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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, 2025

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, 2025, there were 50,623,086 shares of the registrant's common stock, par value $0.0001 per share, issued and outstanding.

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REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2025

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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 and our collaboration with Nippon Shinyaku to develop and commercialize RGX-121 and RGX-111;
- 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®, CAMPSIITE® and other 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 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;
- the impact of any government-imposed tariffs or other trade barriers on cost of goods and services, particularly related to partnered product candidates; 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, 2024 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, NAVXpress, 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, 2025	December 31, 2024
Assets		
Current assets		
Cash and cash equivalents	$ 58,802	$ 57,526
Marketable securities	215,403	177,161
Accounts receivable	25,347	20,473
Prepaid expenses	12,943	9,067
Other current assets	19,472	13,774
Total current assets	331,967	278,001
Marketable securities	27,838	10,179
Accounts receivable	2,765	474
Property and equipment, net	107,515	117,589
Operating lease right-of-use assets	48,820	53,716
Restricted cash	2,030	2,030
Other assets	4,268	4,000
Total assets	$ 525,203	$ 465,989
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 22,266	$ 22,798
Accrued expenses and other current liabilities	38,316	38,070
Deferred revenue	13,195	115
Operating lease liabilities	9,073	7,902
Royalty monetization liabilities	41,718	34,309
Total current liabilities	124,568	103,194
Deferred revenue	21,186	—
Operating lease liabilities	67,433	74,131
Royalty monetization liabilities	149,916	25,378
Other liabilities	648	3,635
Total liabilities	363,751	206,338
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at September 30, 2025 and December 31, 2024	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2025 and December 31, 2024; 50,619 and 49,549 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	1,220,977	1,192,536
Accumulated other comprehensive loss	(652)	(741)
Accumulated deficit	(1,058,878)	(932,149)
Total stockholders' equity	161,452	259,651
Total liabilities and stockholders' equity	$ 525,203	$ 465,989

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,	
	2025	2024	2025	2024
Revenues				
License and royalty revenue	$ 23,605	$ 23,982	$ 129,119	$ 61,172
Service revenue	6,128	215	10,985	942
Total revenues	29,733	24,197	140,104	62,114
Operating Expenses				
Cost of license and royalty revenues	5,725	12,387	14,370	27,249
Research and development	56,101	54,429	168,688	158,142
General and administrative	20,253	19,422	60,483	56,568
Impairment of long-lived assets	—	—	—	2,101
Other operating expenses	65	37	125	32
Total operating expenses	82,144	86,275	243,666	244,092
Loss from operations	(52,411)	(62,078)	(103,562)	(181,978)
Other Income (Expense)				
Interest income from licensing	19	25	65	91
Investment income	3,620	3,276	9,500	9,213
Interest expense	(13,169)	(820)	(32,732)	(3,242)
Total other income (expense)	(9,530)	2,481	(23,167)	6,062
Net loss	$ (61,941)	$ (59,597)	$ (126,729)	$ (175,916)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	98	1,684	89	3,847
Total other comprehensive income	98	1,684	89	3,847
Comprehensive loss	$ (61,843)	$ (57,913)	$ (126,640)	$ (172,069)
Net loss per share, basic and diluted	$ (1.20)	$ (1.17)	$ (2.46)	$ (3.59)
Weighted-average common shares outstanding, basic and diluted	51,689	50,800	51,513	49,051

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, 2025					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2025	50,389	$ 5	$ 1,211,361	$ (750)	$ (996,937)	$ 213,679
Vesting of restricted stock units, net of tax	119	—	(123)	—	—	(123)
Issuance of common stock under employee stock purchase plan	111	—	741	—	—	741
Stock-based compensation expense	—	—	8,998	—	—	8,998
Unrealized gain on available-for-sale securities, net	—	—	—	98	—	98
Net loss	—	—	—	—	(61,941)	(61,941)
Balances at September 30, 2025	50,619	$ 5	$ 1,220,977	$ (652)	$ (1,058,878)	$ 161,452

	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

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, 2025					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2024	49,549	$ 5	$ 1,192,536	$ (741)	$ (932,149)	$ 259,651
Vesting of restricted stock units, net of tax	652	—	(633)	—	—	(633)
Exercise of stock options, net of tax	61	—	228	—	—	228
Issuance of common stock under employee stock purchase plan	158	—	1,048	—	—	1,048
Issuance of warrants, net of transaction costs	—	—	1,610	—	—	1,610
Exercise of pre-funded warrants	199	—	—	—	—	—
Stock-based compensation expense	—	—	26,188	—	—	26,188
Unrealized gain on available-for-sale securities, net	—	—	—	89	—	89
Net loss	—	—	—	—	(126,729)	(126,729)
Balances at September 30, 2025	50,619	$ 5	$ 1,220,977	$ (652)	$ (1,058,878)	$ 161,452

	Nine Months Ended September 30, 2024					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
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

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, 2025	2024
Cash flows from operating activities		
Net loss	$ (126,729)	$ (175,916)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	26,188	28,921
Depreciation and amortization	11,746	12,192
Net accretion of discounts on marketable debt securities	(4,250)	(3,027)
Net realized gain on investments	(62)	—
Impairment of long-lived assets	—	2,101
Non-cash interest expense	7,795	412
Other non-cash adjustments	60	(60)
Changes in operating assets and liabilities		
Accounts receivable	(7,100)	1,574
Prepaid expenses	(3,876)	3,518
Other current assets	(5,586)	(2,804)
Operating lease right-of-use assets	4,989	4,586
Other assets	(268)	475
Accounts payable	(423)	(3,330)
Accrued expenses and other current liabilities	184	(1,448)
Deferred revenue	34,266	(4)
Operating lease liabilities	(5,620)	(6,021)
Other liabilities	(2,987)	(2,670)
Net cash used in operating activities	(71,673)	(141,501)
Cash flows from investing activities		
Purchases of marketable debt securities	(269,819)	(173,916)
Maturities of marketable debt securities	218,257	238,446
Sales of equity securities	62	—
Purchases of property and equipment	(1,906)	(1,357)
Net cash provided by (used in) investing activities	(53,406)	63,173
Cash flows from financing activities		
Proceeds from exercise of stock options	228	1,474
Taxes paid related to net settlement of stock-based awards	(633)	(910)
Proceeds from issuance of common stock under employee stock purchase plan	1,048	1,191
Proceeds from public offering of common stock and pre-funded warrants, net of issuance costs	—	131,067
Offering expenses related to at-the-market offering programs	(334)	(158)
Proceeds from issuance of royalty bond and warrants, net of transaction costs	144,493	—
Repayments under royalty monetization liabilities, net of interest	(18,447)	(32,241)
Net cash provided by financing activities	126,355	100,423
Net increase in cash and cash equivalents and restricted cash	1,276	22,095
Cash and cash equivalents and restricted cash		
Beginning of period	59,556	36,552
End of period	$ 60,832	$ 58,647

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 proprietary AAV vectors. 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, 2025, 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.

Liquidity

The Company has incurred cumulative losses since inception and as of September 30, 2025, had generated an accumulated deficit of $1.06 billion. 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. The Company's ability to continue as a going concern may be impacted by the development and potential commercialization of its product candidates and its ability to raise additional capital to fund its operations. 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, 2025, the Company had cash, cash equivalents and marketable securities of $302.0 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, 2024 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on March 13, 2025. 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, 2024, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024.

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. Estimates are used in the following areas, among others: revenue recognition, the allowance for credit losses, accrued research and development expenses and other accrued liabilities, stock-based compensation expense, interest expense under royalty monetization liabilities, income taxes and fair value measurements.

Reclassifications

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation, including separate presentation of service revenue in the statements of operations and comprehensive loss. As a result of the Company's collaboration and license agreement with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) effective in March 2025, the Company has modified the presentation of its revenues and now presents service revenues separately from license and royalty revenues. The modified presentation has been applied retrospectively to all prior periods presented. The reclassifications 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,	
	2025	2024
Cash and cash equivalents	$ 58,802	$ 56,617
Restricted cash	2,030	2,030
Total cash and cash equivalents and restricted cash	$ 60,832	$ 58,647

Accounts Receivable

Accounts receivable consist of consideration due to the Company resulting from its agreements with customers. Accounts receivable include amounts invoiced to customers as well as rights to consideration which have not yet been invoiced, including unbilled royalties and services, 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 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 balance is 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. The Company did not record an allowance for credit losses on its accounts receivable as of September 30, 2025 and December 31, 2024.

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. Accounting Standards Codification (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.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of ASC 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of ASC 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

The Company licenses its NAVTechnology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of its 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 Company's NAVTechnology 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 payable to the Company under its license and collaboration 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 and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

The Company evaluates its agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company identifies the various transactions with the counterparty and determines if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, the Company applies the five-step model as described in its revenue recognition policies.

The Company's license and collaboration agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each agreement, the Company determines the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to the Company. For each license granted, the Company evaluates these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options.

Performance obligations under the Company's license and collaboration agreements may include (i) the delivery of intellectual property licenses, (ii) development and manufacturing services to be performed by the Company related to licensed products and (iii) options granted to purchase additional goods and services, to the extent the options convey material rights. At the inception of each license agreement which contains performance obligations for development or other services, the Company evaluates whether the license is distinct from the services, which requires judgment. In making this determination, the Company considers, among other things, the stage of development of the licensed products and whether the services will significantly impact further development of the licensed products. If it is determined that the license is not distinct from the services, the license is combined with the services into a single performance obligation. Agreements may provide licensees and collaborators with options to purchase additional goods or other services, including options to purchase commercial supply of licensed products. Options are evaluated at the inception of the agreement to determine whether they provide material rights to the customer. In making this determination, the Company considers whether the options are priced at an incremental discount to the standalone selling price of the underlying goods or services, in which case the option is considered to be a material right. Material rights are accounted for as separate performance obligations under the current arrangement.

The Company evaluates the transaction price of its license and collaboration agreements at contract inception and at each reporting date. The transaction price includes the fixed consideration payable to the Company over the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the agreements may include up-front and annual fees payable over the contract term and fixed fees for development and other services performed by the Company. Variable consideration under the agreements may include development and sales-based milestone payments, payments for development and other services performed by the Company, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by the customer is excluded from the transaction price and not accounted for as part of the arrangement until the option is exercised.

