstraZeneca Ireland Limited, dated as of March 15, 2025.</a>					X
31.1	<a href="#">Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.</a>					X
31.2	<a href="#">Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.</a>					X
32.1+	<a href="#">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.</a>					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					X

+ 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: May 6, 2025

**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/ Nathan Jorgensen, Ph.D.  
Nathan Jorgensen, Ph.D.  
Chief Financial Officer  
(Principal Financial and Accounting Officer)

**PERFORMANCE RESTRICTED STOCK UNIT AWARD AGREEMENT  
FOR COMPANY EMPLOYEES  
UNDER VOYAGER THERAPEUTICS, INC.  
2015 STOCK OPTION AND INCENTIVE PLAN**

Pursuant to the Voyager Therapeutics, Inc. 2015 Stock Option and Incentive Plan as amended through the date hereof (the "Plan"), Voyager Therapeutics, Inc. (the "Company") hereby grants an award of the number of Performance Restricted Stock Units listed on the cover page hereof (an "Award") to the Grantee named on the cover page hereof. Each Performance Restricted Stock Unit shall relate to one share of Common Stock, par value \$0.001 per share (the "Stock") of the Company.

1. Restrictions on Transfer of Award. This Award may not be sold, transferred, pledged, assigned or otherwise encumbered or disposed of by the Grantee, and any shares of Stock issuable with respect to the Award may not be sold, transferred, pledged, assigned or otherwise encumbered or disposed of until (i) the Performance Restricted Stock Units have vested as provided in Paragraph 2 of this Agreement and (ii) shares of Stock have been issued to the Grantee in accordance with the terms of the Plan and this Agreement.

2. Vesting of Performance Restricted Stock Units. The restrictions and conditions of Paragraph 1 of this Agreement shall lapse on the Vesting Date or Vesting Dates specified on Exhibit A so long as the Grantee remains an employee of the Company or a Subsidiary on such Vesting Dates. If a series of Vesting Dates is specified, then the restrictions and conditions in Paragraph 1 shall lapse only with respect to the number of Performance Restricted Stock Units specified as vested on such date. The Administrator may at any time accelerate the vesting schedule specified in this Paragraph 2.

3. Termination of Employment. If the Grantee's employment with the Company and its Subsidiaries terminates for any reason (including death or disability) prior to the satisfaction of the vesting conditions set forth in Paragraph 2 above, any Performance Restricted Stock Units that have not vested as of such date shall automatically and without notice terminate and be forfeited, and neither the Grantee nor any of his or her successors, heirs, assigns, or personal representatives will thereafter have any further rights or interests in such unvested Performance Restricted Stock Units.

4. Issuance of Shares of Stock. As soon as practicable following each Vesting Date (but in no event later than two and one-half months after the end of the year in which the Vesting Date occurs), the Company shall issue to the Grantee the number of shares of Stock equal to the aggregate number of Performance Restricted Stock Units that have vested pursuant to Paragraph 2 of this Agreement on such date and the Grantee shall thereafter have all the rights of a stockholder of the Company with respect to such shares.

5. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Agreement shall be subject to and governed by all the terms and conditions of the Plan, including the powers of the Administrator set forth in Section 2(b) of the Plan. Capitalized terms in this Agreement shall have the meaning specified in the Plan, unless a different meaning is specified herein.

6. Tax Withholding. The Grantee acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Grantee any federal, state, local or other taxes of any kind required by law to be withheld with respect to the vesting of the Performance Restricted Stock Units. At

such time as the Grantee is not aware of any material nonpublic information about the Company or the Stock, the Grantee shall execute the instructions set forth in Schedule 1 attached hereto (the “Durable Automatic Sale Instructions”) as the means of satisfying such tax obligation. If the Grantee does not execute the Durable Automatic Sale Instructions prior to an applicable Vesting Date, then the Grantee agrees that if under applicable law the Grantee will owe taxes at such Vesting Date on the portion of the Award then vested the Company shall be entitled to immediate payment from the Grantee of the amount of any tax required to be withheld by the Company. The Company shall not deliver any shares of Stock to the Participant until it is satisfied that all required withholdings have been made.

