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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2024

OR

☐ **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 001-37553

REGENXBIO Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-1851754**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
9804 Medical Center Drive Rockville, MD	**20850**
(Address of principal executive offices)	**(Zip Code)**

(240) 552-8181
(Registrant's telephone number, including area code)
Not Applicable
(Former name, former address and former fiscal year, if changed since last report)

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

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	RGNX	The 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 ☒

As of October 31, 2024, there were 49,545,071 shares of the registrant's common stock, par value $0.0001 per share, issued and outstanding.

REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2024

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INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "anticipate," "assume," "believe," "continue," "could," "design," "estimate," "expect," "forecast," "goal," "intend," "may," "objective," "plan," "position," "potential," "predict," "project," "seek," "should," "will," "would" or variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations, estimates and assumptions and analyses in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- our ability to establish and maintain development partnerships, including our collaboration with AbbVie to develop and commercialize ABBV-RGX-314;
- our ability to obtain and maintain regulatory approval of our product candidates and the labeling for any approved products;
- the timing of enrollment, commencement, completion and the success of our AAVIATE®, AFFINITY BEYOND®, AFFINITY DUCHENNE®, ALTITUDE®, ASCENT™, ATMOSPHERE® and CAMPSIITE® clinical trials;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain, maintain and enforce intellectual property protection for our product candidates and technology, and defend against third-party intellectual property-related claims;
- our expectations regarding the development and commercialization of product candidates currently being developed by third parties that utilize our technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our products that are approved;
- our expectations regarding our expenses and revenue;
- our strategic pipeline prioritization and corporate restructuring, including plans for advancing our product candidates, the expected charges and cost savings associated with our restructuring and any future cost reduction measures;
- our ability to execute strategic alternatives for our de-prioritized rare neurodegenerative disease clinical-stage programs;
- our expectations regarding our need for additional financing and our ability to obtain additional financing;
- our expectations regarding the outcome of legal proceedings;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- changes in the financial markets and banking system that may affect the availability and terms on which we may obtain financing and our ability to accurately predict how long our existing cash resources will be sufficient to fund our anticipated operating expenses.

You should carefully read the factors discussed in the sections titled "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and the factors discussed elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2023 and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance, and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this Quarterly Report on Form 10-Q. Except as required by law, we disclaim any duty to update any forward-looking statements, whether as a result of new information, future events or otherwise.

Available Information

Our principal offices are located at 9804 Medical Center Drive, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. The information contained in, or that can be accessed through, our website is not a part of, or incorporated by reference in, this Quarterly Report on Form 10-Q. We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. You may obtain any reports, proxy and information statements, and other information that we file electronically with the SEC at www.sec.gov.

You also may view and download copies of our SEC filings free of charge at our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and is not considered part of, this Quarterly Report on Form 10-Q. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our business. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

As used in this Quarterly Report on Form 10-Q, the terms "REGENXBIO," "we," "us," "our" or the "Company" mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

AAVIATE, AFFINITY BEYOND, AFFINITY DUCHENNE, ALTITUDE, ATMOSPHERE, CAMPSIITE, NAV, NAVXCELL, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective holders.

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share data)

	September 30, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 56,617	$ 34,522
Marketable securities	198,843	240,736
Accounts receivable, net	23,604	24,790
Prepaid expenses	11,002	14,520
Other current assets	23,330	20,403
Total current assets	313,396	334,971
Marketable securities	23,108	38,871
Accounts receivable	404	701
Property and equipment, net	120,551	132,103
Operating lease right-of-use assets	55,293	60,487
Restricted cash	2,030	2,030
Other assets	4,332	4,807
Total assets	$ 519,114	$ 573,970
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 19,522	$ 22,786
Accrued expenses and other current liabilities	48,642	49,703
Deferred revenue	144	148
Operating lease liabilities	7,720	7,068
Liability related to sale of future royalties	26,697	50,567
Total current liabilities	102,725	130,272
Operating lease liabilities	76,342	82,222
Liability related to sale of future royalties	35,052	43,485
Other liabilities	3,579	6,249
Total liabilities	217,698	262,228
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2024 and December 31, 2023; 49,534 and 44,046 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	5	4
Additional paid-in capital	1,182,956	1,021,214
Accumulated other comprehensive loss	(582)	(4,429)
Accumulated deficit	(880,963)	(705,047)
Total stockholders' equity	301,416	311,742
Total liabilities and stockholders' equity	$ 519,114	$ 573,970

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Revenues				
License and royalty revenue	$ 24,197	$ 28,914	$ 62,114	$ 68,029
Total revenues	24,197	28,914	62,114	68,029
Operating Expenses				
Cost of revenues	12,387	12,388	27,249	25,975
Research and development	54,429	58,183	158,142	176,585
General and administrative	19,422	23,083	56,568	69,415
Impairment of long-lived assets	—	—	2,101	—
Other operating expenses	37	220	32	279
Total operating expenses	86,275	93,874	244,092	272,254
Loss from operations	(62,078)	(64,960)	(181,978)	(204,225)
Other Income (Expense)				
Interest income from licensing	25	56	91	166
Investment income	3,276	4,660	9,213	8,953
Interest expense	(820)	(1,624)	(3,242)	(5,499)
Total other income	2,481	3,092	6,062	3,620
Net loss	$ (59,597)	$ (61,868)	$ (175,916)	$ (200,605)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	1,684	2,685	3,847	7,988
Total other comprehensive income	1,684	2,685	3,847	7,988
Comprehensive loss	$ (57,913)	$ (59,183)	$ (172,069)	$ (192,617)
Net loss per share, basic and diluted	$ (1.17)	$ (1.41)	$ (3.59)	$ (4.60)
Weighted-average common shares outstanding, basic and diluted	50,800	43,945	49,051	43,644

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands)

	Three Months Ended September 30, 2024					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2024	49,317	$ 5	$ 1,171,894	$ (2,266)	$ (821,366)	$ 348,267
Vesting of restricted stock units, net of tax	31	—	—	—	—	—
Exercise of stock options, net of tax	108	—	398	—	—	398
Issuance of common stock under employee stock purchase plan	78	—	780	—	—	780
Stock-based compensation expense	—	—	9,884	—	—	9,884
Unrealized gain on available-for-sale securities, net	—	—	—	1,684	—	1,684
Net loss	—	—	—	—	(59,597)	(59,597)
Balances at September 30, 2024	49,534	$ 5	$ 1,182,956	$ (582)	$ (880,963)	$ 301,416

	Three Months Ended September 30, 2023					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2023	43,621	$ 4	$ 996,239	$ (10,098)	$ (580,290)	$ 405,855
Vesting of restricted stock units, net of tax	34	—	—	—	—	—
Exercise of stock options, net of tax	6	—	92	—	—	92
Issuance of common stock under employee stock purchase plan	73	—	1,243	—	—	1,243
Issuance of common stock upon private placement, net of transaction costs of $126	257	—	4,874	—	—	4,874
Stock-based compensation expense	—	—	10,219	—	—	10,219
Unrealized gain on available-for-sale securities, net	—	—	—	2,685	—	2,685
Net loss	—	—	—	—	(61,868)	(61,868)
Balances at September 30, 2023	43,991	$ 4	$ 1,012,667	$ (7,413)	$ (642,158)	$ 363,100

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands)

	Nine Months Ended September 30, 2024					
	Common Stock		Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Loss	Deficit	Equity
Balances at December 31, 2023	44,046	$ 4	$ 1,021,214	$ (4,429)	$ (705,047)	$ 311,742
Vesting of restricted stock units, net of tax	340	—	(910)	—	—	(910)
Exercise of stock options, net of tax	281	—	1,474	—	—	1,474
Issuance of common stock under employee stock purchase plan	105	—	1,191	—	—	1,191
Issuance of common stock and pre-funded warrants upon public offering, net of transaction costs of $534	4,565	1	131,066	—	—	131,067
Exercise of pre-funded warrants	197	—	—	—	—	—
Stock-based compensation expense	—	—	28,921	—	—	28,921
Unrealized gain on available-for-sale securities, net	—	—	—	3,847	—	3,847
Net loss	—	—	—	—	(175,916)	(175,916)
Balances at September 30, 2024	49,534	$ 5	$ 1,182,956	$ (582)	$ (880,963)	$ 301,416

	Nine Months Ended September 30, 2023					
	Common Stock		Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Loss	Deficit	Equity
Balances at December 31, 2022	43,299	$ 4	$ 973,145	$ (15,401)	$ (441,553)	$ 516,195
Vesting of restricted stock units, net of tax	161	—	(419)	—	—	(419)
Exercise of stock options, net of tax	171	—	1,312	—	—	1,312
Issuance of common stock under employee stock purchase plan	103	—	1,826	—	—	1,826
Issuance of common stock upon private placement, net of transaction costs of $126	257	—	4,874	—	—	4,874
Stock-based compensation expense	—	—	31,929	—	—	31,929
Unrealized gain on available-for-sale securities, net	—	—	—	7,988	—	7,988
Net loss	—	—	—	—	(200,605)	(200,605)
Balances at September 30, 2023	43,991	$ 4	$ 1,012,667	$ (7,413)	$ (642,158)	$ 363,100

