REGENXBIO Reports Second 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)*
  - *New, positive data from Phase II AFFINITY DUCHENNE® trial of RGX-202 demonstrates consistent high expression of microdystrophin across treated patients in all age groups*
  - *Successful End-of-Phase II meeting with FDA for RGX-202 supports plans for using the accelerated approval pathway and pivotal initiation in Q4 2024*
  - *End-of-Phase II meeting for ABBV-RGX-314 for diabetic retinopathy accelerated to Q4 2024 to support global pivotal program initiation in H1 2025*
- *$327 million in cash, cash equivalents and marketable securities as of June 30, 2024, expected to fund operational runway into 2026*
- *Conference call Thursday, August 1, at 4:30 p.m. ET*

ROCKVILLE, Md., Aug. 1, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the second quarter ending June 30, 2024. Recent operational highlights, including acceleration of the late-stage pipeline, support meaningful value generation from the Company's leading portfolio of AAV Therapeutics.

"In the first half of 2024, REGENXBIO has made remarkable progress in accelerating and advancing the development of AAV Therapeutics that are well-positioned to make a profound impact for patients," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "Looking ahead, we have multiple, exciting catalysts across our pipeline, including enrolling our first patient aged 1-3 in the AFFINITY DUCHENNE® trial and advancing RGX-202 into pivotal stage to address the significant ongoing unmet need in this community, initiating a rolling BLA for RGX-121 as the only one-time treatment for Hunter syndrome, and accelerating our End-of-Phase II meeting with the FDA for ABBV-RGX-314 in diabetic retinopathy. Each of these programs represent differentiated therapies that we expect will drive significant value."

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is an investigational one-time AAV Therapeutic designed to deliver a novel microdystrophin gene for improved function and outcomes in Duchenne.

- REGENXBIO recently announced new, positive data from two patients treated with dose level 2 (DL2), demonstrating consistent, high microdystrophin expression across treated patients in the Phase II AFFINITY DUCHENNE® trial of RGX-202. In patients aged 5.8 and 8.5 at dosing, RGX-202 microdystrophin expression was measured to be 77.2%, and 46.5%, respectively, compared to control at three months. As of July 8, 2024, RGX-202 has been well tolerated with no serious adverse events. All seven patients who completed three-month trial assessments indicate meaningful increases in expression of RGX-202 microdystrophin and reduction from baseline in serum creatinine kinase levels, supporting evidence of clinical improvement.
- REGENXBIO expects to complete enrollment in the DL2 expansion cohort in early Q3 2024 and has initiated enrollment in the cohort for boys aged 1-3. The Company remains on track to share initial strength and functional assessment data for both dose levels of the AFFINITY DUCHENNE trial in the second half of 2024.
- REGENXBIO recently held a successful End-of-Phase II (EOP2) meeting with the FDA and is moving forward with plans to initiate a pivotal trial in Q4 2024. Discussions with the FDA continue to support use of microdystrophin as a surrogate endpoint reasonably likely to predict

clinical benefit for accelerated approval. The Company expects to share the pivotal trial design in late Q3 to early Q4 2024.
- RGX-202 is manufactured using REGENXBIO's proprietary, high-yielding NAVXpress™ platform process. This suspension-based manufacturing process has demonstrated scalability up to 2,000L with consistent yield and product purity. The REGENXBIO Manufacturing Innovation Center has the capacity and yields to produce up to 2,500 doses of RGX-202 per year to support future commercialization.

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

- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of DR
    - Based on positive interim results from the Phase II ALTITUDE® trial to date, the design and evaluation of two pivotal trials is on-going. With AbbVie, REGENXBIO has accelerated a planned EOP2 meeting with the FDA, now expected in Q4 2024. The Company expects to initiate the first global pivotal trial in the first half of 2025.
    - The ALTITUDE trial is now enrolling a new cohort of patients with center-involved diabetic macular edema (DME). Patients will receive a one-time, in-office injection of ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye) with short course prophylactic steroid eye drops. DME is a vision-threatening complication of diabetic retinopathy; an estimated 34 million people globally have DME.
- ABBV-RGX-314 Subretinal Delivery for the Treatment of Wet AMD
    - Enrollment is on track in the ATMOSPHERE® and ASCENT™ pivotal trials and these trials are expected to support global regulatory submissions with the FDA and European Medicines Agency in the first half of 2026.
    - The open label fellow eye study evaluating ABBV-RGX-314 in patients treated in the subretinal Phase I/IIa study is now fully enrolled. This study is designed to monitor safety, immune responses, and efficacy of ABBV-RGX-314 treatment in the fellow eye and these study data are intended to support the inclusion of bilateral use in the product label. Bilateral disease impacts a significant number of patients with wet AMD.
- ABBV-RGX-314 Suprachoroidal Delivery for Treatment of Wet AMD
    - As of July 29, 2024, ABBV-RGX-314 at dose level 3 with short course prophylactic steroid eye drops continues to be well tolerated with no drug-related SAEs and no cases of intraocular inflammation, endophthalmitis, vasculitis, retinal artery occlusion, choroidal effusion, or hypotony. Mild episcleritis occurred in 3 patients, all resolved and completed treatment with topical steroids. There were no cases of elevated intraocular pressure.
    - Based on this favorable safety profile and to evaluate dose levels for a planned pivotal program, the Phase II AAVIATE® trial is initiating enrollment in a new cohort to evaluate ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short course prophylactic steroid eye drops.

