REGENXBIO Reports First Quarter 2025 Financial Results and Recent Operational Highlights

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- *RGX-202 in Duchenne muscular dystrophy on track for BLA submission mid-2026*
  - *Pivotal trial more than half enrolled, with completion expected in 2025*
  - *Additional Phase I/II functional data expected 1H 2025*
  - *Initiating commercial supply manufacturing in Q3 2025*
- *FDA acceptance of Biologics License Application (BLA) for clemidsogene lanparvovec (RGX-121, partnered with Nippon Shinyaku) expected in May 2025*
- *Pivotal data evaluating the safety and efficacy of the subretinal delivery of surabgene lomparvovec (ABBV-RGX-314, partnered with AbbVie) in patients with wet age-related macular degeneration are expected in 2026 and planning of diabetic retinopathy pivotal study continues*
- *Conference call today at 4:30 p.m. ET*

ROCKVILLE, Md., May 12, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and recent operational highlights for the first quarter ended March 31, 2025.

"We have made tremendous progress towards delivering multiple commercial gene therapies, starting this year," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "Each of our late-stage assets is demonstrating differentiation against standard of care or available treatments, giving us a pipeline of potential first- or best-in-class gene therapies for rare and retinal diseases. With advanced clinical programs, commercial-ready manufacturing in-house at our Rockville, MD headquarters, and a strong balance sheet, REGENXBIO is well-equipped to deliver potentially transformative gene therapies to patients in need."

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is designed to use the NAV® AAV8 vector to deliver a differentiated, novel microdystrophin gene for improved muscle function and outcomes for patients living with Duchenne. It is the only investigational or approved microdystrophin gene therapy construct to include the C-Terminal (CT) domain found in naturally occurring dystrophin.

- The pivotal Phase I/II/III AFFINITY DUCHENNE® trial of RGX-202 is ongoing in ambulatory patients and is expected to enroll approximately 30 patients aged 1+ in the U.S. and Canada by 2025, with more than half already enrolled to support the pivotal dataset.
- REGENXBIO expects to share topline data in the first half of 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026.
- Commercial readiness activities are underway to support an expected launch in 2027, when the majority of the prevalent market will still be available. REGENXBIO has manufactured RGX-202 supply for both clinical and confirmatory trials with the highest product purity levels in Duchenne gene therapies and expects to initiate commercial supply manufacturing in Q3 2025, with all production sourced in the U.S.
- Data continue to show consistent, robust expression and transduction of RGX-202 microdystrophin across all ages, supported by new biomarker data from two patients who received the pivotal dose of RGX-202 in the Phase I/II portion of the trial, which were presented at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in March.
  - In a patient aged 3 years at dosing, microdystrophin expression was measured to be 122.3% compared to control. In a patient aged 7 years old, RGX-202 microdystrophin

expression was measured to be 31.5% compared to control.
- REGENXBIO expects to share additional functional, efficacy, and safety data in the first half of 2025.

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.

- This month, REGENXBIO expects the U.S. Food and Drug Administration (FDA) to accept the BLA submitted in March 2025, and potential approval of clemidsogene lanparvovec in the second half of 2025. FDA approval could result in receipt of a Priority Review Voucher (PRV), to which REGENXBIO retains full rights.
- Clemidsogene lanparvovec remains on track to be the potential first gene therapy and one-time treatment approved for MPS II.

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 DR (Suprachoroidal Delivery)

- AbbVie and REGENXBIO announced in January 2025 that they will plan a Phase III clinical program. The program is expected to support global regulatory submissions.
- The Phase II ALTITUDE® trial is enrolling a cohort of patients with center-involved diabetic macular edema (DME). Patients will receive a one-time, in-office injection of sura-vec at dose level 4 (1.5x10e12 GC/eye) with short-course prophylactic steroid eye drops.

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

- Enrollment is ongoing in the ATMOSPHERE® and ASCENT™ pivotal trialsREGENXBIO and AbbVie expect to share topline results in 2026. Sura-vec is on track to be the first approved gene therapy for wet AMD.

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

- The Phase II AAVIATE® trial continues enrolling a new cohort to evaluate sura-vec at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short-course prophylactic steroid eye drops.