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2024	2023
Cash flows from operating activities		
Net loss	$ (175,916)	$ (200,605)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	28,921	31,929
Depreciation and amortization	12,192	12,985
Net amortization of premiums (accretion of discounts) on marketable debt securities	(3,027)	929
Net realized gain on investments	—	(2,205)
Impairment of long-lived assets	2,101	—
Non-cash interest expense	412	(569)
Other non-cash adjustments	(60)	(1)
Changes in operating assets and liabilities		
Accounts receivable	1,574	566
Prepaid expenses	3,518	1,339
Other current assets	(2,804)	(13,573)
Operating lease right-of-use assets	4,586	4,245
Other assets	475	1,728
Accounts payable	(3,330)	(4,011)
Accrued expenses and other current liabilities	(1,448)	(1,546)
Deferred revenue	(4)	(1,218)
Operating lease liabilities	(6,021)	(5,004)
Other liabilities	(2,670)	(2,636)
Net cash used in operating activities	(141,501)	(177,647)
Cash flows from investing activities		
Purchases of marketable debt securities	(173,916)	(67,925)
Maturities of marketable debt securities	238,446	231,753
Sales of equity securities	—	1,975
Purchases of property and equipment	(1,357)	(8,834)
Net cash provided by investing activities	63,173	156,969
Cash flows from financing activities		
Proceeds from exercise of stock options	1,474	1,312
Taxes paid related to net settlement of stock-based awards	(910)	(419)
Proceeds from issuance of common stock under employee stock purchase plan	1,191	1,826
Proceeds from public offering of common stock and pre-funded warrants, net of issuance costs	131,067	—
Proceeds from private placement of common stock, net of issuance costs	—	4,874
Offering expenses related to at-the-market offering program	(158)	(51)
Repayments under liability related to sale of future royalties, net of imputed interest	(32,241)	(30,546)
Net cash provided by (used in) financing activities	100,423	(23,004)
Net increase (decrease) in cash and cash equivalents and restricted cash	22,095	(43,682)
Cash and cash equivalents and restricted cash		
Beginning of period	36,552	98,982
End of period	$ 58,647	$ 55,300

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

REGENXBIO INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

1. Nature of Business

REGENXBIO Inc. (the Company) is a clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's investigational gene therapies use adeno-associated virus (AAV) vectors from its proprietary gene delivery platform (NAV Technology Platform). The NAV® Technology Platform consists of exclusive rights to a large portfolio of AAV vectors, including commonly used AAV8 and AAV9. The Company has developed a broad pipeline of gene therapy product candidates using the NAV Technology Platform as a one-time treatment to address an array of diseases. In addition to its internal product development efforts, the Company also selectively licenses the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies (NAV Technology Licensees). As of September 30, 2024, the NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma®, and in the preclinical and clinical development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

The Company has incurred cumulative losses since inception and as of September 30, 2024, had generated an accumulated deficit of $881.0 million. The Company's ability to transition to recurring profitability is dependent upon achieving a level of revenues adequate to support its cost structure, which depends heavily on the successful development, approval and commercialization of its product candidates. The Company may never achieve recurring profitability, and unless and until it does, will continue to need to raise additional capital through equity offerings, licensing and collaboration arrangements, or other non-dilutive financings. There is no assurance that the Company will be able to raise sufficient capital or obtain financing on favorable terms, or at all. If the Company is unable to raise capital sufficient to meet its working capital needs in the future, it may be forced to delay expenditures, reduce the scope of its development activities or make other changes to its operating plans. As of September 30, 2024, the Company had cash, cash equivalents and marketable securities of $278.6 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). The interim unaudited consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2023 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, which was filed with the SEC on February 27, 2024. Certain information and footnote disclosures required by GAAP, which are normally included in the Company's annual consolidated financial statements, have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which are normal and recurring in nature, necessary for a fair statement of the results of operations for the periods presented.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Management bases its estimates on historical experience and various other factors that it believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates. Significant estimates are used in the following areas, among others: license and royalty revenue, the allowance

for credit losses, accrued research and development expenses and other accrued liabilities, stock-based compensation expense, interest expense under the liability related to the sale of future royalties, income taxes and fair value measurements.

Reclassifications

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation. These reclassifications are not material and have no effect on previously reported financial position, results of operations and cash flows.

Restricted Cash

Restricted cash consists of deposits held at financial institutions that are used to collateralize irrevocable letters of credit required under the Company's lease agreements and certain other agreements with third parties. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	As of September 30,	
	2024	2023
Cash and cash equivalents	$ 56,617	$ 53,045
Restricted cash	2,030	2,255
Total cash and cash equivalents and restricted cash	$ 58,647	$ 55,300

Accounts Receivable

Accounts receivable primarily consist of consideration due to the Company resulting from its license agreements with customers. Accounts receivable include amounts invoiced to licensees as well as rights to consideration which have not yet been invoiced, including unbilled royalties, and for which payment is conditional solely upon the passage of time. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any accounts receivable from the licensee which are not contractually payable to the Company are charged off as a reduction of license revenue in the period of the termination. Accounts receivable which are not expected to be received by the Company within 12 months from the reporting date are stated net of a discount to present value and recorded as non-current assets on the consolidated balance sheets. The present value discount is recognized as a reduction of revenue in the period in which the accounts receivable are initially recorded and is accreted as interest income from licensing over the term of the receivables.

Accounts receivable are stated net of an allowance for credit losses, if deemed necessary based on the Company's evaluation of collectability and potential credit losses. Management assesses the collectability of its accounts receivable using the specific identification of account balances, and considers the credit quality and financial condition of its significant customers, historical information regarding credit losses and the Company's evaluation of current and expected future economic conditions. If necessary, an allowance for credit losses is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. Accounts receivable balances are written off against the allowance for credit losses when the potential for collectability is considered remote. Please refer to Note 9 for further information regarding the allowance for credit losses related to accounts receivable.

Leases

The Company accounts for its lease arrangements in accordance with Accounting Standards Codification (ASC) 842, *Leases* (ASC 842). Under ASC 842, the Company classifies its leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the Company. Lease classification is evaluated at the inception of the lease agreement. Regardless of classification, the Company records a right-of-use asset and a lease liability for all leases with a term greater than 12 months. All of the Company's leases have been classified as operating leases. Operating lease expense is recognized on a straight-line basis over the term of the lease, with the exception of variable lease expenses which are recognized as incurred.

The Company identifies leases in its contracts if the contract conveys the right to control the use of identified property, plant or equipment for a period of time in exchange for consideration. The Company does not allocate lease consideration between lease and nonlease components and records a lease liability equal to the present value of the remaining fixed consideration under the lease. The interest rates implicit in the Company's leases are generally not readily determinable. Accordingly, the Company uses its estimated incremental borrowing rate at the commencement date of the lease to determine the present value discount of the lease liability. The Company estimates its incremental borrowing rate for each lease based on an evaluation of its expected credit rating and the prevailing

market rates for collateralized debt in a similar economic environment with similar payment terms and maturity dates commensurate with the term of the lease. The right-of-use asset for each lease is equal to the lease liability, adjusted for unamortized initial direct costs and lease incentives and prepaid or accrued rent. Initial direct costs of entering into a lease are included in the right-of-use asset and amortized as lease expense over the term of the lease. Lease incentives, such as tenant improvement allowances, are recorded as a reduction of the right-of-use asset and amortized as a reduction of lease expense over the term of the lease. The Company excludes options to extend or terminate leases from the calculation of the lease liability unless it is reasonably certain the option will be exercised.

The Company evaluates its right-of-use assets for impairment in accordance with its policy for long-lived assets. To the extent an impairment of a right-of-use asset is recognized, the remaining carrying value of the asset is subsequently amortized as lease expense on a straight-line basis from the date of impairment to the earlier of the end of the right-of-use asset's useful life or the end of the lease term.

The Company determines the classification of subleases at the inception of the sublease, as well as whether the Company has been relieved of its primary obligation under the original lease. All of the Company's subleases have been classified as operating leases and, in each case, the Company has not been relieved of its primary obligation under the original lease and continues to account for the original lease as it did prior to the commencement of the sublease. Sublease income is recognized on a straight-line basis over the term of the sublease as a reduction of the related lease expense of the original lease. Initial direct costs of entering into a sublease are deferred and amortized on a straight-line basis over the term of the sublease as a reduction of sublease income.

Impairment of Long-lived Assets

The Company's long-lived assets consist primarily of property and equipment and operating lease right-of-use assets. The Company evaluates its long-lived assets for impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to estimated future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. Please refer to Note 5 and Note 6 for further information on impairment of long-lived assets.

Fair Value Measurements

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. ASC 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for assets and liabilities categorized in Level 3. The level within the fair value hierarchy of an asset or liability measured at fair value is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 financial instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third-party pricing providers or other market observable data. Please refer to Note 4 for further information on the Company's fair value measurements.

Pre-funded Warrants

Warrants are accounted for based on the specific terms of the warrant agreements. The Company's pre-funded warrants are indexed to the Company's common stock and meet the criteria to be classified as equity. Proceeds from the issuance of pre-funded warrants are recorded within additional paid-in capital and are not subject to remeasurement. Please refer to Note 8 for further information regarding pre-funded warrants issued by the Company.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting the weighted-average common shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of computing both basic and diluted net loss per share, pre-funded warrants are considered outstanding shares upon issuance because the shares may be issued for nominal consideration and are exercisable after the original issuance date. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation of diluted net loss per share if their effect would be anti-dilutive.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

The Company did not adopt any new accounting standards during the three and nine months ended September 30, 2024 and 2023 which had a material impact on the consolidated financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In November 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which enhances certain interim and annual disclosure requirements of reportable segment information, including information about significant segment expenses. Additionally, the standard requires entities with a single reportable segment to provide all disclosures required by ASC 280, *Segment Reporting*. The standard is effective for the Company for annual periods beginning January 1, 2024 and interim periods beginning January 1, 2025, with early adoption permitted. Upon the adoption of this standard, the Company will modify its disclosures for segment reporting, as applicable. The Company does not believe the adoption of this standard will have a material impact on its consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which enhances the disclosure of an entity's effective tax rate reconciliation and requires the disclosure of income taxes paid to be disaggregated by jurisdiction. The standard is effective for the Company beginning January 1, 2025, with early adoption permitted. Upon the adoption of this standard, the Company will modify its disclosures for income taxes, as applicable. The Company does not believe the adoption of this standard will have a material impact on its consolidated financial statements.

