REGENXBIO Reports Fourth Quarter and Full Year 2023 Financial Results and Recent Operational Highlights

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- *Focus on clinical stage AAV Therapeutic product candidates addressing large commercial opportunities and value generation*
- *Prioritized pipeline is expected to further progress to pivotal stage and first BLA filing in 2024*
- *New updates planned for Duchenne and in-office delivery retinal disease programs starting in March*
- *$314 million in cash, cash equivalents and marketable securities as of December 31, 2023, expected to fund operational runway into the second half of 2025*
- *Conference call Tuesday, February 27, at 4:30 p.m. ET*

ROCKVILLE, Md., Feb. 27, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the fourth quarter and full year ended December 31, 2023, and recent operational highlights, including progress on the strategic pipeline prioritization and corporate restructuring intended to significantly reduce operating expenses and support meaningful value generation from the Company's strong pipeline of AAV Therapeutics.

"We started 2024 with amazing data from our AbbVie-partnered eye care programs and our treatments for Duchenne and Hunter syndrome. Our strategic pipeline prioritization at the end of 2023 created a sharpened focus for us and today we are rapidly advancing products through late-stage clinical trials. We believe this is the best way to support the creation of meaningful value," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We expect more important data readouts and milestones to be achieved across all programs and the initiation of new pivotal trials this year. 2024 will be a turning point in our journey to bring ground-breaking AAV Therapeutics to millions of patients."

PROGRAM HIGHLIGHTS AND MILESTONES

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 that is designed to continually produce an anti-VEGF protein in the eye. ABBV-RGX-314 is currently being evaluated in patients with wet AMD and DR in nine ongoing clinical trials, including two pivotal trials. A single ABBV-RGX-314 treatment has the potential to become a new standard-of-care option among anti-VEGF treatments by sustaining vision health long term and overcoming the clinical challenges of managing retinal disease due to the treatment burden of chronic anti-VEGF injections.

- 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 U.S. Food and Drug Administration and the European Medicines Agency in late 2025 through 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 mid-2024.
  - In January 2024, REGENXBIO presented data from the AAVIATE® trial demonstrating that, at six months, patients treated with ABBV-RGX-314 continue to demonstrate stable vision and retinal anatomy while a meaningful reduction in anti-VEGF treatment burden was observed. The highest reduction was seen in dose level 3, demonstrating an 80% reduction in annualized injection rate with 50% of patients remaining injection-free.
- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of DR
  - REGENXBIO expects to share new program and data updates for the Phase II ALTITUDE® trial in Q2 2024.
  - In November 2023, REGENXBIO presented data from the ALTITUDE trial showing that,

at one year, dose level 2 in non-proliferative DR patients prevented disease progression as measured by the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale. Dose level 2 reduced the risk of patients developing vision-threatening events by 89%.

Neuromuscular Disease: RGX-202 is an investigational one-time AAV therapeutic designed to meaningfully impact disease by delivering a gene for a novel microdystrophin with important biology most similar to naturally occurring dystrophin that protects from the muscle degradation associated with Duchenne.

- REGENXBIO plans to share new updates from the Phase I/II AFFINITY DUCHENNE® trial at the Muscular Dystrophy Association Clinical and Scientific Meeting being held next week in Orlando, FL and virtually.
- REGENXBIO expects to make a pivotal dose determination in mid-2024. The Company also expects to share initial strength and functional assessment data for both dose levels and the initiation of a pivotal program in the second half of 2024.
- In February 2024, REGENXBIO reported interim data from the AFFINITY DUCHENNE trial, demonstrating that all three patients at dose level 1 indicate encouraging increases in expression of RGX-202 microdystrophin at three months and reduction from baseline in serum creatinine kinase levels, supporting evidence of clinical improvement. In the third patient, RGX-202 microdystrophin expression was measured to be 83.4%.
- REGENXBIO plans to use RGX-202 microdystrophin expression as a surrogate endpoint to support a Biologics License Application (BLA) filing using the accelerated approval pathway.

Neurodegenerative Disease: RGX-121 is an investigational one-time AAV therapeutic designed to change the course of disease by restoring the gene missing in 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.
- In February 2024, REGENXBIO reported the pivotal phase of the CAMPSIITE® trial achieved its primary endpoint, as treated patients achieved decreased cerebrospinal fluid (CSF) levels of D2S6 below maximum attenuated disease levels at 16 weeks (p value of 0.00016). Patients treated with RGX-121 have showed continued improvement in neurodevelopmental skill acquisition up to four years and discontinued intravenous enzyme therapy.

NAV® TECHNOLOGY PLATFORM LICENSEE PROGRAM HIGHLIGHTS

Novartis AG reported fourth quarter and full year 2023 global sales of Zolgensma, for the treatment of spinal muscular atrophy, of $286 million and $1.21 billion, respectively. 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 $314.1 million as of December 31, 2023, compared to $565.2 million as of December 31, 2022. The decrease was primarily driven by cash used to fund operating activities during the year ended December 31, 2023.