The transaction price of the Company's license and collaboration arrangements is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Variable consideration payable based on services performed by the Company is allocated directly to the performance obligation for such services. Consideration allocated to performance obligations for the delivery of intellectual property licenses is recognized as license and royalty revenue in full upon the delivery of the license. Consideration allocated to performance obligations for development and manufacturing services is recognized as service revenue as the services are performed by the Company. Consideration allocated to performance obligations for material rights to purchase additional goods and services is recognized as revenue upon the satisfaction of the performance obligations underlying the optional goods and services purchased by the customer. Service revenue is recognized using a measure of progress that best reflects the pattern of satisfaction of the performance obligations. At each reporting date, the Company re-evaluates the measure of progress and adjusts service revenue on a cumulative catch-up basis to reflect its best estimate of the services performed to date versus the total services to be performed under the arrangement.

Development milestone payments are payable to the Company upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of each outstanding development milestone and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. The increase to the transaction price as a result of any such adjustments is then allocated to the underlying performance obligations in a manner similar to the allocation of the initial transaction price and, to the extent the performance obligations are satisfied, recognized as revenue on a cumulative catch-up basis in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as license and royalty revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under the Company's license agreement with Novartis Gene Therapies, Inc. (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the development and commercialization of treatments for spinal muscular atrophy (SMA). 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.

The Company receives payments from licensees and collaborators based on the billing schedules established in the associated agreements. Amounts recognized as revenue which have not yet been received from the customer are recorded as accounts receivable when the Company's rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from customers are recorded as contract assets when the Company's rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets if the consideration is expected to be realized within 12 months from the reporting date, or as other assets if the consideration is expected to be realized in periods beyond 12 months from the reporting date. 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 consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of revenue in the period of the termination. Amounts received by the Company prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations by the Company. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

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 underlying 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, 2025 and 2024 which had a material impact on the consolidated financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In December 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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 for annual periods beginning January 1, 2025. The Company is currently in the process of evaluating the impact of this standard on its consolidated financial statements and related disclosures.

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. In January 2025, the FASB issued ASU 2025-01, which clarifies the effective date of ASU 2024-03 with respect to interim periods. 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 currently in the process of 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, 2025				
U.S. government and agency securities	$ 123,444	$ 66	$ (1)	$ 123,509
Corporate bonds	119,589	158	(15)	119,732
	$ 243,033	$ 224	$ (16)	$ 243,241

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2024				
U.S. government and agency securities	$ 44,281	$ 11	$ (77)	$ 44,215
Certificates of deposit	1,466	—	(4)	1,462
Corporate bonds	141,474	234	(45)	141,663
	$ 187,221	$ 245	$ (126)	$ 187,340

As of September 30, 2025 and December 31, 2024, no available-for-sale debt securities had remaining maturities greater than two 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, 2025 and December 31, 2024, 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, 2025 and 2024, 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, 2025						
U.S. government and agency securities	$ 11,485	$ (1)	$ —	$ —	$ 11,485	$ (1)
Corporate bonds	34,235	(15)	—	—	34,235	(15)
	$ 45,720	$ (16)	$ —	$ —	$ 45,720	$ (16)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2024						
U.S. government and agency securities	$ —	$ —	$ 22,917	$ (77)	$ 22,917	$ (77)
Certificates of deposit	—	—	1,221	(4)	1,221	(4)
Corporate bonds	21,317	(45)	—	—	21,317	(45)
	$ 21,317	$ (45)	$ 24,138	$ (81)	$ 45,455	$ (126)

As of September 30, 2025, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 11 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, 2025 or December 31, 2024, and no impairment or credit losses on available-for-sale debt securities were recorded during the three and nine months ended September 30, 2025 and 2024.

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, 2025				
Cash equivalents:				
Money market mutual funds	$ —	$ 49,384	$ —	$ 49,384
Total cash equivalents	—	49,384	—	49,384
Marketable securities:				
U.S. government and agency securities	—	123,509	—	123,509
Corporate bonds	—	119,732	—	119,732
Total marketable securities	—	243,241	—	243,241
Total cash equivalents and marketable securities	$ —	$ 292,625	$ —	$ 292,625

	Level 1	Level 2	Level 3	Total
December 31, 2024				
Cash equivalents:				
Money market mutual funds	$ —	$ 43,895	$ —	$ 43,895
U.S. government and agency securities	—	2,498	—	2,498
Total cash equivalents	—	46,393	—	46,393
Marketable securities:				
U.S. government and agency securities	—	44,215	—	44,215
Certificates of deposit	—	1,462	—	1,462
Corporate bonds	—	141,663	—	141,663
Total marketable securities	—	187,340	—	187,340
Total cash equivalents and marketable securities	$ —	$ 233,733	$ —	$ 233,733

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. Certain 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, 2025 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 values of its royalty monetization liabilities approximate fair value. As discussed in Note 7, the carrying values of royalty monetization liabilities are based on the Company's estimate of future royalties, milestones and other consideration to be paid over the life of the arrangement, which are considered Level 3 inputs, as well as any remaining repayment obligations upon maturity of the instruments.

Long-lived assets, if determined to be 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, 2025 and 2024.

5. Property and Equipment, Net

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

	September 30, 2025	December 31, 2024
Laboratory and manufacturing equipment	$ 78,017	$ 77,141
Computer equipment and software	4,468	4,244
Furniture and fixtures	7,036	7,031
Leasehold improvements	101,533	101,465
Total property and equipment	191,054	189,881
Accumulated depreciation and amortization	(83,539)	(72,292)
Property and equipment, net	$ 107,515	$ 117,589

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 Lease and 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 expires 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, 2025, total undiscounted future minimum lease payments to be received by the Company over the term of the New York Sublease were $0.8 million. The Company recognized sublease income under the New York Sublease of $0.1 million and $0.3 million during the three and nine months ended September 30, 2025, respectively, and $0.1 million and $0.2 million 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 impairment losses on long-lived assets were recorded during the three and nine months ended September 30, 2025 and the three months ended September 30, 2024.

7. Royalty Monetization Liabilities

Royalty monetization liabilities are accounted for as debt and consist of the following (in thousands):

	September 30, 2025	December 31, 2024
2020 Royalty Purchase Agreement	$ 40,149	$ 59,687
2025 Royalty Bond	151,485	—
Total	$ 191,634	$ 59,687
Current portion of royalty monetization liabilities	$ 41,718	$ 34,309
Non-current portion of royalty monetization liabilities	149,916	25,378
Total	$ 191,634	$ 59,687

2020 Royalty Purchase Agreement

In December 2020, the Company entered into a royalty purchase agreement (the 2020 Royalty Purchase Agreement) with entities managed by Healthcare Royalty Management, LLC (collectively and with other affiliated entities, HCR). Under the 2020 Royalty Purchase Agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement with Novartis Gene Therapies (the Novartis License), including $4.0 million of royalty payments received by the Company in the fourth quarter of 2020. In consideration for these rights, HCR paid the Company $200.0 million (the Purchase Price), less $4.0 million representing the payment of the royalties received in the fourth quarter of 2020 to HCR. Beginning upon the effective date of the 2020 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 2020 Royalty Purchase Agreement, the total amount of Zolgensma royalty payments to be paid to HCR was subject to an increasing cap (the Cap Amount) equal to (i) $260.0 million applicable for the period from the effective date of the 2020 Royalty Purchase Agreement through November 7, 2024 (the First Cap Amount), and (ii) $300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the Novartis License (the Second Cap Amount). If, on or prior to the defined dates for each Cap Amount, the total amount of Zolgensma royalties paid to HCR equals or exceeds the Cap Amount applicable to such date, the 2020 Royalty Purchase Agreement will automatically terminate. The First Cap Amount was not achieved prior to November 7, 2024, therefore the 2020 Royalty Purchase Agreement will remain in effect until the achievement of the Second Cap Amount or the termination of the Novartis License, if earlier. The Company has no obligation to repay any amounts to HCR under the 2020 Royalty Purchase Agreement if 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 royalties under the 2020 Royalty Purchase Agreement for a repurchase price equal to, as of the option exercise date, $300.0 million minus the total amount of royalty payments paid to HCR.

The proceeds received from HCR under the 2020 Royalty Purchase Agreement 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 paid to HCR, subject to the Cap Amount, over the life of the arrangement. The total amount of royalty payments paid to HCR, 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 arrangement.

The Company estimates the effective interest rate used to record interest expense under the 2020 Royalty Purchase Agreement based on its estimate of total Zolgensma royalties to be paid HCR under the arrangement. At each reporting date, the Company reassesses its estimate of total future royalty payments to be paid to HCR at the applicable Cap Amount, and prospectively adjusts the effective interest rate and amortization of the liability as necessary. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of royalty payments actually paid to HCR, which may differ from the Company's forecasts. The estimated effective interest rate in effect as of September 30, 2025 and December 31, 2024 was 77.1% and 65.5%, respectively, which was based on the amortized balance of the liability and the estimated remaining royalties to be paid to HCR under the arrangement. The estimated interest rate is subject to adjustments in the future based on actual royalties paid to HCR and changes in the royalty forecast. As of September 30, 2025, the estimated effective interest rate over the life of the 2020 Royalty Purchase Agreement, taking into account actual royalties paid to date and the estimated remaining royalties to be paid under the arrangement, was 16.2%.

The following table presents the changes in the royalty monetization liability under the 2020 Royalty Purchase Agreement with HCR (in thousands):

	2020 Royalty Purchase Agreement
Balance at December 31, 2024	$ 59,687
Zolgensma royalties paid to HCR	(43,384)
Interest expense recognized	23,846
Balance at September 30, 2025	40,149
Current portion	(38,665)
Non-current portion	$ 1,484

2025 Royalty Bond

In May 2025, the Company entered into a loan agreement with HCR pursuant to which HCR will provide the Company with an aggregate limited recourse loan of up to $250.0 million (the 2025 Royalty Bond). The 2025 Royalty Bond is disbursable to the Company in three tranches, with $150.0 million funded on the closing date in May 2025, $50.0 million available to be funded if sales of a specified product exceed a specified threshold prior to December 31, 2026, and $50.0 million available to be funded if both parties exercise an option in 2027. Loan proceeds under the 2025 Royalty Bond are funded to the Company net of an original issue discount of 2.25% and reimbursement of certain expenses to HCR. Proceeds received by the Company from the initial funding tranche of the 2025 Royalty Bond in May 2025, net of discounts and transaction costs, were $144.5 million.