In November 2024, the FASB issued ASU 2024-03, *Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*. The new standard requires disclosures about specific types of expenses included in the expense captions presented on the face of the income statement as well as disclosures about selling expenses. The standard is effective for the Company for annual periods beginning January 1, 2027 and interim periods beginning January 1, 2028, with early adoption permitted. The standard may be applied either prospectively to financial statements issued for reporting periods after the effective date or retrospectively to any or all prior periods presented in the financial statements. The Company is evaluating the impact of this standard on its consolidated financial statements and related disclosures.

3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist solely of available-for-sale debt securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
September 30, 2024				
U.S. government and agency securities	$ 50,350	$ 16	$ (233)	$ 50,133
Certificates of deposit	4,404	—	(15)	4,389
Corporate bonds	166,919	583	(73)	167,429
	$ 221,673	$ 599	$ (321)	$ 221,951

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2023				
U.S. government and agency securities	$ 71,811	$ 6	$ (1,248)	$ 70,569
Certificates of deposit	6,572	—	(106)	6,466
Corporate bonds	204,793	143	(2,364)	202,572
	$ 283,176	$ 149	$ (3,718)	$ 279,607

As of September 30, 2024 and December 31, 2023, no available-for-sale debt securities had remaining maturities greater than three years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of September 30, 2024 and December 31, 2023, the balance in accumulated other comprehensive loss consisted solely of unrealized gains and losses on available-for-sale debt securities, net of reclassification adjustments for realized gains and losses and income tax effects. The Company uses the aggregate portfolio approach to release the tax effects of unrealized gains and losses on available-for-sale debt securities in accumulated other comprehensive loss. Realized gains and losses from the sale or maturity of marketable securities are based on the specific identification method and are included in results of operations as investment income. The Company did not recognize any realized gains or losses on available-for-sales securities during the three and nine months ended September 30, 2024 and 2023, and no income tax effects or reclassification adjustments were recorded in accumulated other comprehensive loss during the periods.

The following tables present the fair values and unrealized losses of available-for-sale debt securities held by the Company in an unrealized loss position for less than 12 months and 12 months or greater (in thousands):

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
September 30, 2024						
U.S. government and agency securities	$ —	$ —	$ 30,252	$ (233)	$ 30,252	$ (233)
Certificates of deposit	484	(1)	3,170	(14)	3,654	(15)
Corporate bonds	4,995	(1)	21,290	(72)	26,285	(73)
	$ 5,479	$ (2)	$ 54,712	$ (319)	$ 60,191	$ (321)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2023						
U.S. government and agency securities	$ 12,877	$ (16)	$ 52,686	$ (1,232)	$ 65,563	$ (1,248)
Certificates of deposit	965	(2)	5,257	(104)	6,222	(106)
Corporate bonds	25,051	(48)	144,642	(2,316)	169,693	(2,364)
	$ 38,893	$ (66)	$ 202,585	$ (3,652)	$ 241,478	$ (3,718)

As of September 30, 2024, available-for-sale debt securities held by the Company in an unrealized loss position consisted of 36 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and based on the credit quality of the issuers and low severity of each unrealized loss position relative to its amortized cost basis, the Company did not identify any credit losses associated with its available-for-sale debt securities. The Company did not record an allowance for credit

losses on its available-for-sale debt securities as of September 30, 2024 or December 31, 2023, and no impairment or credit losses on available-for-sale debt securities were recorded during the three and nine months ended September 30, 2024 and 2023.

4. Fair Value Measurements

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the fair value hierarchy discussed in Note 2 (in thousands):

	Level 1	Level 2	Level 3	Total
September 30, 2024				
Cash equivalents:				
Money market mutual funds	$ —	$ 43,771	$ —	$ 43,771
Total cash equivalents	—	43,771	—	43,771
Marketable securities:				
U.S. government and agency securities	—	50,133	—	50,133
Certificates of deposit	—	4,389	—	4,389
Corporate bonds	—	167,429	—	167,429
Total marketable securities	—	221,951	—	221,951
Total cash equivalents and marketable securities	$ —	$ 265,722	$ —	$ 265,722

	Level 1	Level 2	Level 3	Total
December 31, 2023				
Cash equivalents:				
Money market mutual funds	$ —	$ 13,024	$ —	$ 13,024
Total cash equivalents	—	13,024	—	13,024
Marketable securities:				
U.S. government and agency securities	—	70,569	—	70,569
Certificates of deposit	—	6,466	—	6,466
Corporate bonds	—	202,572	—	202,572
Total marketable securities	—	279,607	—	279,607
Total cash equivalents and marketable securities	$ —	$ 292,631	$ —	$ 292,631

Management estimates that the carrying values of its current accounts receivable, other current assets, accounts payable, accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Accounts receivable which contain non-current portions and certain non-current payables reported as other liabilities are recorded at their present values using a discount rate that is based on prevailing market rates on the date the amounts were initially recorded. Management does not believe there have been any significant changes in market conditions or credit quality that would cause the discount rates initially used to be materially different from those that would be used as of September 30, 2024 to determine the present value of these instruments. Accordingly, management estimates that the carrying values of its non-current accounts receivable and other liabilities approximate the fair value of those instruments. Management estimates that the carrying value of the liability related to the sale of future royalties approximates fair value. As discussed in Note 7, the carrying value of the liability related to the sale of future royalties is based on the Company's estimate of future royalties expected to be paid by the Company over the life of the arrangement, which are considered Level 3 inputs.

Long-lived assets, if determined to be not recoverable and impaired, are measured at fair value on a nonrecurring basis using Level 3 inputs. Please refer to Note 6 for further information on nonrecurring fair value measurements of long-lived assets during the three and nine months ended September 30, 2024 and 2023.

5. Property and Equipment, Net

Property and equipment, net consists of the following (in thousands):

	September 30, 2024	December 31, 2023
Laboratory and manufacturing equipment	$ 76,788	$ 75,632
Computer equipment and software	4,748	4,700
Furniture and fixtures	7,024	7,052
Leasehold improvements	101,340	101,927
Total property and equipment	189,900	189,311
Accumulated depreciation and amortization	(69,349)	(57,208)
Property and equipment, net	$ 120,551	$ 132,103

In March 2024, the Company entered into an agreement to sublease its office facilities in New York, New York. In connection with the sublease, the Company recorded impairment of property and equipment of $0.7 million in the first quarter of 2024 related to furniture and fixtures and leasehold improvements located at the subleased facility. Please refer to Note 6 for further information regarding the sublease agreement and associated impairment of long-lived assets.

6. Leases

New York Sublease

In May 2016, the Company entered into an operating lease for office space in New York, New York (the New York Lease), which has since been amended to include additional office space and extend the term of the lease. The lease term commenced in July 2016 and expires in April 2027.

In March 2024, the Company entered into an agreement to sublease its office space under the New York Lease (the New York Sublease) to a third-party subtenant. The sublease term commenced in April 2024 and will expire in April 2027 concurrent with the expiration of the New York Lease. Monthly payments under the New York Sublease commenced in July 2024 and escalate annually in accordance with the sublease agreement. As of September 30, 2024, total undiscounted future minimum lease payments to be received by the Company over the term of the New York Sublease were $1.3 million. The Company recognized sublease income of $0.1 million and $0.2 million under the New York Sublease during the three and nine months ended September 30, 2024, respectively.

The New York Sublease is classified as an operating lease and the Company was not relieved of its primary obligation under the New York Lease. The Company continues to account for the New York Lease as it did prior to the commencement of the sublease.

As a result of the New York Sublease, the Company determined an impairment indicator was present as of March 31, 2024 related to the long-lived asset group subject to the sublease, which included the right-of-use asset under the New York Lease, leasehold improvements and other property and equipment allocable to the New York Sublease. The Company concluded the carrying value of the asset group as of March 31, 2024 was not recoverable, as it exceeded the sum of the estimated undiscounted cash flows to be generated by the assets over their remaining lives. The Company estimated the fair value of the asset group as of March 31, 2024 using a discounted cash flow method, which incorporated unobservable inputs including the net identifiable cash flows over the term of the New York Sublease and an estimated borrowing rate of a market participant subtenant. The estimated fair value of the asset group as of March 31, 2024 represents a Level 3 nonrecurring fair value measurement. The Company concluded the carrying value of the asset group of $3.4 million exceeded its estimated fair value of $1.3 million as of March 31, 2024. As such, the Company recognized impairment losses of $2.1 million during the three months ended March 31, 2024 and the nine months ended September 30, 2024 on the long-lived asset group associated with the New York Sublease. The impairment losses were allocated to the various assets within the long-lived asset group based on their relative carrying values and consisted of $1.4 million recorded to the right-of-use assets and $0.7 million recorded to property and equipment. No material impairment losses on long-lived assets were recorded during the three months ended September 30, 2024 and the three and nine months ended September 30, 2023.

7. Liability Related to Sale of Future Royalties

In December 2020, the Company entered into a royalty purchase agreement (the Royalty Purchase Agreement) with entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Under the Royalty Purchase Agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement (the Novartis License) with Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), including $4.0 million of royalty payments received by the Company in the fourth quarter of 2020 (the Pledged Royalties). In consideration for these rights, HCR paid the Company $200.0 million (the Purchase Price), less $4.0 million representing the payment of the Pledged Royalties to HCR. Beginning upon the effective date of the Royalty Purchase Agreement, Zolgensma royalty payments, up to a specified threshold, shall be paid to HCR, net of upstream royalties payable by the Company to certain licensors in accordance with existing license agreements.