7. Section 409A of the Code. This Agreement shall be interpreted in such a manner that all provisions relating to the settlement of the Award are exempt from the requirements of Section 409A of the Code as “short-term deferrals” as described in Section 409A of the Code.

8. No Obligation to Continue Employment. Neither the Company nor any Subsidiary is obligated by or as a result of the Plan or this Agreement to continue the Grantee in employment and neither the Plan nor this Agreement shall interfere in any way with the right of the Company or any Subsidiary to terminate the employment of the Grantee at any time.

9. Integration. This Agreement constitutes the entire agreement between the parties with respect to this Award and supersedes all prior agreements and discussions between the parties concerning such subject matter.

10. Data Privacy Consent. In order to administer the Plan and this Agreement and to implement or structure future equity grants, the Company, its subsidiaries and affiliates and certain agents thereof (together, the “Relevant Companies”) may process any and all personal or professional data, including but not limited to Social Security or other identification number, home address and telephone number, date of birth and other information that is necessary or desirable for the administration of the Plan and/or this Agreement (the “Relevant Information”). By entering into this Agreement, the Grantee (i) authorizes the Company to collect, process, register and transfer to the Relevant Companies all Relevant Information; (ii) waives any privacy rights the Grantee may have with respect to the Relevant Information; (iii) authorizes the Relevant Companies to store and transmit such information in electronic form; and (iv) authorizes the transfer of the Relevant Information to any jurisdiction in which the Relevant Companies consider appropriate. The Grantee shall have access to, and the right to change, the Relevant Information. Relevant Information will only be used in accordance with applicable law.

11. Notices. Notices hereunder shall be mailed or delivered to the Company at its principal place of business and shall be mailed or delivered to the Grantee at the address on file with the Company or, in either case, at such other address as one party may subsequently furnish to the other party in writing.

[Remainder of Page Intentionally Left Blank]

SCHEDULE 1

**DURABLE AUTOMATIC SELL-TO-COVER INSTRUCTION**

This Durable Automatic Sell-to-Cover Instruction (this “Instruction”), which is being delivered to Voyager Therapeutics, Inc. (the “Company”) by the undersigned on the date set forth below (the “Adoption Date”), relates to the Covered RSUs (as defined following my signature below). This Instruction provides for “eligible sell-to-cover transactions” (as described in Rule 10b5-1(c)(1)(ii)(D)(3) under the Securities Exchange Act of 1934 (the “Exchange Act”)) and is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c)(1) under the Exchange Act.

I acknowledge that upon vesting and settlement of any Covered RSUs in accordance with the applicable RSU’s terms, whether vesting is based on the passage of time or the achievement of performance goals, I will have compensation income equal to the fair market value of the shares of the Company’s common stock subject to the RSUs that are settled on such settlement date and that the Company is required to withhold income and employment taxes in respect of that compensation income.

I desire to establish a plan and process to satisfy such withholding obligation in respect of all Covered RSUs through an automatic sale of the number of the shares of the Company’s common stock that would otherwise be issuable to me on each applicable settlement date in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

I understand that the Company has arranged for the administration and execution of its equity incentive programs and the sale of securities by participants thereunder pursuant to a platform administered by a third party (the “Administrator”) and the Administrator’s designated brokerage partner.

Upon the settlement of any of my Covered RSUs after the 30<sup>th</sup> day following the Adoption Date (or if I am an officer of the Company on the Adoption Date, after the 120<sup>th</sup> day following the Adoption Date) (the “Cooling-Off Period”), I hereby appoint the Administrator (or any successor administrator) to automatically sell such number of shares of the Company’s common stock issuable with respect to such RSUs that vested and settled as is sufficient to generate net proceeds sufficient to satisfy the Company’s minimum statutory withholding obligations with respect to the income recognized by me in connection with the vesting and settlement of such RSUs (based on minimum statutory withholding rates for all tax purposes, including payroll and social security taxes, that are applicable to such income), and the Company shall receive such net proceeds in satisfaction of such tax withholding obligation.