Prior to the maturity date, interest and principal under the 2025 Royalty Bond shall be paid quarterly to HCR solely using proceeds received from certain specified royalties, milestone payments, license fees and other consideration payable to the Company under specified license agreements (collectively, the Royalty Interest), including (i) the Novartis License for Zolgensma, (ii) the collaboration and license agreement with Nippon Shinyaku for RGX-121 and RGX-111, and (iii) NAV Technology Platform license agreements with Rocket Pharmaceuticals, Inc. and Ultragenyx Pharmaceutical Inc. Zolgensma royalties under the Novartis License shall only be included in the Royalty Interest after full repayment of the applicable Cap Amount under the 2020 Royalty Purchase Agreement with HCR. The Royalty Interest excludes, and the Company retains the rights to, certain other consideration payable under the license agreements including certain milestone payments, license fees and reimbursement of costs as applicable. The Royalty Interest is payable to HCR net of upstream royalty and sublicense fee obligations payable by the Company to applicable licensors. The 2025 Royalty Bond is collateralized by a security interest and lien on the Royalty Interest.

The 2025 Royalty Bond bears interest at a rate of 9.75% plus the 3-month secured overnight financing rate as administered by the Federal Reserve Bank of New York (SOFR), with a minimum interest rate of 14.0%. Interest payments are due quarterly using proceeds received under the Royalty Interest. At each payment date, any proceeds received under the Royalty Interest in excess of the interest payment due will be applied to outstanding principal. If the proceeds received under the Royalty Interest are insufficient to pay the interest due, unpaid interest will accrue to the principal balance.

The 2025 Royalty Bond matures in May 2035, subject to potential extension, unless repaid in full at an earlier date. The maturity date may be extended by two years to May 2037 subject to a potential patent term extension of a specific patent. Upon maturity, the outstanding principal and interest shall be due and payable to HCR. Additionally, upon repayment in full prior to the maturity date, or at the maturity date, the Company shall pay to HCR an additional amount equal to 5.0% of the total outstanding principal as of the applicable determination date. Other than through the payment of proceeds received under the Royalty Interest, the 2025 Royalty Bond may not be prepaid prior to maturity.

In connection with the loan agreement for the 2025 Royalty Bond, the Company also issued HCR warrants to purchase 268,096 shares of its common stock at an exercise price per share of $14.92 (the May 2025 Warrants). The May 2025 Warrants are exercisable upon issuance and expire 10 years from the closing date of the 2025 Royalty Bond. The Company evaluated the May 2025 Warrants and concluded the warrants are indexed to the Company's common stock and meet the criteria to be classified as equity. The net proceeds received by the Company under the loan agreement of $144.5 million were allocated between the 2025 Royalty Bond and the May 2025 Warrants based on their relative fair values. The fair value of the 2025 Royalty Bond was determined based on the carrying amount of the loan on the closing date. The fair value of the May 2025 Warrants was determined using a Black-Scholes option-pricing model on the closing date, resulting in an estimated fair value of the warrants of $1.7 million. Based on the relative fair values of these instruments, $1.6 million of the net proceeds were allocated to the warrants and recorded as additional paid-in capital. The net proceeds allocated to the 2025 Royalty Bond were $142.9 million, resulting in a total debt discount of $7.1 million which is recorded as a reduction of the carrying value of the debt and will be amortized as interest expense over the life of the 2025 Royalty Bond.

The effective interest rate of the 2025 Royalty Bond is partially estimated based on the Company's estimate of future payments under the Royalty Interest to be paid HCR. At each reporting date, the Company reassesses its estimate of total future payments to HCR under the arrangement and prospectively adjusts the effective interest rate and amortization of the debt and associated discount as necessary. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the actual Royalty Interest payments to HCR and fluctuations in the variable interest rate, which may differ from the Company's forecasts. The estimated effective interest rate in effect as of September 30, 2025 was 14.9%, which was based on the amortized balance of the liability, the current coupon rate and the estimated remaining payments to HCR under the arrangement.

The following table presents the changes in the royalty monetization liability under the 2025 Royalty Bond with HCR (in thousands):

	2025 Royalty Bond
Balance at December 31, 2024	$ —
Proceeds from royalty bond, net of discount and issuance costs	142,883
Unpaid interest accrued to principal	8,027
Amortization of debt discount and issuance costs	575
Balance at September 30, 2025	151,485
Current portion	(3,053)
Non-current portion	$ 148,432

8. Commitments and Contingencies

GlaxoSmithKline

In March 2009, the Company entered into a license agreement, which was amended in April 2009 (as amended, the GSK License), with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents underlying the Company's NAV Technology Platform which are owned by The Trustees of the University of Pennsylvania (Penn) and exclusively licensed to GSK. Pursuant to the GSK License, the Company is obligated to pay GSK royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse GSK for certain costs incurred related to the maintenance of the licensed patents. The Company was also obligated to pay $1.5 million to GSK upon the achievement of various milestones, all of which have been achieved and paid.

In March 2022, the Company entered into a letter agreement (the Penn Letter Agreement) with Penn to buy out the Company's obligation to pay sublicense fees under its license agreement with Penn. In connection with the execution of the Penn Letter Agreement in March 2022, the Company's royalty obligations under the GSK License were assigned by GSK to Penn. Beginning upon the effective date of the Penn Letter Agreement in March 2022, any royalties payable by the Company under the GSK License shall be paid to Penn rather than GSK. The Company remains obligated to pay GSK sublicense fees and reimbursement of certain patent maintenance costs in accordance with the GSK License.

Expenses incurred by the Company related to the GSK License were recorded as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Cost of license and royalty revenues:				
Royalties on net sales of Zolgensma	$ 5,709	$ 12,330	$ 13,495	$ 27,168
Other	1	7	244	31
Total cost of license and royalty revenues	5,710	12,337	13,739	27,199
General and administrative	30	70	137	147
	$ 5,740	$ 12,407	$ 13,876	$ 27,346

As of September 30, 2025 and December 31, 2024, the Company had recorded $6.4 million and $6.3 million, respectively, payable under the GSK License.

The Company has been notified of a dispute with GSK over the amount of sublicense fees paid by the Company to GSK under the GSK License. GSK claims there has been a significant underpayment by the Company as they are entitled to a sublicense payment on all amounts received by the Company from sublicensees, including royalties, and not just amounts received for GSK's sublicensed patents. The Company disagrees with GSK's interpretation of the GSK License. The Company does not believe that a loss is probable, and no reasonable range of loss is estimable, related to this matter. No liabilities related to this matter were recorded as of September 30, 2025 and December 31, 2024.

9. Capitalization

March 2024 Public Offering and Pre-funded Warrants

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 (the March 2024 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.

The rights and privileges of the March 2024 Pre-funded Warrants are set forth in the warrant agreement between the Company and each of the respective warrant holders. The March 2024 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%. The March 2024 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 March 2024 Pre-funded Warrants 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 199,300 shares of common stock upon the exercise of March 2024 Pre-funded Warrants during the nine months ended September 30, 2025, and there were no exercises of March 2024 Pre-funded Warrants during the three months ended September 30, 2025. The Company issued 197,000 shares of common stock upon the exercise of March 2024 Pre-funded Warrants during the nine months ended September 30, 2024, and there were no exercises of March 2024 Pre-funded Warrants during the three months ended September 30, 2024. As of September 30, 2025, 1,125,440 of the March 2024 Pre-funded Warrants remained outstanding.

May 2025 Warrants

In May 2025, in connection with issuance of the 2025 Royalty Bond, the Company issued to HCR the May 2025 Warrants to purchase 268,096 shares of its common stock at an exercise price per share of $14.92. The May 2025 Warrants are exercisable upon issuance and have a contractual term of 10 years. The Company evaluated the May 2025 Warrants 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 Company allocated $1.6 million of the net proceeds from the 2025 Royalty Bond to the issuance of the May 2025 Warrants, which were recorded as additional paid-in capital. Please refer to Note 7 for further information on the May 2025 Warrants issued in connection with the 2025 Royalty Bond. As of September 30, 2025, none of the May 2025 Warrants had been exercised and 268,096 of the May 2025 Warrants remained outstanding.

At-the-Market Offering Program

In December 2024, the Company entered into a Sales Agreement with Leerink Partners LLC (Leerink) 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 Leerink, acting as the Company's sales agent (the Leerink ATM Program). As of September 30, 2025, no shares of common stock had been sold under the Leerink ATM Program.

10. License and Collaboration Agreements

License and Collaboration Revenues

As of September 30, 2025, 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 and commercialization of certain product candidates. Consideration payable to the Company under its license and collaboration 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 and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators. 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.

Revenues earned under license and collaboration agreements consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
License and royalty revenue:				
Zolgensma royalties	$ 23,586	$ 23,872	$ 59,002	$ 60,849
Nippon Shinyaku licenses	—	—	69,979	—
Other license and royalty revenue	19	110	138	323
Total license and royalty revenue	23,605	23,982	129,119	61,172
Service revenue:				
Nippon Shinyaku services	5,932	—	10,426	—
Other service revenue	196	215	559	942
Total service revenue	6,128	215	10,985	942
Total revenues	$ 29,733	$ 24,197	$ 140,104	$ 62,114

Outstanding development milestone payments are evaluated each reporting period and are only included in the transaction price of each license 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, 2025, the Company's license and collaboration agreements contained unachieved milestones which could result in aggregate milestone payments to the Company of up to $2.18 billion, including (i) $548.2 million upon the commencement of various stages of clinical trials, (ii) $106.3 million upon the submission of regulatory approval filings or upon regulatory approval of licensed products and (iii) $1.53 billion 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.