Pursuant to the Royalty Purchase Agreement, the total amount of royalty payments to be received by HCR is subject to an increasing cap (the Cap Amount) equal to (i) $260.0 million applicable for the period from the effective date of the Royalty Purchase Agreement through November 7, 2024, and (ii) $300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the Novartis License. If, on or prior to the defined dates for each Cap Amount, the total amount of royalty payments received by HCR equals or exceeds the Cap Amount applicable to such date, the Royalty Purchase Agreement will automatically terminate and all rights to the Zolgensma royalty payments will revert back to the Company. The Company has no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to achieve the applicable Cap Amount prior to the termination of the Novartis License.

The Company has a call option to repurchase its rights to the purchased royalties from HCR for a repurchase price equal to, as of the option exercise date, $300.0 million minus the total amount of royalty payments received by HCR; provided, however, that with respect to a call option exercised on or before November 7, 2024, in the event that the then applicable Cap Amount minus the total amount of royalty payments received by HCR is less than $1.0 million, the repurchase price shall equal such difference.

The proceeds received from HCR of $196.0 million were recorded as a liability, net of transaction costs of $3.5 million, which is amortized over the estimated life of the arrangement using the effective interest method. In order to determine the amortization of the liability, the Company is required to estimate the total amount of future royalty payments to be received by HCR, subject to the Cap Amount, over the life of the arrangement. The total amount of royalty payments received by HCR under the Royalty Purchase Agreement, less the net proceeds received by the Company of $192.5 million, is recorded as interest expense over the life of the arrangement using the effective interest method. Due to its continuing involvement in the Novartis License, the Company continues to recognize royalty revenue on net sales of Zolgensma and records the royalty payments to HCR as a reduction of the liability when paid. As such payments are made to HCR, the balance of the liability will be effectively repaid over the life of the Royalty Purchase Agreement.

The Company estimates the effective interest rate used to record interest expense under the Royalty Purchase Agreement based on its estimate of future royalty payments to be received by HCR. As of September 30, 2024, the estimated effective interest rate under the Royalty Purchase Agreement was 4.2%. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the royalty payments received by HCR and changes in the Company's forecasted royalties. At each reporting date, the Company reassesses its estimate of total future royalty payments to be received by HCR at the applicable Cap Amount, and prospectively adjusts the effective interest rate and amortization of the liability, as necessary.

The following table presents the changes in the liability related to the sale of future royalties under the Royalty Purchase Agreement with HCR (in thousands):

	Liability Related to Sale of Future Royalties
Balance at December 31, 2023	$ 94,052
Zolgensma royalties paid to HCR	(35,072)
Interest expense recognized	2,769
Balance at September 30, 2024	61,749
Current portion of liability related to sale of future royalties	(26,697)
Liability related to sale of future royalties, non-current	$ 35,052

8. Capitalization

March 2024 Public Offering

In March 2024, the Company completed a public offering of 4,565,260 shares of its common stock at a price of $23.00 per share and 1,521,740 pre-funded warrants to purchase shares of its common stock at a price of $22.9999 per pre-funded warrant, which equaled the public offering price per share of the common stock less the $0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received by the Company from the offering were $131.1 million, net of underwriting discounts and commissions and offering expenses payable by the Company.

The rights and privileges of the pre-funded warrants issued under the March 2024 offering are set forth in the warrant agreement between the Company and each of the respective warrant holders. The pre-funded warrants are exercisable at the option of the warrant holder at any time and do not expire. However, as set forth in the warrant agreements with each holder, the number of pre-funded warrants that may be exercised at any given time may be limited if, upon exercise, the warrant holder and any of its affiliates would beneficially own more than 9.99% of the Company's common stock, or have voting power of more than 9.99% of the Company's common stock. The limitation threshold may be increased or decreased by the warrant holder, with advance notice to the Company, to any other percentage not less than 4.99% nor in excess of 19.99%. Pre-funded warrants do not provide any of the rights or privileges provided by the Company's common stock, including any voting rights, until the pre-funded warrants are exercised and settled in underlying shares of common stock.

The Company evaluated the pre-funded warrants issued under the March 2024 offering and concluded the warrants are indexed to the Company's common stock, meet the criteria to be classified as equity and are not subject to remeasurement. The proceeds received from the issuance of the pre-funded warrants were recorded as additional paid-in capital. The Company issued 197,000 shares of common stock upon the exercise of pre-funded warrants during the nine months ended September 30, 2024. There were no exercises of pre-funded warrants during the three months ended September 30, 2024. As of September 30, 2024, 1,324,740 pre-funded warrants remained outstanding.

At-the-Market Offering Program

In September 2023, the Company entered into an ATM Equity Offering℠ Sales Agreement with BofA Securities, Inc. (BofA) pursuant to which the Company may offer and sell shares of its common stock having an aggregate offering price of up to $150.0 million from time to time through BofA, acting as the Company's sales agent (the ATM Program). As of September 30, 2024, no shares of common stock had been sold under the ATM Program.

9. License and Collaboration Agreements

License and Royalty Revenue

As of September 30, 2024, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma, and in the development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development of certain product candidates. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees. Sublicense fees vary by license and range from a mid-single digit percentage to a low-double digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single digit percentage to a low double-digit percentage of net sales by licensees.

License and royalty revenue consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Zolgensma royalties	$ 23,872	$ 28,364	$ 60,849	$ 63,454
Other license and royalty revenue	325	550	1,265	4,575
Total license and royalty revenue	$ 24,197	$ 28,914	$ 62,114	$ 68,029

Outstanding development milestone payments are evaluated each reporting period and are only included in the transaction price of each license and recognized as license revenue to the extent the milestones are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as royalty revenue in the period of achievement. As of September 30, 2024, the Company's license agreements, excluding additional licenses that could be granted upon the exercise of options by licensees, contained unachieved milestones which could result in aggregate milestone payments to the Company of up to $1.51 billion, including (i) $524.9 million upon the commencement of various stages of clinical trials, (ii) $113.8 million upon the submission of regulatory approval filings or upon regulatory approval of licensed products and (iii) $870.0 million upon the achievement of specified sales targets for licensed products, including milestones payable upon the first commercial sale of licensed products. To the extent the milestone payments are realized by the Company, the Company may be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of these milestones is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

Changes in Accounts Receivable, Contract Assets and Deferred Revenue

The following table presents the balances of the Company's net accounts receivable, contract assets and deferred revenue, as well as other information regarding revenue recognized during the periods presented (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Accounts receivable, net, current and non-current:				
Beginning of period	$ 23,273	$ 22,523	$ 25,491	$ 29,586
End of period	$ 24,008	$ 29,121	$ 24,008	$ 29,121
Contract assets:				
Beginning of period	$ 194	$ 2,000	$ —	$ —
End of period	$ 259	$ 2,000	$ 259	$ 2,000
Deferred revenue:				
Beginning of period	$ 21	$ 448	$ 148	$ 1,829
End of period	$ 144	$ 442	$ 144	$ 442
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	$ 21	$ 28	$ 148	$ 1,409
Performance obligations satisfied in previous periods	$ 23,875	$ 28,365	$ 60,912	$ 65,405

As of September 30, 2024, the Company had recorded deferred revenue of $0.1 million which represents consideration received or unconditionally due from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations as of September 30, 2024 consisted of research and development services to be performed by the Company related to licensed products, which will be satisfied as the services are performed. As of September 30, 2024, the aggregate transaction price of the Company's license agreements allocated to performance obligations not yet satisfied, or partially satisfied, was $1.5 million, which is expected to be satisfied over a period of approximately two to three years.

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma royalties and changes in the transaction prices of the Company's license agreements. Changes in transaction prices were primarily attributable to development milestones achieved or deemed probable of achievement during the periods which were previously not considered probable of achievement, resulting in a cumulative catch-up adjustment to revenue. Revenue recognized during the nine months ended September 30, 2023 included $2.0 million in cumulative catch-up adjustments for changes in the probability of achievement of development milestones. There were no cumulative catch-up adjustments to revenue for changes in the probability of achievement of development milestones during the three and nine months ended September 30, 2024 or during the three months ended September 30, 2023.

Accounts Receivable, Contract Assets and the Allowance for Credit Losses

Accounts receivable, net consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Current accounts receivable:		
Billed to customers	$ 105	$ 265
Unbilled Zolgensma royalties	23,138	24,128
Due from Abeona, net of present value discount	4,942	4,587
Other unbilled	361	397
Allowance for credit losses	(4,942)	(4,587)
Current accounts receivable, net	23,604	24,790
Non-current accounts receivable:		
Other unbilled	404	701
Non-current accounts receivable, net	404	701
Total accounts receivable, net	$ 24,008	$ 25,491

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the nine months ended September 30, 2024 (in thousands):

	Allowance for Credit Losses	
	Accounts Receivable	Contract Assets
Balance at December 31, 2023	$ 4,587	$ —
Changes in present value discount of receivables	355	—
Balance at September 30, 2024	$ 4,942	$ —

The Company's allowance for credit losses as of September 30, 2024 and December 31, 2023 was related solely to accounts receivable from Abeona Therapeutics Inc. (Abeona). Please refer to the section below, "Settlement Agreement with Abeona Therapeutics", for further information regarding amounts due from Abeona and the associated allowance for credit losses. The Company did not record a provision for credit losses for the three and nine months ended September 30, 2024 and 2023.

Zolgensma License with Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement (as amended, the Novartis License) with Novartis Gene Therapies. Under the Novartis License, the Company granted Novartis Gene Therapies an exclusive, worldwide commercial license, with rights to sublicense, to the NAV Technology Platform, as well as other certain rights, for the treatment of spinal muscular atrophy (SMA) in humans by *in vivo* gene therapy. In 2019, Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the Novartis License. In accordance with the Novartis License, the Company receives royalties on net sales of Zolgensma.

