Table of Contents

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

FORM 10-Q

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

For the quarterly period ended June 30, 2024

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

For the transition period from _____ to _____

Commission file number: 001-37625

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

Delaware (State or other jurisdiction of incorporation or organization)	**46-3003182** (I.R.S. Employer Identification No.)
75 Hayden Avenue, Lexington, Massachusetts (Address of principal executive offices)	**02421** (Zip Code)

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

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

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

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

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

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

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

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

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

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of August 1, 2024 was 54,533,254.

Forward-Looking Statements

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

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

- our plans to develop and commercialize our product candidates based on adeno-associated virus, or AAV, gene therapy and our proprietary antibodies;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) discovery platform and our vectorized antibody platform, our proprietary antibody program, and our gene therapy and vectorized antibody programs;
- our ability to identify and optimize product candidates and proprietary AAV capsids;
- our strategic collaborations and licensing agreements with, and funding from, our collaboration partners Neurocrine Biosciences, Inc. and Novartis Pharma AG, and our licensee Alexion, AstraZeneca Rare Disease (successor-in-interest to former licensee Pfizer Inc.);
- our planned and initiated clinical trials and ongoing and planned preclinical development efforts, related timelines and studies;
- our ability to enter into future collaborations, strategic alliances, or option and license arrangements;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for our product candidates, including the ability to submit investigational new drug, applications for our programs;
- our estimates regarding revenue, expenses, contingent liabilities, future revenues, 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;

- 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 February 28, 2024, particularly in "Part I, Item 1A — Risk Factors," and, if applicable, our Quarterly Reports on Form 10-Q, particularly in "Part II, Item 1A — Risk Factors," that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, strategic collaborations, licenses, joint ventures or investments we may make.

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

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 TM 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

PART I. FINANCIAL INFORMATION

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 76,127	$ 68,802
Marketable securities, current	294,894	162,073
Accounts receivable	1,702	80,150
Related party collaboration receivable	1,549	3,341
Prepaid expenses and other current assets	7,298	5,318
Total current assets	381,570	319,684
Property and equipment, net	16,267	16,494
Deposits and other non-current assets	2,874	1,593
Operating lease, right-of-use assets	35,514	13,510
Total assets	$ 436,225	$ 351,281
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,605	$ 1,604
Accrued expenses	10,273	16,823
Other current liabilities	6,736	3,200
Deferred revenue, current	20,516	42,881
Total current liabilities	40,130	64,508
Deferred revenue, non-current	19,473	32,359
Other non-current liabilities	41,205	18,094
Total liabilities	100,808	114,961
Commitments and contingencies (see note 7)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized at June 30, 2024 and December 31, 2023; no shares issued and outstanding at June 30, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized at June 30, 2024 and December 31, 2023; 54,472,113 and 44,038,333 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively	54	44
Additional paid-in capital	618,538	497,506
Accumulated other comprehensive loss	(522)	(48)
Accumulated deficit	(282,653)	(261,182)
Total stockholders' equity	335,417	236,320
Total liabilities and stockholders' equity	$ 436,225	$ 351,281

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Collaboration revenue	$ 29,578	$ 4,853	$ 49,094	$ 155,333
Operating expenses:				
Research and development	34,452	21,985	61,544	40,553
General and administrative	10,151	8,294	18,758	17,322
Total operating expenses	44,603	30,279	80,302	57,875
Operating (loss) income	(15,025)	(25,426)	(31,208)	97,458
Interest income	4,888	3,274	9,755	5,138
Other income	20	3	20	3
Total other income, net	4,908	3,277	9,775	5,141
(Loss) income before income taxes	(10,117)	(22,149)	(21,433)	102,599
Income tax provision	24	59	38	763
Net (loss) income	$ (10,141)	$ (22,208)	$ (21,471)	$ 101,836
Other comprehensive (loss) income:				
Net unrealized (loss) gain on available-for-sale securities	(16)	(1)	(474)	86
Total other comprehensive (loss) income	(16)	(1)	(474)	86
Comprehensive (loss) income	$ (10,157)	$ (22,209)	$ (21,945)	$ 101,922
Net (loss) income per share, basic	$ (0.18)	$ (0.51)	$ (0.37)	$ 2.42
Net (loss) income per share, diluted	$ (0.18)	$ (0.51)	$ (0.37)	$ 2.33
Weighted-average common shares outstanding, basic	57,721,934	43,520,137	57,419,490	42,102,101
Weighted-average common shares outstanding, diluted	57,721,934	43,520,137	57,419,490	43,770,999

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

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

	Common Stock Shares	Amount	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Stockholders' Equity
Balance at December 31, 2022	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	59,020
Exercises of vested stock options	51,993	—	185	—	—	185
Vesting of restricted stock units	374,417	—	—	—	—	—
Issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	4,395,588	5	31,116	—	—	31,121
Stock-based compensation expense	—	—	2,504	—	—	2,504
Unrealized gain on available-for-sale securities, net of tax	—	—	—	87	—	87
Net income	—	—	—	—	124,044	124,044
Balance at March 31, 2023	43,435,889	$ 43	$ 486,518	$ (132)	$ (269,468)	$ 216,961
Exercises of vested stock options	198,348	1	1,228	—	—	1,229
Vesting of restricted stock units	62,828	—	—	—	—	—
Issuance of common stock under ESPP	62,344	—	418	—	—	418
Stock-based compensation expense	—	—	2,627	—	—	2,627
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(22,208)	(22,208)
Balance at June 30, 2023	43,759,409	$ 44	$ 490,791	$ (133)	$ (291,676)	$ 199,026
Balance at December 31, 2023	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)
Balance at March 31, 2024	54,318,133	$ 54	$ 613,850	$ (506)	$ (272,512)	$ 340,886
Exercises of vested stock options	25,958	—	90	—	—	90
Vesting of restricted stock units	56,549	—	—	—	—	—
Issuance of common stock under ESPP	71,473	—	639	—	—	639
Stock-based compensation expense	—	—	3,959	—	—	3,959
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(16)	—	(16)
Net loss	—	—	—	—	(10,141)	(10,141)
Balance at June 30, 2024	54,472,113	$ 54	$ 618,538	$ (522)	$ (282,653)	$ 335,417

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

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

	Six Months Ended June 30,	
	2024	**2023**
Cash flow from operating activities		
Net (loss) income	$ (21,471)	$ 101,836
Adjustments to reconcile net (loss) income to net cash provided by operating activities:		
Stock-based compensation expense	7,615	5,230
Depreciation	2,588	2,161
Impairment charge on leased facility	2,776	—
Amortization of premiums and discounts on marketable securities	(4,395)	(25)
Loss on disposal of fixed assets	286	124
Changes in operating assets and liabilities:		
Accounts receivable	78,448	—
Related party collaboration receivable	1,792	(3,093)
Prepaid expenses and other current assets	(1,980)	(686)
Operating lease, right-of-use asset	2,432	957
Accounts payable	1,001	(907)
Accrued expenses	(6,550)	1,296
Operating lease liabilities	(104)	(1,385)
Deferred revenue	(35,251)	17,222
Net cash provided by operating activities	27,187	122,730
Cash flow from investing activities		
Purchases of property and equipment	(3,108)	(1,719)
Purchases of marketable securities	(293,859)	(28,453)
Proceeds from sales and maturities of marketable securities	164,959	20,000
Net cash used in investing activities	(132,008)	(10,172)
Cash flow from financing activities		
Proceeds from the exercise of stock options	168	1,414
Proceeds from the issuance of common stock 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	—
Proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	—	31,121
Proceeds from the purchase of common stock under ESPP	481	319
Net cash provided by financing activities	113,427	32,854
Net increase in cash, cash equivalents, and restricted cash	8,606	145,412
Cash, cash equivalents, and restricted cash, beginning of period	70,395	100,474
Cash, cash equivalents, and restricted cash, end of period	$ 79,001	$ 245,886
Supplemental disclosure of cash and non-cash activities		
Capital expenditures incurred but not yet paid	$ —	$ 52
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. The Company's pipeline includes programs for Alzheimer's disease; amyotrophic lateral sclerosis; Parkinson's disease, and multiple other diseases of the central nervous system. Many of the Company's programs are derived from its TRACER™ adeno-associated virus ("AAV") capsid discovery platform, which the Company has used to generate novel capsids ("TRACER Capsids") and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of the Company's programs are wholly-owned, and some are advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease; Novartis Pharma AG, ("Novartis"); and Neurocrine Biosciences, Inc. ("Neurocrine").