I hereby appoint the Chief Executive Officer, the Chief Financial Officer and the General Counsel, and any of them acting alone and with full power of substitution, to serve as my attorneys-in-fact to arrange for the sale of shares of the Company’s common stock in accordance with this Instruction. I agree to execute and deliver such documents, instruments and certificates as may reasonably be required in connection with the sale of the shares of common stock pursuant to this Instruction.

Unless the third and final box in the definition of Covered RSUs below is checked, if I have previously adopted an automatic sale or sell-to-cover instruction relating to Covered RSUs, this Instruction shall be void *ab initio*.

**I hereby certify that, as of the Adoption Date:**

**(i) I am not prohibited from entering into this Instruction by the Company's insider trading policy or otherwise;**

**(ii) I am not aware of any material nonpublic information about the Company or its common stock; and**

**(iii) I am adopting this Instruction in good faith and not as part of a plan or scheme to evade the prohibitions of Rule 10b-5 under the Exchange Act.**

\_\_\_\_\_  
Print Name: \_\_\_\_\_

Date: \_\_\_\_\_

**Covered RSUs:**

The following restricted stock units ("RSUs") are covered by this Instruction.

Check all applicable boxes:

The first award of RSUs granted to me on or after \_\_\_\_\_ [*insert date of grant of current RSUs the grant of which is triggering the execution of this Instruction; if instruction is being executed in advance of a grant of RSUs, insert the Adoption Date*] and any RSUs that may, from time to time following such date, be granted to me by the Company, other than any future granted RSUs which by the terms of the applicable award agreement require the Company to withhold shares for tax withholding obligations in connection with the vesting and settlement of such RSUs, and therefore do not permit sell-to-cover transactions.

Any outstanding RSUs that were granted to me by the Company prior to the Adoption Date that (1) are not subject to any prior automatic sale or sell-to-cover instruction and (2) for which the next vesting date is after the Cooling-Off Period, other than any previously granted RSUs which by the terms of the applicable award agreement require the Company to withhold shares for tax withholding obligations in connection with the vesting and settlement of such RSUs, and therefore do not permit sell-to-cover transactions.

With respect to any RSUs, whether or not granted to me by the Company prior to the Adoption Date, that already are subject to an automatic sale or sell-to-cover instruction (a "Prior Instruction"), I elect to have such sales effected pursuant to this Instruction and confirm that doing so does not modify or change the amount, price, or timing of such sales from those provided by the Prior Instruction (and, as a result the Cooling-Off Period is not applicable to sales pursuant to this Instruction that were previously subject to the Prior Instruction).

**SECOND AMENDMENT TO OPTION AND LICENSE AGREEMENT**

This Second Amendment to Option and License Agreement (this “Amendment”) is entered into and made effective as of March 15, 2025 (the “Amendment Effective Date”) by and between (i) Voyager Therapeutics, Inc., a Delaware corporation having its principal place of business at 75 Hayden Avenue, Lexington, MA 02421 (“Voyager”), and (ii) AstraZeneca Ireland Limited, a limited company incorporated in Ireland, with its registered office at College Business & Technology Park, Blachardstown Road North, Dublin 15, Ireland (“Alexion”). Voyager and Alexion are referred to herein individually as a “Party” and collectively as the “Parties”. Capitalized terms used in this Amendment that are not otherwise defined herein shall have the meanings set forth in the Agreement (as defined below).

**RECITALS**

WHEREAS, Alexion desires to extend the Research Term for the CNS Capsid, and the Parties have agreed to amend the Agreement in accordance with Section 11.11 thereof.