Accounts Receivable, Contract Assets and Deferred Revenue

The following table presents the balances of the Company's 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,	
	2025	2024	2025	2024
Accounts receivable, net, current and non-current:				
Beginning of period	$ 21,783	$ 23,273	$ 20,947	$ 25,491
End of period	$ 28,112	$ 24,008	$ 28,112	$ 24,008
Contract assets:				
Beginning of period	$ 28	$ 194	$ 239	$ —
End of period	$ 93	$ 259	$ 93	$ 259
Deferred revenue, current and non-current:				
Beginning of period	$ 37,781	$ 21	$ 115	$ 148
End of period	$ 34,381	$ 144	$ 34,381	$ 144
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	$ 3,401	$ 21	$ 115	$ 148
Performance obligations satisfied in previous periods	$ 23,590	$ 23,875	$ 59,010	$ 60,912

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma royalties.

As of September 30, 2025, the Company had recorded deferred revenue of $34.4 million which represents consideration received or unconditionally due from licensees and collaboration partners for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations as of September 30, 2025 consisted of (i) development services to be performed related to licensed products, which will be satisfied as the services are performed, and (ii) material rights granted to purchase commercial supply of licensed products, which will be satisfied upon delivery of the commercial supply. As of September 30, 2025, the aggregate transaction price of the Company's license and collaboration agreements allocated to performance obligations not yet satisfied or partially satisfied was $36.2 million, primarily associated with the Company's collaboration and license agreement with Nippon Shinyaku, the substantial majority of which is expected to be satisfied over a period of approximately five years.

Accounts receivable consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Current accounts receivable:		
Billed to customers	$ 20	$ 65
Unbilled Zolgensma royalties	22,761	20,106
Unbilled Nippon Shinyaku services	2,494	—
Other unbilled	72	302
Current accounts receivable	25,347	20,473
Non-current accounts receivable:		
Unbilled Nippon Shinyaku services	2,292	—
Other unbilled	473	474
Non-current accounts receivable	2,765	474
Total accounts receivable	$ 28,112	$ 20,947

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 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,	
	2025	2024	2025	2024
Zolgensma royalties	$ 23,586	$ 23,872	$ 59,002	$ 60,849
Total license and royalty revenue	$ 23,586	$ 23,872	$ 59,002	$ 60,849
Interest income from licensing	$ 10	$ 6	$ 31	$ 20

As of September 30, 2025 and December 31, 2024, the Company had recorded total accounts receivable of $23.1 million and $20.4 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, 2025 included $16.6 million expected to be paid to HCR in accordance with the 2020 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.

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 (as amended, 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. Global development expenses for ABBV-RGX-314 are shared by the parties in accordance with the AbbVie Collaboration Agreement, with AbbVie being responsible for the majority of total 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 mutually agreed supply agreements. If requested by AbbVie, the Company will manufacture up to a specified portion of ABBV-RGX-314 commercial supply for sales 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.

In August 2025, the Company and AbbVie entered into an amendment to the AbbVie Collaboration Agreement which modified the development plan and milestone payment structure for the ABBV-RGX-314 DR program. Under the amendment, the Company will conduct the first registration enabling trial for DR suprachoroidal (SCS) treatment as a combined Phase IIb/III trial performed in two parts (Part 1 and Part 2), and AbbVie will conduct the second registration enabling trial as a separate, standalone Phase III trial. In

lieu of the $200.0 million development milestone due to the Company under the original AbbVie Collaboration Agreement upon first patient dosed in the first registration enabling trial for DR SCS treatment, AbbVie will pay the Company $100.0 million upon first patient dosed in the Phase IIb/III trial for DR SCS treatment and an additional $100.0 million upon first patient dosed in the subsequent Phase III trial. Also pursuant to the amendment, AbbVie will lead a new Phase III randomized controlled study (the ACHIEVE Study) to assess the injection burden, adverse events, change in disease activity, and long-term preservation of visual acuity of ABBV-RGX-314 in adult participants with neovascular AMD. The Company will be responsible for its development expenses to conduct Part 1 of the Phase IIb/III trial for DR and the parties will share the development expenses related to Part 2 of the Phase IIb/III trial and the subsequent Phase III trial for DR in accordance with the existing terms of the AbbVie Collaboration Agreement. AbbVie will be responsible for all development expenses related to the ACHIEVE Study.

The Company applied the requirements of 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, 2025 and December 31, 2024, 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, 2025 and 2024.

The Company applied the requirements of 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, 2025 and December 31, 2024, the Company had recorded $17.0 million and $11.3 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,	
	2025	2024	2025	2024
Net cost reimbursement to (from) AbbVie included in:				
Research and development expense	$ (17,742)	$ (20,082)	$ (49,479)	$ (66,330)
General and administrative expense	712	341	1,860	1,168
Total net cost reimbursement to (from) AbbVie	$ (17,030)	$ (19,741)	$ (47,619)	$ (65,162)

Nippon Shinyaku Collaboration and License Agreement

In January 2025, the Company entered into a collaboration and license agreement with Nippon Shinyaku for the development and commercialization of RGX-121, the Company's product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), and RGX-111, the Company's product candidate for the treatment of Mucopolysaccharidosis Type I (MPS I) (the Nippon Shinyaku Collaboration Agreement). The Nippon Shinyaku Collaboration Agreement became effective in March 2025.

Pursuant to the Nippon Shinyaku Collaboration Agreement, the Company granted Nippon Shinyaku a license to develop and exclusively commercialize RGX-121 and RGX-111 in the United States and certain countries in Asia. The Company is responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. The Company is responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement and mutually agreed supply agreements. Nippon Shinyaku will be responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. The Company reserves the right to develop and commercialize RGX-121 and RGX-111 in countries outside the licensed territories. The Nippon Shinyaku Collaboration Agreement contains provisions for termination, including termination for convenience by Nippon Shinyaku.

In consideration for the rights granted and services to be performed under the Nippon Shinyaku Collaboration Agreement, Nippon Shinyaku paid the Company an up-front fee of $110.0 million upon the effective date of the agreement in March 2025 and is required to pay to the Company up to $700.0 million upon the achievement of specified development and sales-based milestones, of which $40.0 million are based on development milestones and $660.0 million are sales-based milestones. Nippon Shinyaku is also required to pay to the Company double-digit royalties on net sales of RGX-121 and RGX-111 in the licensed territories, subject to specified offsets and reductions. The Company retains all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111.

The Company evaluated its various commitments under the Nippon Shinyaku Collaboration Agreement and identified the distinct units of account under the arrangement. For each of the distinct units of account identified, the Company determined whether the transactions should be accounted for as a contract with a customer within the scope of ASC 606 or as a collaborative arrangement within the scope of ASC 808. The Company concluded that each of the distinct units of account identified should be accounted for as revenue under ASC 606, as Nippon Shinyaku is deemed to be a customer for each of the various transactions. The Company identified the following material performance obligations under the agreement: (i) delivery of intellectual property licenses to develop and commercialize RGX-121 and RGX-111 in the United States and Asia territories, (ii) development services for RGX-121 and RGX-111 in the Unites States, including manufacturing of clinical supply and commercial supply prior to regulatory approval, and (iii) material rights granted to Nippon Shinyaku to purchase commercial supply for sales in licensed territories.

The intellectual property licensed to Nippon Shinyaku 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. In determining the distinct performance obligations under the agreements, the Company concluded that the licenses granted to Nippon Shinyaku to develop and commercialize RGX-121 and RGX-111 are distinct from the other goods and services promised under the agreement, as Nippon Shinyaku can benefit from the license on a standalone basis and, based on the stage of development of the product candidates, the underlying licensed products and know-how are not expected to be significantly modified as a result of other goods and services promised under the agreement. The Company evaluated all options granted to Nippon Shinyaku under the agreement to determine whether the options represent material rights. Management concluded the options to purchase commercial supply convey material rights granted to Nippon Shinyaku, and therefore are accounted for as separate performance obligations under the current arrangement. The Company identified various promises under the Nippon Shinyaku Collaboration Agreement which were determined to be immaterial in the context of the contract and will not be accounted for as separate performance obligations.

As of September 30, 2025, the transaction price of the Nippon Shinyaku Collaboration Agreement included fixed consideration of $110.0 million for the up-front payment and variable consideration of $5.3 million for estimated reimbursable manufacturing costs which are deemed not to be constrained. Variable consideration which has been excluded from the transaction price includes $40.0 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement, and other reimbursable manufacturing costs which are contingent on events occurring that are outside the Company's control. The transaction price also excludes sales-based milestone payments of $660.0 million and royalties on net sales of RGX-121 and RGX-111 in the United States and Asia territories. Development milestones will be added to the transaction price upon achievement, or if deemed probable of achievement, and other variable consideration may be added to the transaction price in the future as uncertainties regarding payment of the consideration are resolved. 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 fixed transaction price of the Nippon Shinyaku Collaboration Agreement between the effective date of the agreement and September 30, 2025.

The fixed transaction price of the Nippon Shinyaku Collaboration Agreement of $110.0 million was allocated to the various performance obligations based on their relative standalone selling prices, which requires significant judgment. The selling prices of the intellectual property licenses were determined based on discounted cash flow models for each of the licensed products in the respective licensed territories and were adjusted for the probability of developmental, regulatory and commercial success. Significant

assumptions and judgments were required to estimate the future cash flows, discount rates and probabilities of success for each of the licensed products and territories. The selling prices of development services and commercial supply were determined based on the expected cost plus a reasonable margin. The selling prices of the material rights to purchase commercial supply were determined based on the incremental discount to the standalone selling prices of the commercial supply and were adjusted to take into account the likelihood of exercise of the material rights. Significant assumptions and judgments were required to estimate the future costs of development and manufacturing, an appropriate margin for such services, and the likelihood of exercise of the material rights to purchase commercial supply based on anticipated demand and risks. The $5.3 million of variable consideration included in the transaction price is allocated directly to performance obligations for development services related to the manufacturing of commercial supply prior to regulatory approval since the consideration is directly associated with reimbursement of such costs. Consideration contingent upon the future exercise of options to purchase commercial supply is excluded from the transaction price until exercised.

