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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, 2026

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 30, 2026, there were 66,122,600 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, 2026

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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 (surabgene lomparvovec, sura-vec) and our collaboration with Nippon Shinyaku Co., Ltd. to develop and commercialize RGX-121 (clemidsogene lanparvovec) and RGX-111;
- our ability to obtain, including under accelerated approval, and maintain regulatory approval of our product candidates, the labeling for any approved products and our ability to receive priority review vouchers for approved products;
- our ability to address any deficiencies raised by the U.S. Food and Drug Administration (the FDA) regarding RGX-121 as part of the Complete Response Letter or subsequent clinical hold;
- our ability to resolve the partial clinical hold for RGX-111 to the satisfaction of the FDA;
- the timing of enrollment, commencement, completion and the success of our AAVIATE®, AFFINITY BEYOND®, AFFINITY DUCHENNE®, AFFINITY® RISE, ALTITUDE®, ASCENT®, ATMOSPHERE®, CAMPSIITE®, NAAVIGATE 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;

- our ability to continue as a going concern; 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, 2025 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, AFFINITY BEYOND, AFFINITY DUCHENNE, ALTITUDE, ASCENT, ATMOSPHERE, CAMPSITE, 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, 2026	December 31, 2025
Assets		
Current assets		
Cash and cash equivalents	$ 95,795	$ 34,466
Marketable securities	155,181	195,604
Accounts receivable	8,536	26,379
Prepaid expenses	13,009	11,927
Restricted cash	225	—
Other current assets	48,146	12,905
Total current assets	320,892	281,281
Marketable securities	—	10,785
Accounts receivable	450	2,312
Property and equipment, net	95,347	104,855
Operating lease right-of-use assets	42,246	47,156
Restricted cash	1,805	2,030
Other assets	5,300	4,613
Total assets	$ 466,040	$ 453,032
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 13,482	$ 21,358
Accrued expenses and other current liabilities	29,611	38,390
Deferred revenue	5,985	10,452
Operating lease liabilities	6,492	8,286
Royalty monetization liabilities	12,114	39,609
Total current liabilities	67,684	118,095
Deferred revenue	16,221	18,943
Operating lease liabilities	60,054	65,215
Royalty monetization liabilities	173,564	147,408
Other liabilities	475	638
Total liabilities	317,998	350,299
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2026 and December 31, 2025; 66,119 and 50,892 shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively	7	5
Additional paid-in capital	1,383,187	1,229,442
Accumulated other comprehensive loss	(1,045)	(687)
Accumulated deficit	(1,234,107)	(1,126,027)
Total stockholders' equity	148,042	102,733
Total liabilities and stockholders' equity	$ 466,040	$ 453,032

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

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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,	
	2026	2025	2026	2025
Revenues				
License and royalty revenue	$ 5,244	$ 23,605	$ 114,175	$ 129,119
Service revenue	3,918	6,128	9,399	10,985
Total revenues	9,162	29,733	123,574	140,104
Operating Expenses (Income)				
Cost of license and royalty revenues	1,141	5,725	13,257	14,370
Research and development	61,009	56,101	174,434	168,688
General and administrative	20,579	20,253	63,501	60,483
Gain on litigation settlement	(38,026)	—	(38,026)	—
Other operating expenses (income)	(7)	65	41	125
Total operating expenses	44,696	82,144	213,207	243,666
Loss from operations	(35,534)	(52,411)	(89,633)	(103,562)
Other Income (Expense)				
Interest income from licensing	16	19	49	65
Investment income	2,252	3,620	5,477	9,500
Interest expense	(7,472)	(13,169)	(23,973)	(32,732)
Total other income (expense)	(5,204)	(9,530)	(18,447)	(23,167)
Net loss	$ (40,738)	$ (61,941)	$ (108,080)	$ (126,729)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(170)	98	(358)	89
Total other comprehensive income (loss)	(170)	98	(358)	89
Comprehensive loss	$ (40,908)	$ (61,843)	$ (108,438)	$ (126,640)
Net loss per share, basic and diluted	$ (0.62)	$ (1.20)	$ (1.90)	$ (2.46)
Weighted-average common shares outstanding, basic and diluted	65,263	51,689	56,884	51,513

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, 2026					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2026	54,164	$ 5	$ 1,266,004	$ (875)	$ (1,193,369)	$ 71,765
Vesting of restricted stock units, net of tax	120	—	(255)	—	—	(255)
Exercise of stock options, net of tax	76	—	647	—	—	647
Issuance of common stock under employee stock purchase plan	88	—	896	—	—	896
Issuance of common stock and pre-funded warrants upon public offering, net of transaction costs of $360	11,671	2	107,775	—	—	107,777
Stock-based compensation expense	—	—	8,120	—	—	8,120
Unrealized loss on available-for-sale securities, net	—	—	—	(170)	—	(170)
Net loss	—	—	—	—	(40,738)	(40,738)
Balances at September 30, 2026	66,119	$ 7	$ 1,383,187	$ (1,045)	$ (1,234,107)	$ 148,042

	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

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, 2026					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2025	50,892	$ 5	$ 1,229,442	$ (687)	$ (1,126,027)	$ 102,733
Vesting of restricted stock units, net of tax	957	—	(1,055)	—	—	(1,055)
Exercise of stock options, net of tax	99	—	816	—	—	816
Issuance of common stock under employee stock purchase plan	181	—	1,544	—	—	1,544
Issuance of common stock and pre-funded warrants upon public offering, net of transaction costs of $360	11,671	2	107,775	—	—	107,777
Issuance of common stock under at-the-market offering program, net of transaction costs of $624	2,319	—	18,875	—	—	18,875
Stock-based compensation expense	—	—	25,790	—	—	25,790
Unrealized loss on available-for-sale securities, net	—	—	—	(358)	—	(358)
Net loss	—	—	—	—	(108,080)	(108,080)
Balances at September 30, 2026	66,119	$ 7	$ 1,383,187	$ (1,045)	$ (1,234,107)	$ 148,042

	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

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, 2026	2025
Cash flows from operating activities		
Net loss	$ (108,080)	$ (126,729)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	25,790	26,188
Depreciation and amortization	11,129	11,746
Net accretion of discounts on marketable debt securities	(1,683)	(4,250)
Net realized gain on investments	(16)	(62)
Non-cash interest expense	15,922	7,795
Other non-cash adjustments	(8)	60
Changes in operating assets and liabilities		
Accounts receivable	19,754	(7,100)
Prepaid expenses	(1,082)	(3,876)
Other current assets	(35,417)	(5,586)
Operating lease right-of-use assets	5,000	4,989
Other assets	(687)	(268)
Accounts payable	(6,989)	(423)
Accrued expenses and other current liabilities	(9,051)	184
Deferred revenue	(7,189)	34,266
Operating lease liabilities	(7,045)	(5,620)
Other liabilities	(163)	(2,987)
Net cash used in operating activities	(99,815)	(71,673)
Cash flows from investing activities		
Purchases of marketable debt securities	(138,704)	(269,819)
Maturities of marketable debt securities	179,788	218,257
Sales of marketable debt securities	11,465	—
Sales of equity securities	—	62
Purchases of property and equipment	(2,189)	(1,906)
Net cash provided by (used in) investing activities	50,360	(53,406)
Cash flows from financing activities		
Proceeds from exercise of stock options	844	228
Taxes paid related to net settlement of stock-based awards	(1,083)	(633)
Proceeds from issuance of common stock under employee stock purchase plan	1,544	1,048
Proceeds from sales of common stock under at-the-market offering program, net of commissions	19,499	—
Expenses related to at-the-market offering program	(601)	(334)
Proceeds from public offering of common stock and pre-funded warrants, net of issuance costs	107,777	—
Proceeds from issuance of royalty bond and warrants, net of transaction costs	—	144,493
Repayments under royalty monetization liabilities, net of interest	(17,196)	(18,447)
Net cash provided by financing activities	110,784	126,355
Net increase in cash and cash equivalents and restricted cash	61,329	1,276
Cash and cash equivalents and restricted cash		
Beginning of period	36,496	59,556
End of period	$ 97,825	$ 60,832
Supplemental disclosures of non-cash investing and financing information		
Purchases of property and equipment in accounts payable and accrued expenses and other current liabilities	$ 330	$ 150

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 has consisted 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. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

The Company's lead product candidates include ABBV-RGX-314 (surabgene lomparvovec, sura-vec) for the treatment of wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR), which is being developed and commercialized in collaboration with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., and RGX-202 for the treatment of Duchenne muscular dystrophy. Additional clinical-stage candidates include RGX-121 (clemidsogene lanparvovec) for the treatment of Mucopolysaccharidosis Type II (MPS II) and RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I), which are being developed and commercialized in collaboration with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku).

In addition to its internal product development efforts, the Company has also selectively licensed the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies (NAV Technology Licensees). As of September 30, 2026, the NAV Technology Platform was being applied by NAV Technology Licensees in two commercial products, Zolgensma® and Itvisma®, and in the preclinical and clinical development of various other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates.

Liquidity

The Company has incurred cumulative losses since inception and as of September 30, 2026, had generated an accumulated deficit of $1.23 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.