The Company recognized the following amounts under the Novartis License (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Zolgensma royalties	$ 23,872	$ 28,364	$ 60,849	$ 63,454
Total license and royalty revenue	$ 23,872	$ 28,364	$ 60,849	$ 63,454
Interest income from licensing	$ 6	$ 7	$ 20	$ 22

As of September 30, 2024 and December 31, 2023, the Company had recorded total accounts receivable of $23.3 million and $24.3 million, respectively, from Novartis Gene Therapies under the Novartis License, which consisted primarily of Zolgensma royalties receivable. The Zolgensma royalties receivable recorded as of September 30, 2024 included $11.1 million expected to be paid to HCR in accordance with the Royalty Purchase Agreement discussed in Note 7. The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

Settlement Agreement with Abeona Therapeutics

In November 2021, the Company entered into a settlement agreement and mutual release with Abeona (the Settlement Agreement) related to claims associated with a license agreement between the parties which was terminated in May 2020. The Settlement Agreement resolved all arbitration and legal proceedings and mutually released each party from any and all claims under the terminated license agreement. Pursuant to the Settlement Agreement, Abeona will pay the Company a total of $30.0 million as follows: (i) $20.0 million which was paid in November 2021, (ii) $5.0 million which was paid in November 2022, and (iii) $5.0 million payable on the earlier of the third anniversary of the Settlement Agreement in November 2024 or the closing of a specified type of transaction by Abeona.

As of September 30, 2024 and December 31, 2023, the Company had recorded accounts receivable of $4.9 million and $4.6 million, respectively, associated with the remaining amounts due from Abeona under the Settlement Agreement. The receivable of $4.9 million as of September 30, 2024 consisted of the $5.0 million payment due by November 2024, net of discount to present value. While the Company anticipates taking appropriate measures to enforce the full collection of all amounts due from Abeona under the Settlement Agreement, the Company assessed the collectability of the accounts receivable from Abeona as it relates to credit risk. In performing this assessment, the Company evaluated Abeona's credit profile and financial condition, as well its expectations regarding Abeona's future cash flows and ability to satisfy the contractual obligations of the Settlement Agreement. As a result of its analysis, the Company recorded an allowance for credit losses of $4.9 million and $4.6 million as of September 30, 2024 and December 31, 2023, respectively, related to the accounts receivable due from Abeona. No credit losses or recoveries were recorded on the Abeona receivable during the three and nine months ended September 30, 2024 and 2023. The present value discount of the Abeona receivable is accreted as interest income from licensing through the contractual due date using the effective interest method. The Company has elected to record increases in the allowance for credit losses associated with the accretion of the present value discount as a reduction of the associated interest income, resulting in no interest income recognized during the periods related to the accretion of the present value discount on the Abeona receivable.

Collaboration Agreements

AbbVie Collaboration and License Agreement

In September 2021, the Company entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314, the Company's product candidate for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal diseases (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement became effective in November 2021.

Pursuant to the AbbVie Collaboration Agreement, the Company granted AbbVie a co-exclusive license to develop and commercialize ABBV-RGX-314 in the United States and an exclusive license to develop and commercialize ABBV-RGX-314 outside the United States. The Company and AbbVie will collaborate to develop ABBV-RGX-314 in the United States, and AbbVie will be responsible for the development of ABBV-RGX-314 in specified markets outside the United States. Through December 31, 2022, the Company was responsible for the development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the parties shared the additional development expenses related to ABBV-RGX-314. Beginning on January 1, 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of ABBV-RGX-314 for commercial supply outside the United States. Manufacturing expenses will be allocated between the parties in accordance with the terms of the AbbVie Collaboration Agreement and supply agreements determined in accordance with the agreement. If requested by AbbVie, the Company will manufacture up to a specified portion of ABBV-RGX-314 for commercial supply outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of ABBV-RGX-314 globally, and the Company will participate in U.S. commercialization efforts as provided under a commercialization plan determined in accordance with the agreement. The Company and AbbVie will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of ABBV-RGX-314.

In consideration for the rights granted under the AbbVie Collaboration Agreement, AbbVie paid the Company an up-front fee of $370.0 million upon the effective date of the agreement in November 2021 and is required to pay to the Company up to $1.38 billion upon the achievement of specified development and sales-based milestones, of which $562.5 million are based on development milestones and $820.0 million are sales-based milestones. AbbVie is also required to pay to the Company tiered royalties on net sales of ABBV-RGX-314 outside the United States at percentages in the mid-teens to low twenties, subject to specified offsets and reductions.

The Company applied the requirements of ASC 606, *Revenue from Contracts with Customers* (ASC 606) to the AbbVie Collaboration Agreement for the units of account in which AbbVie was deemed to be a customer. The Company determined that there is only one material performance obligation under the agreement for the delivery of the intellectual property license to develop and commercialize ABBV-RGX-314 globally. The intellectual property licensed to AbbVie includes the rights to certain patents, data, know-how and other rights developed and owned by the Company, as well as other intellectual property rights exclusively licensed by the Company from various third parties. As of September 30, 2024 and December 31, 2023, the transaction price of the AbbVie Collaboration Agreement was $370.0 million, which consisted solely of the up-front payment received from AbbVie in November 2021. The $370.0 million transaction price was fully recognized as revenue upon the delivery of the license to AbbVie in November 2021. Variable consideration under the AbbVie Collaboration Agreement, which has been excluded from the transaction price, includes $562.5 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement. Additionally, the transaction price excludes sales-based milestone payments of $820.0 million and royalties on net sales of ABBV-RGX-314 outside the United States. Development milestones will be added to the transaction price and recognized as revenue upon achievement, or if deemed probable of achievement. In accordance with the sale- or usage-based royalty exception under ASC 606, royalties on net sales and sales-based milestones will be recognized as revenue in the period the underlying sales occur or milestones are achieved. There were no changes in the transaction price of the AbbVie Collaboration Agreement, and no revenue was recognized, during the three and nine months ended September 30, 2024 and 2023.

The Company applied the requirements of ASC 808, *Collaborative Arrangements* (ASC 808) to the AbbVie Collaboration Agreement for the units of account which were deemed to be a collaborative arrangement. Both the Company and AbbVie will perform various activities related to the development, manufacturing and commercialization of ABBV-RGX-314 in the United States. Development costs are shared between the parties in accordance with the terms of the AbbVie Collaboration Agreement, and the parties will share equally in the net profits and losses derived from sales of ABBV-RGX-314 in the United States. The Company accounts for payments to and from AbbVie for the sharing of development and commercialization costs in accordance with its accounting policy for collaborative arrangements. Amounts owed to AbbVie for the Company's share of development costs or commercialization costs incurred by AbbVie are recorded as research and development expense or general and administrative expense, respectively, in the period the costs are incurred. Amounts owed to the Company for AbbVie's share of development costs or commercialization costs incurred by the Company are recorded as a reduction of research and development expense or general and administrative expense, respectively, in the period the costs are incurred. At the end of each reporting period, the Company records a net amount due to or from AbbVie as a result of the cost-sharing arrangement. As of September 30, 2024 and December 31, 2023, the Company had recorded $20.3 million and $17.7 million, respectively, due from AbbVie for net reimbursement of costs incurred for activities performed under AbbVie Collaboration Agreement, which was included in other current assets on the consolidated balance sheets.

The Company recognized the following amounts under the AbbVie Collaboration Agreement (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Net cost reimbursement to (from) AbbVie included in:				
Research and development expense	$ (20,082)	$ (19,608)	$ (66,330)	$ (56,300)
General and administrative expense	341	615	1,168	1,007
Total net cost reimbursement to (from) AbbVie	$ (19,741)	$ (18,993)	$ (65,162)	$ (55,293)

10. Stock-based Compensation

Effective in January 2024, an additional 1,761,849 shares were authorized for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). As of September 30, 2024, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 19,118,989, of which 1,511,556 remained available for future grants under the 2015 Plan.

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Stock options	$ 6,051	$ 7,275	$ 17,969	$ 22,868
Restricted stock units	3,755	2,843	10,536	8,475
Employee stock purchase plan	78	101	416	586
	$ 9,884	$ 10,219	$ 28,921	$ 31,929

As of September 30, 2024, the Company had $69.5 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.4 years.

The Company recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	$ 4,301	$ 5,131	$ 13,893	$ 16,670
General and administrative	5,583	5,088	15,028	15,259
	$ 9,884	$ 10,219	$ 28,921	$ 31,929

Stock Options

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2023	8,581	$ 32.62	6.2	$ 7,011
Granted	2,263	$ 16.24		
Exercised	(281)	$ 5.25		
Cancelled or forfeited	(546)	$ 35.56		
Outstanding at September 30, 2024	10,017	$ 29.53	6.4	$ 529
Exercisable at September 30, 2024	6,609	$ 34.38	5.1	$ 529
Vested and expected to vest at September 30, 2024	10,017	$ 29.53	6.4	$ 529

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at the dates reported.

The weighted-average grant date fair value per share of options granted during the nine months ended September 30, 2024 was $10.06. During the nine months ended September 30, 2024, the total number of stock options exercised was 280,753, resulting in total proceeds of $1.5 million. The total intrinsic value of options exercised during the nine months ended September 30, 2024 was $3.0 million.

Restricted Stock Units

The following table summarizes restricted stock unit activity under the 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2023	1,309	$ 25.89
Granted	1,188	$ 16.22
Vested	(390)	$ 27.20
Forfeited	(72)	$ 22.45
Unvested balance at September 30, 2024	2,035	$ 20.11

The total intrinsic value of restricted stock units vested during the nine months ended September 30, 2024 was $6.7 million.

Employee Stock Purchase Plan

As of September 30, 2024, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 912,964 remained available for future issuance. During the nine months ended September 30, 2024, 105,400 shares of common stock were issued under the 2015 ESPP.

11. Income Taxes

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of September 30, 2024 and December 31, 2023. Based on the Company's history of operating losses, and other relevant facts and circumstances, the Company concluded that it was more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company provided a full valuation allowance for its net deferred tax assets as of September 30, 2024 and December 31, 2023.