The portion of the $110.0 million fixed transaction price allocated to the delivery of the intellectual property licenses was recognized as license and royalty revenue upon the delivery of the license to Nippon Shinyaku in March 2025. The portion of the fixed transaction price allocated to development services will be recognized as service revenue as the services are performed using an input method based on costs incurred versus total estimated costs to perform the services, which is re-assessed at each reporting date. The portion of the fixed transaction price allocated to material rights to purchase commercial supply will be recognized as revenue proportionally with the total expected commercial supply revenue expected to be recognized under the arrangement, which is re-assessed at each reporting date. Commercial supply revenue will be recognized as revenue upon delivery to Nippon Shinyaku, or otherwise upon transfer of control to Nippon Shinyaku as defined in the associated supply agreements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
License and royalty revenue	$ —	$ —	$ 69,979	$ —
Service revenue	5,932	—	10,426	—
Total revenues	$ 5,932	$ —	$ 80,405	$ —

As of September 30, 2025, the Company had recorded total accounts receivable of $4.8 million for reimbursement of manufacturing-related development costs and other services under the Nippon Shinyaku Collaboration Agreement, of which $2.5 million was included in current assets and $2.3 million was included in non-current assets. As of September 30, 2025, the Company had recorded total deferred revenue of $34.4 million for development services and material rights which have not yet been satisfied under the Nippon Shinyaku Collaboration Agreement, of which $13.2 million was included in current liabilities and $21.2 million was included in non-current liabilities.

11. Stock-based Compensation

Effective in January 2025, an additional 1,981,975 shares were authorized for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). The 2015 Plan expired in June 2025, upon which no further awards may be issued under the plan. Any awards outstanding under the 2015 Plan as of its expiration shall remain outstanding and effective pursuant to the contractual terms of the awards. Additionally, as of September 30, 2025, no awards remained outstanding or available for issuance under the 2014 Stock Plan (the 2014 Plan), the predecessor plan to the 2015 Plan.

In May 2025, the Company adopted the 2025 Equity Incentive Plan (the 2025 Plan), which replaced the 2015 Plan upon its expiration in June 2025. The total number of shares of common stock authorized for issuance under the 2025 Plan upon its adoption was 5,500,000. The number of shares authorized for issuance under the 2025 Plan shall automatically increase for any shares of common stock underlying awards outstanding under the 2015 Plan, as of the adoption date of the 2025 Plan, which are not issued due to forfeiture, expiration, termination or cancellation of the award. Shares of common stock that are withheld, tendered, or otherwise not issued in connection with the settlement of awards outstanding under the 2015 Plan do not increase the number of shares authorized for issuance under the 2025 Plan. As of September 30, 2025, the total number of shares of common stock reserved for issuance under the 2025 Plan and the 2015 Plan was 20,167,367, of which 5,537,889 remained available for future grants under the 2025 Plan.

The 2025 Plan and 2015 Plan provide for the issuance of stock options, stock appreciation rights, restricted and unrestricted stock and unit awards, and performance cash awards to employees, members of the Board of Directors and consultants of the Company. Stock options generally expire 10 years following the date of grant. Options typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Stock options have an exercise price at least equal to the estimated fair value of the Company's common stock on the date of grant. Restricted stock units typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Upon vesting, restricted stock units are settled in common stock of the Company. Awards granted under the 2025 Plan generally have a minimum vesting requirement of one year from the grant date.

Shares of common stock underlying awards granted under the 2025 Plan which are not issued due to forfeiture, expiration, termination or cancellation of the award are added to the number of shares of common stock available for issuance under the 2025 Plan, except for shares that are withheld, tendered or otherwise not issued in connection with the settlement of the award. Shares available for issuance under the 2025 Plan and 2015 Plan may be either authorized but unissued shares of the Company's common stock or common stock reacquired by the Company and held in treasury. The 2025 Plan expires in May 2035, 10 years from its adoption date, unless earlier terminated.

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,	
	2025	2024	2025	2024
Stock options	$ 4,777	$ 6,051	$ 14,262	$ 17,969
Restricted stock units	4,051	3,755	11,432	10,536
Employee stock purchase plan	170	78	494	416
	$ 8,998	$ 9,884	$ 26,188	$ 28,921

As of September 30, 2025, the Company had $57.2 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 2.3 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,	
	2025	2024	2025	2024
Research and development	$ 4,245	$ 4,301	$ 12,181	$ 13,893
General and administrative	4,753	5,583	14,007	15,028
	$ 8,998	$ 9,884	$ 26,188	$ 28,921

Stock Options

The following table summarizes stock option activity under the Company's equity incentive plans (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, 2024	9,994	$ 29.48	5.9	$ 240
Granted	2,441	$ 8.02		
Exercised	(61)	$ 3.76		
Cancelled or forfeited	(648)	$ 28.73		
Outstanding at September 30, 2025	11,726	$ 25.19	6.3	$ 3,961
Exercisable at September 30, 2025	7,693	$ 31.89	5.0	$ 125
Vested and expected to vest at September 30, 2025	11,726	$ 25.19	6.3	$ 3,961

(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, 2025 was $4.96. During the nine months ended September 30, 2025, the total number of stock options exercised was 60,554, resulting in total proceeds of $0.2 million. The total intrinsic value of options exercised during the nine months ended September 30, 2025 was $0.3 million.

Restricted Stock Units

The following table summarizes restricted stock unit activity under the Company's equity incentive plans (in thousands, except per share data):

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2024	2,042	$ 19.95
Granted	1,696	$ 8.56
Vested	(732)	$ 21.34
Forfeited	(102)	$ 15.99
Unvested balance at September 30, 2025	2,904	$ 13.09

The total intrinsic value of restricted stock units vested during the nine months ended September 30, 2025 was $5.9 million.

Employee Stock Purchase Plan

As of September 30, 2025, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 755,306 remained available for future issuance. During the nine months ended September 30, 2025, 157,658 shares of common stock were issued under the 2015 ESPP.

12. Income Taxes

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of September 30, 2025 and December 31, 2024. 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, 2025 and December 31, 2024.

In July 2025, the United States enacted tax reform legislation through the One Big Beautiful Bill Act (OBBBA). The OBBBA amends U.S. tax laws, including extending or making permanent certain provisions of the Tax Cuts and Jobs Act of 2017 and repealing certain clean energy initiatives, in addition to other changes. The Company expects changes to various gross deferred tax assets and liabilities as a result of the enactment of the OBBBA, including changes to its deferred tax assets for net operating loss carryforwards and capitalized research and development costs. However, due to projected taxable losses and a full valuation allowance against its deferred tax assets, the Company expects no material impact to its income tax provision and net deferred tax assets for the year ended December 31, 2025 as a result of the enactment, and there was no impact to the Company's income tax provision for the three and nine months ended September 30, 2025. The Company continues to evaluate the impact the OBBBA will have on its consolidated financial statements in future periods.

13. Net Loss Per Share

Since the Company incurred net losses for the three and nine months ended September 30, 2025 and 2024, 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,	
	2025	2024
Stock options issued and outstanding	11,726	10,017
Unvested restricted stock units outstanding	2,904	2,035
Employee stock purchase plan	60	47
May 2025 Warrants outstanding	268	—
	14,958	12,099

14. Segment Information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker (CODM), or decision-making group, in making decisions on how to allocate resources and assess performance. The Company's CODM, its Chief Executive Officer, views the Company's operations and manages the business as one operating segment focused on the development and commercialization of gene therapies to treat an array of diseases. The determination of a single operating segment is consistent with the consolidated financial information regularly provided to the CODM

The CODM reviews and evaluates consolidated net income (loss) for purposes of assessing performance, making operating decisions and allocating resources. The CODM uses net income (loss) to assess performance versus operating budgets and in the preparation of near-term and long-range operating plans to inform decisions on resource and capital allocation. The CODM reviews consolidated cash, cash equivalents and marketable securities as a measure of segment assets. As of September 30, 2025 and December 31, 2024, the Company's cash, cash equivalents and marketable securities were $302.0 million and $244.9 million, respectively.

The following table presents information about the Company's segment revenues, significant segment expenses regularly provided to the CODM, other segment items and consolidated net income (loss) (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenues	$ 29,733	$ 24,197	$ 140,104	$ 62,114
Less:				
Cost of license and royalty revenues	5,725	12,387	14,370	27,249
Research and development expense				
Personnel	18,464	16,367	55,153	49,467
Direct development and support (a)	26,800	27,306	81,860	74,664
Facilities	2,946	2,742	8,467	8,672
Stock-based compensation	4,245	4,301	12,181	13,893
Depreciation and amortization	3,646	3,713	11,027	11,446
Total research and development expense	56,101	54,429	168,688	158,142
General and administrative expense				
Personnel	5,567	5,512	17,055	15,905
Other general and administrative (b)	8,331	6,839	24,751	21,301
Facilities	1,362	1,240	3,951	3,588
Stock-based compensation	4,753	5,583	14,007	15,028
Depreciation and amortization	240	248	719	746
Total general and administrative expense	20,253	19,422	60,483	56,568
Other segment items (c)	(9,595)	2,444	(23,292)	3,929
Net loss	$ (61,941)	$ (59,597)	$ (126,729)	$ (175,916)

(a)Direct development and support includes external goods and services for the development of product candidates and early-stage research activities, laboratory costs, consulting, development cost reimbursement to and from collaborators and other expenses in support of research and development activities.

(b)Other general and administrative expenses include professional and administrative services, consulting, commercial cost reimbursement to and from collaborators and other corporate overhead expenses.

(c)Other segment items include impairment of long-lived assets, other operating expenses, interest income from licensing, investment income and interest expense.

The Company’s interest income included interest income from licensing as presented in the consolidated statements of operations and comprehensive loss, as well as interest income from investments of $3.6 million and $9.4 million during the three and nine months ended September 30, 2025, respectively, and $3.3 million and $9.2 million during the three and nine months ended September 30, 2024, respectively, which is included within investment income in the consolidated statements of operations and comprehensive loss.

