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

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 06, 2025

REGENXBIO Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-37553**	**47-1851754**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

9804 Medical Center Drive **Rockville, Maryland** **(Address of Principal Executive Offices)**	**20850** **(Zip Code)**

Registrant's Telephone Number, Including Area Code: (240) 552-8181

N/A
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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 is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

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. ☐

Item 2.02. Results of Operations and Financial Condition.

On November 6, 2025, REGENXBIO Inc. (the "Company") issued a press release regarding its results of operations and financial condition for the quarter ended September 30, 2025. The press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated by reference herein.

The information in Item 2.02 of this Current Report on Form 8-K and Exhibit 99.1 attached hereto shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to liability under that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release dated November 6, 2025 relating to REGENXBIO Inc.'s financial results.
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL.

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 hereunto duly authorized.

REGENXBIO INC.

Date: November 6, 2025

By: /s/ Patrick J. Christmas II
Patrick J. Christmas II

Executive Vice President, Chief Strategy & Legal Officer

REGENXBIO Reports Third Quarter 2025 Financial Results and Operational Highlights

- *RGX-202 program for Duchenne muscular dystrophy advancing rapidly, topline results expected early Q2 2026 and BLA submission mid-2026*
  - *Pivotal trial enrollment completed in October; confirmatory trial open and enrolling*
  - *New 12-month analysis shows all participants demonstrate improved functional outcomes across multiple natural history methods of measurement, when decline is expected*
- *Clemidsogene lanparvovec (RGX-121) on track to be first gene therapy and one-time treatment for MPS II; PDUFA date February 8, 2026*
- *Surabgene lomparvovec (sura-vec, ABBV-RGX-314) on track to be first gene therapy for chronic retinal disease*
  - *Enrollment completed in pivotal trials evaluating subretinal delivery of sura-vec in wet AMD; topline data expected Q4 2026*
  - *Sura-vec for diabetic retinopathy using suprachoroidal delivery advancing to global pivotal program, supported by positive 2-year Phase II trial data*
- *Conference call today at 8:00 a.m. ET*

ROCKVILLE, Md., November 6, 2025 (PR Newswire) -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and operational highlights for the third quarter ended September 30, 2025.

“The positive data and significant clinical milestones achieved across each of our late-stage programs underscore the meaningful progress we’re making towards delivering potentially transformative gene therapies,” said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. “Our strong momentum is driven by our differentiated, in-house end-to-end capabilities, including commercial-ready manufacturing with capacity to seize blockbuster opportunities, and a deep commitment to improve the lives of patients with Duchenne, Hunter syndrome, wet AMD and diabetic retinopathy. With the rapid advancement of our programs and leading technology platform, we are well-positioned to become a commercial company early next year.”

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is a potential best-in-class gene therapy for Duchenne muscular dystrophy (Duchenne). RGX-202 is designed to address the underlying cause of Duchenne by enabling targeted expression of a novel microdystrophin that is closest to naturally occurring dystrophin. It is the only microdystrophin that includes the C-Terminal domain, which has been shown to protect and preserve muscle function. The differentiated therapeutic approach behind RGX-202, including the novel construct, a proactive immune suppression regimen, and suspension-based manufacturing process delivering industry-leading product purity levels, is designed for improved muscle function, durability and safety outcomes for patients.

- Enrollment in the AFFINITY DUCHENNE® pivotal trial of RGX-202 was completed in October (n=30). The confirmatory study will continue enrolling ambulatory patients aged 1+ in the U.S. and Canada, with full clinical supply available.
- New analysis of the positive Phase I/II data reported in June were presented at the International Congress of the World Muscle Society in October and showed individual patient improvement on the North Star Ambulatory Assessment (NSAA) using the established cTAP disease progression model from the Collaborative Trajectory Analysis Project. These results add to the multiple methods evaluating RGX-202 at 12 months post-dosing that demonstrate meaningful functional improvements compared to expected disease trajectory, further supporting the potential of RGX-202 to serve as a differentiated, best-in-class gene therapy.
- REGENXBIO has manufactured the first batches of RGX-202 intended for commercial supply at its in-house Manufacturing Innovation Center and expects to imminently complete its Process Performance Qualification campaign in support of an expected commercial launch in 2027, when the vast majority of the prevalent market is expected to be available.
- REGENXBIO expects to share topline data in early Q2 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026.

Neurodegenerative Disease: Clemidsogene lanparvovec (RGX-121) is a potential first-in-class treatment for MPS II, also known as Hunter syndrome, being developed and potentially commercialized in partnership with Nippon Shinyaku.

- In September, REGENXBIO presented 12-month pivotal data that were submitted to the U.S. Food and Drug Administration (FDA) in response to an information request in the ongoing BLA review of RGX-121. These data further demonstrated the ability of one-time RGX-121 treatment to improve outcomes for patients with MPS II.
    - More than 80% reduction in CSF levels of HS D2S6, a key biomarker of MPS II brain disease, was sustained through 1 year.
    - Strong correlation between HS D2S6 levels and neurodevelopmental outcomes at 1 year, supporting the use of CSF HS D2S6 as a surrogate endpoint that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway.
- FDA PDUFA date is February 8, 2026. FDA approval would result in receipt of a Priority Review Voucher (PRV), to which REGENXBIO has full rights.