Revenues: Revenues were $22.2 million and $90.2 million for the three months and full year ended December 31, 2023, respectively, compared to $31.3 million and $112.7 million for the three months and full year ended December 31, 2022, respectively. The decreases were primarily attributable to Zolgensma royalty revenues, which decreased from $101.9 million for the year ended December 31, 2022 to $85.3 million for the year ended December 31, 2023.

Research and Development Expenses: Research and development expenses were $55.7 million and $232.3 million for the three months and full year ended December 31, 2023, respectively, compared to $62.5 million and $242.5 million for the three months and full year ended December 31, 2022, respectively. The decreases were primarily attributable to clinical trial and manufacturing expenses for ABBV-RGX-314 resulting from an increase in development cost reimbursement from AbbVie under our eye care collaboration and were partially offset by increased clinical trial expenses for our other lead product candidates.

General and Administrative Expenses: General and administrative expenses were $19.1 million and $88.5 million for the three months and full year ended December 31, 2023, respectively, compared to $21.2 million and $85.3 million for the three months and full year ended December 31, 2022, respectively. The increase for the full year ended December 31, 2023 was primarily attributable to personnel-related costs, expenses for professional services and other corporate overhead costs, and was partially offset by a decrease in these costs in the fourth quarter of 2023 as compared to the fourth quarter of 2022.

Net Loss: Net loss was $62.9 million, or $1.43 basic and diluted net loss per share, for the three months ended December 31, 2023, compared to a net

loss of $59.9 million, or $1.38 basic and diluted net loss per share, for the three months ended December 31, 2022. Net loss was $263.5 million, or $6.02 basic and diluted net loss per share, for the year ended December 31, 2023, compared to a net loss of $280.3 million, or $6.50 basic and diluted net loss per share, for the year ended December 31, 2022.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $314.1 million as of December 31, 2023 to fund its operations into the second half of 2025. 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.

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 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, which will be filed with the SEC in the first quarter of 2024 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)

	December 31, 2023	December 31, 2022
Assets		
Current assets		
Cash and cash equivalents	$ 34,522	$ 96,952
Marketable securities	240,736	267,690
Accounts receivable, net	24,790	28,082
Prepaid expenses	14,520	13,900
Other current assets	20,403	9,352
Total current assets	334,971	415,976
Marketable securities	38,871	200,560
Accounts receivable, net	701	1,504
Property and equipment, net	132,103	141,685
Operating lease right-of-use assets	60,487	65,116

Restricted cash	2,030	2,030
Other assets	4,807	6,397
Total assets	$ 573,970	$ 833,268
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 22,786	$ 27,213
Accrued expenses and other current liabilities	49,703	46,794
Deferred revenue	148	1,829
Operating lease liabilities	7,068	5,997
Liability related to sale of future royalties	50,567	48,601
Total current liabilities	130,272	130,434
Operating lease liabilities	82,222	88,802
Liability related to sale of future royalties	43,485	89,005
Other liabilities	6,249	8,832
Total liabilities	262,228	317,073
Stockholders' equity		
Preferred stock; no shares issued and outstanding at December 31, 2023 and 2022	—	—
Common stock; 44,046 and 43,299 shares issued and outstanding at December 31, 2023 and 2022, respectively	4	4
Additional paid-in capital	1,021,214	973,145
Accumulated other comprehensive loss	(4,429)	(15,401)
Accumulated deficit	(705,047)	(441,553)
Total stockholders' equity	311,742	516,195
Total liabilities and stockholders' equity	$ 573,970	$ 833,268

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,	
	2023	**2022**	**2023**	**2022**
Revenues				
License and royalty revenue	$ 22,213	$ 31,345	$ 90,242	$ 112,724
Total revenues	22,213	31,345	90,242	112,724
Operating Expenses				
Cost of revenues	11,238	12,783	37,213	54,545
Research and development	55,681	62,505	232,266	242,453
General and administrative	19,079	21,210	88,494	85,281
Other operating expenses (income)	118	(7,382)	397	(6,679)
Total operating expenses	86,116	89,116	358,370	375,600
Loss from operations	(63,903)	(57,771)	(268,128)	(262,876)
Other Income (Expense)				
Interest income from licensing	(141)	77	25	342
Investment income	2,366	2,026	11,319	5,383
Interest expense	(1,363)	(4,310)	(6,862)	(23,254)
Total other income (expense)	862	(2,207)	4,482	(17,529)
Loss before income taxes	(63,041)	(59,978)	(263,646)	(280,405)
Income Tax Benefit	152	43	152	84
Net loss	$ (62,889)	$ (59,935)	$ (263,494)	$ (280,321)
Other Comprehensive Loss				
Unrealized gain (loss) on available-for-sale securities, net	2,984	2,855	10,972	(12,832)
Total other comprehensive income (loss)	2,984	2,855	10,972	(12,832)
Comprehensive loss	$ (59,905)	$ (57,080)	$ (252,522)	$ (293,153)
Net loss per share, basic and diluted	$ (1.43)	$ (1.38)	$ (6.02)	$ (6.50)
Weighted-average common shares outstanding, basic and diluted	44,001	43,296	43,734	43,152

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