As of September 30, 2026, the Company had cash, cash equivalents and marketable securities of $251.0 million. In October 2026, the Company received a payment of $39.0 million pursuant to a settlement agreement with Sarepta Therapeutics, Inc. (Sarepta). Please refer to Note 8 for further information regarding the settlement agreement with Sarepta. Management believes the Company's cash, cash equivalents and marketable securities as of September 30, 2026, along with the settlement proceeds received in October 2026, are sufficient to fund operations into the late fourth quarter of 2027. This estimate is based on the Company's current operating plan and excludes the potential effects of any future financings or material milestone payments that may be received under the Company's licensing and collaboration arrangements. The Company has based this estimate on assumptions that may prove to be wrong, and it could exhaust its capital resources sooner than expected. These conditions raise substantial doubt about the Company's ability to continue as a going concern within 12 months from the date these consolidated financial statements were issued. The Company’s ability to continue as a going concern will depend heavily on the successful development, approval and 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.

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, 2025 included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2025, which was filed with the SEC on March 5, 2026. 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, 2025, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025.

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.

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,	
	2026	2025
Cash and cash equivalents	$ 95,795	$ 58,802
Restricted cash, current	225	—
Restricted cash, non-current	1,805	2,030
Total cash and cash equivalents and restricted cash	$ 97,825	$ 60,832

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, 2026 and December 31, 2025.

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.

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, 2026 and 2025 which had a material impact on the consolidated financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In November 2024, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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, 2026				
U.S. government and agency securities	$ 103,152	$ 2	$ (100)	$ 103,054
Corporate bonds	52,214	1	(88)	52,127
	$ 155,366	$ 3	$ (188)	$ 155,181

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2025				
U.S. government and agency securities	$ 83,085	$ 59	$ —	$ 83,144
Corporate bonds	123,131	131	(17)	123,245
	$ 206,216	$ 190	$ (17)	$ 206,389

As of September 30, 2026, no available-for-sale debt securities had remaining maturities greater than one year. As of December 31, 2025, 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, 2026 and December 31, 2025, 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. Unrealized gain (loss) on available-for-sale securities, net, presented in the statements of operations and comprehensive loss consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Unrealized gain (loss) before reclassifications	$ (170)	$ 98	$ (342)	$ 89
Realized gains reclassified to investment income	—	—	(16)	—
Unrealized gain (loss) on available-for-sale securities, net	$ (170)	$ 98	$ (358)	$ 89

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, 2026						
U.S. government and agency securities	$ 78,407	$ (100)	$ —	$ —	$ 78,407	$ (100)
Corporate bonds	47,133	(88)	—	—	47,133	(88)
	$ 125,540	$ (188)	$ —	$ —	$ 125,540	$ (188)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2025						
U.S. government and agency securities	$ 4,676	$ —	$ —	$ —	$ 4,676	$ —
Corporate bonds	32,289	(17)	—	—	32,289	(17)
	$ 36,965	$ (17)	$ —	$ —	$ 36,965	$ (17)

As of September 30, 2026, available-for-sale debt securities held by the Company in an unrealized loss position consisted of 33 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, 2026 or December 31, 2025, and no impairment or credit losses on available-for-sale debt securities were recorded during the three and nine months ended September 30, 2026 and 2025.

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, 2026				
Cash equivalents:				
Money market mutual funds	$ —	$ 65,972	$ —	$ 65,972
U.S. government and agency securities	—	4,978	—	4,978
Total cash equivalents	—	70,950	—	70,950
Marketable securities:				
U.S. government and agency securities	—	103,054	—	103,054
Corporate bonds	—	52,127	—	52,127
Total marketable securities	—	155,181	—	155,181
Total cash equivalents and marketable securities	$ —	$ 226,131	$ —	$ 226,131

	Level 1	Level 2	Level 3	Total
December 31, 2025				
Cash equivalents:				
Money market mutual funds	$ —	$ 21,418	$ —	$ 21,418
U.S. government and agency securities	—	2,496	—	2,496
Total cash equivalents	—	23,914	—	23,914
Marketable securities:				
U.S. government and agency securities	—	83,144	—	83,144
Corporate bonds	—	123,245	—	123,245
Total marketable securities	—	206,389	—	206,389
Total cash equivalents and marketable securities	$ —	$ 230,303	$ —	$ 230,303

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 non-current accounts receivable 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, 2026 to determine the present value of these instruments. Accordingly, management estimates that the carrying values of its non-current accounts receivable 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.

5. Property and Equipment, Net

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

	September 30, 2026	December 31, 2025
Laboratory and manufacturing equipment	$ 79,728	$ 78,825
Computer equipment and software	3,807	4,573
Furniture and fixtures	7,059	7,039
Leasehold improvements	101,739	101,610
Total property and equipment	192,333	192,047
Accumulated depreciation and amortization	(96,986)	(87,192)
Property and equipment, net	$ 95,347	$ 104,855

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, 2026, total undiscounted future minimum lease payments to be received by the Company over the remaining term of the New York Sublease were $0.3 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, 2026, respectively, and $0.1 million and $0.3 million during the three and nine months ended September 30, 2025, respectively.

7. Royalty Monetization Liabilities

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

	September 30, 2026	December 31, 2025
2020 Royalty Purchase Agreement	$ 9,416	$ 29,672
2025 Royalty Bond	176,262	157,345
Total	$ 185,678	$ 187,017
Current portion of royalty monetization liabilities	$ 12,114	$ 39,609
Non-current portion of royalty monetization liabilities	173,564	147,408
Total	$ 185,678	$ 187,017

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 and Itvisma royalty payments under the Company's license agreement with Novartis Innovative Technologies Inc. (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 and Itvisma 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 and Itvisma 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 royalties paid to HCR equals or exceeds the Cap Amount applicable to such date, the 2020 Royalty Purchase Agreement would 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 and Itvisma royalty payments are not sufficient to achieve the Second 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 applicable 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 Itvisma 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 and Itvisma royalties to be paid to 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 as of September 30, 2026 and December 31, 2025 was 38.3% and 85.2%, 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, 2026, 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, 2025	$ 29,672
Novartis royalties paid to HCR	(25,246)
Interest expense recognized	4,990
Balance at September 30, 2026	9,416
Current portion	(9,416)
Non-current portion	$ —

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 and Itvisma, (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 and Itvisma royalties earned

under the Novartis License shall only be included in the Royalty Interest after full repayment of the Second 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 the 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 as of September 30, 2026 and December 31, 2025 was 15.0% and 15.1%, respectively, 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, 2025	$	157,345
Unpaid interest accrued to principal		17,770
Amortization of debt discount and issuance costs		1,147
Balance at September 30, 2026		176,262
Current portion		(2,698)
Non-current portion	$	173,564

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.

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,	
	2026	2025	2026	2025
Cost of license and royalty revenues:				
Royalties on net sales of Zolgensma and Itvisma	$ 869	$ 5,709	$ 2,678	$ 13,495
Settlement payment	—	—	10,000	—
Other	—	1	140	244
Total cost of license and royalty revenues	869	5,710	12,818	13,739
General and administrative	(29)	30	50	137
	$ 840	$ 5,740	$ 12,868	$ 13,876

As of September 30, 2026 and December 31, 2025, the Company had recorded $0.9 million and $5.7 million, respectively, payable under the GSK License.

GSK Settlement Agreement

The Company was notified of a dispute with GSK over the amount of sublicense fees paid by the Company to GSK under the GSK License. GSK claimed there had been a significant underpayment by the Company with respect to sublicense fees. In February 2026, the Company and GSK engaged in non-binding mediation regarding the dispute. In March 2026, the Company and GSK entered into a settlement and release agreement to resolve the matter. The settlement agreement provides for the mutual release of all past claims and certain future claims by the parties under the GSK License. Pursuant to the settlement agreement, the Company paid $10.0 million to GSK in March 2026, which was recorded as cost of license and royalty revenues in the first quarter of 2026. Additionally, the Company will continue to pay sublicense fees to GSK on license fees earned under existing sublicense agreements, utilizing the Company's historical allocation methodologies for such sublicense payments.

Sarepta Therapeutics Settlement Agreement

In September 2026, the Company and Penn entered into a settlement agreement with Sarepta and affiliated entities, and Catalent, Inc. to resolve all litigation alleging infringement of certain U.S. patents related to Sarepta's manufacture, use, offer to sell and sale of SRP-9001 (also known as Elevidys®).

Pursuant to the terms of the settlement agreement, Sarepta made a payment of $39.0 million to the Company following the effective date of the agreement. The parties have terminated with prejudice all ongoing patent litigation regarding the asserted patents. The settlement agreement includes the release by the Company and Penn of all current claims of infringement and a covenant not to sue for future patent claims relating to any Sarepta gene therapy product that uses an AAVrh74-based capsid, including Elevidys, based upon certain patents and patent applications. In addition, the Company agreed not to sue Sarepta or its affiliates, licensees or commercial partners over any Company patents or patent applications that cover the existing form of Elevidys or the AAVrh74-based capsid sequence; but this does not extend to patents or patent applications that cover other aspects of gene therapy products.

The Company recorded a gain of $38.0 million in the third quarter of 2026 related to the settlement agreement, which consisted of the $39.0 million settlement payment due from Sarepta, net of proceeds payable to certain licensors as a result of the settlement. The $39.0 million settlement payment was recorded as other current assets on the consolidated balance sheet as of September 30, 2026 and was received in full from Sarepta in October 2026.

