



REGENXBIO Reports Third Quarter 2025 Financial Results and Operational Highlights

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- *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., Nov. 6, 2025 /PRNewswire/ -- 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 (1.5x10¹² 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		<u>19,472</u>		<u>13,774</u>
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		<u>4,268</u>		<u>4,000</u>
Total assets	\$	<u>525,203</u>	\$	<u>465,989</u>

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		<u>41,718</u>		<u>34,309</u>
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		<u>648</u>		<u>3,635</u>
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	<u>(1,058,878)</u>	<u>(932,149)</u>
Total stockholders' equity	<u>161,452</u>	<u>259,651</u>
Total liabilities and stockholders' equity	\$ <u>525,203</u>	\$ <u>465,989</u>

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

	Three Months		Nine Months	
	Ended September 30,		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	<u>\$ (61,941)</u>	<u>\$ (59,597)</u>	<u>\$ (126,729)</u>	<u>\$ (175,916)</u>
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	<u>\$ (61,843)</u>	<u>\$ (57,913)</u>	<u>\$ (126,640)</u>	<u>\$ (172,069)</u>
Net loss per share, basic and diluted	<u>\$ (1.20)</u>	<u>\$ (1.17)</u>	<u>\$ (2.46)</u>	<u>\$ (3.59)</u>
Weighted-average common shares outstanding, basic and diluted	<u>51,689</u>	<u>50,800</u>	<u>51,513</u>	<u>49,051</u>



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