The Company has a history of incurring annual net operating losses. As of June 30, 2024, the Company had an accumulated deficit of $282.7 million. The Company has not generated any product revenue and has financed its operations primarily through public offerings and private placements of its equity securities, funding from fees, option exercise payments, and milestone payments, and cost reimbursements associated with its prior and ongoing collaborations and license agreements.

As of June 30, 2024, the Company had cash, cash equivalents, and marketable securities of $371.0 million. Based upon the Company's current operating plans, the Company expects that its existing cash, cash equivalents, and marketable securities at June 30, 2024 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, 2023 as filed with the Securities and Exchange Commission ("SEC") on February 28, 2024. 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 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, 2023. 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 year ended December 31, 2023.

3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of June 30, 2024 and December 31, 2023 are as follows:

Assets	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
June 30, 2024		*(in thousands)*		
Money market funds included in cash and cash equivalents	$ 63,854	$ 63,854	$ —	$ —
Marketable securities:				
U.S. Treasury notes	22,714	22,714	—	—
U.S. Government agency securities	126,706	126,706	—	—
Certificates of deposit	5,449	—	5,449	—
Corporate bonds	113,661	—	113,661	—
Commercial paper	26,364	—	26,364	—
Total money market funds and marketable securities	$ 358,748	$ 213,274	$ 145,474	$ —
December 31, 2023				
Money market funds included in cash and cash equivalents	$ 65,589	$ 65,589	$ —	$ —
Marketable securities:				
U.S. Treasury notes	103,044	103,044	—	—
U.S. Government agency securities	31,075	31,075	—	—
Corporate bonds	23,970	—	23,970	—
Commercial paper	3,985	—	3,985	—
Total money market funds and marketable securities	$ 227,663	$ 199,708	$ 27,955	$ —

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

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

Cash, cash equivalents, and marketable securities included the following at June 30, 2024 and December 31, 2023:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
		(in thousands)		
As of June 30, 2024				
Money market funds included in cash and cash equivalents	$ 63,854	$ —	$ —	$ 63,854
Marketable securities:				
U.S. Treasury notes	22,763	—	(49)	22,714
U.S. Government agency securities	126,820	1	(115)	126,706
Certificates of deposit	5,447	2		5,449
Corporate bonds	113,885	2	(226)	113,661
Commercial paper	26,373	1	(10)	26,364
Total money market funds and marketable securities	$ 359,142	$ 6	$ (400)	$ 358,748
As of December 31, 2023				
Money market funds included in cash and cash equivalents	$ 65,589	$ —	$ —	$ 65,589
Marketable securities:				
U.S. Treasury notes	102,966	81	(3)	103,044
U.S. Government agency securities	31,068	10	(3)	31,075
Corporate bonds	23,975	2	(7)	23,970
Commercial paper	3,985	—	—	3,985
Total money market funds and marketable securities	$ 227,583	$ 93	$ (13)	$ 227,663

All of the Company's marketable securities as of June 30, 2024 have a contractual maturity of one year or less.

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

The Company held $268.2 million and $44.2 million in marketable securities that were in an unrealized loss position as of June 30, 2024 and December 31, 2023, respectively. The unrealized losses at June 30, 2024 and December 31, 2023 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 such amounts shown in the condensed consolidated statements of cash flows:

	As of June 30, 2024	As of December 31, 2023
	(in thousands)	
Cash and cash equivalents	$ 76,127	$ 68,802
Restricted cash included in deposits and other non-current assets	2,874	1,593
Total cash, cash equivalents, and restricted cash	$ 79,001	$ 70,395

5. Accrued expenses

Accrued expenses as of June 30, 2024 and December 31, 2023 consist of the following:

	As of June 30, 2024	As of December 31, 2023
	(in thousands)	
Employee compensation costs	$ 4,764	$ 6,614
Research and development costs	3,657	5,225
Accrued goods and services	912	4,229
Professional services	940	755
Total	$ 10,273	$ 16,823

6. Lease obligation

Operating Leases

As of June 30, 2024, 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 through November 30, 2026.

On August 11, 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 Company received $1.8 million of leasehold improvement incentives associated with the First Amendment. The Company gained control of the space on February 1, 2024 and recorded a $26.7 million right-of-use asset and a $26.7 million operating lease liability, accordingly, which reflect the leasehold improvement incentive.

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 and are included in deposits and other non-current assets in the accompanying condensed consolidated balance sheets.

During the three months ended June 30, 2024, the Company vacated its leased office and laboratory space in Cambridge, Massachusetts. The Company recorded an impairment charge of $2.8 million to operating expenses during the three months ended June 30, 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 which was executed in August 2024, which is disclosed in Note 13 to the unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. The impairment charge reduced the carrying value of the leased office and laboratory space asset group by $2.8 million.

During the three and six months ended June 30, 2024, the Company incurred lease expenses of $4.4 million and $6.1 million, respectively. During the three and six months ended June 30, 2023, the Company incurred lease expenses

of $0.9 million and $1.8 million, respectively, for operating leases. As of June 30, 2024, the weighted average remaining lease term was 5.2 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 6.9%.

7. Commitments, contingencies and other liabilities

As of June 30, 2024 and December 31, 2023, other current and non-current liabilities consisted of the following:

	As of June 30, 2024	As of December 31, 2023
	(in thousands)	
Other current liabilities		
Lease liability	6,736	3,200
Total other current liabilities	$ 6,736	$ 3,200
Other non-current liabilities		
Lease liability	$ 40,205	$ 17,093
Other	1,000	1,001
Total other non-current liabilities	$ 41,205	$ 18,094

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 provides that the Company repay amounts received under certain circumstances including termination of the agreement, and to pay an amount up to 2.6 times the funding received upon successful development and commercialization of any products developed. 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 June 30, 2024, or December 31, 2023. The Company did not have contingency reserves established for any litigation liabilities as of June 30, 2024, or December 31, 2023.

8. Significant agreements

The Company's significant agreements are described in Note 9 of the December 31, 2023 consolidated financial statements included in its Annual Report on Form 10-K for the year ended December 31, 2023. During the three and six months ended June 30, 2024, there were no material changes to the Company's collaboration agreements or option and license agreements and no new collaboration or license agreements. The Company recorded collaboration revenue of $29.6 million and $4.9 million during the three months ended June 30, 2024 and 2023, respectively. The Company recorded collaboration revenue of $49.1 million and $155.3 million during the six months ended June 30, 2024 and 2023, respectively.

2023 Neurocrine Collaboration Agreement

In April 2024, the Company announced that the joint steering committee with Neurocrine selected a development candidate for the glucocerebrosidase 1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases (the "GBA1 program") under the collaboration and license agreement with Neurocrine entered into in January 2023 (the "2023 Neurocrine Collaboration Agreement"). The joint steering committee selection of a

development candidate for the GBA1 Program triggered a $3.0 million milestone payment to the Company. The Company recorded the $3.0 million as collaboration revenue during the three months ended June 30, 2024.

2023 Novartis Stock Purchase Agreement

Under the stock purchase agreement entered into in December 2023 (the "2023 Novartis Stock Purchase Agreement"), Novartis purchased 2,145,002 shares of common stock of the Company (the "Novartis Shares") for an aggregate purchase price of approximately $20.0 million. The issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement in January 2024 resulted in a premium of $0.7 million. The premium was allocated to the development and commercialization licenses granted to Novartis for two programs pursuant to the license and collaboration agreement with Novartis entered into in December 2023 and was recognized as collaboration revenue during the first quarter of 2024, upon the issuance of the Novartis Shares under the 2023 Novartis Stock Purchase Agreement.

