REGENXBIO Reports Fourth Quarter and Full Year 2024 Financial Results and Recent Operational Updates

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- *Biologics Licensing Application (BLA) for clemidsogene lanparvovec (RGX-121) submitted and on track for potential FDA approval 2H 2025; strategic partnership with Nippon Shinyaku aims to expand potential access and commercial opportunity in MPS II and MPS I*
- *Pivotal trial of RGX-202 for Duchenne Muscular Dystrophy progressing rapidly; enrollment completion expected in 2025 with BLA filing in mid-2026*
- *AbbVie-partnered retinal franchise continues advancing; pivotal data evaluating the safety and efficacy of the subretinal delivery of surabgene lomparvovec (ABBV-RGX-314) 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., March 13, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and recent operational highlights for the fourth quarter and year ended December 31, 2024.

"REGENXBIO is on the cusp of delivering potential first- or best-in-class gene therapies to market. In 2025, we have already submitted our first BLA and will accelerate late-stage development and advance commercial preparations for multiple potential product launches," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "RGX-202 is progressing rapidly through pivotal study, continues to demonstrate a differentiated profile, and remains on track to be the next gene therapy in Duchenne. Our partnership with AbbVie continues to advance multiple large, global commercial opportunities. Now partnered with Nippon Shinyaku, RGX-121 is poised to bring another meaningful revenue stream to REGENXBIO if approved in the coming months. We remain focused on bringing multiple potentially transformative new medicines to patients."

PROGRAM HIGHLIGHTS AND MILESTONES

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

- In March 2025, REGENXBIO completed its Biologics License Application (BLA) submission to the U.S. Food and Drug Administration (FDA), seeking accelerated approval of clemidsogene lanparvovec.
- REGENXBIO expects potential approval of clemidsogene lanparvovec in the second half of 2025.
- FDA approval could result in receipt of a Priority Review Voucher (PRV). REGENXBIO retains full rights to the PRV.
- Clemidsogene lanparvovec remains on track to be the potential first gene therapy and one-time treatment approved for MPS II.

Neuromuscular Disease: RGX-202 is designed to deliver a differentiated, novel microdystrophin gene for improved function and outcomes for patients living with Duchenne.

- The pivotal Phase I/II/III AFFINITY DUCHENNE® trial is ongoing in ambulatory patients and will enroll approximately 30 patients aged 1+ in the U.S. and Canada. In November 2024, REGENXBIO announced the first patient had been dosed in the pivotal phase.
- The pivotal trial is nearly 50% enrolled, and REGENXBIO expects to complete enrollment in the study in 2025, share top line data in the first half of 2026, and submit a BLA under the accelerated approval pathway in mid-2026.

- Results to date from the ongoing Phase I/II trial demonstrate a favorable safety profile with no SAEs or AEs of special interest, robust microdystrophin expression, and improved functional outcomes at 9 and 12 months, supporting the potential of RGX-202 to be a differentiated gene therapy in Duchenne. The latest results were published in November 2024 (press release).
- REGENXBIO expects to share additional Phase I/II biomarker data at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, including the first biomarker data from the cohort of patients aged 1-3. The company expects to share additional efficacy and safety data, including additional functional data, in the first half of 2025.

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.
- Enrollment in the Phase II fellow eye sub-study evaluating the subretinal delivery of sura-vec in patients with bilateral wet AMD is complete. Positive results from this study were presented at the 2024 American Academy of Ophthalmology annual meeting, supporting the potential of sura-vec to treat bilateral disease at an expected commercial launch (press release).

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

- In March 2025, REGENXBIO announced the successful closing of its strategic partnership with Nippon Shinyaku to develop and commercialize RGX-121 for the treatment of MPS II and RGX-111 for MPS I in the United States and Asia. Per the agreement, REGENXBIO will receive $110 million up front and up to an additional $700 million if certain milestones are achieved.
- REGENXBIO announced the promotions of two key leaders:
  - Ram Palanki, PharmD, from Executive Vice President of Commercial Strategy and Operations to Executive Vice President, Chief Commercial Officer. Dr. Palanki joined the company in 2018.
  - Craig Malzahn from Senior Vice President, Technical Operations, to Executive Vice President, Product Development and Chief Technology Officer. Mr. Malzahn joined the company in 2019.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $244.9 million as of December 31, 2024, compared to $314.1 million as of December 31, 2023. The decrease was primarily driven by cash used to fund operating activities during the year ended December 31, 2024, and was partially offset by $131.1 million of aggregate net proceeds received from the follow-on public offering of the Company's common stock and pre-funded warrants completed in March 2024.

Revenues: Revenues were $21.2 million and $83.3 million for the three months and full year ended December 31, 2024, respectively, compared to $22.2 million and $90.2 million for the three months and full year ended December 31, 2023, respectively. The decreases were primarily attributable to Zolgensma royalty revenues, which decreased from $85.3 million in 2023 to $81.5 million in 2024.