Litigation

In February 2026, a putative securities class action complaint was filed by Andre Kuik (Kuik) against the Company and certain of its current officers and directors in the United States District Court for the District of Maryland. The complaint asserted claims under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder, on behalf of a putative class of persons who purchased or otherwise acquired the Company's securities during the period from February 9, 2022

through January 27, 2026. The complaint alleged that the Company misled investors concerning the viability and safety of RGX-111, and that the Company's stock price declined following the announcement of the clinical hold imposed by the U.S. Food and Drug Administration (FDA) on the Company's RGX-111 program on January 28, 2026. In August 2026, an amended complaint was filed focusing on interactions with FDA and Company statements prior to the Company's receipt of a Complete Response Letter for RGX-121 in February 2026. The amended complaint asserts claims under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder, on behalf of a putative class of persons who purchased or otherwise acquired the Company's securities during the period from February 7, 2024 through March 3, 2026. The plaintiff seeks unspecified compensatory damages, attorneys' fees, expert fees and other costs, and other relief as the court may deem just and proper. The Company believes that it has meritorious defenses to the claims asserted and intends to vigorously defend against them. 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, 2026.

In March 2026, a purported stockholder derivative complaint, as revised, was filed by plaintiff Roberto Medina (Medina) against the Company as nominal defendant and certain of its current officers and directors in the United States District Court for the District of Maryland. The complaint alleges breach of fiduciary duty and claims of unjust enrichment, abuse of control, gross mismanagement, waste of corporate assets, and alleged violations of Sections 10(b), 14(a) and 21D of the Securities Exchange Act of 1934 during the period from February 9, 2022 through January 27, 2026. The complaint alleges that the Company misled investors concerning the viability and safety of RGX-111 and that the Company's stock price declined following the announcement of the clinical hold imposed by the FDA on the Company's RGX-111 program on January 28, 2026. The complaint seeks, among other relief, damages in favor of the Company, disgorgement of alleged profits, restitution, attorneys' fees, expert fees, corporate governance reforms, and other relief as the court may deem just and proper. In May 2026 a substantially similar suit was filed by plaintiff Matthew Hay (Hay) in the United States District Court for the District of Maryland, containing largely similar factual and legal contentions and requested relief. In June 2026 the Medina and Hay derivative suits were consolidated and stayed through the resolution of the planned motion to dismiss in the Kuik securities suit. The Company believes the claims are procedurally defective. 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, 2026.

9. Capitalization

Public Offerings of Common Stock and Pre-funded Warrants

In July 2026, the Company completed a public offering of 11,671,139 shares of its common stock at a price of $9.00 per share and 1,111,111 pre-funded warrants (the July 2026 Pre-funded Warrants) to purchase shares of its common stock at a price of $8.9999 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 $107.8 million, net of underwriting discounts and commissions and offering expenses.

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 March 2024 Pre-funded Warrants and July 2026 Pre-funded Warrants (collectively, the Pre-funded Warrants) have similar terms and conditions, are exercisable at the option of the warrant holder at any time, subject to certain limitations, and do not expire. The Company evaluated the 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. No Pre-funded Warrants were exercised during the nine months ended September 30, 2026. As of September 30, 2026, there were 1,980,714 Pre-funded Warrants 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 ATM Program). During the nine months ended September 30, 2026, the Company sold 2,318,735 shares of common stock under the ATM Program for aggregate net proceeds of $18.9 million after deducting commissions and offering expenses. No shares of common stock were sold under the ATM Program prior to the second quarter of 2026.

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, 2026, no May 2025 Warrants had been exercised.

10. License and Collaboration Agreements

License and Collaboration Revenues

As of September 30, 2026, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in two commercial products, Zolgensma and Itvisma, and in the development of various 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,	
	2026	2025	2026	2025
License and royalty revenue:				
Zolgensma and Itvisma royalties	$ 5,244	$ 23,586	$ 14,137	$ 59,002
AbbVie development milestone	—	—	100,000	—
Nippon Shinyaku licenses	—	—	—	69,979
Other	—	19	38	138
Total license and royalty revenue	5,244	23,605	114,175	129,119
Service revenue:				
Nippon Shinyaku services	3,309	5,932	8,040	10,426
Other	609	196	1,359	559
Total service revenue	3,918	6,128	9,399	10,985
Total revenues	$ 9,162	$ 29,733	$ 123,574	$ 140,104

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, 2026, the Company's license and collaboration agreements contained unachieved milestones which could result in aggregate milestone payments to the Company of up to $2.06 billion, including (i) $444.0 million upon the commencement of various stages of clinical trials, (ii) $86.5 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 Company realizes the milestone payments, 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,	
	2026	2025	2026	2025
Accounts receivable, current and non-current:				
Beginning of period	$ 107,189	$ 21,783	$ 28,691	$ 20,947
End of period	$ 8,986	$ 28,112	$ 8,986	$ 28,112
Contract assets (included in other current assets):				
Beginning of period	$ 523	$ 28	$ 104	$ 239
End of period	$ 372	$ 93	$ 372	$ 93
Deferred revenue, current and non-current:				
Beginning of period	$ 25,286	$ 37,781	$ 29,395	$ 115
End of period	$ 22,206	$ 34,381	$ 22,206	$ 34,381
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	$ 3,080	$ 3,401	$ 7,189	$ 115
Performance obligations satisfied in previous periods	$ 6,189	$ 23,590	$ 116,998	$ 59,010

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma and Itvisma royalties and changes in the transaction prices of the Company's license agreements. Changes in transaction prices were primarily attributable to the achievement of development milestones, or the resolution of related uncertainties such that it became probable that a significant reversal of cumulative revenue would not occur if the development milestone consideration was included in the transaction price. These changes were recognized as cumulative catch-up adjustments to revenue. Revenue recognized during the nine months ended September 30, 2026 included $100.0 million in cumulative catch-up adjustments for changes in the probability of achievement of development milestones, which was associated with a $100.0 million development milestone achieved under the Company's collaboration and license agreement with AbbVie. No cumulative catch-up adjustments for development milestones were recorded in revenue during the three months ended September 30, 2026 or during the three and nine months ended September 30, 2025.

As of September 30, 2026, the Company had recorded deferred revenue of $22.2 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, 2026 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, 2026, the aggregate transaction price of the Company's license and collaboration agreements allocated to performance obligations not yet satisfied or partially satisfied was $23.3 million, primarily associated with development services under 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 four years.

Accounts receivable consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Current accounts receivable:		
Billed to customers	$ 1,173	$ 557
Unbilled Novartis royalties	5,244	23,806
Unbilled Nippon Shinyaku services	2,097	1,997
Other unbilled	22	19
Current accounts receivable	8,536	26,379
Non-current accounts receivable:		
Unbilled Nippon Shinyaku services	—	1,858
Other unbilled	450	454
Non-current accounts receivable	450	2,312
Total accounts receivable	$ 8,986	$ 28,691

Zolgensma and Itvisma License with Novartis

In March 2014, the Company entered into an exclusive license agreement (as amended, the Novartis License) with Novartis Innovative Technologies Inc. (formerly, Novartis Gene Therapies, Inc.), a wholly owned subsidiary of Novartis AG (Novartis). Under the Novartis License, the Company granted Novartis an exclusive, worldwide commercial license, with rights to sublicense, to the NAV Technology Platform, as well as other certain rights, for the treatment of spinal muscular atrophy (SMA) in humans by *in vivo* gene therapy. In 2019, Novartis launched commercial sales of Zolgensma for the treatment of SMA in patients under the age of two years old. In the fourth quarter of 2025, Novartis launched commercial sales of Itvisma for the treatment of SMA in patients two years and older. Zolgensma and Itvisma are licensed products under the Novartis License, pursuant to which the Company receives royalties on certain net sales of the licensed products.

In January 2026, licensed patents for Zolgensma under the Novartis License expired in the United States. The Company is entitled to continued royalties on net sales of Zolgensma in approximately 20 countries where licensed patents remain active. Licensed product made prior to patent expiration but sold after expiration may also be subject to royalties. Licensed patents covering the use of Itvisma have issued in the United States and certain other countries, and the Company is entitled to ongoing royalties on certain net sales of Itvisma in these territories.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Zolgensma royalties	$ 2,431	$ 23,586	$ 8,933	$ 59,002
Itvisma royalties	2,813	—	5,204	—
Total license and royalty revenue	$ 5,244	$ 23,586	$ 14,137	$ 59,002
Interest income from licensing	$ 10	$ 10	$ 30	$ 31

As of September 30, 2026 and December 31, 2025, the Company had recorded total accounts receivable of $5.5 million and $24.1 million, respectively, from Novartis under the Novartis License, which consisted primarily of Zolgensma and Itvisma royalties receivable. Royalties receivable as of September 30, 2026 included $4.2 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 and Itvisma in the period in which the underlying products are sold by Novartis, 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 to jointly develop and commercialize sura-vec (or ABBV-RGX-314), the Company's product candidate for the treatment of wet AMD, 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 sura-vec in the United States and an exclusive license to develop and commercialize sura-vec outside the United States. The Company and AbbVie will collaborate to develop sura-vec in the United States, and AbbVie will be responsible for the development of sura-vec in specified markets outside the United States. Global development expenses for sura-vec 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 sura-vec for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of sura-vec 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 sura-vec commercial supply for sales outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of sura-vec 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 sura-vec in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of sura-vec.