12. Restructuring

In November 2023, the Company implemented a strategic pipeline prioritization and corporate restructuring designed to reduce operating expenses and prioritize the development of ABBV-RGX-314, RGX-202 for the treatment of Duchenne muscular dystrophy, and RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II), while pursuing strategic alternatives for the Company's other clinical stage programs. The restructuring included a reduction in workforce and other planned operating expenses, primarily in rare neurodegenerative disease development, early research and other general and administrative areas.

In connection with the restructuring, the Company implemented a reduction in workforce of approximately 15%, which was substantially completed in the fourth quarter of 2023. The Company recorded restructuring costs of $3.7 million in the fourth quarter of 2023, which primarily consisted of employee severance, continuing healthcare benefits and other employee-related costs. During the nine months ended September 30, 2024, the Company recorded reductions in the restructuring liability of $0.4 million associated with changes in total estimated restructuring costs. As of September 30, 2024, all of the restructuring costs had been paid by the Company and no restructuring liability was recorded. The following table presents the details of the Company's restructuring liability as of September 30, 2024 (in thousands):

	Restructuring Liability
Balance at December 31, 2022	$ —
Restructuring charges	3,731
Cash payments	(1,925)
Balance at December 31, 2023	1,806
Cash payments	(1,370)
Other adjustments	(436)
Balance at September 30, 2024	$ —

13. Net Loss Per Share

Since the Company incurred net losses for the three and nine months ended September 30, 2024 and 2023, common stock equivalents were excluded from the calculation of diluted net loss per share for such periods as their effect would be anti-dilutive. Accordingly, basic and diluted net loss per share were the same for such periods. The weighted-average number of common shares outstanding used in the basic and diluted net loss per share calculations includes the weighted-average effect of pre-funded warrants to purchase shares of the Company's common stock, as the pre-funded warrants are exercisable at any time for nominal cash consideration. The following potentially dilutive common stock equivalents outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

	Three and Nine Months Ended September 30,	
	2024	2023
Stock options issued and outstanding	10,017	8,856
Unvested restricted stock units outstanding	2,035	1,394
Employee stock purchase plan	47	36
	12,099	10,286

14. Supplemental Disclosures

Other Current Assets

Other current assets consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Net cost reimbursement due from collaborators	$ 20,302	$ 17,745
Accrued interest on investments	1,578	1,551
Other	1,450	1,107
	$ 23,330	$ 20,403

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Accrued external research and development expenses	$ 16,715	$ 13,762
Accrued sublicense fees and royalties	14,628	14,234
Accrued personnel costs	14,335	18,146
Accrued external general and administrative expenses	1,843	2,717
Accrued purchases of property and equipment	334	386
Other accrued expenses and current liabilities	787	458
	$ 48,642	$ 49,703

Supplemental Disclosures of Non-cash Investing and Financing Activities

Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities were $0.4 million as of September 30, 2024, a net decrease of less than $0.1 million from December 31, 2023, and $0.5 million as of September 30, 2023, a net decrease of $2.0 million from December 31, 2022.

Offering expenses for the ATM Program included in accounts payable and accrued expenses and other liabilities were $0.4 million as of September 30, 2023. No such amounts were recorded as of September 30, 2024.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2023, which we filed with the SEC on February 27, 2024. In addition, you should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2023 for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our investigational gene therapies are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, gene therapy could potentially alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects.

Overview of Product Candidates

We have developed a broad pipeline of gene therapy programs using our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) as a one-time treatment to address an array of diseases. Our lead programs and product candidates are described below:

•**ABBV-RGX-314:** We are developing ABBV-RGX-314 in collaboration with AbbVie as a potential one-time treatment for chronic retinal conditions which cause total or partial vision loss, including wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR). ABBV-RGX-314 is currently being evaluated in multiple ongoing clinical trials, including two pivotal trials (ATMOSPHERE and ASCENT), one Phase II bridging study, one long-term follow-up study, and a fellow eye treatment study in patients with wet AMD, all utilizing subretinal delivery. Additionally, two Phase II clinical trials in patients with wet AMD (AAVIATE) and DR (ALTITUDE) are ongoing along with two corresponding long-term follow-up studies, all utilizing in-office suprachoroidal delivery. Within the Phase II study in DR, we are also evaluating ABBV-RGX-314 in diabetic macular edema (DME). ABBV-RGX-314 uses the NAV® AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF). We have licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. (Clearside) to deliver gene therapy treatments to the suprachoroidal space of the eye.

Wet AMD

Subretinal Delivery

Enrollment continues to be on track in the ATMOSPHERE® and ASCENT™ pivotal trials for the treatment of patients with wet AMD using subretinal delivery. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) in the first half of 2026. In October 2024, we reported positive data from a Phase II fellow eye sub-study at the American Academy of Ophthalmology (AAO) 2024 annual meeting. The sub-study evaluated subretinal delivery of ABBV-RGX-314 in patients who received ABBV-RGX-314 in the Phase I/IIa or bridging studies and elected to receive treatment in their second eye. As of September 11, 2024, ABBV-RGX-314 was well tolerated in the treated fellow eye, with no drug-related serious adverse events and no cases of intraocular inflammation observed. At nine months post-administration, patients saw a 97% reduction in anti-VEGF treatment burden and sustained vision and anatomy. These data demonstrate the potential of ABBV-RGX-314 to preserve vision long-term for patients with wet AMD as a one-time treatment for both eyes. Bilateral disease impacts a significant number of patients with wet AMD.

Suprachoroidal Delivery

The AAVIATE® trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 for the treatment of wet AMD. As of July 29, 2024, ABBV-RGX-314 at dose level 3 with short course prophylactic steroid eye drops continues to be well tolerated with no drug-related serious adverse events (SAEs) and no cases of intraocular inflammation, endophthalmitis, vasculitis, retinal artery occlusion, choroidal effusion, or hypotony. Mild episcleritis occurred in three patients, all resolved and completed treatment with topical steroids. There were no cases of elevated intraocular pressure. Based on this favorable safety profile, the Phase II AAVIATE trial is initiating enrollment in a new cohort to evaluate ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short course prophylactic steroid eye drops.

DR and DME

The ALTITUDE® trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of ABBV-RGX-314 using suprachoroidal delivery for the treatment of DR. Based on positive interim results from this trial, we and AbbVie have accelerated a planned End-of-Phase II (EOP2) meeting with the FDA expected to occur in the fourth quarter of 2024. We expect to initiate the first global pivotal trial in the first half of 2025.

The ALTITUDE trial is now enrolling a new cohort of patients with center-involved DME. DME is a vision-threatening complication of DR; an estimated 34 million people globally have DME. Patients will receive a one-time, in-office injection of ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye) with short course prophylactic steroid eye drops.

•**RGX-202:** We are developing RGX-202 as an investigational one-time AAV therapeutic for the treatment of Duchenne muscular dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

AFFINITY DUCHENNE® is a Phase I/II multicenter, open-label dose escalation and dose expansion clinical study to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in patients with Duchenne aged 1 to 11 years old. In March 2024 and May 2024, we reported interim safety and efficacy data from the trial, including RGX-202 microdystrophin expression for the first two patients who received RGX-202 at dose level 2 (DL2).

In August 2024, we announced new data from two patients, aged 5.8 and 8.5 years, who received RGX-202 at DL2. RGX-202 microdystrophin expression in these patients was measured to be 77.2% and 46.5%, respectively, compared to control at three months. As of July 8, 2024, RGX-202 has been well tolerated with no SAEs. Data from all seven patients who completed three-month trial assessments indicate meaningful increases in expression of RGX-202 microdystrophin and reduction from baseline in serum creatinine kinase levels, supporting evidence of clinical improvement.

In November 2024, we announced that the last patient has been dosed in the dose level 2 (pivotal dose) expansion cohort for ages 4 to 11 years old and the first patient has been dosed in the cohort for ages 1 to 3 years old. A clinical trial application for RGX-202 has been authorized by Health Canada, and we expect to initiate sites in Canada in the first half of 2025.

Following a successful EOP2 meeting with the FDA earlier this year, we plan to share a full program update in November 2024, including pivotal trial design and plans for accelerated approval, as well as initial strength and functional assessment data for both dose levels of the AFFINITY DUCHENNE trial.

RGX-202 is manufactured using our proprietary, high-yielding NAVXpress™ platform process. This suspension-based manufacturing process has demonstrated scalability up to 2,000 liters with consistent yield and product purity. Our Manufacturing Innovation Center has the capacity and yields to produce up to 2,500 doses of RGX-202 per year to support future commercialization.

•**RGX-121:** We are developing RGX-121 as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase enzyme.

CAMPSIITE® is a Phase I/II/III multi-center, open-label trial to evaluate the efficacy, safety, tolerability and pharmacodynamics of RGX-121 in patients with MPS II aged 4 months up to 5 years old. We continue to follow patients in the trial and, in February 2024, reported that the pivotal phase of the CAMPSIITE trial achieved its primary endpoint, a reduction in cerebrospinal fluid Heparan sulfate levels of D2S6, a biomarker indicative of brain disease activity, with statistical significance. In September 2024, we announced positive data from the pivotal dose level of RGX-121 demonstrating long-term systemic effect. We plan to use levels of cerebrospinal fluid Heparan sulfate D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit for accelerated approval and have initiated a rolling biologics license application (BLA) submission using the accelerated approval pathway. The BLA submission is expected to be complete in the first quarter of 2025.

We believe that RGX-121 is likely to be eligible for priority review, especially if no other gene therapy product for MPS II is approved before submission of a BLA for RGX-121, and potential approval of the Company's planned BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025, assuming the statutory criteria are met, potentially making RGX-121 the first approved gene therapy and one-time treatment for MPS II.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of September 30, 2024, our NAV Technology Platform was being applied in one commercial product, Zolgensma®, and the preclinical and clinical development of a number of other licensed products. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform and creating potential additional revenue opportunities.