The substantial majority of the Company’s assets reside in the United States.

15. Supplemental Disclosures

Other Current Assets

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

	September 30, 2025	December 31, 2024
Net cost reimbursement due from AbbVie	$ 17,031	$ 11,304
Accrued interest on investments	1,120	1,094
Other	1,321	1,376
	$ 19,472	$ 13,774

Accrued Expenses and Other Current Liabilities

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

	September 30, 2025	December 31, 2024
Accrued personnel costs	$ 15,076	$ 17,607
Accrued external research and development expenses	11,089	8,998
Accrued sublicense fees and royalties	9,080	8,658
Accrued external general and administrative expenses	2,064	2,002
Accrued purchases of property and equipment	78	156
Other	929	649
	$ 38,316	$ 38,070

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 $0.2 million as of September 30, 2025, a net decrease of $0.2 million from December 31, 2024, and $0.4 million as of September 30, 2024, a net decrease of less than $0.1 million from December 31, 2023.

During the nine months ended September 30, 2025, accrued interest of $8.0 million was converted to principal balance under the 2025 Royalty Bond.

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, 2024, which we filed with the SEC on March 13, 2025. 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, 2024 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 (surabgene lomparvovec, sura-vec) in collaboration with AbbVie as a potential one-time treatment for chronic retinal conditions that 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 clinical trials, including two pivotal trials (ATMOSPHERE and ASCENT), one Phase II bridging study, one long-term follow-up study and a fellow eye sub-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 in the ATMOSPHERE® and ASCENT™ pivotal trials for the treatment of patients with wet AMD using subretinal delivery was completed in October 2025. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Topline data from these trials are expected to be shared in the fourth quarter of 2026.

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.

In January 2024, we announced data from the AAVIATE trial demonstrating ABBV-RGX-314 suprachoroidal delivery was well tolerated across 106 patients with no drug-related serious adverse events (SAEs) were reported. Patients treated with ABBV-RGX-314 continued to demonstrate stable best corrected visual acuity (BCVA) and central retinal thickness (CRT) at six months. In addition, a meaningful reduction in anti-VEGF treatment burden was observed following administration of ABBV-RGX-314. The highest reduction was seen in dose level 3, demonstrating an 80% reduction in annualized injection rate with 50% of patients remaining injection-free.

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 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 continues to enroll 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. In November 2023, we announced data showing ABBV-RGX-314 was well tolerated at dose levels 1 and 2 and positive signals of efficacy, including 20.8% of patients exhibiting >2-step Diabetic Retinopathy Severity Scale (DRSS) improvement without additional DR treatment at one year.

In August 2025, we announced new data from the ALTITUDE trial and plans to initiate a pivotal program. New ALTITUDE trial data demonstrate a durable safety and efficacy profile observed in patients with non-proliferative DR through two years with a single, in-office injection. As of June 9, 2025, ABBV-RGX-314 was well tolerated at dose levels 1, 2 and 3, with no drug-related SAEs. No intraocular inflammation was observed through two years at dose level 3 (1.0x10e12 GC/eye) (n=15) with short-course topical prophylactic steroids. In August 2025, we and AbbVie executed an amendment to our collaboration agreement and announced plans to initiate a pivotal two-part sham injection-controlled Phase IIb/III trial, with the primary endpoint being ≥2-step DRSS improvement at one year. Site selection for the Phase IIb/III trial is in progress.

The ALTITUDE trial includes a new cohort of patients with center-involved DME evaluating ABBV-RGX-314 at dose level 4. Enrollment completed in this cohort in June 2025. 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 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 domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne. Other differentiating elements of RGX-202 include the proactive immune suppression regimen and in-house, state-of-the-art manufacturing that has demonstrated leading purity levels in Duchenne (>80% full capsids).

AFFINITY DUCHENNE® is a multicenter, open-label Phase I/II/III trial to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous dose of RGX-202 in patients with Duchenne aged one and older. The initiation of the pivotal study, which was designed to enroll approximately 30 patients in the U.S. and Canada, as well as positive interim safety and efficacy data from the Phase I/II portion of the study were announced in November 2024. These data included positive biomarker data from the first nine patients, which demonstrated consistent, robust microdystrophin and transduction, as well as positive initial functional data. Subsequent findings were presented in March 2025 at the 2025 Muscular Dystrophy Association Clinical & Scientific Conference, in June 2025 via a Company webcast and in October 2025 at the International Congress of the World Muscle Society. In sum, these data were positive and demonstrate potential for RGX-202 to serve as a differentiated gene therapy for Duchenne. As of May 2025, we had reported positive microdystrophin data on 12 patients and positive initial functional data from five patients. We also reported a favorable safety profile with no serious adverse events or adverse events of special interest observed (n=13).

In October 2025, we announced that enrollment in the AFFINITY DUCHENNE pivotal trial had completed and that we continue to enroll participants in the planned confirmatory trial. We also announced that the first batches of RGX-202 intended for commercial supply have been manufactured at our Manufacturing Innovation Center and that we expect to imminently complete the Process Performance Qualification (PPQ) campaign. We expect to share topline data in early second quarter 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026.

We are also recruiting patients in the AFFINITY BEYOND® trial, an observational screening study. The primary objective is to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

•**RGX-121:** We are developing RGX-121 (clemidsogene lanparvovec) in collaboration with Nippon Shinyaku in the United States and certain countries in Asia 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.

In February 2024, we announced that, in the pivotal phase of the Phase I/II/III CAMPSIITE® trial, RGX-121 achieved its primary endpoint, a reduction in cerebrospinal fluid Heparan sulfate (HS) 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 HS D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit for accelerated approval.

A BLA for RGX-121 seeking accelerated approval was submitted to the FDA in March 2025. The FDA subsequently granted priority review of the BLA and successfully completed mid-cycle meeting, Pre-license inspection (PLI) and Bioresearch monitoring information (BIMO) inspections. The PLI and BIMO inspections were completed with no observations. In August 2025, we announced that the FDA review timeline has been extended following submission of 12-month clinical data for all patients in the pivotal study of RGX-121 (n=13) in response to an FDA information request. The Prescription Drug User Fee Act (PDUFA) goal date has been extended from November 9, 2025 to February 8, 2026.

The longer-term data submitted to the FDA were presented at the International Congress of Inborn Errors of Metabolism (ICIEM) in September 2025. These results showed that in the pivotal phase of the CAMPSIITE trial (n=13), participants through one year sustained an 82% median reduction of cerebrospinal fluid (CSF) levels of HS D2S6. These longer-term data were consistent with previously reported topline pivotal results from the CAMPSIITE trial. Potential approval of the BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher (PRV), assuming the statutory criteria are met. If approved, RGX-121 would be the first approved gene therapy and one-time treatment for MPS II.

•**RGX-111:** We are developing RGX-111 in collaboration with Nippon Shinyaku in the United States and certain countries in Asia as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type I (MPS I), also known as Hurler syndrome, using the NAV AAV9 vector to deliver the IDUA gene.

In November 2023, future development of RGX-111 was halted as a result of a strategic pipeline prioritization and corporate restructuring. Prior to that announcement, RGX-111 demonstrated to be well tolerated and indicated encouraging biomarker and neurodevelopmental results in a Phase I/II study. Efforts to continue development of RGX-111 as part of the strategic partnership with Nippon Shinyaku are ongoing.

AbbVie Collaboration for ABBV-RGX-314

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 (as amended, the AbbVie Collaboration Agreement). Pursuant to the AbbVie Collaboration Agreement, both we and AbbVie are active participants in the development of ABBV-RGX-314 and development expenses are shared between the parties in accordance with the agreement. The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the global commercialization of ABBV-RGX-314. We received an up-front fee of $370.0 million from AbbVie upon the effective date of the AbbVie Collaboration Agreement in November 2021, and we are eligible to receive up to $1.38 billion from AbbVie upon the achievement of specified development and sales-based milestones. Additionally, the parties will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States, and we are eligible to receive tiered royalties on net sales by AbbVie of ABBV-RGX-314 outside the United States. For additional information regarding the AbbVie Collaboration Agreement, please refer to Note 10, “License and Collaboration Agreements—AbbVie Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

In August 2025, we and AbbVie entered into an amendment to the AbbVie Collaboration Agreement which modified the development plan and milestone payment structure for the ABBV-RGX-314 DR program. Under the amendment, we will conduct the first registration enabling trial for DR suprachoroidal (SCS) treatment as a combined Phase IIb/III trial performed in two parts (Part 1 and Part 2), and AbbVie will conduct the second registration enabling trial as a separate, standalone Phase III trial. In lieu of the $200.0 million milestone due to us under the original AbbVie Collaboration Agreement upon first patient dosed in the first registration enabling trial for DR SCS treatment, AbbVie will pay us $100.0 million upon first patient dosed in the Phase IIb/III trial for DR SCS treatment and an additional $100.0 million upon first patient dosed in the subsequent Phase III trial. Also pursuant to the amendment, AbbVie will lead a new Phase III randomized controlled study (the ACHIEVE Study) to assess the injection burden, adverse events,

change in disease activity, and long-term preservation of visual acuity of ABBV-RGX-314 in adult participants with neovascular AMD. We will be responsible for our development expenses to conduct Part 1 of the Phase IIb/III trial for DR and the parties will share the development expenses related to Part 2 of the Phase IIb/III trial and the subsequent Phase III trial for DR in accordance with the existing terms of the AbbVie Collaboration Agreement. AbbVie will be responsible for all development expenses related to the ACHIEVE Study.

Nippon Shinyaku Collaboration for RGX-121 and RGX-111

In January 2025, we entered into a collaboration and license agreement with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) for the development and commercialization of RGX-121 and RGX-111 (the Nippon Shinyaku Collaboration Agreement). Pursuant to the Nippon Shinyaku Collaboration Agreement, we are responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. We are responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement. Nippon Shinyaku is responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. Under the terms of the Nippon Shinyaku Collaboration Agreement, we received an up-front payment of $110.0 million from Nippon Shinyaku following the effective date of the agreement in March 2025 and are eligible to receive up to $700.0 million from Nippon Shinyaku upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions. We retain all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111.