Retinal Disease: Surabgene lomparvovec (sura-vec, ABBV-RGX-314), developed in collaboration with AbbVie, is potentially the first-in-class treatment for wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR).

Sura-vec for the Treatment of Wet AMD (Subretinal Delivery)

- Enrollment of more than 1,200 participants in the in the ATMOSPHERE® and ASCENT® pivotal trials was completed in October, representing the largest global gene therapy program ever conducted.
- Topline results are expected in Q4 2026. Sura-vec is on track to be the first approved gene therapy for wet AMD.

Sura-vec for the Treatment of DR (Suprachoroidal Delivery)

- REGENXBIO will initiate a two-part sham injection-controlled Phase IIb/III trial; the primary endpoint will be 2-step DRSS improvement. Site selection is in progress.

- The pivotal program is supported by the durable safety and efficacy profile observed in patients with non-proliferative DR through two years in the Phase II ALTITUDE® trial.

Sura-vec for the Treatment of Wet AMD (Suprachoroidal Delivery)

- The Phase II AAVIATE® trial continues enrolling a cohort to evaluate sura-vec at Dose Level 4 (1.5x10e12 GC/eye).

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $302.0 million as of September 30, 2025, compared to $244.9 million as of December 31, 2024. The increase was primarily attributable to the $110.0 million upfront payment received under the Nippon Shinyaku partnership in March 2025 and $144.5 million in net proceeds received from the royalty monetization with HCRx in May 2025, and was partially offset by cash used to fund operating activities during the nine months ended September 30, 2025.

Revenues: Revenues were $29.7 million for the three months ended September 30, 2025, compared to $24.2 million for the three months ended September 30, 2024. The increase was primarily attributable to $5.9 million of development service revenue under the Nippon Shinyaku partnership in the third quarter of 2025.

Research and Development Expenses: Research and development expenses were $56.1 million for the three months ended September 30, 2025, compared to $54.4 million for the three months ended September 30, 2024. The increase was primarily attributable to personnel costs and manufacturing-related expenses for clemidsogene lanparvovec.

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

Net Income: Net loss was $61.9 million, or $1.20 basic and diluted net loss per share, for the three months ended September 30, 2025, compared to a net loss of $59.6 million, or $1.17 basic and diluted net loss per share, for the three months ended September 30, 2024.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $302.0 million as of September 30, 2025 to fund its operations into early 2027. This cash runway guidance is based on the Company’s current operational plans and excludes the impact of any material payments that may potentially be received from partners or licensees upon the achievement of development or regulatory milestones, or upon the approval or commercialization of product candidates, and excludes potential monetization of a PRV that would be received upon potential approval of clemidsogene lanparvovec.

CONFERENCE CALL

In connection with this announcement, REGENXBIO will host a conference call and webcast at 8:00 a.m. ET today. Listeners can register for the webcast via this **link**. Analysts wishing to participate in the question and answer session should use this **link**. A replay of the webcast will be available via the company's investor website approximately two hours after the call's conclusion. Those who plan on participating are advised to join 15 minutes prior to the start time.

ABOUT REGENXBIO Inc.
REGENXBIO is a biotechnology company on a mission to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the field of AAV gene therapy. REGENXBIO is advancing a late-stage pipeline of one-time treatments for rare and retinal diseases, including RGX-202 for the treatment of Duchenne; clemidsogene lanparvovec (RGX-121) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku; and surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA®. REGENXBIO's investigational gene therapies have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.REGENXBIO.com.

FORWARD-LOOKING STATEMENTS
This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timing or likelihood of payments from AbbVie or Nippon Shinyaku, the monetization of any priority review voucher, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2024, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the SEC and are available on the SEC's website at **WWW.SEC.GOV**. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. 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 REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. Except as required by law, REGENXBIO does not

undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Zolgensma® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands)

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Revenues				
License and royalty revenue	$ 23,605	$ 23,982	$ 129,119	$ 61,172
Service revenue	6,128	215	10,985	942
Total revenues	29,733	24,197	140,104	62,114
Operating Expenses				
Cost of license and royalty revenues	5,725	12,387	14,370	27,249
Research and development	56,101	54,429	168,688	158,142
General and administrative	20,253	19,422	60,483	56,568
Impairment of long-lived assets	—	—	—	2,101
Other operating expenses	65	37	125	32
Total operating expenses	82,144	86,275	243,666	244,092
Loss from operations	(52,411)	(62,078)	(103,562)	(181,978)
Other Income (Expense)				
Interest income from licensing	19	25	65	91
Investment income	3,620	3,276	9,500	9,213
Interest expense	(13,169)	(820)	(32,732)	(3,242)
Total other income (expense)	(9,530)	2,481	(23,167)	6,062
Net loss	$ (61,941)	$ (59,597)	$ (126,729)	$ (175,916)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	98	1,684	89	3,847
Total other comprehensive income	98	1,684	89	3,847
Comprehensive loss	$ (61,843)	$ (57,913)	$ (126,640)	$ (172,069)
Net loss per share, basic and diluted	$ (1.20)	$ (1.17)	$ (2.46)	$ (3.59)
Weighted-average common shares outstanding, basic and diluted	51,689	50,800	51,513	49,051

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