REGENXBIO Reports Fourth Quarter and Full Year 2025 Financial Results and Operational Highlights

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- *Late-stage gene therapy pipeline for rare and retinal diseases advancing toward key catalysts*
  - *RGX-202 for Duchenne muscular dystrophy: New Phase I/II data at MDA, topline pivotal results expected early Q2 2026*
    - *Robust patient enrollment continues in confirmatory trial*
  - *Surabgene lomparvovec (sura-vec, ABBV-RGX-314) subretinal wet AMD topline pivotal data expected in Q4 2026; first patient dosed in pivotal Phase IIb/III trial in diabetic retinopathy expected in Q2 2026*
- *Company to engage FDA on potential path forward for RGX-121*
- *Conference call today at 8:00 a.m. ET*

ROCKVILLE, Md., March 5, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and operational highlights for the fourth quarter and year ended December 31, 2025.

"We are rapidly advancing our late-stage pipeline of gene therapies to treat rare and retinal diseases with significant unmet need, with multiple near-term catalysts in 2026," said Curran Simpson, President and Chief Executive Officer, REGENXBIO. "We will drive continued momentum across our programs – powered by our fully in-house, end-to-end capabilities, commercial-ready manufacturing, and global partners. We remain focused on executing on our mission to deliver meaningful new medicines to patients in need through the curative potential of gene therapy."

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 includes a novel construct, a proactive immune suppression regimen, and a suspension-based manufacturing process that delivers industry-leading product purity levels. RGX-202 is designed for improved muscle function, durability and safety outcomes for patients.

- Positive 18-month functional data from patients treated with the pivotal dose in the Phase I/II portion of the AFFINITY DUCHENNE® trial (n=4) were reported in January 2026. All patients exceeded expected disease trajectory on the North Star Ambulatory Assessment (NSAA) using the established cTAP disease progression model. RGX-202 recipients improved an average of 7.4 points compared to cTAP. These same patients improved an average of 6.6 points compared to cTAP at 12 months post-treatment.
- The Company plans to share additional Phase I/II safety, biomarker, and functional data at the MDA Clinical and Scientific Conference on March 11, 2026.
- REGENXBIO continues to manufacture RGX-202 intended for commercial supply at its in-house Manufacturing Innovation Center.
- REGENXBIO expects to share pivotal topline data in early Q2 2026.
- REGENXBIO plans to request a pre-BLA meeting in mid-2026 to align with FDA on the BLA submission. Additional regulatory interactions with the FDA and European Medical Association (EMA) are planned for 1H 2026.
- Following the completion of enrollment in the pivotal trial (n=30) in October 2025, the Company continues to enroll in the confirmatory trial (n=30) and expects to have the majority of this trial enrolled at the time of BLA submission.

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)

- REGENXBIO expects to share topline data with AbbVie from ATMOSPHERE® and ASCENT® pivotal trials of sura-vec using subretinal delivery in Q4 2026.
- Global regulatory submissions are expected in 2027.

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

- REGENXBIO is activating U.S. clinical sites in the pivotal Phase IIb/III NAAVIGATE study. The Company will receive a $100 million milestone payment from AbbVie upon first patient dosed in the Phase IIb portion, expected Q2 2026.
- NAAVIGATE is a Phase IIb/III multicenter, randomized, masked, sham-controlled study to evaluate the safety and efficacy of sura-vec in subjects with non-proliferative DR (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. Following an interim analysis, REGENXBIO and AbbVie will initiate a Phase III expansion, which will include two Phase III trials, including a U.S. trial and a parallel global trial, led by AbbVie.
- All subjects will receive sura-vec at $1.0x10^{12}$ genome copies (GC)/eye, which was evaluated as Dose Level 3 in the Phase II ALTITUDE® trial of sura-vec, and short-course topical prophylactic steroids.
- At two years post treatment with Dose Level 3 and short-course prophylactic topical steroids in ALTITUDE, no intraocular inflammation was observed (n = 15). The majority of subjects achieved DRSS improvement, with 50% achieving ≥2-step improvement without additional DR treatment. Sura-vec at Dose Level 3 also reduced the risk of disease progression, demonstrating a ≥70% risk reduction in vision-threatening complications compared to historical controls.

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

- Enrollment is complete in the Phase II AAVIATE® trial.

Neurodegenerative Disease: Clemidsogene lanparvovec (RGX-121) is a potential first-in-class treatment for Mucopolysaccharidosis (MPS) Type II, also known as Hunter syndrome. RGX-111 is an investigational one-time treatment for severe MPS I, also known as Hurler syndrome. These programs are partnered with Nippon Shinyaku.

- In January 2026, the FDA placed a clinical hold on RGX-111 following preliminary analysis of a single case of neoplasm (intraventricular CNS tumor) in a participant treated in its Phase I/II study.
- The FDA also placed a clinical hold on RGX-121 citing similarities to RGX-111, study populations, and shared risk between the clinical studies.
- In February 2026, the FDA issued a complete response letter (CRL) for the RGX-121 BLA. REGENXBIO is working to address concerns in the CRL with the goal of resubmitting the BLA.