NOW, THEREFORE, in consideration of the premises and mutual covenants set forth in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

**1. AMENDMENT OF THE AGREEMENT**

The Parties hereby agree that the Agreement is amended as set forth below, effective as of the Amendment Effective Date.

1.1 Amended Definition. Section 1.103 and of the Agreement are hereby deleted in its entirety and is replaced with the following:

1.103 “Research Term” means, (i) for any TRACER Capsid Candidate designed to target the central nervous system, the period commencing on the Effective Date and ending on October 1, 2025, and (ii) for the Cardiology Capsid, the period commencing on the Effective Date and ending on the first (1<sup>st</sup>) anniversary of the Effective Date.

1.2 Capsid Substitution During Research Term; Reporting. The Parties acknowledge and agree that during the Research Term, pursuant to the terms and conditions of Section 2.3 and Section 2.5 of the Agreement (without limiting the terms of Sections 2.3 and Section 2.5 except as amended by this Amendment), (i) Alexion may conduct additional Evaluation of TRACER Capsid Candidates that are CNS Capsids during the Research Term, and (ii) Alexion may elect to substitute any TRACER Capsid Candidate for any Licensed Capsid during the Research Term by providing written notice to Voyager in accordance with Section 11.7 of the Agreement, identifying the Substitute Capsid and the specific Pfizer Transgene for which the Substitute Capsid will replace the previously designated Licensed Capsid. Alexion will provide to Voyager written reports summarizing all results (including the Pfizer Evaluation Data) of the Evaluation conducted by Alexion during the Research Term; provided that Alexion, in its sole discretion, may choose to redact, mask, or not

provide any information related to a Pfizer Transgene or Manufacturing, in each case, pursuant to Section 2.3.2 of the Agreement, and Alexion will provide the foregoing results within thirty (30) days following the completion of the Research Term.

- 1.3 No Further Options. The Parties acknowledge and agree that Pfizer's rights to exercise the Option set forth in Section 2.4 have been exercised or expired as of the Amendment Effective Date, and accordingly no further Option rights exist under the Agreement as of the Amendment Effective Date. For clarity, an Option does not include the substitution rights under Section 2.5, which continue to apply as set forth herein.

## **2. MISCELLANEOUS**

- 2.1 Scope. This Amendment supersedes all proposals, negotiations, conversations and/or discussions between or among Parties relating to the subject matter of this Amendment and all past dealing or industry custom. This Amendment shall be integrated in and form part of the Agreement effective as of the Amendment Effective Date. Except for the foregoing modifications, the Agreement is hereby ratified and confirmed in accordance with its original terms.
- 2.2 Counterparts. This Amendment may be executed in one or more counterparts, each of which will be an original and all of which together will constitute one instrument.

*[Remainder of this page is intentionally left blank]*

IN WITNESS WHEREOF, and intending to be legally bound hereby, the Parties have caused this Amendment to be executed by their duly authorized representatives as of the Amendment Effective Date.

**VOYAGER THERAPEUTICS, INC.**

By: /s/ Alfred Sandrock

Name: Alfred W. Sandrock, Jr., M.D., Ph.D.

Title: President and CEO

**ASTRAZENECA IRELAND LIMITED**

By: /s/ Becky Jorgenson

Name: Becky Jorgenson

Title: Vice President, Finance

---

## Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended March 31, 2025 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: May 6, 2025

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

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

---

## Certification

I, Nathan Jorgensen, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended March 31, 2025 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: May 6, 2025

*/s/ Nathan Jorgensen, Ph.D.*

Nathan Jorgensen

Chief Financial Officer

*(Principal Financial and Accounting Officer)*

---

**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 March 31, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 that to his or her knowledge:

- 1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- 2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 6, 2025

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

---

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

Date: May 6, 2025

*/s/ Nathan Jorgensen, Ph.D.*

---

Nathan Jorgensen  
Chief Financial Officer  
(Principal Financial and Accounting Officer)

---