Financial Overview

Revenues

Our revenues to date consist primarily of license and royalty revenue resulting from the licensing of our NAV Technology Platform and other intellectual property rights. We have not generated any revenues from commercial sales of our own products. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval and adequate labeling, our ability to generate future revenues will be materially compromised.

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of our product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration from licensees under our license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees.

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products, which is uncertain, and revenues may fluctuate significantly from period to period. Additionally, we may never receive consideration in our license agreements that is contemplated on option fees, development and sales-based milestone payments, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments. Our revenues are concentrated among a low number of licensees and licenses are terminable at the option of the licensee. The termination of our licenses by licensees may materially impact the amount of revenue we recognize in future periods.

Zolgensma Royalties

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is marketed by Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the treatment of spinal muscular atrophy (SMA). Zolgensma is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA using the NAV Technology Platform.

Collaboration and License Agreement with AbbVie

In September 2021, we entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314 (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement may materially impact our future revenues, research and development expenses, other operating expenses and operating cash flows associated with the development and commercialization of ABBV-RGX-314. For additional information regarding the AbbVie Collaboration Agreement, please refer to Note 9, “License and Collaboration Agreements—AbbVie Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

Operating Expenses

Our operating expenses consist primarily of cost of revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, wages, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee or using other reasonable allocation methodologies.

Cost of Revenues

Our cost of revenues consists primarily of upstream fees due to our licensors as a result of revenue generated from the licensing of our NAV Technology Platform and other intellectual property rights, including sublicense fees and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from licensees and are recognized in the period that the underlying license revenue is recognized. Royalties are based on a percentage of net sales of licensed products by licensees and are recognized in the period that the underlying sales occur. Future costs of revenues are uncertain due to the nature of our license agreements and significant fluctuations in cost of revenues may occur from period to period.

Research and Development Expense

Our research and development expenses consist primarily of:

- salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for our scientific personnel and others performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- fees paid to consultants and other third-parties who support our product candidate development;
- other costs in seeking regulatory approval of our product candidates; and
- direct costs and allocated costs related to laboratories and facilities, depreciation expense, information technology and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities, as well as associated milestone payments, are charged to research and development expense as incurred if the technology licensed has no alternative future use.

We expect to continue to incur significant research and development expenses for the foreseeable future as we continue the development of our product candidates and engage in early research and development for prospective product candidates and new technologies. The following table summarizes our research and development expenses incurred during the three and nine months ended September 30, 2024 and 2023 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Direct Expenses				
ABBV-RGX-314	$ 10,803	$ 6,022	$ 28,773	$ 16,391
RGX-202	3,875	2,088	9,053	8,233
RGX-121	4,017	4,991	11,798	12,623
Other product candidates	1,174	2,187	3,059	6,725
Total direct expenses	19,869	15,288	52,683	43,972
Unallocated Expenses				
Platform and new technologies	5,977	10,306	17,627	33,211
Personnel-related	20,789	23,784	63,803	73,201
Facilities and depreciation expense	6,823	7,274	20,946	21,623
Other unallocated	971	1,531	3,083	4,578
Total unallocated expenses	34,560	42,895	105,459	132,613
Total research and development	$ 54,429	$ 58,183	$ 158,142	$ 176,585

Direct expenses related to the development of ABBV-RGX-314 include net cost reimbursement from AbbVie under our eye care collaboration of $20.1 million and $66.3 million for the three and nine months ended September 30, 2024, respectively, and $19.6 million and $56.3 million for the three and nine months ended September 30, 2023, respectively, which were recorded as a reduction of research and development expenses. Net cost reimbursement from AbbVie includes reimbursement of personnel and overhead costs attributable to the development of ABBV-RGX-314, the underlying costs of which are reported as unallocated expenses in the table above. We typically utilize our employee and infrastructure resources across our development programs. As a result, we generally do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

Platform and new technologies reported in the table above include direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform used across programs, process development, manufacturing analytics and early research and development for prospective product candidates and new technologies.

Direct expenses related to the development of product candidates for which we have discontinued internal development are included in other product candidates in the table above. We expect to continue to incur minor development expenses associated with long-term follow up studies for certain discontinued product candidates.

General and Administrative Expense

Our general and administrative expenses consist primarily of salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for employees performing functions other than research and development. This includes certain personnel in executive, commercial, corporate development, finance, legal, human resources, information technology, facilities and administrative support functions. Additionally, general and administrative expenses include costs associated with accounting, legal, commercial and other corporate advisory services, obtaining and maintaining patents, insurance, information systems and other general corporate activities, as well as facility-related costs and other corporate overhead costs not otherwise allocated to research and development expense. We expect that our general and administrative expenses will increase as we continue to develop, and potentially commercialize, our product candidates.

Other Income (Expense)

Interest Income from Licensing

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees as well as interest income accrued on unpaid balances due from licensees.

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents, marketable securities and non-marketable equity securities. Cash equivalents are comprised of money market mutual funds and highly liquid debt securities with original maturities of 90 days or less at acquisition. Marketable securities are comprised of available-for-sale debt securities.

Interest Expense

Interest expense consists primarily of interest imputed on the liability related to the sale of future Zolgensma royalties to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Interest expense is recognized using the effective interest method, based on our estimate of total royalty payments expected to be received by HCR under the royalty purchase agreement. For further information regarding the royalty purchase agreement with HCR, please refer to Note 7, “Liability Related to Sale of Future Royalties” to the accompanying unaudited consolidated financial statements.

Critical Accounting Policies and Estimates

This Management’s Discussion and Analysis of Financial Condition and Results of Operations is based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities

for the periods presented. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

Our significant accounting policies are fully described in Note 2 to the accompanying unaudited consolidated financial statements and in Note 2 to our audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023. There have been no significant changes in our critical accounting policies and estimates since December 31, 2023.

Results of Operations

Our consolidated results of operations were as follows (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change
Revenues						
License and royalty revenue	$ 24,197	$ 28,914	$ (4,717)	$ 62,114	$ 68,029	$ (5,915)
Total revenues	24,197	28,914	(4,717)	62,114	68,029	(5,915)
Operating Expenses						
Cost of revenues	12,387	12,388	(1)	27,249	25,975	1,274
Research and development	54,429	58,183	(3,754)	158,142	176,585	(18,443)
General and administrative	19,422	23,083	(3,661)	56,568	69,415	(12,847)
Impairment of long-lived assets	—	—	—	2,101	—	2,101
Other operating expenses	37	220	(183)	32	279	(247)
Total operating expenses	86,275	93,874	(7,599)	244,092	272,254	(28,162)
Loss from operations	(62,078)	(64,960)	2,882	(181,978)	(204,225)	22,247
Other Income (Expense)						
Interest income from licensing	25	56	(31)	91	166	(75)
Investment income	3,276	4,660	(1,384)	9,213	8,953	260
Interest expense	(820)	(1,624)	804	(3,242)	(5,499)	2,257
Total other income	2,481	3,092	(611)	6,062	3,620	2,442
Net loss	$ (59,597)	$ (61,868)	$ 2,271	$ (175,916)	$ (200,605)	$ 24,689

Comparison of the Three Months Ended September 30, 2024 and 2023

License and Royalty Revenue. License and royalty revenue decreased by $4.7 million, from $28.9 million for the three months ended September 30, 2023 to $24.2 million for the three months ended September 30, 2024. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased from $28.4 million for the third quarter of 2023 to $23.9 million for the third quarter of 2024. As reported by Novartis, sales of Zolgensma for the third quarter of 2024 were $308 million, consistent with sales for the third quarter of 2023, and the product continues to treat mainly incident patients in established markets, translating into stable sales for the third quarter of 2024. The decrease in Zolgensma royalties was primarily attributable to fluctuations in the effective royalty rate under the license agreement with Novartis.

Research and Development Expense. Research and development expenses decreased by $3.8 million, from $58.2 million for the three months ended September 30, 2023 to $54.4 million for the three months ended September 30, 2024. The decrease was primarily attributable to the following:

- a decrease of $3.0 million in personnel-related costs for research and development personnel, including a $0.8 million decrease in stock-based compensation expense, largely driven by the reduction in workforce associated with our corporate restructuring implemented in the fourth quarter of 2023;
- a decrease of $2.4 million in preclinical activities and other early-stage research and development;
- a decrease of $1.9 million in costs for laboratories and facilities used by research and development personnel, including a $0.4 million decrease in depreciation expense allocated to research and development functions, primarily driven by a decrease in laboratory supplies and consumables; and
- a decrease of $1.6 million in manufacturing expenses and other costs of clinical supply for our lead product candidates.

The decrease in research and development expenses was partially offset by an increase of $5.8 million in costs associated with clinical trial activities, largely driven by clinical trial expenses for ABBV-RGX-314 and RGX-202.

General and Administrative Expense. General and administrative expenses decreased by $3.7 million, from $23.1 million for the three months ended September 30, 2023 to $19.4 million for the three months ended September 30, 2024. The decrease was primarily attributable to professional services and consulting fees, including legal and other corporate advisory services.

Comparison of the Nine Months Ended September 30, 2024 and 2023

License and Royalty Revenue. License and royalty revenue decreased by $5.9 million, from $68.0 million for the nine months ended September 30, 2023 to $62.1 million for the nine months ended September 30, 2024. The decrease was primarily attributable to non-recurring development milestone revenue recognized in the first nine months of 2023 and Zolgensma royalty revenues, which decreased from $63.5 million for the first nine months of 2023 to $60.8 million for the first nine months of 2024. As reported by Novartis, sales of Zolgensma for the first nine months of 2024 were $952 million, an increase of 3% from the first nine months of 2023, and the product continues to treat mainly incident patients in established markets. The decrease in Zolgensma royalties was primarily attributable to fluctuations in the effective royalty rate under the license agreement with Novartis.