2019 Neurocrine Collaboration Agreement

In February 2024, the Company announced that the joint steering committee with Neurocrine selected a lead development candidate for the gene therapy program for Friedreich's ataxia (the "FA Program") under the collaboration and license agreement with Neurocrine entered into in January 2019 (the "2019 Neurocrine Collaboration Agreement"), which triggered a $5.0 million milestone payment to the Company that was received in the first quarter of 2024. The Company included the $5.0 million that had previously been constrained in the transaction price allocated to the FA Program performance obligation in the three months ended March 31, 2024, accordingly, which resulted in a cumulative catch-up adjustment to collaboration revenue of $4.4 million.

Related Party Collaboration Receivable

The following table presents changes in the balances of the Company's related party collaboration receivable and contract liabilities for the 2023 Neurocrine Collaboration Agreement and the 2019 Neurocrine Collaboration Agreement during the six months ended June 30, 2024:

	Balance at December 31, 2023	Additions	Deductions	Balance at June 30, 2024
		(in thousands)		
Related party collaboration receivables	$ 3,341	$ 8,446	$ (10,238)	$ 1,549
Contract liabilities:				
Deferred revenue	$ 75,240	$ 586	$ (35,837)	$ 39,989

The change in the related party collaboration receivable balance for the three months ended June 30, 2024 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected during the period, for the 2023 and 2019 Neurocrine Collaboration Agreements. Deferred revenue activity for the period includes the recording of $0.6 million of deferred revenue during the six months ended June 30, 2024 related to the fixed transaction price allocation increase for the FA Program, offset by $35.8 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 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) income was as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(in thousands)			
Research and development	$ 1,626	$ 690	$ 2,906	$ 1,553
General and administrative	2,416	1,982	4,709	3,677
Total stock-based compensation expense	$ 4,042	$ 2,672	$ 7,615	$ 5,230

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(in thousands)			
Stock options	$ 2,577	$ 1,903	$ 4,985	$ 3,566
Restricted stock awards and units	1,382	724	2,471	1,565
Employee stock purchase plan awards	83	45	159	99
Total stock-based compensation expense	$ 4,042	$ 2,672	$ 7,615	$ 5,230

Restricted Stock Units

A summary of the status of and changes in unvested restricted stock unit activity under the Company's equity award plans for the six months ended June 30, 2024 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2023	1,370,897	$ 6.65
Granted	851,908	$ 8.16
Vested	(381,069)	$ 6.06
Forfeited	(38,196)	$ 6.51
Unvested restricted stock units as of June 30, 2024	1,803,540	$ 7.49

Stock-based compensation of restricted stock units is based on the fair value of the Company's common stock on the date of grant and is recognized over the vesting period. All of the restricted stock units granted in the six months ended June 30, 2024 vest in equal amounts, annually over three years. The stock-based compensation expense related to restricted stock units was $1.4 million and $2.5 million for the three and six months ended June 30, 2024, respectively. The stock-based compensation expense related to restricted stock units was $0.7 million and $1.6 million for the three and six months ended June 30, 2023, respectively.

As of June 30, 2024, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $11.1 million, which is expected to be recognized over the remaining average vesting period of 2.3 years.

Stock Options

The following is a summary of stock option activity for the six months ended June 30, 2024:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value *(in thousands)*
Outstanding at December 31, 2023	7,425,444	$ 8.52		
Granted	1,999,926	$ 7.81		
Exercised	(58,458)	$ 4.47		
Cancelled or forfeited	(500,794)	$ 14.40		
Outstanding at June 30, 2024	8,866,118	$ 8.05	7.4	9,481
Exercisable at June 30, 2024	4,212,635	$ 8.57	6.4	$ 5,862

As of June 30, 2024, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $22.0 million which is expected to be recognized over the remaining weighted-average vesting period of 2.8 years.

10. Net (loss) income per share

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Unvested restricted common stock awards	22,500	45,000	22,500	45,000
Unvested restricted common stock units	1,803,540	1,313,500	1,803,540	725,876
Outstanding stock options	8,866,118	7,334,771	8,866,118	6,253,497
Total	10,692,158	8,693,271	10,692,158	7,024,373

Basic net (loss) income and diluted weighted-average shares outstanding are as follows for the three and six months ended June 30, 2024 and 2023:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Numerator:				
Net (loss) income *(in thousands)*	$ (10,141)	$ (22,208)	$ (21,471)	$ 101,836
Denominator for basic net (loss) income per share:				
Weighted average shares outstanding-basic	57,721,934	43,520,137	57,419,490	42,102,101
Denominator for diluted net (loss) income per share:				
Weighted average shares outstanding-basic	57,721,934	43,520,137	57,419,490	42,102,101
Common stock options and restricted stock units	—	—	—	1,668,898
Weighted average shares outstanding-diluted	57,721,934	43,520,137	57,419,490	43,770,999
Net (loss) income per share, basic:	$ (0.18)	$ (0.51)	$ (0.37)	$ 2.42
Net (loss) income per share, diluted:	$ (0.18)	$ (0.51)	$ (0.37)	$ 2.33

The pre-funded warrants issued in connection with the underwritten public offering discussed in Note 11 are included in basic and diluted weighted average shares outstanding for the three and six months ended June 30, 2024.

11. Underwritten public offering

On January 4, 2024, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Citigroup Global Markets Inc. and Guggenheim Securities, LLC, as representatives of the 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 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.

12. Related-party transactions

During the three and six months ended June 30, 2024, the Company received scientific advisory board and other scientific advisory services from Dinah Sah, Ph.D., the Company's former Chief Scientific Officer. The total amount of fees paid to Dr. Sah for services provided during the three and six months ended June 30, 2024, was $150,000 and $300,000, respectively. The total amount of fees paid to Dr. Sah for services provided during the three and six months ended June 30, 2023 was $184,000 and $383,800, respectively. During the second quarter of 2023, the Company and Dr. Sah agreed to a fee of $50,000 per month for advisory services from Dr. Sah pursuant to an amendment to the Company's consulting agreement with Dr. Sah that became effective in June 2023.

Under each of the Company's collaboration agreements with Neurocrine, the Company and Neurocrine have agreed to conduct research, development, and commercialization activities for certain of the Company's AAV gene therapy product candidates. Amounts due from Neurocrine are reflected as related party collaboration receivables. As of June 30, 2024, the Company had approximately $0.9 million in related party collaboration receivables associated with the 2023 Neurocrine Collaboration Agreement and approximately $0.7 million in related party collaboration receivables associated with the 2019 Neurocrine Collaboration Agreement.

13. Subsequent Events

In August 2024, the Company entered into an agreement (the "Sublease") to sublease the office and laboratory space leased by the Company at 64 Sidney Street in Cambridge, Massachusetts to a third party (the "Subtenant"). The term of the Sublease is approximately two years and the Company expects to receive approximately $2.6 million from the Subtenant over the term. Prior to entering the Sublease, the Company had fully vacated the space at 64 Sidney Street in Cambridge, Massachusetts.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2023, which was filed with the Securities and Exchange Commission, or the SEC, on February 28, 2024.