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 sura-vec 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 sura-vec 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 (NAAVIGATE) which will be 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 a $200.0 million development milestone payable 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 NAAVIGATE trial (the First DR Milestone) and an additional $100.0 million upon first patient dosed in the subsequent Phase III trial (the Second DR Milestone). Also pursuant to the amendment, AbbVie will lead a new Phase III randomized controlled study (ACHIEVE) to assess the injection burden, adverse events, change in disease activity, and long-term preservation of visual acuity of sura-vec in adult participants with neovascular AMD. The Company will be responsible for its development expenses to conduct Part 1 of the NAAVIGATE trial and the parties will share the development expenses related to Part 2 of the NAAVIGATE 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.

In June 2026, the Company dosed the first patient in the NAAVIGATE trial, resulting in a $100.0 million milestone payment from AbbVie for achievement of the First DR Milestone.

The Company applied the requirements of ASC 606, *Revenue from Contracts with Customers* (ASC 606) to the AbbVie Collaboration Agreement for the units of account in which AbbVie was deemed to be a customer. The Company determined that there is only one material performance obligation under the agreement for the delivery of the intellectual property license to develop and commercialize sura-vec 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, 2026, the transaction price of the AbbVie Collaboration Agreement was $470.0 million, which consisted of (i) the $370.0 million up-front payment received from AbbVie in November 2021 and (ii) the $100.0 million development milestone payment earned upon achievement of the First DR Milestone in June 2026 and received from AbbVie in July 2026. As of September 30, 2026, variable consideration excluded from the transaction price of the AbbVie Collaboration Agreement includes $462.5 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 sura-vec 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. The $470.0 million transaction price of the AbbVie Collaboration Agreement has been fully recognized as license and royalty revenue since the intellectual property licenses were delivered to AbbVie at the inception of the agreement in November 2021. The transaction price of the AbbVie Collaboration Agreement increased by $100.0 million during the nine months ended September 30, 2026 as a result of the achievement of the First DR Milestone in June 2026, as the milestone payment was previously constrained and excluded from the transaction price. The $100.0 million increase in the transaction price was fully recognized as a cumulative catch-up adjustment to revenue upon the achievement of the First DR Milestone in June 2026. There were no changes in the transaction price of the AbbVie Collaboration Agreement, and no revenue was recognized, during the three months ended September 30, 2026 or the three and nine months ended September 30, 2025.

The Company applied the requirements of ASC 808, *Collaborative Arrangements* (ASC 808) to the AbbVie Collaboration Agreement for the units of account which were deemed to be a collaborative arrangement. Both the Company and AbbVie will perform various activities related to the development, manufacturing and commercialization of sura-vec 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 sura-vec 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, 2026 and December 31, 2025, the Company had recorded $7.5 million and $10.9 million, respectively, due from AbbVie for net reimbursement of costs incurred for activities performed under the 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,	
	2026	2025	2026	2025
License and royalty revenue	$ —	$ —	$ 100,000	$ —
Net cost reimbursement to (from) AbbVie included in:				
Research and development expense	$ (9,336)	$ (17,742)	$ (34,022)	$ (49,479)
General and administrative expense	1,911	712	3,662	1,860
Total net cost reimbursement to (from) AbbVie	$ (7,425)	$ (17,030)	$ (30,360)	$ (47,619)

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 MPS II, and RGX-111, the Company's product candidate for the treatment of 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.

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 concluded that each of the distinct units of account identified under the Nippon Shinyaku Collaboration Agreement 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 United 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.

As of September 30, 2026, the transaction price of the Nippon Shinyaku Collaboration Agreement included fixed consideration of $110.0 million for the up-front payment and estimated variable consideration of $5.6 million for reimbursable costs of manufacturing and other services 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 reimbursable costs of manufacturing and other services which are contingent on events occurring that are outside the Company's control and are deemed to be constrained. 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 for manufacturing and other services 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 during the three and nine months ended September 30, 2026 and 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 $5.6 million of estimated variable consideration included in the transaction price is allocated directly to performance obligations for the manufacturing of commercial supply prior to regulatory approval and other service obligations since the consideration is directly associated with the performance of such services and reimbursement of applicable 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 licenses 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 of commercial supply 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,	
	2026	2025	2026	2025
License and royalty revenue	$ —	$ —	$ —	$ 69,979
Service revenue	3,309	5,932	8,040	10,426
Total revenues	$ 3,309	$ 5,932	$ 8,040	$ 80,405

As of September 30, 2026 and December 31, 2025, the Company had recorded total accounts receivable of $2.5 million and $4.1 million, respectively, for reimbursement of manufacturing-related development costs and other services under the Nippon Shinyaku Collaboration Agreement.

As of September 30, 2026, the Company had recorded total deferred revenue of $22.2 million for development services and material rights which have not yet been satisfied under the Nippon Shinyaku Collaboration Agreement, of which $6.0 million was included in current liabilities and $16.2 million was included in non-current liabilities. As of December 31, 2025, the Company had recorded total deferred revenue of $29.4 million for development services and material rights which have not yet been satisfied under the Nippon Shinyaku Collaboration Agreement, of which $10.5 million was included in current liabilities and $18.9 million was included in non-current liabilities.

11. Stock-based Compensation

The total number of shares of common stock authorized for issuance under the 2025 Equity Incentive Plan (the 2025 Plan) upon its adoption in May 2025 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 Equity Incentive Plan (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, including an exchange of the award (to the extent that surrendered awards exceed newly issued awards in such exchange). 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, 2026, the total number of shares of common stock reserved for issuance under the 2025 Plan and 2015 Plan was 18,732,094, of which 4,985,411 remained available for future grants under the 2025 Plan.

The Company's equity incentive plans 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. As of September 30, 2026, the Company has issued only stock options, restricted stock units (RSUs) and performance stock units (PSUs) under its equity plans. Stock options generally expire 10 years following the date of grant and typically vest over a four-year service period, but contractual terms and 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. RSUs typically vest over a four-year service period, but vesting provisions can vary by award based on the discretion of the Board of Directors. In January 2026, the Company granted PSUs to certain executive employees. PSUs have similar terms and conditions as RSUs, except that vesting is contingent upon the achievement of specified performance conditions in addition to service conditions over a period of one year. Upon vesting, RSUs and PSUs 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.

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,	
	2026	2025	2026	2025
Stock options	$ 3,345	$ 4,777	$ 10,751	$ 14,262
RSUs	3,966	4,051	12,530	11,432
PSUs	657	—	1,914	—
Employee stock purchase plan	152	170	595	494
	$ 8,120	$ 8,998	$ 25,790	$ 26,188

As of September 30, 2026, the Company had $45.8 million of unrecognized stock-based compensation expense related to stock options, RSUs, PSUs 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,	
	2026	2025	2026	2025
Research and development	$ 3,377	$ 4,245	$ 10,988	$ 12,181
General and administrative	4,743	4,753	14,802	14,007
	$ 8,120	$ 8,998	$ 25,790	$ 26,188

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, 2025	11,566	$ 25.26	6.0	$ 17,523
Granted	1,752	$ 11.86		
Exercised	(325)	$ 11.84		
Cancelled or forfeited	(2,435)	$ 33.17		
Outstanding at September 30, 2026	10,558	$ 21.62	6.2	$ 233
Exercisable at September 30, 2026	6,957	$ 26.93	5.1	$ 6
Vested and expected to vest at September 30, 2026	10,558	$ 21.62	6.2	$ 233

(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, 2026, excluding options granted under the stock option exchange program as discussed below, was $6.87. During the nine months ended September 30, 2026, the total number of stock options exercised was 325,339, resulting in total proceeds of $0.8 million. The number of stock options exercised during the period includes shares withheld to cover option cost and taxes. The total intrinsic value of options exercised during the nine months ended September 30, 2026 was $0.5 million.

Stock Option Exchange Program

On June 3, 2026, the Company commenced an offer to exchange (the Offer to Exchange) certain eligible stock options held by eligible non-executive employees for replacement stock options under a voluntary stock option exchange program (the Stock Option Exchange Program). Under the Stock Option Exchange Program, eligible employees were offered the opportunity to exchange outstanding stock options granted under the 2015 Plan that have an exercise price equal to or greater than $18.00 per share (Eligible Options), for a lesser number of replacement stock options to be granted under the 2025 Plan (Replacement Options). The number of Replacement Options granted in exchange for Eligible Options surrendered was calculated based on specified exchange ratios which vary based on the grant date and exercise price of the Eligible Options. The exchange ratios were established in order to result in a fair value of the Replacement Options that was generally equal, on an aggregate basis, to the fair value of the Eligible Options surrendered.

The Offer to Exchange expired on July 1, 2026. Pursuant to the terms and conditions of the Offer to Exchange, a total of 184 eligible employees elected to exchange a total of 1,940,394 Eligible Options with a weighted-average exercise price of $35.13 per share, representing approximately 67.0% of the total Eligible Options outstanding. On July 2, 2026, all Eligible Options that were tendered were cancelled and, in exchange therefor, the Company granted Replacement Options to purchase 775,401 shares of common stock with an exercise price of $13.28 per share, which was the closing price of the Company's common stock on July 2, 2026. The Replacement Options expire eight years from the grant date and are subject to new vesting schedules based on continued service over a period of two years from the grant date.

