REGENXBIO Reports First Quarter 2024 Financial Results and Recent Operational Highlights

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- *Company remains on track for its first BLA filing in 2024 and is accelerating progress toward pivotal trial initiation for Duchenne (H2 2024) and diabetic retinopathy (H1 2025)*
- *Dose level 2 selected as pivotal dose for RGX-202 treatment of Duchenne*
  - *New positive interim efficacy and safety data announced for second boy at DL2*
  - *AFFINITY DUCHENNE® trial expansion phase is underway with third and fourth boys dosed at DL2*
  - *End-of-Phase II meeting with FDA planned for early Q3 and pivotal trial initiation anticipated in late Q3 to early Q4 2024*
- *Positive interim results from ALTITUDE® trial for diabetic retinopathy support plans for anticipated End-of-Phase II meeting with FDA in Q1 2025*
- *$381 million in cash, cash equivalents and marketable securities as of March 31, 2024, expected to fund operational runway into 2026*
- *Conference call Wednesday, May 8, at 4:30 p.m. ET*

ROCKVILLE, Md., May 8, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the first quarter ending March 31, 2024. Recent operational highlights, including acceleration of the prioritized pipeline supports meaningful value generation from the Company's strong portfolio of AAV Therapeutics.

"Exciting program and data updates continue in 2024 and demonstrate remarkable progress on how REGENXBIO is rapidly advancing products through late-stage development," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We expect to make significant progress across our prioritized pipeline, including advancing our Duchenne program into pivotal stage and filing a BLA for RGX-121 this year, as well as finalizing plans to initiate a pivotal program in diabetic retinopathy early next year. Regardless of today's landscape of Duchenne treatments, communication from the FDA continues to support the need for alternative gene therapies for rare diseases, including Duchenne. Our new, positive biomarker data from the AFFINITY DUCHENNE trial demonstrates the potential of RGX-202 as a meaningful and differentiated treatment option for the Duchenne community."

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is a potential one-time AAV therapeutic for the treatment of Duchenne.

- As of May 3, 2024, RGX-202 continues to be well tolerated in all patients with no serious adverse events.
- In new data announced today from the second patient, aged 8.1 years, who received RGX-202 at dose level 2, RGX-202 microdystrophin expression was measured to be 20.9% compared to control at three months. A reduction from baseline in serum creatinine kinase (CK) levels of 90% was observed at 10 weeks.
- Dose level 2 has been selected as the pivotal dose and the positive interim results enable rapid acceleration into pivotal development.
- REGENXBIO is now enrolling patients in an expedited dose level 2 expansion phase of the AFFINITY DUCHENNE trial accepted by the U.S. Food and Drug Administration (FDA), and recently dosed two additional boys aged 5.8 and 8.5 years old. The Company expects to enroll up to a total of seven patients at the pivotal dose through early Q3 2024.
- An end-of-Phase II (EOP2) meeting with FDA in early Q3 2024 is expected to support a final pivotal trial design. REGENXBIO plans to use RGX-202 microdystrophin expression as a surrogate endpoint likely to predict clinical benefit to support a Biologics License Application (BLA) filing using the accelerated approval pathway. Initiation of the pivotal trial is expected in

late Q3 2024 to early Q4 2024.
- REGENXBIO also continues to expect to share initial strength and functional assessment data for both dose levels of the AFFINITY DUCHENNE trial in the second half of 2024.

Retinal Disease: ABBV-RGX-314, in collaboration with AbbVie, is a potential one-time treatment for wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal disease.

- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of DR
  - Based on positive interim results from the Phase II ALTITUDE trial to date, design and evaluation of two pivotal trials is on-going. These results also support discussion with the FDA at an EOP2 meeting anticipated in Q1 2025 that can enable rapid acceleration towards pivotal development. The Company expects to initiate the first pivotal trial in the first half of 2025.
- ABBV-RGX-314 Subretinal Delivery for the Treatment of Wet AMD
  - Enrollment is on track in ATMOSPHERE® and ASCENT™ pivotal trials and these trials are expected to support global regulatory submissions with the FDA and the European Medicines Agency in the first half of 2026.
- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of Wet AMD
  - REGENXBIO expects to share new program and data updates for the Phase II AAVIATE® trial in Q3 2024.

Neurodegenerative Disease: RGX-121 is a potential one-time AAV therapeutic for the treatment of boys with MPS II.

- On track to file a BLA in 2024 using the accelerated approval pathway. Approval of the planned BLA could result in receipt of a Priority Review Voucher in 2025.