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 year ended December 31, 2023, 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; amyotrophic lateral sclerosis, or ALS; 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 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 focus on leveraging our expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the genetic medicine and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, and therefore providing clinically meaningful impact to patients. We are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on AD. Our wholly-owned prioritized pipeline programs include an anti-tau antibody for AD; a superoxide dismutase 1, or SOD1, silencing gene therapy for ALS; and a tau silencing gene therapy for AD. We identified a lead development candidate for our anti-tau antibody program in the first quarter of 2023, which we refer to as VY7523 (formerly referred to as VY-TAU01). We submitted an investigational new drug, or IND, application to the U.S. Food and Drug Administration, or the FDA, for VY7523 in March 2024 and we have obtained clearance of the IND. In May 2024, we dosed the first participant in our Phase 1a single ascending dose, or SAD, trial of VY7523 in healthy volunteers. We expect top-line safety and pharmacokinetic data from our SAD trial in the first half of 2025. We expect to initiate a Phase 1b multiple ascending dose, or MAD, trial of VY7523 in patients with early AD in 2025, which has the potential to generate initial data for slowing the spread of pathological tau via tau positron emission tomography, or PET, imaging in the second half of 2026. We identified a lead development candidate for the SOD1 silencing gene therapy program in the fourth quarter of 2023, which we refer to as VY9323, and we expect to submit the IND application for this program in mid-2025. We completed the pre-IND meeting with the FDA for the VY9323 program in May 2024 and initiated Good Laboratory Practice, or GLP, toxicology studies in July 2024. We promoted our tau silencing gene therapy program to a prioritized program in the first quarter of 2024, based on preclinical data demonstrating robust reductions in tau messenger RNA, or mRNA, in a murine model, and we anticipate submission of an IND for this program in 2026. Our proprietary pipeline also includes an early research initiative to develop a gene therapy for the treatment of AD. This program seeks to combine a vectorized anti-amyloid antibody with a TRACER Capsid.

We are also working with our collaboration partners on multiple programs. In January 2019 and January 2023, we entered into collaboration and license agreements with Neurocrine. Under our agreements with Neurocrine, we are

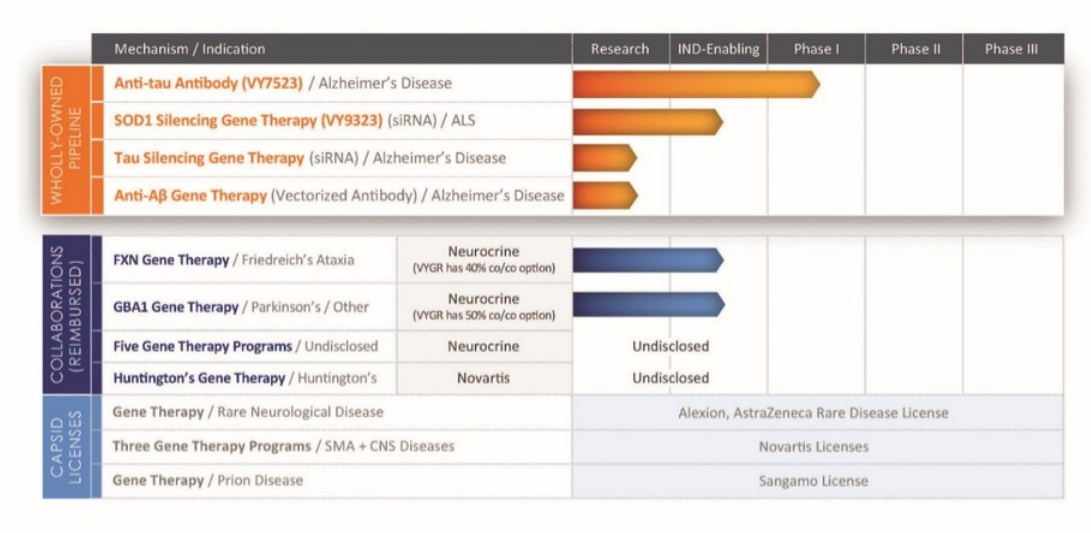
actively advancing two later preclinical stage programs: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, or the GBA1 Program, and a frataxin, or FXN, gene therapy program for Friedreich's ataxia, or the FA Program. The joint steering committee with Neurocrine selected a development candidate for the FA Program in February 2024, and we and Neurocrine expect to file an IND application with the FDA for the FA Program in 2025. The joint steering committee with Neurocrine also selected a development candidate for the GBA1 Program in April 2024, and we and Neurocrine expect to file an IND application with the FDA for the GBA1 Program in 2025. Pursuant to our agreements with Neurocrine, we are also working with Neurocrine on five early-stage programs for the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets. In December 2023, we entered into a license and collaboration agreement with Novartis to provide Novartis certain rights regarding the development of potential gene therapy product candidates for the treatment of spinal muscular atrophy and to collaborate with Novartis to develop gene therapy product candidates for the treatment of Huntington's disease. We have also entered into agreements with licensees including Novartis and Alexion to license or to provide options to receive exclusive licenses to certain TRACER Capsids.

All of the gene therapies in our wholly-owned and collaborative pipeline leverage novel capsids derived from our TRACER™ Capsid discovery platform. TRACER is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier and enhanced CNS tropism in multiple species, including non-human primates.

Overview of Our Pipeline

We have leveraged our TRACER discovery platform and other gene therapy platforms, our expertise with proprietary antibodies, vectorized small interfering RNA, or siRNA, knockdown, gene delivery and our vectorized antibody platform to assemble a pipeline of proprietary antibody, AAV gene therapy and other genetic medicine programs for the treatment of neurological diseases. We have prioritized pipeline programs for our development based on the following criteria: high unmet medical need, target validation, efficient path to human proof of biology, robust preclinical pharmacology, and strong commercial potential. Depending on the disease, we are seeking to develop AAV gene therapies that will use a gene replacement, gene silencing or vectorized antibody approach, and antibodies that will use a passive administration approach.

Our pipeline of programs is summarized in the table below:



Wholly-Owned Programs

Anti-Tau Antibody (VY7523 – formerly VY-TAU01) for the Treatment of Alzheimer's Disease

Our Treatment Approach

We selected VY7523 as our lead humanized anti-tau antibody candidate to advance against AD. VY7523 is an intravenously 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. In contrast to previous N-terminal directed anti-tau antibodies that did not show efficacy in clinical studies, VY7523 targets a distinct C-terminal epitope of tau and has demonstrated robust in vivo inhibition of the spread of pathological tau in a preclinical model. Additional preclinical studies have demonstrated that VY7523 was well-tolerated and demonstrated a favorable pharmacokinetic profile following intravenous administration.

Program Status

In January 2023, we selected VY7523 as our lead humanized anti-tau antibody candidate to advance against AD. We submitted an IND application for VY7523 to the FDA in March 2024 and we obtained clearance of the IND. In May 2024 we dosed the first participant in our Phase 1a SAD trial in healthy volunteers. Enrollment is on track, with a total of approximately 48 participants expected to enroll across multiple cohorts. We expect top-line safety and pharmacokinetic data from our SAD trial in the first half of 2025. A Phase 1b MAD trial in participants with early AD is expected to be initiated in 2025. The MAD trial has the potential to generate initial data for slowing the spread of pathological tau via tau PET imaging in the second half of 2026.

SOD1 Silencing Gene Therapy Program for the Treatment of ALS (VY9323)

Our Treatment Approach

We believe that a therapeutic delivering a vectorized highly potent siRNA construct via intravenous administration of an AAV gene therapy may enable broad CNS knockdown of SOD1, which could potentially slow the decline of functional ability in ALS patients with the SOD1 mutation. We have selected a potent, specific vectorized siRNA transgene targeting SOD1, delivered using a novel TRACER Capsid. We believe that a Phase 1 clinical trial to

demonstrate reductions in SOD1 in the cerebrospinal fluid and in neurofilament light chain in the plasma could provide evidence of target engagement and the attenuation of motor neuron loss, respectively.

Program Status

We have identified VY9323, a potent and specific vectorized siRNA transgene that resulted in substantially extended lifespan and motor function when delivered using a BBB-penetrant capsid in a mouse model. In December 2023, we selected VY9323 as our lead development candidate for our SOD1 program. We plan to submit an IND application to the FDA in mid-2025 for VY9323 and to initiate a Phase 1 clinical trial of VY9323 in subjects with SOD1 ALS for the program as soon as possible thereafter. We completed a pre-IND meeting with the FDA in May 2024 and initiated GLP toxicology studies designed to support a potential IND filing. We expect to evaluate the safety and biological activity of VY9323 in this Phase 1 trial.