CORPORATE UPDATES

Partnership with Nippon Shinyaku

- In March 2025, REGENXBIO announced the successful closing of its strategic partnership with Nippon Shinyaku to develop and commercialize clemidsogene lanparvovec for the treatment of MPS II and RGX-111 for the treatment of MPS I in the United States and Asia. Per the agreement, REGENXBIO received $110 million up front and is eligible to receive up to an additional $700 million if certain milestones are achieved.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $272.7 million as of March 31, 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, and was partially offset by cash used to fund operating activities during the first quarter of 2025, which is generally in line with historical first quarter cash spend.

Revenues: Revenues were $89.0 million for the three months ended March 31, 2025, compared to $15.6 million for the three months ended March 31, 2024. The increase was primarily attributable to $71.8 million of license and service revenue recognized under the collaboration with Nippon Shinyaku in the first quarter of 2025.

Research and Development Expenses: Research and development expenses were $53.1 million for the three months ended March 31, 2025, compared to $54.8 million for the three months ended March 31, 2024. The decrease was primarily attributable to clinical trial expenses for sura-vec, clemidsogene lanparvovec, and RGX-202, preclinical activities and other early-stage research and development. The decrease was partially offset by

an increase in manufacturing-related expenses and other clinical supply costs for the Company's lead product candidates.

General and Administrative Expenses: General and administrative expenses were $20.3 million for the three months ended March 31, 2025, compared to $18.3 million for the three months ended March 31, 2024. The increase was primarily attributable to personnel-related costs, expenses for professional services and other corporate overhead costs.

Net Income: Net income was $6.1 million, or $0.12 basic and diluted net income per share, for the three months ended March 31, 2025, compared to a net loss of $63.3 million, or $1.38 basic and diluted net loss per share, for the three months ended March 31, 2024.

FINANCIAL GUIDANCE
REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $272.7 million as of March 31, 2025 to fund its operations into the second half of 2026. 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 4:30 p.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.

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

	March 31, 2025	December 31, 2024
Assets		
Current assets		
Cash and cash equivalents	$ 130,104	$ 57,526

Marketable securities	137,765	177,161
Accounts receivable	18,192	20,473
Prepaid expenses	11,094	9,067
Other current assets	16,082	13,774
Total current assets	313,237	278,001
Marketable securities	4,858	10,179
Accounts receivable	1,208	474
Property and equipment, net	114,497	117,589
Operating lease right-of-use assets	52,112	53,716
Restricted cash	2,030	2,030
Other assets	2,987	4,000
Total assets	$ 490,929	$ 465,989
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 19,044	$ 22,798
Accrued expenses and other current liabilities	23,597	38,070
Deferred revenue	13,588	115
Operating lease liabilities	7,942	7,902
Liability related to sale of future royalties	42,560	34,309
Total current liabilities	106,731	103,194
Deferred revenue	26,297	—
Operating lease liabilities	71,906	74,131
Liability related to sale of future royalties	11,118	25,378
Other liabilities	680	3,635
Total liabilities	216,732	206,338
Stockholders' equity		
Preferred stock; no shares issued and outstanding at March 31, 2025 and December 31, 2024	—	—
Common stock; 50,117 and 49,549 shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	1,201,020	1,192,536
Accumulated other comprehensive loss	(762)	(741)
Accumulated deficit	(926,066)	(932,149)
Total stockholders' equity	274,197	259,651
Total liabilities and stockholders' equity	$ 490,929	$ 465,989

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

	Three Months Ended March 31,	
	2025	**2024**
Revenues		
License and royalty revenue	$ 87,049	$ 15,344
Service revenue	1,963	278
Total revenues	89,012	15,622
Operating Expenses		
Cost of license and royalty revenues	3,436	4,283
Research and development	53,087	54,844
General and administrative	20,347	18,291
Impairment of long-lived assets	—	2,101
Other operating expenses (income)	15	(34)
Total operating expenses	76,885	79,485
Income (loss) from operations	12,127	(63,863)
Other Income (Expense)		
Interest income from licensing	25	37
Investment income	2,501	2,469
Interest expense	(8,570)	(1,973)
Total other income (expense)	(6,044)	533
Net income (loss)	$ 6,083	$ (63,330)

Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net	(21)	1,200
Total other comprehensive income (loss)	(21)	1,200
Comprehensive income (loss)	$ 6,062	$ (62,130)
Net income (loss) per share:		
Basic	$ 0.12	$ (1.38)
Diluted	$ 0.12	$ (1.38)
Weighted-average common shares outstanding:		
Basic	51,362	45,733
Diluted	51,434	45,733

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