FINANCIAL RESULTS
Cash Position: Cash, cash equivalents and marketable securities were $240.9 million as of December 31, 2025, compared to $244.9 million as of December 31, 2024. The decrease was primarily driven by cash used to fund operating activities during the year ended December 31, 2025, and was partially offset by 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.

Revenues: Revenues were $30.3 million and $170.4 million for the three months and full year ended December 31, 2025, respectively, compared to $21.2 million and $83.3 million for the three months and full year ended December 31, 2024, respectively. The increases were primarily attributable to revenues recognized under our collaboration with Nippon Shinyaku, including $72.9 million of up-front license revenue and $11.8 million of service revenue recognized in 2025, as well as an increase in royalty revenues for Zolgensma and Itvisma.

Research and Development Expenses: Research and development expenses were $59.6 million and $228.3 million for the three months and full year ended December 31, 2025, respectively, compared to $50.4 million and $208.5 million for the three months and full year ended December 31, 2024,

respectively. The increases were primarily attributable to personnel-related costs and clinical trial expenses, largely driven by RGX-202 pivotal trials, for the fourth quarter and full year 2025, as well as an increase in manufacturing-related expenses Sura-vec, RGX-202 and RGX-121 for the full year 2025.

General and Administrative Expenses: General and administrative expenses were $22.4 million and $82.9 million for the three months and full year ended December 31, 2025, respectively, compared to $20.1 million and $76.6 million for the three months and full year ended December 31, 2024, respectively. The increases were largely driven by professional services, consulting and other corporate advisory services.

Net Loss: Net loss was $67.1 million, or $1.30 basic and diluted net loss per share, for the three months ended December 31, 2025, compared to a net loss of $51.2 million, or $1.01 basic and diluted net loss per share, for the three months ended December 31, 2024. Net loss was $193.9 million, or $3.76 basic and diluted net loss per share, for the year ended December 31, 2025, compared to a net loss of $227.1 million, or $4.59 basic and diluted net loss per share, for the year ended December 31, 2024.

FINANCIAL GUIDANCE
REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $240.9 million as of December 31, 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 any additional potential non-dilutive funding opportunities.

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, regulatory interactions following the complete response letter, 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, 2025, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2026 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.

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

	As of December 31,

	2025	2024
Assets		
Current assets		
Cash and cash equivalents	$ 34,466	$ 57,526
Marketable securities	195,604	177,161
Accounts receivable	26,379	20,473
Prepaid expenses	11,927	9,067
Other current assets	12,905	13,774
Total current assets	281,281	278,001
Marketable securities	10,785	10,179
Accounts receivable	2,312	474
Property and equipment, net	104,855	117,589
Operating lease right-of-use assets	47,156	53,716
Restricted cash	2,030	2,030
Other assets	4,613	4,000
Total assets	$ 453,032	$ 465,989
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 21,358	$ 22,798
Accrued expenses and other current liabilities	38,390	38,070
Deferred revenue	10,452	115
Operating lease liabilities	8,286	7,902
Royalty monetization liabilities	39,609	34,309
Total current liabilities	118,095	103,194
Deferred revenue	18,943	—
Operating lease liabilities	65,215	74,131
Royalty monetization liabilities	147,408	25,378
Other liabilities	638	3,635
Total liabilities	350,299	206,338
Stockholders' equity		
Preferred stock; no shares issued and outstanding at December 31, 2025 and 2024	—	—
Common stock; 50,892 and 49,549 shares issued and outstanding at December 31, 2025 and 2024, respectively	5	5
Additional paid-in capital	1,229,442	1,192,536
Accumulated other comprehensive loss	(687)	(741)
Accumulated deficit	(1,126,027)	(932,149)
Total stockholders' equity	102,733	259,651
Total liabilities and stockholders' equity	$ 453,032	$ 465,989

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

	Three Months Ended December 31,		Years Ended December 31,	
	2025	2024	2025	2024
Revenues				
License and royalty revenue	$ 27,148	$ 20,788	$ 156,267	$ 81,960
Service revenue	3,189	426	14,174	1,368
Total revenues	30,337	21,214	170,441	83,328
Operating Expenses				
Cost of license and royalty revenues	5,928	6,318	20,298	33,567
Research and development	59,611	50,380	228,299	208,522
General and administrative	22,380	20,051	82,863	76,619
Credit losses (recoveries)	—	(5,000)	—	(5,000)
Impairment of long-lived assets	—	—	—	2,101
Other operating expenses	54	833	179	865
Total operating expenses	87,973	72,582	331,639	316,674
Loss from operations	(57,636)	(51,368)	(161,198)	(233,346)
Other Income (Expense)				

Interest income from licensing	18	83	83	174
Investment income	2,745	9,516	12,245	18,729
Interest expense	(12,276)	(9,417)	(45,008)	(12,659)
Total other income (expense)	(9,513)	182	(32,680)	6,244
Net loss	$ (67,149)	$ (51,186)	$ (193,878)	$ (227,102)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(35)	(159)	54	3,688
Total other comprehensive income (loss)	(35)	(159)	54	3,688
Comprehensive loss	$ (67,184)	$ (51,345)	$ (193,824)	$ (223,414)
Net loss per share, basic and diluted	$ (1.30)	$ (1.01)	$ (3.76)	$ (4.59)
Weighted-average common shares outstanding, basic and diluted	51,752	50,871	51,573	49,509

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