Tau Silencing Gene Therapy Program for the Treatment of AD

Our Treatment Approach

We have maintained a long-standing focus on developing proprietary and complimentary approaches to disrupt the progression of tau pathology believed to be central to AD and other tauopathies. A reduction of toxic tau aggregates may slow disease progression and cognitive decline in these diseases. In addition to our aforementioned anti-tau antibody program, we are advancing a gene therapy that leverages an intravenously delivered TRACER Capsid containing a vectorized siRNA, specifically targeting tau mRNA.

Program Status

In the first quarter of 2024, we promoted the tau silencing gene therapy program to a prioritized program on our wholly-owned pipeline, based on its demonstration on in vivo proof-of-concept and expected advancement to IND within two to three years. We are evaluating the optimal combination of payload and capsid for this program, to enable selection of a development candidate. We expect to file an IND for this program in 2026.

Vectorized Anti-Amyloid Antibody Early Research Program for the Treatment of AD

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

Collaboration Programs

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

Our Treatment Approach

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

Our Program Status

Under the collaboration and license agreement with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement, we are developing VY-FXN01 for the treatment of Friedreich's ataxia. VY-FXN01 is currently in preclinical development. In February 2024, the joint steering committee with Neurocrine selected a development candidate combining an FXN gene replacement payload with a novel TRACER Capsid for its FA Program and we and Neurocrine expect to file an IND application with the FDA for the FA Program in 2025. The

selection of a lead development candidate triggered a $5.0 million milestone payment to us, which we received in March 2024.

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

Our Treatment Approach

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

Program Status

Under the collaboration and license agreement with Neurocrine entered into in January 2023, or the 2023 Neurocrine Collaboration Agreement, we are developing gene therapy products directed to the gene that encodes GBA1 for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program. The GBA1 Program is currently in preclinical development. In April 2024, the joint steering committee with Neurocrine selected a development candidate for the GBA1 Program and we and Neurocrine expect to file an IND application with the FDA for the GBA1 Program in 2025. The selection of this development candidate triggered a $3.0 million milestone payment, which we received in the second quarter of 2024.

HD Program (2023 Novartis Collaboration Agreement)

Program Status

On December 28, 2023, or the 2023 Novartis Collaboration Agreement Effective Date, we entered into a license and collaboration agreement with Novartis, or the 2023 Novartis Collaboration Agreement. Under the 2023 Novartis Collaboration Agreement, we and Novartis have agreed to collaborate to develop AAV gene therapy products and product candidates intended for the treatment of Huntington's disease, which we refer to as the Novartis HD Program. The Novartis HD Program is currently in preclinical development. From and after the first IND application filing for the Novartis HD Program, we and Novartis have agreed that Novartis will assume sole responsibility for the development and commercialization of gene therapy products and product candidates under the Novartis HD Program, including all further preclinical and clinical development and any commercialization of the Novartis HD Program products and product candidates.

Collaboration Programs and Licensing Agreements

2023 Novartis Collaboration Agreement

On the 2023 Novartis Collaboration Agreement Effective Date, as described above we entered into the 2023 Novartis Collaboration Agreement, with Novartis 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 under the Novartis HD Program, in each case, leveraging TRACER Capsids and other intellectual property controlled by us.

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 year ended December 31, 2023 under the caption "2023 Novartis 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, or the 2023 Novartis Investment Closing Date.

2023 Novartis Investor Agreement

We and Novartis also entered into an investor agreement on December 28, 2023, or the 2023 Novartis Investor Agreement, which became effective as of the 2023 Novartis Investment Closing Date, providing for standstill and lock-up restrictions.

Pursuant to the terms of the 2023 Novartis Investor Agreement, Novartis has agreed not to, without the prior written approval of us and subject to specified conditions, directly or indirectly acquire shares of our outstanding common stock, publicly seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents to vote any voting securities that we have issued, or undertake other specified actions related to the potential acquisition of additional equity interests in us. Further, Novartis has also agreed not to, and to cause its affiliates not to sell or transfer any of the Novartis Shares without our prior approval, subject to specified conditions.

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 Payloads.

Under the terms of the 2022 Novartis Option and License Agreement, Novartis paid us an upfront payment of $54.0 million. 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. With Novartis' option exercise on two Initial Novartis Targets, we received a $25.0 million option exercise payment in April 2023, and are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of products containing the corresponding Novartis Payload, or the Novartis Licensed Products, incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in our wholly-owned and partnered pipeline. In addition, during the research term, Novartis retains the right to expand the agreement to include options to license capsids for up to two other targets, or the Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and tiered mid- to high-single digit royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids.

Novartis elected not to license a capsid for one Initial Novartis Target under the 2022 Novartis Option and License Agreement 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 Initial Novartis Target has terminated, the research term for this Initial Novartis Target has expired, and we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this Initial Novartis Target. All capsid rights with respect to that Initial Novartis Target have returned to us. 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 year ended December 31, 2023 under the caption "2022 Novartis Option and License Agreement."

2023 Neurocrine Collaboration Agreement

In January 2023, we entered into a collaboration agreement, or the 2023 Neurocrine Collaboration Agreement, with Neurocrine for the research, development, manufacture and commercialization of certain of our AAV gene therapy products. 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.

Under the terms of the 2023 Neurocrine Collaboration Agreement, Neurocrine paid us an upfront payment of approximately $136.0 million and approximately $39.0 million as consideration for an equity purchase of 4,395,588 shares of our common stock in February 2023. 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. The joint steering committee's selection of the development candidate for the GBA1 Program in April 2024 triggered a $3.0 million milestone payment, which we received in May 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. On a country-by-country and 2023 Neurocrine Program-by-2023 Neurocrine Program basis, the parties have agreed royalty payments would commence on the first commercial sale of a 2023 Collaboration Product in such country and terminate upon the latest of (x) the expiration, invalidation or the abandonment of the last patent covering the composition of the 2023 Collaboration Product or its approved method of use in such country, (y) ten years from the first commercial sale of the 2023 Collaboration Product in such country and (z) the expiration of regulatory exclusivity in such country, or the 2023 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patent rights related to a 2023 Collaboration Product, approval of biosimilar products in a given country, or required payment of licensing fees to third parties related to the development and commercialization of any 2023 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and 2023 Collaboration Product-by-2023 Collaboration Product basis upon the expiration of the 2023 Royalty Term applicable to the 2023 Collaboration Product in such country.

The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023. On February 23, 2023, we received the upfront payment, and the shares of our common stock were issued and sold to Neurocrine pursuant to the applicable stock purchase agreement. 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 year ended December 31, 2023 under the caption "2023 Neurocrine Collaboration Agreement."

2019 Neurocrine Collaboration

In January 2019, we entered into the 2019 Neurocrine Collaboration Agreement for the research, development and commercialization of certain of our AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2019 Neurocrine Programs: the NBIb-1817 (VY-AADC) program for the treatment of Parkinson's disease, or the VY-AADC Program; the FA Program, and two other undisclosed programs, which we refer to as the 2019 Discovery Programs.

Under the terms of the 2019 Neurocrine Collaboration Agreement, Neurocrine has paid us an upfront payment of $115.0 million. In connection with the 2019 Neurocrine Collaboration Agreement, Neurocrine also paid us $50.0 million as consideration for an equity purchase of 4,179,728 shares of our common stock. The 2019 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for the research, development, manufacture, and commercialization of gene therapy products, or the 2019 Collaboration Products, under (a) the FA Program of up to $195.0 million, and (b) each of the two 2019 Discovery Programs of up to $130.0 million per 2019 Discovery Program. We may be entitled to receive aggregate commercial milestone payments 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 joint steering committee'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.

Neurocrine has also agreed to pay us royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (b) for each 2019 Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a 2019 Collaboration Product and terminate on the later of (x) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (y) 10 years from the first commercial sale of the 2019 Collaboration Product in such country and (z) the expiration of regulatory exclusivity in such country, or the 2019 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patent rights related to a 2019 Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any 2019 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the 2019 Royalty Term applicable to such 2019 Collaboration Product in such country. 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 year ended December 31, 2023 under the caption "2019 Neurocrine Collaboration Agreement."