Research and Development Expense. Research and development expenses decreased by $18.4 million, from $176.6 million for the nine months ended September 30, 2023 to $158.1 million for the nine months ended September 30, 2024. The decrease was primarily attributable to the following:

- a decrease of $13.4 million in manufacturing expenses and other costs of clinical supply for our lead product candidates, largely driven by ABBV-RGX-314 and RGX-202 clinical supply costs;
- a decrease of $9.4 million in personnel-related costs for research and development personnel, including a $2.8 million decrease in stock-based compensation expense, largely driven by the reduction in workforce associated with our corporate restructuring implemented in the fourth quarter of 2023;
- a decrease of $6.1 million in costs for laboratories and facilities used by research and development personnel, including a $0.8 million decrease in depreciation expense allocated to research and development functions, primarily driven by a decrease in laboratory supplies and consumables; and
- a decrease of $5.5 million in preclinical activities and other early-stage research and development.

The decrease in research and development expenses was partially offset by an increase of $17.5 million in costs associated with clinical trial activities, largely driven by clinical trial expenses for ABBV-RGX-314 and RGX-202.

General and Administrative Expense. General and administrative expenses decreased by $12.8 million, from $69.4 million for the nine months ended September 30, 2023 to $56.6 million for the nine months ended September 30, 2024. The decrease was primarily attributable to the following:

- a decrease of $10.3 million in professional services and consulting fees, including legal and other corporate advisory services; and
- a decrease of $1.4 million in personnel-related costs for general and administrative personnel, including a $0.2 million decrease in stock-based compensation expense, largely driven by the reduction in workforce associated with our corporate restructuring implemented in the fourth quarter of 2023.

Liquidity and Capital Resources

Sources of Liquidity

As of September 30, 2024, we had cash, cash equivalents and marketable securities of $278.6 million, which were primarily derived from the sale of our common stock and pre-funded warrants described below and license fees received under the AbbVie Collaboration Agreement. We expect that our cash, cash equivalents and marketable securities as of September 30, 2024 will enable us to fund our operating expenses and capital expenditure requirements, and are sufficient to meet our financial commitments and obligations, for at least the next 12 months from the date of this report based on our current business plan.

In March 2024, we completed a public offering of 4,565,260 shares of our common stock at a price of $23.00 per share and 1,521,740 pre-funded warrants to purchase shares of our common stock at a price of $22.9999 per pre-funded warrant, which equaled

the public offering price per share of the common stock less the $0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received from the offering were $131.1 million, net of underwriting discounts and commissions and offering expenses.

We intend to devote the majority of our current capital to preclinical research, clinical development, seeking regulatory approval of our product candidates and, if approved, commercialization of our product candidates, as well as additional capital expenditures needed to support these activities. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the total amount of operating expenditures and capital outlays necessary to complete the development of our product candidates. While we expect the pipeline prioritization and corporate restructuring implemented in November 2023 to result in cost savings, we may also incur other charges or cash expenditures not currently contemplated due to events that may occur as a result of, or associated with, the restructuring. In addition, we may not achieve the expected benefits of any cost reduction measures on our currently anticipated timeline, or at all. Furthermore, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect which could accelerate our liquidity needs.

At-the-Market Offering Program

In September 2023, we entered into an ATM Equity Offering℠ Sales Agreement with BofA Securities, Inc. (BofA) pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to $150.0 million from time to time through BofA, acting as our sales agent (the ATM Program). We intend to use proceeds obtained from the sale of shares under the ATM Program, if any, for general corporate purposes. As of September 30, 2024, no shares of common stock had been sold under the ATM Program.

Cash Flows

Our consolidated cash flows were as follows (in thousands):

	Nine Months Ended September 30,	
	2024	2023
Net cash used in operating activities	$ (141,501)	$ (177,647)
Net cash provided by investing activities	63,173	156,969
Net cash provided by (used in) financing activities	100,423	(23,004)
Net increase (decrease) in cash and cash equivalents and restricted cash	$ 22,095	$ (43,682)

Cash Flows from Operating Activities

Our net cash used in operating activities for the nine months ended September 30, 2024 decreased by $36.1 million from the nine months ended September 30, 2023, largely as a result of lower operating expenses and increased cost reimbursement received from AbbVie under our ABBV-RGX-314 collaboration in the first nine months of 2024. We expect to continue to incur regular net cash outflows from operations for the foreseeable future as we continue the development and advancement of our product candidates and other research programs.

For the nine months ended September 30, 2024, our net cash used in operating activities of $141.5 million consisted of a net loss of $175.9 million and unfavorable changes in operating assets and liabilities of $6.1 million, offset by adjustments for non-cash items of $40.5 million. The changes in operating assets and liabilities include a decrease in total accounts payable, accrued expenses and other current liabilities, and other liabilities of $7.5 million, which was driven largely by decreases in accrued personnel costs, sublicense fees payable to licensors and amounts payable to suppliers as of the end of the period. Other changes in operating working capital occurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $28.9 million and depreciation and amortization expense of $12.2 million.

For the nine months ended September 30, 2023, our net cash used in operating activities of $177.6 million consisted of a net loss of $200.6 million and unfavorable changes in operating assets and liabilities of $20.1 million, offset by adjustments for non-cash items of $43.1 million. The changes in operating assets and liabilities include an increase in other current assets of $13.6 million, which was driven primarily by an increase in net cost reimbursement due from AbbVie under our ABBV-RGX-314 collaboration, and a decrease in total accounts payable and accrued expenses and other current liabilities of $5.6 million, which was driven primarily by a decrease in amounts payable to suppliers as of the end of the period. Other changes in operating working capital occurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $31.9 million and depreciation and amortization expense of $13.0 million.

Cash Flows from Investing Activities

For the nine months ended September 30, 2024, our net cash provided by investing activities consisted of $238.4 million in maturities of marketable debt securities, offset by $173.9 million used to purchase marketable debt securities and $1.4 million used to purchase property and equipment.

For the nine months ended September 30, 2023, our net cash provided by investing activities consisted of $231.8 million in maturities of marketable debt securities and $2.0 million in proceeds received from uniQure N.V. (uniQure) in connection with the achievement of milestones associated with their acquisition of Corlieve Therapeutics SAS (Corlieve), offset by $67.9 million used to purchase marketable debt securities and $8.8 million used to purchase property and equipment.

Cash Flows from Financing Activities

For the nine months ended September 30, 2024, our net cash provided by financing activities primarily consisted of $131.1 million in net proceeds received from the public offering of common stock and pre-funded warrants completed in March 2024, net of underwriting discounts and commissions and other offering expenses paid during the period, and $2.7 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan. Our net cash provided by financing activities was partially offset by $32.2 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR.

For the nine months ended September 30, 2023, our net cash used in financing activities primarily consisted of $30.5 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR. Our net cash used in financing activities was partially offset by $4.9 million in net proceeds received from a private placement of our common stock in July 2023 and $3.1 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Additional Capital Requirements

Our material capital requirements from known contractual and other obligations primarily relate to vendor service contracts and purchase commitments, in-license agreements, operating lease agreements and our Zolgensma royalty purchase agreement with HCR. Our material commitments and obligations are further described in Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" of our Annual Report on Form 10-K for the year ended December 31, 2023, and in the notes to the audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023. Other than the changes described in the notes to the unaudited consolidated financial statements accompanying this Quarterly Report on Form 10-Q, including Note 6, "Leases," there have been no material changes to our commitments and obligations since December 31, 2023.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $881.0 million as of September 30, 2024. Our transition to recurring profitability is dependent upon achieving a level of revenues adequate to support our cost structure, which depends heavily on the successful development, approval and commercialization of our product candidates. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect to continue to incur significant research and development and general and administrative expenses for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect to continue to incur capital expenditures associated with building out additional laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;

- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;
- revenue received from commercial sales of Zolgensma and the timing and amount of Zolgensma royalties paid to HCR under our royalty purchase agreement;
- revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, and other revenue received under our licensing agreements and collaborations;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration Agreement, and our ability to timely achieve any milestones set forth in such agreements or collaborations;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and 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 milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform licensing is dependent in part on the clinical and commercial success of our licensing partners, including the commercialization of Zolgensma, and on maintaining our license agreements with our licensor partners, including GlaxoSmithKline LLC and The Trustees of the University of Pennsylvania. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or others that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Off-Balance Sheet Arrangements

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

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

For information regarding market risk, refer to Item 7A, "Quantitative and Qualitative Disclosures About Market Risk," included in our Annual Report on Form 10-K for the year ended December 31, 2023. There have been no material changes to our exposure to market risk during the nine months ended September 30, 2024.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures 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 accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of September 30, 2024, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended September 30, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

Item 1A. Risk Factors.

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2023. There have been no material changes from the risk factors previously disclosed in such filing.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

Rule 10b5-1 Trading Plans

During the three months ended September 30, 2024, none of our directors or Section 16 reporting officers adopted or terminated any Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement (as such terms are defined in Item 408 of the SEC's Regulation S-K).

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	6/7/21	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
10.1*	Employment Separation Agreement, effective as of September 21, 2024, between the Registrant and Vittal Vasista				X
10.2*	Consulting Agreement, effective as of September 16, 2024, between the Registrant and Vittal Vasista				X
31.1	Certification of the Chief Executive Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2024 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Loss (iii) Consolidated Statements of Stockholders' Equity (iv) Consolidated Statements of Cash Flows (v) Notes to Consolidated Financial Statements				X
104	The cover page from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2024 formatted in Inline XBRL (included in Exhibit 101)				

* Management contract or compensatory plan or arrangement.

The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

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.

REGENXBIO Inc.

Dated: November 6, 2024

/s/ Curran Simpson
Curran Simpson
President and Chief Executive Officer
(Principal Executive Officer)

Dated: November 6, 2024

/s/ Mitchell Chan
Mitchell Chan
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)