We recognized $80.4 million of revenue under the Nippon Shinyaku Collaboration Agreement during the nine months ended September 30, 2025. For additional information regarding the agreement with Nippon Shinyaku, please refer to Note 10, “License and Collaboration Agreements—Nippon Shinyaku Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

In May 2025, we entered into a loan agreement with entities managed by Healthcare Royalty Management, LLC (collectively and with other affiliated entities, HCR). Pursuant to the terms of the loan agreement, future royalties, sales-based milestone payments and certain development milestone payments earned under the Nippon Shinyaku Collaboration Agreement, along with consideration earned under various other NAV Technology Platform license agreements, shall be used to repay principal and interest owed to HCR. For additional information regarding the May 2025 loan agreement with HCR, please refer to Note 7, “Royalty Monetization Liabilities—2025 Royalty Bond” to the accompanying unaudited consolidated financial statements.

NAV Technology Licensing 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, 2025, 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 additional revenue opportunities.

Financial Overview

Revenues

Our revenues to date have been primarily generated from the licensing of our NAV Technology Platform and other intellectual property rights to NAV Technology Licensees and collaborators. 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 to the Company under our license and collaboration 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 and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

Future revenues under our license and collaboration arrangements 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 under our license or collaboration agreements that is contemplated on optional goods and services, development and sales-based milestones, 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 collaborators and the arrangements are terminable at the option of the counterparty. The termination of our license and collaborations arrangements 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. (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.

Operating Expenses

Our operating expenses consist primarily of cost of license and royalty 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 License and Royalty Revenues

Our cost of license and royalty 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 license and royalty 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, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024*	2025	2024*
Direct Expenses				
ABBV-RGX-314	$ 6,068	$ 11,197	$ 27,322	$ 29,824
RGX-202	6,908	3,929	16,345	9,258
RGX-121	4,678	4,050	11,015	11,885
Other product candidates	1,183	1,962	4,484	5,397
Total direct expenses	18,837	21,138	59,166	56,364
Unallocated Expenses				
Platform and early research	7,963	6,168	22,694	18,300
Personnel	18,464	16,367	55,153	49,467
Facilities	2,946	2,742	8,467	8,672
Stock-based compensation	4,245	4,301	12,181	13,893
Depreciation and amortization	3,646	3,713	11,027	11,446
Total unallocated expenses	37,264	33,291	109,522	101,778
Total research and development	$ 56,101	$ 54,429	$ 168,688	$ 158,142

* Certain amounts reported in prior years have been reclassified to conform to the current year's presentation.

Direct expenses related to the development of ABBV-RGX-314 include $17.7 million and $49.5 million for the three and nine months ended September 30, 2025, respectively, and $20.1 million and $66.3 million for the three and nine months ended September 30, 2024, respectively, in net cost reimbursement from AbbVie under our eye care collaboration, which were recorded as a reduction of research and development expenses. In addition to reimbursement of direct 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 early research reported in the table above includes direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform used across programs, process and analytical development, early research and development for prospective product candidates and new technologies, and other costs in support of research and development activities.

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. Specifically, we expect general and administrative costs associated with the potential commercialization of our product candidates to increase in future periods as we and/or our commercial partners prepare for and carry out product launch efforts, in particular for the potential commercialization of our RGX-202 and ABBV-RGX-314 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.

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 is primarily associated with our royalty monetization liabilities, including our December 2020 Zolgensma royalty purchase agreement (2020 Royalty Purchase Agreement) and May 2025 loan agreement (2025 Royalty Bond) with HCR. For further information regarding our royalty monetization liabilities and associated interest expense, please refer to Note 7, "Royalty Monetization Liabilities" 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, 2024. Other than the accounting policies described below, there have been no significant changes in our critical accounting policies and estimates since December 31, 2024.

Revenue Recognition

We recognize revenue in accordance with Accounting Standards Codification (ASC) 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of ASC 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of ASC 606 only when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, for contracts within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an interest rate which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

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 payable to us under our license and collaboration 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 and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

We evaluate our agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). For collaboration arrangements within the scope of ASC 808 that contain multiple elements, we identify the various transactions with the counterparty and determine if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, we apply the five-step model as described in our revenue recognition policies.

Our license and collaboration agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each agreement, we determine the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to us. For each license granted, we evaluate these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options.

Performance obligations under our license and collaboration agreements may include (i) the delivery of intellectual property licenses, (ii) development and manufacturing services to be performed by us related to licensed products and (iii) options granted to purchase additional goods and services, to the extent the options convey material rights. At the inception of each license agreement which contains performance obligations for development or other services, we evaluate whether the license is distinct from the services, which requires judgment. In making this determination, we consider, among other things, the stage of development of the licensed products and whether the services will significantly impact further development of the licensed products. If it is determined that the license is not distinct from the services, the license is combined with the services into a single performance obligation. Agreements may provide licensees and collaborators with options to purchase additional goods or other services, including options to purchase commercial supply of licensed products. Options are evaluated at the inception of the agreement to determine whether they provide material rights to the customer. In making this determination, we consider whether the options are priced at an incremental discount to the standalone selling price of the underlying goods or services, in which case the option is considered to be a material right. Material rights are accounted for as separate performance obligations under the current arrangement.

We evaluate the transaction price of our license and collaboration agreements at contract inception and at each reporting date. The transaction price includes the fixed consideration payable to us over the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the agreements may include up-front and annual fees payable to us over the contract term and fixed fees for development and other services. Variable

consideration under the agreements may include development and sales-based milestone payments, payments for development and other services, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by the customer is excluded from the transaction price and not accounted for as part of the arrangement until the option is exercised.

The transaction price of our license and collaboration arrangements is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Variable consideration payable based on services performed is allocated directly to the performance obligation for such services. Consideration allocated to performance obligations for the delivery of intellectual property licenses is recognized as license and royalty revenue in full upon the delivery of the license. Consideration allocated to performance obligations for development and manufacturing services is recognized as service revenue as we perform the services. Consideration allocated to performance obligations for material rights to purchase additional goods and services is recognized as revenue upon the satisfaction of the performance obligations underlying the optional goods and services purchased by the customer. Service revenue is recognized using a measure of progress that best reflects the pattern of satisfaction of the performance obligations. At each reporting date, we re-evaluate the measure of progress and adjust service revenue on a cumulative catch-up basis to reflect our best estimate of the services performed to date versus the total services to be performed under the arrangement.

Development milestone payments are payable to us upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price. Milestone payments contingent on the achievement of development milestones that are not within our control or the control of the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of each outstanding development milestone and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. The increase to the transaction price as a result of any such adjustments is then allocated to the underlying performance obligations in a manner similar to the allocation of the initial transaction price and, to the extent the performance obligations are satisfied, recognized as revenue on a cumulative catch-up basis in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as license and royalty revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA. We recognize 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 us to estimate royalty revenue for periods of net sales which have not yet been reported to us. 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.

We receive payments from licensees and collaborators based on the billing schedules established in the associated agreements. Amounts recognized as revenue which have not yet been received from the customer are recorded as accounts receivable when our rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from customers are recorded as contract assets when our rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets if the consideration is expected to be realized within 12 months from the reporting date, or as other assets if the consideration is expected to be realized in periods beyond 12 months from the reporting date. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of revenue in the period of the termination. Amounts received by us prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Results of Operations

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Revenues						
License and royalty revenue	$ 23,605	$ 23,982	$ (377)	$ 129,119	$ 61,172	$ 67,947
Service revenue	6,128	215	5,913	10,985	942	10,043
Total revenues	29,733	24,197	5,536	140,104	62,114	77,990
Operating Expenses						
Cost of license and royalty revenues	5,725	12,387	(6,662)	14,370	27,249	(12,879)
Research and development	56,101	54,429	1,672	168,688	158,142	10,546
General and administrative	20,253	19,422	831	60,483	56,568	3,915
Impairment of long-lived assets	—	—	—	—	2,101	(2,101)
Other operating expenses	65	37	28	125	32	93
Total operating expenses	82,144	86,275	(4,131)	243,666	244,092	(426)
Loss from operations	(52,411)	(62,078)	9,667	(103,562)	(181,978)	78,416
Other Income (Expense)						
Interest income from licensing	19	25	(6)	65	91	(26)
Investment income	3,620	3,276	344	9,500	9,213	287
Interest expense	(13,169)	(820)	(12,349)	(32,732)	(3,242)	(29,490)
Total other income (expense)	(9,530)	2,481	(12,011)	(23,167)	6,062	(29,229)
Net loss	$ (61,941)	$ (59,597)	$ (2,344)	$ (126,729)	$ (175,916)	$ 49,187

Comparison of the Three Months Ended September 30, 2025 and 2024

License and Royalty Revenue. License and royalty revenue decreased by $0.4 million, from $24.0 million for the three months ended September 30, 2024 to $23.6 million for the three months ended September 30, 2025. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased by $0.3 million, from $23.9 million for the third quarter of 2024 to $23.6 million for the third quarter of 2025. Novartis reported Zolgensma sales of $301 million for the third quarter of 2025, a decrease of 2% from the third quarter of 2024, driven by lower incidence of SMA during the period.

Service Revenue. Service revenue increased by $5.9 million, from $0.2 million for the three months ended September 30, 2024 to $6.1 million for the three months ended September 30, 2025. The increase was primarily attributable to $5.9 million of development service revenue recognized under our collaboration with Nippon Shinyaku in the third quarter of 2025.

Research and Development Expense. Research and development expenses increased by $1.7 million, from $54.4 million for the three months ended September 30, 2024 to $56.1 million for the three months ended September 30, 2025. The increase was primarily attributable to the following:

- an increase of $2.3 million in personnel-related costs due to increased headcount of development personnel, net of a $0.1 million decrease in stock-based compensation expense;
- an increase of $1.9 million in manufacturing-related expenses and other clinical supply costs for our lead product candidates, largely driven by manufacturing of RGX-121; and
- an increase of $0.5 million in preclinical activities and other early-stage research and development.