Other License Agreements

Alexion License Agreement

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

cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to us.

Effective upon the closing of a transaction on September 20, 2023, Alexion, AstraZeneca Rare Disease, or Alexion, acquired all of Pfizer's rights under the Pfizer Agreement and became the successor-in-interest to Pfizer thereunder. We refer to the Pfizer Agreement following the acquisition, as the Alexion Agreement. The acquisition does not impact the material terms of the option and license agreement.

Under the terms of the Alexion Agreement, we have received an upfront payment of $30 million and a payment of $10 million in connection with the exercise of the Pfizer License Option, which we also refer to as the Alexion License Option, for a rare neurological disease during the fourth quarter of 2022. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115 million for the first Pfizer Licensed CNS Product, which we also refer to as an Alexion Licensed CNS Product, to achieve the applicable milestone. On an Alexion Licensed CNS Product-by-Alexion Licensed CNS Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175 million per Alexion Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Alexion Licensed CNS Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits. For a further description of the Alexion Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023 under the caption "Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)."

Touchlight IP Limited License Agreement

In November 2022, we and Touchlight IP Limited, or Touchlight, entered into a license agreement, or the Touchlight License Agreement, to authorize historical use by us of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process. The terms of the Touchlight License Agreement include a one-time, non-refundable technology access fee of $5.0 million, paid to Touchlight during the fourth quarter of 2022. The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight by us if we or our program collaborators or licensees choose to utilize in a therapeutic product certain TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, we are obligated to pay low single-digit royalties to Touchlight on future payments we receive in connection with licensing of certain TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any of our therapeutic programs.

2024 Underwritten Public Offering

In January 2024, we issued and sold 7,777,778 shares of our common stock and, in lieu of common stock to certain investors, pre-funded warrants to purchase 3,333,333 shares of common stock in a public offering, or the 2024 Public Offering, at a public offering price of $9.00 per share of common stock and $8.999 per pre-funded warrant. The 2024 Public Offering resulted in net proceeds to the Company of approximately $93.5 million after deducting underwriting discounts and commissions and offering expenses.

Each pre-funded warrant has an exercise price of $0.001 per share and is exercisable for one share of common stock from the date of issuance until the pre-funded warrant is exercised in full. Under the terms of the pre-funded warrants, we may not effect the exercise of any such warrant, and a holder will not be entitled to exercise any portion of any such warrant, that, upon giving effect to or immediately prior to, would cause: (1) the aggregate number of shares of our common stock beneficially owned by such holder (together with its affiliates) to exceed 9.99% of the number of shares of our common stock outstanding immediately after giving effect to the exercise; or (2) the combined voting power of our securities beneficially owned by such holder (together with its affiliates) to exceed 9.99% of the combined voting power of all of our securities outstanding immediately after giving effect to the exercise, as such percentage ownership is determined in accordance with the terms of the pre-funded warrants. However, any holder of a pre-funded warrant may increase or decrease such percentage to any other percentage not in excess of 19.99% provided that any such increase will not be effective until the 61st day after notice from the holder is delivered to us.

Accumulated Deficit; Expenses

Despite reporting $132.3 million in net income for the year ended December 31, 2023, we have a history of incurring significant losses. As of June 30, 2024, we had an accumulated deficit of $282.7 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct clinical trials in connection with our anti-tau antibody program;
- conduct preclinical development activities and initiate IND application-enabling studies and clinical trials in connection with our SOD1 ALS gene therapy program;
- continue investing in our proprietary antibody program, gene therapy and vectorized antibody platforms and programs, and other research and development initiatives;
- increase our investment in and support for TRACER, our proprietary discovery platform to facilitate the selection of AAV capsids and expand our investment to discover TRACER Capsids with broad tropism in CNS and other tissues with cell-specific transduction properties for particular therapeutic applications;
- increase our investment in the 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;
- continue 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 June 30, 2024, we recognized $27.6 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $1.1 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, and $0.9 million of collaboration revenue from the 2023 Novartis Collaboration Agreement. For the six months ended June 30, 2024, we recognized $39.1 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $7.6 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, and $2.4 million of collaboration revenue from the 2023 Novartis Collaboration Agreement.

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

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 program and gene therapy and vectorized antibody 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 six months ended June 30, 2024, 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 clinical trial to evaluate VY7523 (formerly referred to as VY-TAU01) which we initiated in the first half of 2024, 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 FDA or the European Medicines Agency or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;
- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs; or
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, 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 six months ended June 30, 2024, our general and administrative expenses have increased as compared to the amount recorded in the same period in prior year, primarily due to increased facilities costs and increased employee related costs.

Other Income, Net

Other income consists primarily of interest income on our marketable securities.

Critical Accounting Policies and Estimates

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate. There were no changes to our critical accounting policies during the six months ended June 30, 2024, as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023. 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 "*Critical Accounting Policies and Estimates*" in our Annual Report on Form 10-K, as filed with the SEC on February 28, 2024.

Results of Operations

Comparison of the three months ended June 30, 2024 and 2023

The following table summarizes our results of operations for the three months ended June 30, 2024 and 2023, together with the changes in those items in dollars:

	Three Months Ended June 30,		
	2024	2023	Change
		(in thousands)	
Collaboration revenue	$ 29,578	$ 4,853	$ 24,725
Operating expenses:			
Research and development	34,452	21,985	12,467
General and administrative	10,151	8,294	1,857
Total operating expenses	44,603	30,279	14,324
Other income, net:			
Interest income	4,888	3,274	1,614
Other income	20	3	17
Total other income, net	4,908	3,277	1,631
Net loss before income taxes	$ (10,117)	$ (22,149)	$ 12,032

Collaboration Revenue

Collaboration revenue was $29.6 million and $4.9 million for the three months ended June 30, 2024 and 2023, respectively. During the three months ended June 30, 2024, we recognized collaboration revenue in connection with the following agreements:

- $27.6 million with the 2023 Neurocrine Collaboration Agreement, which includes a $3.0 million milestone payment earned as result of the selection of a development candidate for the GBA1 Program;
- $1.1 million with the 2019 Neurocrine Collaboration Agreement; and
- $0.9 million with the 2023 Novartis Collaboration Agreement.

During the three months ended June 30, 2023, we recognized collaboration revenue in connection with the following agreements:

- $2.9 million with the 2023 Neurocrine Collaboration Agreement;
- $1.6 million with the 2019 Neurocrine Collaboration Agreement; and
- $0.3 million with other agreements.

Research and Development Expense

Research and development expenses increased by $12.5 million from $22.0 million for the three months ended June 30, 2023, to $34.5 million for the three months ended June 30, 2024. The increase in research and development expenses for the three months ended June 30, 2024 was primarily attributable to the following:

- approximately $6.0 million for increased 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, along with our impairment charge on our leased office and laboratory space in Cambridge, Massachusetts;
- approximately $3.7 million for increased employee and consultant related costs associated with higher headcount in research and development functions, including targeted development team hires to support our advancing pipeline as compared to the three months ended June 30, 2023; and
- approximately $2.4 million for external research and development costs related to increased program-related spending, particularly on our anti-tau antibody program and SOD1 program, along with spend on the Novartis HD Program during the second quarter of 2024.

General and Administrative Expense

General and administrative expense increased by $1.9 million from $8.3 million for the three months ended June 30, 2023, to $10.2 million for the three months ended June 30, 2024. The increase in general and administrative expense was primarily attributable to increased facilities costs and increased employee related costs.

Other Income, net

Other income, net increased by approximately $1.6 million. Other income, net during both the three months ended June 30, 2024 and 2023 primarily reflects interest income on marketable securities balances. The increase was due to increased balances of marketable securities during the three months ended June 30, 2024, as compared to the three months ended June 30, 2023.