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

- REGENXBIO completed a successful pre-BLA meeting with the FDA and will initiate a rolling BLA submission using the accelerated approval pathway in Q3 2024, potentially making RGX-121 the first approved gene therapy and one-time treatment for MPS II.
- REGENXBIO expects to share additional safety and efficacy data from the Phase I/II/III CAMPSIITE® trial in the second half of 2024.
- Approval of the planned BLA could result in receipt of a Priority Review Voucher in 2025.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $327.3 million as of June 30, 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 half of 2024.

Revenues: Revenues were $22.3 million for the three months ended June 30, 2024, compared to $20.0 million for the three months ended June 30, 2023. The increase was primarily attributable to Zolgensma® royalty revenues, which increased from $19.0 million for the second quarter of 2023 to $21.8 million for the second quarter of 2024.

Research and Development Expenses: Research and development expenses were $48.9 million for the three months ended June 30, 2024, compared to $59.9 million for the three months ended June 30, 2023. The decrease was largely driven by manufacturing and clinical supply costs for ABBV-RGX-314 and RGX-202, and personnel-related costs as a result of reduced headcount. The decrease was partially offset by increases in clinical trial expenses for ABBV-RGX-314 and RGX-202.

General and Administrative Expenses: General and administrative expenses were $18.9 million for the three months ended June 30, 2024, compared to $23.7 million for the three months ended June 30, 2023. The decrease was primarily attributable to expenses for professional services and other corporate overhead costs.

Net Loss: Net loss was $53.0 million, or $1.05 basic and diluted net loss per share, for the three months ended June 30, 2024, compared to a net loss of $72.1 million, or $1.66 basic and diluted net loss per share, for the three months ended June 30, 2023.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $327.3 million as of June 30, 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. To access the live call by phone, dial (646) 307-1963 (domestic) or (800) 715-9871 (international) and enter the passcode 4849384. To access a live or recorded webcast of the call, please visit the Investors section of the REGENXBIO website at www.regenxbio.com. The recorded webcast will be available for approximately 30 days following the call. 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)

	June 30, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 57,765	$ 34,522
Marketable securities	232,592	240,736
Accounts receivable, net	22,809	24,790
Prepaid expenses	10,021	14,520
Other current assets	23,496	20,403
Total current assets	346,683	334,971
Marketable securities	36,943	38,871
Accounts receivable	464	701
Property and equipment, net	123,969	132,103
Operating lease right-of-use assets	56,344	60,487
Restricted cash	2,030	2,030
Other assets	2,946	4,807
Total assets	$ 569,379	$ 573,970
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 16,362	$ 22,786
Accrued expenses and other current liabilities	42,488	49,703
Deferred revenue	21	148
Operating lease liabilities	7,302	7,068
Liability related to sale of future royalties	32,100	50,567
Total current liabilities	98,273	130,272
Operating lease liabilities	78,234	82,222
Liability related to sale of future royalties	41,079	43,485
Other liabilities	3,526	6,249
Total liabilities	221,112	262,228
Stockholders' equity		
Preferred stock; no shares issued and outstanding at June 30, 2024 and December 31, 2023	—	—
Common stock; 49,317 and 44,046 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively	5	4
Additional paid-in capital	1,171,894	1,021,214
Accumulated other comprehensive loss	(2,266)	(4,429)
Accumulated deficit	(821,366)	(705,047)
Total stockholders' equity	348,267	311,742
Total liabilities and stockholders' equity	$ 569,379	$ 573,970

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenues				
License and royalty revenue	$ 22,295	$ 19,977	$ 37,917	$ 39,115
Total revenues	22,295	19,977	37,917	39,115
Operating Expenses				
Cost of revenues	10,579	9,475	14,862	13,587
Research and development	48,869	59,886	103,713	118,402
General and administrative	18,855	23,698	37,146	46,332
Impairment of long-lived assets	—	—	2,101	—
Other operating expenses (income)	29	26	(5)	59
Total operating expenses	78,332	93,085	157,817	178,380
Loss from operations	(56,037)	(73,108)	(119,900)	(139,265)
Other Income (Expense)				

Interest income from licensing	29	40	66	110
Investment income	3,468	2,127	5,937	4,293
Interest expense	(449)	(1,120)	(2,422)	(3,875)
Total other income	3,048	1,047	3,581	528
Net loss	$ (52,989)	$ (72,061)	$ (116,319)	$ (138,737)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	963	1,524	2,163	5,303
Total other comprehensive income	963	1,524	2,163	5,303
Comprehensive loss	$ (52,026)	$ (70,537)	$ (114,156)	$ (133,434)
Net loss per share, basic and diluted	$ (1.05)	$ (1.66)	$ (2.41)	$ (3.19)
Weighted-average common shares outstanding, basic and diluted	50,601	43,531	48,167	43,491

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