Research and Development Expenses: Research and development expenses were $50.4 million and $208.5 million for the three months and full year ended December 31, 2024, respectively, compared to $55.7 million and $232.3 million for the three months and full year ended December 31, 2023, respectively. The decrease for the full year ended December 31, 2024 was primarily attributable to lower personnel-related costs, manufacturing and clinical supply costs for lead product candidates and early-stage research and development activities, and was partially offset by increases in clinical trial expenses for ABBV-RGX-314 and RGX-202. The decrease for the fourth quarter of 2024 was largely driven by personnel-related costs and clinical trial expenses as compared to the fourth quarter of 2023.

General and Administrative Expenses: General and administrative expenses were $20.1 million and $76.6 million for the three months and full year ended December 31, 2024, respectively, compared to $19.1 million and $88.5 million for the three months and full year ended December 31, 2023, respectively. The decrease for the full year ended December 31, 2024 was primarily attributable professional services, consulting fees and other corporate overhead expenses. The increase for the fourth quarter of 2024 was largely driven by stock-based compensation expense as compared to the fourth quarter of 2023.

Net Loss: Net loss was $51.2 million, or $1.01 basic and diluted net loss per share, for the three months ended December 31, 2024, compared to a net loss of $62.9 million, or $1.43 basic and diluted net loss per share, for the three months ended December 31, 2023. Net loss was $227.1 million, or $4.59 basic and diluted net loss per share, for the year ended December 31, 2024, compared to a net loss of $263.5 million, or $6.02 basic and diluted net loss per share, for the year ended December 31, 2023.

FINANCIAL GUIDANCE
REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $244.9 million as of December 31, 2024 and the $110.0 million upfront payment to be received under the Nippon Shinyaku collaboration 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.

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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2025, 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)

	December 31, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 57,526	$ 34,522
Marketable securities	177,161	240,736
Accounts Receivable, net	20,473	24,790
Prepaid expenses	9,067	14,520
Other current assets	13,774	20,403
Total current assets	278,001	334,971
Marketable securities	10,179	38,871
Accounts receivable	474	701
Property and equipment, net	117,589	132,103
Operating lease right-of-use assets	53,716	60,487
Restricted cash	2,030	2,030
Other assets	4,000	4,807
Total assets	$ 465,989	$ 573,970
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 22,798	$ 22,786
Accrued expenses and other current liabilities	38,070	49,703
Deferred revenue	115	148
Operating lease liabilities	7,902	7,068
Liability related to sale of future royalties	34,309	50,567
Total current liabilities	103,194	130,272
Operating lease liabilities	74,131	82,222
Liability related to sale of future royalties	25,378	43,485
Other liabilities	3,635	6,249
Total liabilities	206,338	262,228
Stockholders' equity		
Preferred stock; no shares issued and outstanding at December 31, 2024 and December 31, 2023	—	—
Common stock; 49,549 and 44,046 shares issued and outstanding at December 31, 2024 and December 31, 2023, respectively	5	4
Additional paid-in capital	1,192,536	1,021,214
Accumulated other comprehensive loss	(741)	(4,429)
Accumulated deficit	(932,149)	(705,047)
Total stockholders' equity	259,651	311,742
Total liabilities and stockholders' equity	$ 465,989	$ 573,970

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,	
	2024	2023	2024	2023
Revenues				
License and royalty revenue	$ 21,214	$ 22,213	$ 83,328	$ 90,242
Total revenues	21,214	22,213	83,328	90,242
Operating Expenses				
Cost of revenues	6,318	11,238	33,567	37,213
Research and development	50,380	55,681	208,522	232,266
General and administrative	20,051	19,079	76,619	88,494

Credit losses (recoveries)	(5,000)	—	(5,000)	—
Impairment of long-lived assets	—	—	2,101	—
Other operating expenses	833	118	865	397
Total operating expenses	72,582	86,116	316,674	358,370
Loss from operations	(51,368)	(63,903)	(233,346)	(268,128)
Other Income (Expense)				
Interest income from licensing	83	(141)	174	25
Investment income	9,516	2,366	18,729	11,319
Interest expense	(9,417)	(1,363)	(12,659)	(6,862)
Total other income	182	862	6,244	4,482
Loss before income taxes	(51,186)	(63,041)	(227,102)	(263,646)
Income Tax Benefit	—	152	—	152
Net loss	$ (51,186)	$ (62,889)	$ (227,102)	$ (263,494)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(159)	2,984	3,688	10,972
Total other comprehensive income (loss)	(159)	2,984	3,688	10,972
Comprehensive loss	$ (51,345)	$ (59,905)	$ (223,414)	$ (252,522)
Net loss per share, basic and diluted	$ (1.01)	$ (1.43)	$ (4.59)	$ (6.02)
Weighted-average common shares outstanding, basic and diluted	50,871	44,001	49,509	43,734

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