The exchange of stock options under the Stock Option Exchange Program was accounted for as a modification of the awards in accordance with ASC 718, *Compensation—Stock Compensation* (ASC 718). The total incremental stock-based compensation expense resulting from the modification of the awards was immaterial and was measured based on the excess fair value of the modified awards over the fair value of the original awards immediately before the modification, calculated using a Black-Scholes option-pricing model. The incremental stock-based compensation expense will be recognized together with any unrecognized compensation cost remaining on the exchanged options over the two-year requisite service period of the Replacement Options granted.

Restricted Stock Units

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

	Shares		Weighted-average Grant Date Fair Value
Unvested RSUs at December 31, 2025	2,850	$	13.08
Granted	1,225	$	13.35
Vested	(1,039)	$	14.95
Forfeited	(152)	$	11.84
Unvested RSUs at September 30, 2026	2,884	$	12.59

The total intrinsic value of RSUs vested during the nine months ended September 30, 2026 was $12.6 million.

Performance Stock Units

In January 2026, the Company granted PSUs to certain executive employees. Vesting of the PSUs is contingent upon the achievement of specified performance conditions pre-determined by the Board of Directors, as well as service conditions over a requisite service period. The performance conditions are based on certain specified business objectives and are non-market based. As determined by the Board of Directors, each performance condition that is achieved during the applicable performance period will result in the vesting of a specified number of PSUs one year from the grant date, subject to the grantee's continued service through the vesting date.

Stock-based compensation expense for PSUs is measured based on the grant date fair value of the award, which is determined based on the fair value of the Company's stock on the grant date, and is recognized over the requisite service period beginning in the period it is probable that the performance conditions will be achieved. As of September 30, 2026, certain specified performance conditions were achieved or deemed probable of achievement but vesting of the associated PSUs remains subject to continued service conditions. No PSUs vested during the nine months ended September 30, 2026.

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

	Shares		Weighted-average Grant Date Fair Value
Unvested PSUs at December 31, 2025	—	$	—
Granted	306	$	14.18
Vested	—	$	—
Forfeited	—	$	—
Unvested PSUs at September 30, 2026	306	$	14.18

Employee Stock Purchase Plan

As of September 30, 2026, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 574,699 remained available for future issuance. During the nine months ended September 30, 2026, 180,607 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, 2026 and December 31, 2025. 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 deferred tax assets as of September 30, 2026 and December 31, 2025.

13. Net Loss Per Share

Since the Company incurred net losses for the three and nine months ended September 30, 2026 and 2025, 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,	
	2026	2025
Stock options outstanding	10,558	11,726
Unvested RSUs	2,884	2,904
Unvested PSUs	184	—
Employee stock purchase plan	68	60
May 2025 Warrants outstanding	268	268
	13,962	14,958

In addition to the common stock equivalents in the table above, the computations of weighted-average diluted common shares for the three and nine months ended September 30, 2026 exclude 122,565 unvested PSUs as of September 30, 2026 for which vesting is contingent upon the future achievement of performance conditions.

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, 2026 and December 31, 2025, the Company’s cash, cash equivalents and marketable securities were $251.0 million and $240.9 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 loss (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues	$ 9,162	$ 29,733	$ 123,574	$ 140,104
Less:				
Cost of license and royalty revenues	1,141	5,725	13,257	14,370
Research and development expense				
Personnel	18,544	18,464	57,483	55,153
Direct development and support (a)	32,478	26,800	86,500	81,860
Facilities	3,293	2,946	8,996	8,467
Stock-based compensation	3,377	4,245	10,988	12,181
Depreciation and amortization	3,317	3,646	10,467	11,027
Total research and development expense	61,009	56,101	174,434	168,688
General and administrative expense				
Personnel	5,815	5,567	17,812	17,055
Other general and administrative (b)	8,560	8,331	26,233	24,751
Facilities	1,263	1,362	3,992	3,951
Stock-based compensation	4,743	4,753	14,802	14,007
Depreciation and amortization	198	240	662	719
Total general and administrative expense	20,579	20,253	63,501	60,483
Other segment items (c)	32,829	(9,595)	19,538	(23,292)
Net loss	$ (40,738)	$ (61,941)	$ (108,080)	$ (126,729)

(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, commercialization expenses, commercial cost reimbursement to and from collaborators and other corporate overhead expenses.

(c)Other segment items include gain on litigation settlement, other operating expenses (income), 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 $2.3 million and $5.5 million during the three and nine months ended September 30, 2026, respectively, and $3.6 million and $9.4 million during the three and nine months ended September 30, 2025, 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, 2026	December 31, 2025
Settlement payment due from Sarepta	$ 39,000	$ —
Net cost reimbursement due from AbbVie	7,493	10,871
Accrued interest on investments	961	1,004
Other	692	1,030
	$ 48,146	$ 12,905

Accrued Expenses and Other Current Liabilities

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

	September 30, 2026	December 31, 2025
Accrued personnel costs	$ 16,037	$ 17,418
Accrued external research and development expenses	8,412	10,229
Accrued external general and administrative expenses	1,880	1,301
Accrued royalties and sublicense fees	1,150	8,652
Accrued purchases of property and equipment	242	33
Other	1,890	757
	$ 29,611	$ 38,390

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, 2025, which we filed with the SEC on March 5, 2026. 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, 2025 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.

Sura-vec (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). In wet AMD, sura-vec is currently being evaluated in multiple clinical trials utilizing subretinal delivery, including two pivotal trials (ATMOSPHERE and ASCENT) where enrollment has been completed, one long-term follow-up study and a fellow eye sub-study. In DR, we are actively enrolling a pivotal, two-part Phase IIb/III study (NAAVIGATE) using suprachoroidal delivery. In addition to these late-stage pivotal programs, sura-vec is being evaluated in two Phase II clinical trials in patients with wet AMD (AAVIATE) and DR (ALTITUDE), which 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 sura-vec in diabetic macular edema (DME). Sura-vec 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.

Sura-vec for Treatment of Wet AMD

Subretinal Delivery

ATMOSPHERE® and ASCENT® are multi-center, randomized, active-controlled trials evaluating sura-vec versus monthly ranibizumab and aflibercept, respectively. The primary endpoint in both studies is non-inferiority based on change from baseline in best-corrected visual acuity (BCVA) at 54 weeks. Secondary endpoints include safety and tolerability, change in central retinal thickness (CRT) and need for supplemental anti-VEGF injections in the treatment arms.

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, which together enrolled over 1,300 participants across more than 200 sites, are expected to support global regulatory submissions including with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Topline data from these trials are expected to be announced in the fourth quarter of 2026 in partnership with AbbVie, with global regulatory submissions expected in 2027.

The clinical development program evaluating subretinal sura-vec in wet AMD encompasses multiple clinical studies and sub-studies which together are designed to establish a broad and robust body of clinical evidence. The program has enrolled a diverse spectrum of patients, including those with high disease activity and bilateral disease, with the intent of supporting the potential applicability of sura-vec across a broad patient population. The program also includes ODYSSEY, a retrospective comparative analysis evaluating outcomes in sura-vec-treated patients against matched real-world controls. Additionally, AbbVie is enrolling ACHIEVE, a global, randomized, controlled Phase 3b study to assess the injection burden, efficacy, safety and long-term preservation of vision in a real-world context.

In July 2026, we presented long-term follow-up data from the Phase I/IIa trial that demonstrated a durable safety and efficacy profile through five years, with participants in Cohorts 3 and 4, who received subretinal sura-vec at doses similar to those being studied in the ATMOSPHERE and ASCENT pivotal trials, demonstrating stable to improved visual acuity and meaningful reductions in anti-VEGF treatment burden, with the exception of one participant in Cohort 4 with polypoidal choroidal vasculopathy refractory to anti-VEGF therapy.

In October 2026, we held an investor event during which we reviewed the clinical development to date, including data from select Phase I/II studies, and discussed the unmet need and potential role of sura-vec in wet AMD with retina experts. A copy of that presentation is available on our website.

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 sura-vec for the treatment of wet AMD. Based on the favorable safety profile observed as of July 29, 2024, the Phase II AAVIATE trial enrolled a cohort to evaluate sura-vec at dose level 4 ($1.5x10^{12}$ GC/eye). Patients in this cohort received short course prophylactic steroid eye drops. Enrollment of the AAVIATE trial has been completed.

Sura-vec for Treatment of DR and DME

In August 2025, we and AbbVie announced an amendment to our collaboration agreement and plans to initiate a pivotal program consisting of a Phase IIb/III trial (NAAVIGATE) as well as a second Phase III trial to evaluate sura-vec for the treatment of DR. NAAVIGATE is a two-part, multicenter, randomized, masked, sham-controlled Phase IIb/III study to evaluate the safety and efficacy of a one-time, in-office administration of sura-vec in subjects with non-proliferative diabetic retinopathy (NPDR) without center-involved diabetic macular edema (CI-DME). The primary endpoint is >2-step improvement on the Diabetic Retinopathy Severity Scale (DRSS) at one year. We are actively enrolling the Phase IIb/III NAAVIGATE trial and, in June 2026, dosed the first patient in the Phase IIb portion of the trial, upon which we earned a $100.0 million milestone payment that was received from AbbVie in July 2026. Following an interim analysis of part one (Phase IIb) of the NAAVIGATE trial, we and AbbVie expect to initiate a Phase III expansion, including part two (Phase III) of the U.S. NAAVIGATE trial and a parallel global Phase III trial led by AbbVie.