Comparison of the six months ended June 30, 2024 and 2023

The following table summarizes our results of operations for the six months ended June 30, 2024 and 2023, together with the changes in those items in dollars:

	Six Months Ended June 30,		
	2024	2023	Change
		(in thousands)	
Collaboration revenue	$ 49,094	$ 155,333	$ (106,239)
Operating expenses:			
Research and development	61,544	40,553	20,991
General and administrative	18,758	17,322	1,436
Total operating expenses	80,302	57,875	22,427
Other income, net:			
Interest income	9,755	5,138	4,617
Other income	20	3	17
Total other income, net	9,775	5,141	4,634
Net (loss) income before income taxes	$ (21,433)	$ 102,599	$ (124,032)

Collaboration Revenue

Collaboration revenue was $49.1 million and $155.3 million for the six months ended June 30, 2024 and 2023, respectively. During the six months ended June, 30 2024, we recognized collaboration revenue in connection with the following agreements:

- $39.1 million with the 2023 Neurocrine Collaboration Agreement, which includes a $3.0 million milestone payment earned as result of the selection of a development candidate for the GBA1 Program;
- $7.6 million with the 2019 Neurocrine Collaboration Agreement; and
- $2.4 million with the 2023 Novartis Collaboration Agreement.

During the six months ended June 30, 2023, we recognized collaboration revenue in connection with the following agreements:

- $79.0 million with Novartis' decision to exercise two Novartis License Options, along with the expiration of a third Novartis License Option;
- $72.4 million with the 2023 Neurocrine Collaboration Agreement;
- $3.6 million with the 2019 Neurocrine Collaboration Agreement; and
- $0.3 million with other agreements.

Research and Development Expense

Research and development expense increased by $21.0 million from $40.6 million for the six months ended June 30, 2023 to $61.5 million for the six months ended June 30, 2024. The increase in research and development expense was primarily attributable to the following:

- approximately $8.4 million for increased 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, along with our impairment charge on our leased office and laboratory space in Cambridge, Massachusetts;
- approximately $6.6 million for increased employee and consultant related costs associated with higher headcount in research and development functions as compared to the same period in the prior year; and
- approximately $5.1 million for external research and development costs related to increased program-related spending, particularly on our anti-tau antibody program and SOD1 program and spend on the Novartis HD Program.

General and Administrative Expense

General and administrative expense increased by $1.4 million from $17.3 million for the six months ended June 30, 2023, to $18.8 million for the six months ended June 30, 2024. The increase in general and administrative expense was primarily attributable to increased facilities costs and increased employee related costs.

Other Income, net

Other income, net increased by approximately $4.6 million. Other income, net during both the six months ended June 30, 2024 and 2023 primarily reflects interest income on marketable securities balances. The increase was due to

increased balances of marketable securities during the six months ended June 30, 2024, as compared to the six months ended June 30, 2023.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings of our common stock and pre-funded warrants to acquire our common stock, private placements of our common stock, and strategic collaborations and option and license arrangements, including our strategic collaborations and option and license agreements with Neurocrine, Novartis, and Alexion.

During the six months ended June 30, 2024, the 2024 Public Offering resulted in net proceeds to the Company 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 2,145,002 shares of our common stock 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 six months ended June 30, 2024 and 2023:

	Six Months Ended June 30,	
	2024	2023
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ 27,187	$ 122,730
Investing activities	(132,008)	(10,172)
Financing activities	113,427	32,854
Net increase in cash, cash equivalents, and restricted cash	$ 8,606	$ 145,412

Net Cash Provided by Operating Activities

Net cash provided by operating activities was $27.2 million during the six months ended June 30, 2024, compared to $122.7 million of net cash provided by operating activities during the six months ended June 30, 2023. The decrease was primarily due to our net loss during the 2024 period as compared to net income during the 2023 period.

Net Cash Used in Investing Activities

Net cash used in investing activities was $132.0 million during the six months ended June 30, 2024, compared to $10.2 million during the six months ended June 30, 2023. The change was primarily due to increased purchases of marketable securities during the six months ended June 30, 2024 as compared to the six months ended June 30, 2023.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $113.4 million during the six months ended June 30, 2024, driven by proceeds from the issuance of common stock and pre-funded warrants in connection with the 2024 Public Offering and the issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement. Net cash provided by financing activities during the six months ended June 30, 2023 was driven by proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement.

Funding Requirements

Our expenses increased during the six months ended June 30, 2024, as compared with the six months ended June 30, 2023, as our development programs progressed and we increased headcount. We expect our expenses to continue to increase as we continue the research and development of, conduct clinical trials of, and seek marketing approval for our product candidates, including our Phase 1a SAD clinical trial to evaluate VY7523 initiated in the first half of 2024, and as we continue to perform our obligations 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 for our wholly-owned programs and to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators, as applicable. 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 market 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 June 30, 2024, we had cash, cash equivalents, and marketable securities of $371.0 million. Based upon our current operating plans, we expect that our existing cash, cash equivalents, and marketable securities at June 30, 2024, 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 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 Phase 1a SAD clinical trial to evaluate VY7523 initiated in the first half of 2024;
- 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 future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty payments, under any collaboration or license agreements to which we might become a party, such as the Touchlight License Agreement;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;

- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company and maintaining adequate product, clinical trial, and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

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

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

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

Contractual Obligations

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

Our agreements to license intellectual property include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. We may also be required to pay annual maintenance fees or minimum amounts payable ranging from low-four digits to low five-digits depending upon the terms of the applicable agreement. In certain instances, we are also obligated to pay our licensors royalties based on sales of products, if approved, using the intellectual property licensed under the applicable agreement.

We also have non-cancelable operating lease commitments arising from our leases of office and laboratory space at our facilities in Cambridge and Lexington, Massachusetts. For more information, refer to Note 6 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Off-Balance Sheet Arrangements

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

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to market risk related to changes in interest rates. We have policies requiring us to invest in high-quality issuers, limit our exposure to any individual issuer, and ensure adequate liquidity. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of money market funds and marketable securities and are invested in U.S. Treasury notes. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, 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 six months ended June 30, 2024.

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 June 30, 2024. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of June 30, 2024, 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 June 30, 2024, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of any such matters cannot be predicted with certainty, as of June 30, 2024, we were not party to any material pending proceedings. No material governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

ITEM 1A. RISK FACTORS

We are subject to a number of risks that could adversely affect our business, results of operations financial condition and future prospects including those identified in our Annual Report on Form 10-K for the year ended December 31, 2023, filed with the SEC on February 28, 2024.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

On April 1, 2024, we granted to an executive a restricted stock unit award settleable for an aggregate of 105,000 shares of our common stock. This restricted stock unit award was granted outside of our 2015 Stock Option and Incentive Plan as an inducement material to such individual's acceptance of an offer of employment with us in accordance with Nasdaq Listing Rule 5635(c)(4). We intend to file a registration statement on Form S-8 to register the shares of common stock underlying this award prior to the time at which the award becomes settleable.

ITEM 5. OTHER INFORMATION

(c) Director and Officer Trading Arrangements

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as such terms are defined in Items 408(a) and 408(c) of Regulation S-K, respectively) during the quarterly period covered by this report.

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*	First Amendment to Collaboration Agreement, by and between Registrant and Neurocrine Biosciences, Inc., dated April 3, 2024.					X
10.2	Employment Agreement, by and between the Registrant and Nathan Jorgensen, Ph.D., dated as of June 11, 2024.	8-K	10.1	06/13/2024	001-37625	
31.1	Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.					X
31.2	Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.					X
32.1+	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act Rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.					X
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.					X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.					X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.					X
104	Cover Page Interactive Data File – The cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document					

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

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

SIGNATURES

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

Date: August 6, 2024

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)

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

FIRST AMENDMENT TO COLLABORATION AND LICENSE AGREEMENT

This **FIRST AMENDMENT TO THE COLLABORATION AND LICENSE AGREEMENT** (this"**Amendment**") is effective as of April 3, 2024 (the "**Amendment Effective Date**") by and between **VOYAGER THERAPEUTICS, INC.**, a Delaware corporation ("**Voyager**"), having its principal place of business at 75 Hayden Avenue, Lexington, MA 02421, and **NEUROCRINE BIOSCIENCES, INC.**, a Delaware corporation ("**Neurocrine**"), having its principal place of business at 12780 El Camino Real, San Diego, CA 92130. Voyager and Neurocrine may each be referred to herein individually as a "**Party**" and collectively as the "**Parties**".