The increase in research and development expenses was partially offset by a decrease of $3.7 million in costs associated with clinical trials and regulatory activities, largely driven by a decrease in clinical trial expenses for ABBV-RGX-314 pivotal trials.

General and Administrative Expense. General and administrative expenses increased by $0.8 million, from $19.4 million for the three months ended September 30, 2024 to $20.3 million for the three months ended September 30, 2025. The increase was largely driven by professional services, consulting and other corporate advisory services.

Interest Expense. Interest expense increased by $12.3 million, from $0.8 million for the three months ended September 30, 2024 to $13.2 million for the three months ended September 30, 2025. The increase was primarily attributable to interest expense under our royalty monetization liabilities, driven largely by an increase in forecasted Zolgensma royalties expected to be paid to HCR under the 2020 Royalty Purchase Agreement and interest expense incurred to date under the 2025 Royalty Bond issued in May 2025.

Comparison of the Nine Months Ended September 30, 2025 and 2024

License and Royalty Revenue. License and royalty revenue increased by $67.9 million, from $61.2 million for the nine months ended September 30, 2024 to $129.1 million for the nine months ended September 30, 2025. The increase was primarily attributable to $70.0 million of up-front license revenue recognized under our collaboration with Nippon Shinyaku in the first quarter of 2025.

Service Revenue. Service revenue increased by $10.0 million, from $0.9 million for the nine months ended September 30, 2024 to $11.0 million for the nine months ended September 30, 2025. The increase was primarily attributable to $10.4 million of development service revenue recognized under our collaboration with Nippon Shinyaku in the first nine months of 2025.

Research and Development Expense. Research and development expenses increased by $10.5 million, from $158.1 million for the nine months ended September 30, 2024 to $168.7 million for the nine months ended September 30, 2025. The increase was primarily attributable to the following:

- an increase of $10.9 million in manufacturing-related expenses and other clinical supply costs for our lead product candidates, largely driven by ABBV-RGX-314 clinical supply and purchases of raw materials; and
- an increase of $4.2 million in personnel-related costs due to increased headcount of development personnel, net of a $1.7 million decrease in stock-based compensation expense.

The increase in research and development expenses was partially offset by a decrease of $4.6 million in costs associated with clinical trials and regulatory activities, largely driven by a decrease in clinical trial expenses for ABBV-RGX-314 and RGX-121 pivotal trials.

General and Administrative Expense. General and administrative expenses increased by $3.9 million, from $56.6 million for the nine months ended September 30, 2024 to $60.5 million for the nine months ended September 30, 2025. The increase was largely driven by professional services, consulting and other corporate advisory services.

Interest Expense. Interest expense increased by $29.5 million, from $3.2 million for the nine months ended September 30, 2024 to $32.7 million for the nine months ended September 30, 2025. The increase was primarily attributable to interest expense under our royalty monetization liabilities, driven largely by an increase in forecasted Zolgensma royalties expected to be paid to HCR under the 2020 Royalty Purchase Agreement and interest expense incurred to date under the 2025 Royalty Bond issued in May 2025.

Liquidity and Capital Resources

Sources of Liquidity

As of September 30, 2025, we had cash, cash equivalents and marketable securities of $302.0 million, which were primarily derived from the royalty monetization in May 2025 and the up-front payment received under the Nippon Shinyaku Collaboration Agreement in March 2025, each as described below, and the sale of our common stock and pre-funded warrants in March 2024. We expect that our cash, cash equivalents and marketable securities as of September 30, 2025 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 May 2025, we entered into a loan agreement with HCR pursuant to which HCR will provide us with an aggregate limited recourse loan of up to $250.0 million (the 2025 Royalty Bond). The 2025 Royalty Bond is disbursable to us in three tranches, with $150.0 million funded on the closing date in May 2025, $50.0 million available to be funded if sales of a specified product exceed a specified sales threshold prior to December 31, 2026, and $50.0 million available to be funded if both parties exercise an option in 2027. Proceeds received from the initial funding tranche of the 2025 Royalty Bond in May 2025, net of discounts and transaction costs, were $144.5 million. The 2025 Royalty Bond matures in 2035, subject to potential extension, and bears interest at a rate of 9.75% plus the 3-month secured overnight financing rate as administered by the Federal Reserve Bank of New York (SOFR), with a minimum interest rate of 14.0%. Prior to the maturity date, interest and principal under the 2025 Royalty Bond shall be paid quarterly to HCR solely using proceeds received, net of upstream obligations to licensors, from certain specified royalties, milestone payments, license fees and other consideration payable to us under the Zolgensma license with Novartis Gene Therapies, the Nippon Shinyaku Collaboration Agreement and certain other NAV Technology Platform license agreements.

In January 2025, we entered into the Nippon Shinyaku Collaboration Agreement for the development and commercialization of RGX-121 and RGX-111 in the United States and certain countries in Asia. Pursuant the Nippon Shinyaku Collaboration Agreement, we received an up-front payment of $110.0 million following the effective date of the agreement in March 2025 and are eligible to receive up to $700.0 million upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions.

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. Additionally, 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 December 2024, we entered into a Sales Agreement with Leerink Partners LLC (Leerink) 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 Leerink, acting as our sales agent (the Leerink ATM Program). As of September 30, 2025, no shares of common stock had been sold under the Leerink ATM Program. We intend to use proceeds obtained from the sale of shares under the Leerink ATM Program, if any, for general corporate purposes.

Cash Flows

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

	Nine Months Ended September 30,	
	2025	2024
Net cash used in operating activities	$ (71,673)	$ (141,501)
Net cash provided by (used in) investing activities	(53,406)	63,173
Net cash provided by financing activities	126,355	100,423
Net increase in cash and cash equivalents and restricted cash	$ 1,276	$ 22,095

Cash Flows from Operating Activities

Our net cash used in operating activities for the nine months ended September 30, 2025 decreased by $69.8 million from the nine months ended September 30, 2024, largely as a result of the $110.0 million up-front fee received from Nippon Shinyaku in March 2025. 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, 2025, our net cash used in operating activities of $71.7 million consisted of a net loss of $126.7 million, offset by adjustments for non-cash items of $41.5 million and favorable changes in operating assets and liabilities of $13.6 million. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $26.2 million and depreciation and amortization expense of $11.7 million. The changes in operating assets and liabilities include an increase in deferred revenue of $34.3 million, which was primarily attributable to the deferred portion of the $110.0 million up-front payment received under our collaboration with Nippon Shinyaku in the first quarter of 2025. The favorable changes in operating assets and liabilities were partially offset by an increase in prepaid expenses and other current assets of $9.5 million, which was largely driven by an increase in net cost reimbursement due from AbbVie under our ABBV-RGX-314 collaboration, and an increase in accounts receivable of $7.1 million, which was driven largely by reimbursable costs due from Nippon Shinyaku under our collaboration for RGX-121 and RGX-111. Other changes in operating working capital occurred in the normal course of business.

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.

Cash Flows from Investing Activities

For the nine months ended September 30, 2025, our net cash used in investing activities primarily consisted of $269.8 million used to purchase marketable debt securities and $1.9 million used to purchase property and equipment, partially offset by $218.3 million in maturities of marketable debt securities.

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.

Cash Flows from Financing Activities

For the nine months ended September 30, 2025, our net cash provided by financing activities primarily consisted of $144.5 million in proceeds received from the issuance of the 2025 Royalty Bond and warrants to HCR in May 2025, net of discounts and transaction costs paid during the period, and was partially offset by $18.4 million of royalties paid, net of interest, under our royalty monetization liabilities.

For the nine months ended September 30, 2024, our net cash provided by financing activities primarily consisted of $131.1 million in 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 royalties paid, net of interest, under our royalty monetization liabilities.

Additional Capital Requirements

Our material capital requirements from known contractual and other obligations primarily relate to our vendor service contracts and purchase commitments, in-license agreements, operating lease agreements and royalty monetization liabilities. 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, 2024, 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, 2024. Other than the changes described in the notes to the unaudited consolidated financial statements accompanying this Quarterly Report on Form 10-Q, including Note 7, "Royalty Monetization Liabilities," and Note 8, “Commitments and Contingencies,” there have been no material changes to our commitments and obligations since December 31, 2024.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $1.06 billion as of September 30, 2025. 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 impact of any government-imposed tariffs or other trade barriers on cost of goods and services, particularly related to partnered 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;
- revenue received from other commercial sales of our licensees' and collaborators' products, should any of the product candidates receive marketing approval, and other revenues received under our licensing agreements and collaborations;
- the timing and amount of Zolgensma royalties and other specified royalties, milestones and consideration under our license agreements and collaborations paid to HCR under our royalty monetization agreements;
- 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 relating to ABBV-RGX-314 and the Nippon Shinyaku Collaboration Agreement relating to RGX-121 and RGX-111, 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.

The issuance of additional securities, whether equity or debt, by us, including through our at-the-market program, 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, 2024. There have been no material changes to our exposure to market risk during the nine months ended September 30, 2025.

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, 2025. 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, 2025, 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, 2025 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, 2024. 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

The adoption or termination of contracts, instructions or written plans for the purchase or sale of our securities by our Section 16 officers and directors for the three months ended September 30, 2025, each of which is intended to satisfy the affirmative defense of Rule 10b5-1(c) (Rule 10b5-1 Plan), were as follows:

Name	Title	Action	Date Adopted	Expiration Date	Rule 10b5-1 Trading Plan Provides for Purchase/Sale	Aggregate # of Securities to be Purchased/Sold (a)
Mitchell Chan	Chief Financial Officer	Adoption	8/12/2025	8/11/2026	Sale	156,401

(a)The aggregate number of shares in this column includes shares that may be forfeited or withheld to satisfy exercise price and/or tax withholding obligations at the time of vesting.

Other than as described above, during the three months ended September 30, 2025, 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).

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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†	First Amendment to Collaboration and License Agreement dated September 10, 2021 between the Registrant and AbbVie Global Enterprises Ltd.				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, 2025 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, 2025 formatted in Inline XBRL (included in Exhibit 101)				

† Portions of this exhibit have been omitted.

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.

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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, 2025

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

Dated: November 6, 2025

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