Concurrent with the August 2025 announcement, we announced positive two-year data from the Phase II ALTITUDE® trial. The ALTITUDE trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of sura-vec using suprachoroidal delivery for the treatment of DR. The positive two-year data showed sura-vec was well tolerated in subjects with NPDR at dose levels 1, 2, and 3. There were no drug-related serious adverse events and no intraocular inflammation was observed through two years at dose level 3 ($1.0x10^{12}$ GC/eye) (n = 15) with short-course topical prophylactic steroids. Additionally, 50% of dose level 3 patients achieved at least a two-step improvement without need for any supplemental treatment.

In 2026, we presented long-term follow-up data from the Phase II ALTITUDE trial that showed, among other findings, that participants at dose level 3 (1.0×10^{12} GC/eye) with short-course prophylactic topical steroids, the same dose being evaluated in the Phase IIb/III NAAVIGATE trial, maintained a durable safety and efficacy profile through 2.5 years and 3 years. At 2.5 years, 55% of dose level 3 participants achieved >2-step improvement on the DRSS without additional treatment, and 70% of participants experienced no vision-threatening events. 60% of all dose level 3 participants with three-year visits (n=6/10) achieved >2-step improvement on the DRSS without additional treatment for diabetic retinopathy. These participants did not experience any vision-threatening events. Additionally, the majority of participants (n=3/4) who achieved a 1-step DRSS improvement at one year without supplemental anti-VEGF injections went on to achieve a >2-step DRSS improvement by three years without additional treatment for diabetic retinopathy. These data are consistent with previously presented two-year dose level 3 NPDR data from the ALTITUDE trial and support the potential of one-time in-office sura-vec to modify the underlying disease and decrease risk of vision-threatening events.

RGX-202 for Treatment of Duchenne

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 an ongoing 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 ambulatory patients with Duchenne aged 1 to 11 years old. Phase I/II results, reported between November 2024 and March 2026, demonstrated RGX-202 to be well tolerated with no serious adverse events or adverse events of special interest among all patients (n=13). Additionally, we reported positive biomarker data showing consistent, high expression and transduction of RGX-202 microdystrophin, with all patients exceeding 10%. Pivotal dose ($2x10^{14}$ GC/kg) participants in the Phase I/II exceeded expected disease trajectory on the North Star Ambulatory Assessment (NSAA) and other timed function tests at one year using multiple validated methods to estimate expected disease progression without treatment (n=7). Notably, five participants for whom functional data have been reported were aged eight or older at dosing, when functional decline is typically expected.

In October 2025, we announced that enrollment had completed in the pivotal portion of AFFINITY DUCHENNE (n=approximately 30).

In May 2026, we announced positive topline results from the pivotal Phase III AFFINITY DUCHENNE trial of RGX-202, including primary endpoint (n=30 at Week 12), interim safety (n=31) and interim functional data (n=9 at 12 months):

- The primary endpoint was achieved with high statistical significance; 93% of patients achieved RGX-202 microdystrophin expression above 10% ($p<0.0001$).
- RGX-202 was well-tolerated and continued to demonstrate a favorable interim safety profile.
- RGX-202 demonstrated functional improvement and evidence of positively impacting disease trajectory at one year post-treatment, as measured by NSAA and timed function tests (Time to Stand, 10 Meter Walk-run, Time to Climb).
- Statistically significant correlation between RGX-202 microdystrophin expression level and functional improvement, supporting validity of surrogate endpoint.

As of May 2026, in recent discussions, the FDA shared that the use of RGX-202 microdystrophin expression as a surrogate endpoint will be based on the correlation analysis with clinical outcomes, which has been clearly demonstrated in our interim data. While the FDA has recommended a randomized controlled trial, it has guided that externally controlled trials may be adequate for demonstrating substantial evidence of effectiveness, especially when the treatment effect is sufficiently large enough to overcome limitations of externally controlled trials.

Given the positive topline pivotal data, continued favorable safety profile, and statistically significant correlation between microdystrophin and functional improvement, we expect to hold a pre-Biologics License Application (BLA) meeting with the FDA in the fourth quarter of 2026 and submit a BLA in the first quarter of 2027 under the accelerated approval pathway, supporting potential FDA approval in the second half of 2027.

In June 2026, we announced we had completed dosing in the confirmatory study of RGX-202. We expect the BLA to include a safety dataset from the AFFINITY DUCHENNE pivotal and confirmatory studies (n=63) as well as efficacy data from the pivotal portion (n=30), including 12-month functional data for at least half of the total participants in the pivotal study.

We expect to initiate AFFINITY® RISE, a new, ex-U.S. randomized, placebo-controlled study to support RGX-202 global regulatory submissions, in the first half of 2027.

We have completed manufacturing the first batches of RGX-202 intended for commercial supply and manufacturing is ongoing to build commercial inventory in advance of a potential commercial launch. The process performance qualification (PPQ) campaign is also complete.

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 for Treatment of MPS II

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.

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 Prescription Drug User Fee Act (PDUFA) goal date was extended from November 9, 2025 to February 8, 2026 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 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.

In January 2026, we announced that the FDA placed the RGX-121 program on partial clinical hold in relation to a serious adverse event in a patient treated in the Phase I/II trial of RGX-111, discussed in further detail below. The FDA cited the similarities in products, study populations, and shared risk between the clinical studies. The partial clinical hold on RGX-121 was lifted by the FDA on April 30, 2026.

In February 2026, we announced that the FDA issued a Complete Response Letter (CRL) for the RGX-121 BLA. The FDA stated in the CRL that it had agreed to the study protocol in principle and outlined several reasons for not approving the gene therapy, including uncertainty regarding the study eligibility criteria to adequately define a population with neuronopathic disease (vs. attenuated disease), the comparability of the natural history external control to the study population, and the appropriateness of CSF HS D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit.

In March 2026, we entered into discussions with FDA senior leadership and filed a Formal Dispute Resolution Request.

In June 2026, we announced alignment with the FDA regarding the next steps needed for a potential accelerated approval of RGX-121. During those discussions, the FDA acknowledged that the existing RGX-121 clinical data was sufficient to be considered for the accelerated approval pathway and that the Company did not need to enroll additional patients or conduct additional studies, including the FDA's previously recommended incorporation of an untreated control arm.

In July 2026, we and the FDA held a positive Type A meeting during which the FDA reaffirmed that no additional studies of RGX-121 are required for BLA resubmission and aligned on resubmission requirements.

In August 2026, we announced that the FDA placed a clinical hold on RGX-121 following the discovery of asymptomatic spine MRI findings in five participants in the CAMPSIITE study. We continue to collect and evaluate additional patient imaging and long-term follow-up data. 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 for Treatment of MPS I

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 in interim results 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.

In January 2026, we announced that the FDA placed the RGX-111 program on partial clinical hold following preliminary analysis of a single case of neoplasm (intraventricular CNS tumor) in a participant treated in the Phase I/II study. The case was identified during a routine brain MRI of an asymptomatic five-year-old participant who received intracisternal RGX-111 four years prior. Preliminary genetic analysis of the resected tumor detected an AAV vector genome integration event associated with overexpression of a proto-oncogene (PLAG1), which is known to be susceptible to chromosomal rearrangements. Final analysis of the resected tumor was conducted by an independent third-party lab and, as previously reported, detected an AAV vector genome integration event associated with overexpression of a PLAG1. Clonal integration of AAV vector elements into the PLAG1 gene was detected in the tumor tissue. Analyses supported classification as a PLAG1-family neuroepithelial tumor and are consistent with the

hypothesis that AAV vector integration at the PLAG1 site contributed to tumor formation. Of note, this participant had a background of factors that could have contributed to risk of oncogenic transformation. For example, the participant underwent unsuccessful stem cell transplant at four months of age, with loss of donor chimerism, and he received chemotherapeutics that may have contributed to DNA damage. The report concluded, based on formal neuropsychologic testing and developmental pediatrician assessment, that the patient's neurocognitive development was above average, which indicated mitigation of MPS I disease, and the patient continued to do well. The analysis was published in The New England Journal of Medicine in May 2026.

AbbVie Collaboration for Sura-vec

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 sura-vec (as amended, the AbbVie Collaboration Agreement). Pursuant to the AbbVie Collaboration Agreement, both we and AbbVie are active participants in the development of sura-vec and development expenses are shared between the parties in accordance with the agreement. The Company will lead the manufacturing of sura-vec for clinical development and U.S. commercial supply, and AbbVie will lead the global commercialization of sura-vec. 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 sura-vec in the United States, and we are eligible to receive tiered royalties on net sales by AbbVie of sura-vec 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 June 2026, we dosed the first patient in the NAAVIGATE trial, resulting in a $100.0 million development milestone payment from AbbVie which was fully recognized as license and royalty revenue in the second quarter of 2026 and received from AbbVie in July 2026.