NAV® TECHNOLOGY PLATFORM LICENSEE PROGRAM HIGHLIGHTS

Novartis AG reported first quarter 2024 global sales of Zolgensma, for the treatment of spinal muscular atrophy, of $295 million. Novartis, Rocket Pharmaceuticals and Ultragenyx Pharmaceutical all have investigational AAV Therapeutics in pivotal phase that have multiple milestones expected throughout 2024. Eli Lilly is also developing several AAV Therapeutics in Phase II for neurodegenerative diseases using REGENXBIO NAV Technology.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $380.5 million as of March 31, 2024, compared to $314.1 million as of December 31, 2023. The increase was primarily attributable to $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, and was partially offset by cash used to fund operating activities during the first quarter of 2024.

Revenues: Revenues were $15.6 million for the three months ended March 31, 2024, compared to $19.1 million for the three months ended March 31, 2023. The decrease was primarily attributable to non-recurring development milestone revenue recognized in the first quarter of 2023, as well as Zolgensma royalty revenues, which decreased from $16.1 million in the first quarter of 2023 to $15.2 million in the first quarter of 2024.

Research and Development Expenses: Research and development expenses were $54.8 million for the three months ended March 31, 2024, compared to $58.5 million for the three months ended March 31, 2023. The decrease was primarily attributable to manufacturing and clinical supply costs for ABBV-RGX-314 and RGX-202, and personnel-related costs as a result of reduced headcount of research and development personnel. The decrease was partially offset by increased clinical trial expenses across the Company's lead product candidates.

General and Administrative Expenses: General and administrative expenses were $18.3 million for the three months ended March 31, 2024, compared to $22.6 million for the three months ended March 31, 2023. The decrease was primarily attributable to personnel-related costs as a result of reduced headcount, expenses for professional services and other corporate overhead costs.

Net Loss: Net loss was $63.3 million, or $1.38 basic and diluted net loss per share, for the three months ended March 31, 2024, compared to a net loss of $66.7 million, or $1.53 basic and diluted net loss per share, for the three months ended March 31, 2023.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $380.5 million as of March 31, 2024 to fund its operations into 2026. This cash runway guidance is based on the Company's current operational plans and excludes the impact of any payments that may be received from AbbVie upon the achievement of development or commercial milestones under our ABBV-RGX-314 collaboration (including a potential, one-time $200.0 million milestone for achievement of first patient dosed in the first pivotal trial for suprachoroidal delivery for treatment of DR) and the potential monetization of a priority review voucher that may be received for RGX-121.

CONFERENCE CALL

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.m. ET. 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 leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for retinal and rare diseases, including ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, RGX-202 for the treatment of Duchenne and RGX-121 for the treatment of MPS II.

Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA® for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics 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, 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, 2023, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (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.

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

	March 31, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 112,975	$ 34,522
Marketable securities	225,728	240,736
Accounts receivable, net	15,828	24,790
Prepaid expenses	13,590	14,520
Other current assets	27,297	20,403
Total current assets	395,418	334,971
Marketable securities	41,807	38,871
Accounts receivable	523	701
Property and equipment, net	127,662	132,103
Operating lease right-of-use assets	57,558	60,487
Restricted cash	2,030	2,030
Other assets	4,217	4,807
Total assets	$ 629,215	$ 573,970
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 31,356	$ 22,786
Accrued expenses and other current liabilities	33,129	49,703
Deferred revenue	13	148
Operating lease liabilities	7,066	7,068
Liability related to sale of future royalties	38,615	50,567
Total current liabilities	110,179	130,272

Operating lease liabilities	80,183	82,222
Liability related to sale of future royalties	44,702	43,485
Other liabilities	3,485	6,249
Total liabilities	238,549	262,228
Stockholders' equity		
Preferred stock; no shares issued and outstanding at March 31, 2024 and December 31, 2023	—	—
Common stock; 49,043 and 44,046 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	5	4
Additional paid-in capital	1,162,267	1,021,214
Accumulated other comprehensive loss	(3,229)	(4,429)
Accumulated deficit	(768,377)	(705,047)
Total stockholders' equity	390,666	311,742
Total liabilities and stockholders' equity	$ 629,215	$ 573,970

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

	Three Months Ended March 31,	
	2024	**2023**
Revenues		
License and royalty revenue	$ 15,622	$ 19,138
Total revenues	15,622	19,138
Operating Expenses		
Cost of revenues	4,283	4,112
Research and development	54,844	58,516
General and administrative	18,291	22,634
Impairment of long-lived assets	2,101	—
Other operating expenses (income)	(34)	33
Total operating expenses	79,485	85,295
Loss from operations	(63,863)	(66,157)
Other Income (Expense)		
Interest income from licensing	37	70
Investment income	2,469	2,166
Interest expense	(1,973)	(2,755)
Total other income (expense)	533	(519)
Net loss	$ (63,330)	$ (66,676)
Other Comprehensive Income		
Unrealized gain on available-for-sale securities, net	1,200	3,779
Total other comprehensive income	1,200	3,779
Comprehensive loss	$ (62,130)	$ (62,897)
Net loss per share, basic and diluted	$ (1.38)	$ (1.53)
Weighted-average common shares outstanding, basic and diluted	45,733	43,451

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