RECITALS

A. Voyager and Neurocrine are parties to a Collaboration and License Agreement, dated January 8, 2023 (the "**Agreement**"), pursuant to which the Parties are collaborating to develop gene therapy products directed to GBA1 and other genetic targets, and under which Voyager has the right to elect to co-develop and co-commercialize Products directed to GBA1 in the United States.

B. The Parties have agreed to amend the Agreement, in accordance with Section 15.12 thereof, to change the trigger for Voyager's exercise of such right and related financial terms.

NOW, THEREFORE, Voyager and Neurocrine agree as follows:

1. AMENDMENT OF THE AGREEMENT

The Parties hereby agree to amend the terms of the Agreement as provided below, effective as of the Amendment Effective Date. Where the Agreement is not explicitly amended, the terms of the Agreement will remain in force. Capitalized terms used in this Amendment that are not otherwise defined herein shall have the meanings such terms are given in the Agreement.

1.1 Section 1.36 of the Agreement (definition of Co-Co Trigger Date) is hereby deleted in its entirety and replaced with the following:

> "Co-Co Trigger Date" means: (a) the date on which Voyager receives topline data from the first Clinical Trial in Parkinson's disease of a Product that is the subject of the GBA1 Program; or (b) in the event that the JSC decides to focus on a different indication other than Parkinson's disease for Development under the GBA1 Program prior to Voyager's receipt of such topline data in Parkinson's disease, the later to occur of (i) the date on which Voyager receives topline data from the first Clinical Trial of a Product that is the subject of the GBA1 Program or (ii) the date on which the JSC decides definitively not to pursue Parkinson's disease as an indication for Development under the GBA1 Program.

1.2 Section 4.1.1 of the Agreement is hereby deleted in its entirety and is replaced with the following:

4.1.1 Voyager's Opt-In Right. Voyager shall have the right to elect to co-Develop and co-Commercialize Products that are the subject of the GBA1 Program in the United States (the "Co-Co Option") by providing Neurocrine with written notice of such election within [**] following the Co-Co Trigger Date. Upon such exercise, the Parties shall negotiate in good faith and enter into an agreement, which shall be based on terms and conditions substantially the same as those set forth in this Section 4.1 and otherwise consistent with this Agreement (each such agreement, a "Co-Co Agreement"), pursuant to which the Parties will jointly Develop and Commercialize and share equally in the Development Costs, Commercialization costs and profit or loss resulting from the Development and Commercialization of such Products in the United States (the "Co-Co Territory"). If Voyager exercises the Co-Co Option, (a) each Product in the GBA1 Program shall be designated a "Co-Co Product" hereunder and the GBA1 Program shall be designated the "Co-Co Program" hereunder, (b) the Parties will share equally in United States Development Costs incurred thereafter, and (c) Voyager will reimburse Neurocrine, as described in Sections 4.1.2(d), 4.1.3 and 7.3, for fifty percent (50%) of all Development Costs incurred by Neurocrine in connection with the Development of Products in the GBA1 Program prior to Voyager's exercise of the Co- Co Option (the "Reimbursable Costs"); provided that following the first Change of Control of Voyager, Voyager will be obligated to pay interest on the Reimbursable Costs equal to [**]% per annum (paid by the same mechanism as the Reimbursable Costs) unless Voyager pays all of the Reimbursable Costs, without interest, within [**] after the later of (i) Voyager's exercise of the Co-Co Option and (ii) effectiveness of such Change of Control. If Voyager does not exercise the Co-Co Option within [**] after the Co-Co Trigger Date, or if Voyager provides written notice to Neurocrine at any time prior to the Co-Co Trigger Date that it waives its right to exercise its Co-Co Option, then any accrued milestone payments would become payable by Neurocrine in accordance with Section 8.2(b) within [**].

1.3 Section 8.2(b) of the Agreement is hereby deleted in its entirety and replaced with the following:

(b) If the Development Milestone described in Section 8.2.1(c) or any subsequent Development Milestone described in Section 8.2.1 is achieved prior to the date that is [**] after the Co-Co Trigger Date, then notwithstanding anything herein to the contrary, the corresponding Milestone Payment will not be due unless and until (i) the Co-Co Trigger Date occurs and Voyager does not exercise its Co-Co Option or (ii) Voyager waives its right to exercise the Co-Co Option as set forth in Section 4.1.1 (the date on which the foregoing (i) or (ii) occurs, the "Co-Co Expiration Date"). In such event, any unpaid Milestone Payment(s) for any Milestone Event(s) achieved prior to the Co-Co Expiration Date will be due within [**] after the Co-Co Expiration Date. If Voyager exercises its Co-Co Option, then notwithstanding anything herein to the contrary, Voyager shall be entitled to receive Milestone Payments only with respect to any Milestone Event that relates to the Territory outside the Co-Co Territory for so long as the GBA1 Program remains a Co-Co Program, as further provided below. If the Co-Co Agreement is terminated and the GBA1 Program is no longer a Co-Co Program, then the tables in Section 8.2.1 (for Development Milestones) and Section 8.2.3 (for Commercial Milestones) shall thereafter apply, but only with respect to Milestone Events achieved after termination of the Co-Co Agreement.

1.4 Section 8.2(c) of the Agreement is hereby deleted in its entirety and replaced with the following:

(c) Subject to Section 8.2(b) above, and except as expressly set forth below, each Milestone Payment shall be deemed earned as of the achievement of the corresponding Milestone Event.

1.5 The following is hereby added to the end of the first paragraph under the tables in Section 8.2.1 of the Agreement:

If Voyager does not exercise its Co-Co Option or waives its right to the Co-Co Option and thereafter any two (2) Products in the GBA1 Program achieves a Milestone Event as set forth in any of Sections 8.2.1(c)-(i) above for [**] as a first Indication, then notwithstanding anything herein to the contrary, such Milestone Event (and any prior Milestone Event not previously paid as further described in the following paragraph) shall be payable as a second Indication and the next Indication for such Product to achieve such Milestone Event shall be payable as a first Indication.

1.6 Section 15.8 is hereby amended to update Voyager's address in two places to 75 Hayden Avenue, Lexington, MA 02421.

1.7 The Parties agree (a) to equally share Development Costs incurred by [**], and (b) that [**]. The Parties will also equally share the Out-of-Pocket Costs incurred by [**]. The Parties agree that if Development Costs incurred by the Parties in connection with [**] were to increase by more than [**], the Parties will seek approval of the JSC for such additional Development Costs.

2. MISCELLANEOUS

2.1 Full Force and Effect. This Amendment amends the terms of the Agreement and is deemed incorporated into the Agreement. The provisions of the Agreement, as amended by this Amendment, remain in full force and effect.

2.2 Entire Agreement. The Agreement and this Amendment constitute the entire agreement, both written and oral, between the Parties with respect to the subject matter hereof, and any and all prior agreements with respect to the subject matter hereof, either written or oral, expressed or implied, are superseded hereby, merged and canceled, and are null and void and of no effect.

2.3 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.

{Signature Page Follows}

IN WITNESS WHEREOF, the Parties have executed this Amendment as of the Amendment Effective Date.

VOYAGER THERAPEUTICS, INC.	**NEUROCRINE BIOSCIENCES, INC.**
By: */s/ Alfred Sandrock*	By: */s/ Kyle Gano*
Name: Alfred Sandrock	Name: Kyle Gano
Title: Chief Executive Officer	Title: Chief Business Development Officer

Exhibit 31.1

Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended June 30, 2024 of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 6, 2024

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

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

Exhibit 31.2

Certification

I, Nathan Jorgensen, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended June 30, 2024 of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 6, 2024

/s/ Nathan Jorgensen, Ph.D.

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

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Voyager Therapeutics, Inc. (the "Company") for the period ended June 30, 2024, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 that to his knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 6, 2024

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

Date: August 6, 2024

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