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) in the United States and certain countries in Asia. 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. For additional information regarding the Nippon Shinyaku Collaboration Agreement, 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, 2026, our NAV Technology Platform was being applied in two commercial products, Zolgensma® and Itvisma®, and the preclinical and clinical development of various 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, as well as from development and manufacturing services performed under such license and collaboration arrangements. 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 have licensed 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.

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 collaboration arrangements may materially impact the amount of revenue we recognize in future periods.

Zolgensma and Itvisma Royalties

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

In January 2026, licensed patents for Zolgensma under the Novartis License expired in the United States. We are entitled to continued royalties on net sales of Zolgensma in approximately 20 countries where licensed patents remain active. Licensed product made prior to patent expiration but sold after expiration may also be subject to royalties. Licensed patents covering the use of Itvisma have issued in the United States and certain other countries, and we are entitled to ongoing royalties on certain net sales of Itvisma in these territories.

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, 2026 and 2025 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Direct Expenses				
ABBV-RGX-314 (sura-vec)	$ 10,439	$ 6,068	$ 25,705	$ 27,322
RGX-202	12,446	6,908	29,269	16,345
RGX-121	2,047	4,678	3,949	11,015
Other product candidates	786	1,183	3,035	4,484
Total direct expenses	25,718	18,837	61,958	59,166
Unallocated Expenses				
Platform and early research	6,760	7,963	24,542	22,694
Personnel	18,544	18,464	57,483	55,153
Facilities	3,293	2,946	8,996	8,467
Stock-based compensation	3,377	4,245	10,988	12,181
Depreciation and amortization	3,317	3,646	10,467	11,027
Total unallocated expenses	35,291	37,264	112,476	109,522
Total research and development	$ 61,009	$ 56,101	$ 174,434	$ 168,688

Direct expenses related to the development of sura-vec include $9.3 million and $34.0 million for the three and nine months ended September 30, 2026, respectively, and $17.7 million and $49.5 million for the three and nine months ended September 30, 2025, respectively, in net cost reimbursement from AbbVie under our sura-vec 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 sura-vec, 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, manufacturing support, 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 our commercial partners prepare for and carry out product launch efforts, in particular for the potential commercialization of our RGX-202 and sura-vec 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 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, 2025. There have been no significant changes in our critical accounting policies and estimates since December 31, 2025.

Results of Operations

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Revenues						
License and royalty revenue	$ 5,244	$ 23,605	$ (18,361)	$ 114,175	$ 129,119	$ (14,944)
Service revenue	3,918	6,128	(2,210)	9,399	10,985	(1,586)
Total revenues	9,162	29,733	(20,571)	123,574	140,104	(16,530)
Operating Expenses (Income)						
Cost of license and royalty revenues	1,141	5,725	(4,584)	13,257	14,370	(1,113)
Research and development	61,009	56,101	4,908	174,434	168,688	5,746
General and administrative	20,579	20,253	326	63,501	60,483	3,018
Gain on litigation settlement	(38,026)	—	(38,026)	(38,026)	—	(38,026)
Other operating expenses (income)	(7)	65	(72)	41	125	(84)
Total operating expenses	44,696	82,144	(37,448)	213,207	243,666	(30,459)
Loss from operations	(35,534)	(52,411)	16,877	(89,633)	(103,562)	13,929
Other Income (Expense)						
Interest income from licensing	16	19	(3)	49	65	(16)
Investment income	2,252	3,620	(1,368)	5,477	9,500	(4,023)
Interest expense	(7,472)	(13,169)	5,697	(23,973)	(32,732)	8,759
Total other income (expense)	(5,204)	(9,530)	4,326	(18,447)	(23,167)	4,720
Net loss	$ (40,738)	$ (61,941)	$ 21,203	$ (108,080)	$ (126,729)	$ 18,649

Comparison of the Three Months Ended September 30, 2026 and 2025

License and Royalty Revenue. License and royalty revenue decreased by $18.4 million, from $23.6 million for the three months ended September 30, 2025 to $5.2 million for the three months ended September 30, 2026. The decrease was primarily attributable to Zolgensma royalty revenues for the third quarter of 2026.

Combined Zolgensma and Itvisma royalties decreased by $18.3 million, from $23.6 million for the third quarter of 2025 to $5.2 million for the third quarter of 2026. Novartis reported combined Zolgensma and Itvisma sales of $404 million for the third quarter of 2026, as compared to $301 million for the third quarter of 2025. Per Novartis, sales growth was driven by continued launch momentum from Itvisma, while Zolgensma maintained market leadership in the incident SMA population. Zolgensma royalties for the third quarter of 2026 were $2.4 million, a decrease of $21.2 million from the third quarter of 2025, which was primarily attributable to the expiration of licensed patents in the United States in January 2026. We are entitled to continued royalties on net sales of Zolgensma in approximately 20 countries where licensed patents remain active. Itvisma royalties for the third quarter of 2026 were $2.8 million. Itvisma was approved in the United States in the fourth quarter of 2025, with U.S. sales commencing in the first quarter of 2026. Itvisma is now also approved in the EU, Japan and multiple Gulf countries. Licensed patents covering the use of Itvisma have issued in the United States and certain other countries and we are entitled to ongoing royalties on certain net sales of Itvisma in these territories.

Research and Development Expense. Research and development expenses increased by $4.9 million, from $56.1 million for the three months ended September 30, 2025 to $61.0 million for the three months ended September 30, 2026. The increase was primarily attributable to a $7.1 million increase in costs associated with clinical trials and regulatory activities, largely driven by expenses for RGX-202 clinical trials. The increase in research and development expenses was partially offset by a decrease of $1.0 million in personnel-related costs, largely driven by a $0.9 million decrease in stock-based compensation expense.

Gain on Litigation Settlement. In September 2026, we entered into a settlement agreement with Sarepta Therapeutics, Inc. (Sarepta) to resolve all patent litigation matters between the parties. Pursuant to the settlement agreement, we received a $39.0 million settlement payment from Sarepta, which was recorded as other current assets as of September 30, 2026 and received in full in October 2026. We recorded a gain of $38.0 million in the third quarter of 2026 related to the settlement, which consisted of the $39.0 million settlement payment from Sarepta, net of proceeds payable by us to certain licensors as a result of the settlement. For further information regarding the settlement agreement with Sarepta, please refer to Note 8, “Commitments and Contingencies—Sarepta Therapeutics Settlement Agreement” to the accompanying unaudited consolidated financial statements.

Interest Expense. Interest expense decreased by $5.7 million, from $13.2 million for the three months ended September 30, 2025 to $7.5 million for the three months ended September 30, 2026. The decrease was primarily attributable to lower interest costs under our royalty monetization liabilities driven by reduced principal outstanding under the 2020 Royalty Purchase Agreement.

Comparison of the Nine Months Ended September 30, 2026 and 2025

License and Royalty Revenue. License and royalty revenue decreased by $14.9 million, from $129.1 million for the nine months ended September 30, 2025 to $114.2 million for the nine months ended September 30, 2026. The decrease was primarily attributable to $70.0 million of non-recurring, upfront license revenue recognized under our collaboration with Nippon Shinyaku in the first quarter of 2025 and a decrease in Zolgensma royalty revenues for the first nine months of 2026. The decrease in license and royalty revenue was partially offset by $100.0 million of license revenue recognized in the second quarter of 2026 upon the achievement of a development milestone under our sura-vec collaboration with AbbVie for the first patient dosed in the NAAVIGATE trial.

Combined Zolgensma and Itvisma royalties decreased by $44.9 million, from $59.0 million for the nine months ended September 30, 2025 to $14.1 million for the nine months ended September 30, 2026. Novartis reported combined Zolgensma and Itvisma sales of $1.07 billion for the nine months ended September 30, 2026, as compared to $925 million for the nine months ended September 30, 2025. Per Novartis, sales growth was driven by continued launch momentum from Itvisma, while Zolgensma maintained market leadership in the incident SMA population. Zolgensma royalties for the nine months ended September 30, 2026 were $8.9 million, a decrease of $50.1 million from the nine months ended September 30, 2025, which was primarily attributable to the expiration of licensed patents in the United States in January 2026. We are entitled to continued royalties on net sales of Zolgensma in approximately 20 countries where licensed patents remain active. Itvisma royalties for the nine months ended September 30, 2026 were $5.2 million. Itvisma was approved in the United States in the fourth quarter of 2025, with U.S. sales commencing in the first quarter of 2026. Itvisma is now also approved in the EU, Japan and multiple Gulf countries. Licensed patents covering the use of Itvisma have issued in the United States and certain other countries and we are entitled to ongoing royalties on certain net sales of Itvisma in these territories.

Cost of License and Royalty Revenues. Cost of license and royalty revenues decreased by $1.1 million, from $14.4 million for the nine months ended September 30, 2025 to $13.3 million for the nine months ended September 30, 2026. The decrease was largely driven by lower upstream royalties on net sales of Zolgensma, consistent with the decrease in Zolgensma royalty revenues. The decrease in cost of license and royalty revenues was partially offset by a non-recurring charge of $10.0 million in the first quarter of 2026 related to a settlement with GlaxoSmithKline LLC (GSK) to resolve a dispute over sublicense fee obligations under our license agreement with GSK. For further information regarding the settlement agreement with GSK, please refer to Note 8, "Commitments and Contingencies—GlaxoSmithKline—GSK Settlement Agreement" to the accompanying unaudited consolidated financial statements.

Research and Development Expense. Research and development expenses increased by $5.7 million, from $168.7 million for the nine months ended September 30, 2025 to $174.4 million for the nine months ended September 30, 2026. The increase was primarily attributable to the following:

- an increase of $6.9 million in costs associated with clinical trials and regulatory activities, largely driven by expenses for RGX-202 clinical trials; and
- an increase of $0.8 million in personnel-related costs, net of a $1.2 million decrease in stock-based compensation expense.

The increase in research and development expenses was partially offset by a decrease of $1.9 million in manufacturing-related expenses and other clinical supply costs for our lead product candidates.

General and Administrative Expense. General and administrative expenses increased by $3.0 million, from $60.5 million for the nine months ended September 30, 2025 to $63.5 million for the nine months ended September 30, 2026. The increase was largely driven by personnel-related costs and commercialization expenses.

Gain on Litigation Settlement. In September 2026, we entered into a settlement agreement with Sarepta to resolve all patent litigation matters between the parties. Pursuant to the settlement agreement, we received a $39.0 million settlement payment from Sarepta, which was recorded as other current assets as of September 30, 2026 and received in full in October 2026. We recorded a gain of $38.0 million in the third quarter of 2026 related to the settlement, which consisted of the $39.0 million settlement payment from Sarepta, net of proceeds payable by us to certain licensors as a result of the settlement. For further information regarding the settlement agreement with Sarepta, please refer to Note 8, "Commitments and Contingencies—Sarepta Therapeutics Settlement Agreement" to the accompanying unaudited consolidated financial statements.

Interest Expense. Interest expense decreased by $8.8 million, from $32.7 million for the nine months ended September 30, 2025 to $24.0 million for the nine months ended September 30, 2026. The decrease was primarily attributable to lower interest cost under our royalty monetization liabilities driven by reduced principal outstanding under the 2020 Royalty Purchase Agreement. The decrease in interest expense was partially offset by an increase in interest expense under the 2025 Royalty Bond since its issuance in May 2025.

Liquidity and Capital Resources

Sources of Liquidity

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $251.0 million, which were primarily derived from our royalty monetization with HCR in May 2025, milestone payments under our license and collaboration agreements, public offerings of common stock and pre-funded warrants, and sales of common stock under our at-the-market offering program.

In June 2026, we dosed the first patient in the NAAVIGATE trial, resulting in a $100.0 million development milestone payment under our sura-vec collaboration with AbbVie. The $100.0 million milestone payment was received from AbbVie in July 2026.

In July 2026, we completed a public offering of 11,671,139 shares of our common stock at a price of $9.00 per share and 1,111,111 pre-funded warrants to purchase shares of our common stock at a price of $8.9999 per pre-funded warrant, which equaled the public offering price per share of the common stock less the $0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received from the offering were $107.8 million, net of underwriting discounts and commissions and offering expenses.

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 ATM Program). During the second quarter of 2026, we sold 2,318,735 shares of common stock under the ATM Program for aggregate net proceeds of $18.9 million after deducting commissions and offering expenses. No shares of common stock were sold under the ATM Program prior to the second quarter of 2026.

In September 2026, we entered into a settlement agreement with Sarepta to resolve all patent litigation matters between the parties. Pursuant to the settlement agreement, we received a $39.0 million settlement payment from Sarepta in October 2026.

Future Liquidity and Ability to Continue as a Going Concern

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 and commercialization of our product candidates.

We expect that our cash, cash equivalents and marketable securities of $251.0 million as of September 30, 2026, along with the $39.0 million in settlement proceeds received from Sarepta in October 2026, are sufficient to fund our operating expenses and capital expenditure requirements, and to meet our financial commitments and obligations, into the late fourth quarter of 2027. This estimate is based on our current operating plan, and excludes the potential effects of any future financings or material milestone payments that may be received under our licensing and collaboration arrangements. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our capital resources sooner than expected. These conditions raise substantial doubt about our ability to continue as a going concern within 12 months from the issuance date of our consolidated financial statements for the three and nine months ended September 30, 2026 included in this Quarterly Report on Form 10-Q. Our ability to continue as a going concern will depend heavily on the successful development, approval and commercialization of our product candidates and our ability to raise additional capital to fund operations. If we are unable to raise capital sufficient to meet our working capital needs in the future, we may be forced to delay expenditures, reduce the scope of our development activities or make other changes to our operating plans.

Cash Flows

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

	Nine Months Ended September 30,	
	2026	2025
Net cash used in operating activities	$ (99,815)	$ (71,673)
Net cash provided by (used in) investing activities	50,360	(53,406)
Net cash provided by financing activities	110,784	126,355
Net increase in cash and cash equivalents and restricted cash	$ 61,329	$ 1,276

Cash Flows from Operating Activities

Our net cash used in operating activities for the nine months ended September 30, 2026 increased by $28.1 million from the nine months ended September 30, 2025. We received a $100.0 million milestone payment from AbbVie in July 2026 and a $110.0 million up-front payment from Nippon Shinyaku in March 2025, each of which partially offset our net cash used in operating activities for the respective periods. We expect to continue to incur regular net cash outflows from operations for the foreseeable future, which from time to time may be offset by non-recurring payments received under our license and collaboration arrangements, as we continue the development and advancement of our product candidates and other research programs.

For the nine months ended September 30, 2026, our net cash used in operating activities of $99.8 million consisted of a net loss of $108.1 million and unfavorable changes in operating assets and liabilities of $42.9 million, offset by adjustments for non-cash items of $51.1 million. The changes in operating assets and liabilities include an increase in other current assets of $35.4 million, which was primarily attributable to the $39.0 million settlement payment due from Sarepta as of September 30, 2026 and received in October 2026. The unfavorable changes in operating assets and liabilities also include a decrease in accrued expenses and other current liabilities of $9.1 million, which was driven largely by decreases in accrued personnel-related expenses, royalties and sublicense fees, and external research and development services, as well as a decrease in deferred revenue of $7.2 million driven by service revenue recognized under our collaboration with Nippon Shinyaku. The unfavorable changes in operating assets and liabilities were partially offset by a decrease in accounts receivable of $19.8 million, which was driven primarily by a decrease in royalties due from Novartis. 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 $25.8 million, depreciation and amortization expense of $11.1 million and non-cash interest expense of $15.9 million.

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 driven largely 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.

Cash Flows from Investing Activities

For the nine months ended September 30, 2026, our net cash provided by investing activities consisted of $191.3 million in sales and maturities of marketable debt securities, offset by $138.7 million used to purchase marketable debt securities and $2.2 million used to purchase property and equipment.

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.

Cash Flows from Financing Activities

For the nine months ended September 30, 2026, our net cash provided by financing activities primarily consisted of $107.8 million in proceeds received from the public offering of common stock and pre-funded warrants completed in July 2026, net of underwriting discounts and commissions and other offering expenses paid during the period, and $18.9 million in proceeds received from sales of common stock under the ATM Program, net of commissions and offering expenses paid during the period. Net cash provided by financing activities was partially offset by $17.2 million of royalties paid, net of interest, under our royalty monetization liabilities.

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.

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 agreements. 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, 2025, 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, 2025. Other than the changes described in the notes to the unaudited consolidated financial statements accompanying this Quarterly Report on Form 10-Q, including Note 8, "Commitments and Contingencies," there have been no material changes to our commitments and obligations since December 31, 2025.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $1.23 billion as of September 30, 2026. 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;
- delays or costs due to clinical holds or CRLs;
- whether we receive a priority review voucher and are able to monetize or otherwise realize any potential value associated with such a voucher;
- the value of any priority review voucher received, and any diminishing value due to a decrease in demand for these vouchers;
- 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 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 and Itvisma, and the timing and amount of Zolgensma and Itvisma royalties paid to HCR under our royalty monetization agreements;
- revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, other revenue received under our licensing agreements and collaborations, and the timing and amount of any such revenues payable 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 sura-vec 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 otherwise 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, 2025. There have been no material changes to our exposure to market risk during the nine months ended September 30, 2026.

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, 2026. 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, 2026, 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, 2026 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. Please see Note 8, "Commitments and Contingencies—Litigation" to the accompanying unaudited consolidated financial statements for additional information.

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, 2025. There have been no material changes from the risk factors previously disclosed in such filing.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

Rule 10b5-1 Trading Plans

During the three months ended September 30, 2026, none of our directors or Section 16 reporting officers adopted or terminated a contract, instruction or written plan for the purchase or sale of our securities that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act (Rule 10b5-1 Plan) or that constituted a "non-Rule 10b5-1 trading arrangement," as such term is defined in Item 408(a) 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	10-K	3.2	3/5/26	
4.1	Form of Pre-funded Warrant dated July 2026	8-K	4.1	7/20/26	
10.1†	Settlement Agreement dated September 23, 2026 between the Registrant, The Trustees of the University of Pennsylvania, Sarepta Therapeutics, Inc. and affiliated entities, and Catalent, Inc.				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, 2026 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, 2026 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 5, 2026

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

Dated: November 5, 2026

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