



REGENXBIO Announces Updated Strategic Plans and Third Quarter 2023 Financial Results

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- *Announces pipeline prioritization and corporate restructuring to focus on clinical stage AAV Therapeutic product candidates addressing large commercial opportunities and value generation*
- *Highest priority programs are 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 MPS II*
- *Restructuring plan, including a 15% reduction in workforce, expected to result in total savings of at least \$100 million*
- *Anticipated cost savings from corporate restructuring, along with \$365 million in cash, cash equivalents and marketable securities as of September 30, 2023, expected to fund operational runway into the second half of 2025*
- *Conference call Wednesday, November 8, 2023, at 4:30 p.m. (EST)*

ROCKVILLE, Md., Nov. 8, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the third quarter ended September 30, 2023, and recent operational highlights, including a strategic pipeline prioritization and corporate restructuring intended to significantly reduce operating expenses while continuing to support meaningful value generation from the Company's strong pipeline of AAV Therapeutics.

"In the past two months, we reported exciting, positive clinical data from investigational treatments for diabetic retinopathy, and Duchenne, as well as had a very encouraging RMAT meeting with the FDA about expediting the BLA for our treatment for MPS II," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "These milestones demonstrate how our science is supporting avenues to accelerate development, and, today, we are following the data and making necessary decisions to focus our capabilities and resources on these differentiated product candidates which represent opportunities to bring ground-breaking AAV Therapeutics to millions of patients."

PIPELINE PRIORITIZATION AND CORPORATE RESTRUCTURING

The following key strategic decisions support the pipeline prioritization and corporate restructuring related to product candidates that are differentiated, can be expedited, and support near-term and long-term value generation.

- **Prioritizing investigational, one-time AAV therapeutic clinical stage programs:**
 - ABBV-RGX-314, being developed in collaboration with AbbVie, for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other additional chronic retinal conditions
 - RGX-202 for the treatment of Duchenne muscular dystrophy (Duchenne)
 - RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II)
- **Pursuing strategic alternatives, including potential partnering, for its other rare neurodegenerative disease clinical stage programs:** RGX-111 for the treatment of severe Mucopolysaccharidosis Type I, RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease and RGX-381 for the treatment of the ocular manifestations of CLN2 disease.
- **Reducing its workforce by approximately 15%, primarily in rare neurodegenerative disease development, early research, and other general and administrative areas.** REGENXBIO expects to incur approximately \$4 million in one-time restructuring costs in the fourth quarter of 2023.

As a result of the portfolio prioritization and corporate restructuring, REGENXBIO anticipates total savings of at least \$100 million over the next two years. These anticipated cost savings along with \$365 million in cash, cash equivalents and marketable securities as of September 30, 2023 are now expected to fund operations into the second half of 2025.

Mr. Mills added, "since our formation, REGENXBIO has been a leader in the field because of our mission to improve lives through the curative potential of gene therapy with our NAV[®] Technology Platform. As we refocus our capital allocation in a challenging economic environment and position the Company to meet important business goals, I want to express my gratitude for the commitment and dedication exhibited by our colleagues and partners in support of this mission."

PROGRAM HIGHLIGHTS AND MILESTONES

ABBV-RGX-314: ABBV-RGX-314 uses the NAV[®] AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF).

ABBV-RGX-314 is currently being evaluated in nine ongoing clinical trials, including two pivotal trials, a Phase II bridging study, a Long-term Follow-up study, and a Fellow Eye treatment study in patients with wet AMD, all utilizing subretinal delivery, as well as two Phase II clinical trials in patients with wet AMD and DR, and two corresponding Long-term Follow-up studies, all utilizing in-office suprachoroidal delivery.

- **ABBV-RGX-314 Subretinal Delivery for the Treatment of Wet AMD**
 - Enrollment continues to be on track in ATMOSPHERE[®] and ASCENT[™] pivotal trials for the treatment of patients with wet AMD using subretinal delivery.
 - These trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) in late 2025 through the first half of 2026.
- **ABBV-RGX-314 Suprachoroidal Delivery for Treatment of Wet AMD and DR**
 - In November 2023, REGENXBIO presented data from the Phase II ALTITUDE[®] trial demonstrating that ABBV-RGX-314 administered using suprachoroidal delivery was well tolerated in dose levels 1 and 2. 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 developing vision-threatening events by 89% in these patients.
 - REGENXBIO expects to report additional interim data from the Phase II AAVIATE[®] trial of ABBV-RGX-314 using suprachoroidal delivery for the treatment of wet AMD, including full six-month results from Cohorts 5 and 6, at the Hawaiian Eye and Retina meeting (January 13-19, 2024).

RGX-202: RGX-202 is an investigational one-time AAV therapeutic for Duchenne, using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

- In October 2023, REGENXBIO reported interim data from the Phase I/II AFFINITY DUCHENNE[®] trial, demonstrating that RGX-202 continued to be well tolerated with no drug-related serious adverse events in three patients at dose level 1. Initial biomarker data in two patients who completed three-month assessment demonstrate robust RGX-202 microdystrophin expression with localization to the muscle cell membrane.
- 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.
- REGENXBIO reported that, based on these interim results, reported from dose level 1, the FDA has supported amending the trial protocol to accelerate development. Escalation to dose level 2 is expected by the end of 2023.
- REGENXBIO expects to share initial strength and functional assessment data for both dose levels in 2024. Additionally, REGENXBIO anticipates pivotal dose determination and the initiation of a pivotal program in 2024.

RGX-121: RGX-121 is an investigational one-time AAV therapeutic for the treatment of MPS II, also known as Hunter syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase enzyme.

- REGENXBIO continues to follow patients in the Phase I/II/III CAMPSIITE[®] trial, with topline results from pivotal phase expected in the first quarter of 2024.
- A recent positive Regenerative Medicine Advanced Therapy (RMAT) meeting held in October

with FDA confirmed alignment on manufacturing strategy, adequacy of safety database, and confirmatory study design, which are key elements for an expedited plan for BLA filing using the accelerated approval pathway in 2024.

- Based on an expected priority review, potential approval of the Company's planned BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025.

OPERATIONAL UPDATES

- The REGENXBIO Manufacturing Innovation Center in Maryland is using the NAVXpress™ platform process to produce GMP lots intended for upcoming regulatory submissions and initial launch supply for ABBV-RGX-314 and RGX-121. REGENXBIO is one of only a few gene therapy companies worldwide with a cGMP facility capable of production at scales up to 2,000 liters.

NAV® TECHNOLOGY PLATFORM LICENSEE PROGRAM HIGHLIGHTS

As of September 30, 2023, REGENXBIO's NAV Technology Platform was being applied in one marketed product and multiple clinical stage partnered programs, with the potential to impact a broad range of therapeutic areas and disease indications.

- Zolgensma®, a one-time AAV Therapeutic for the treatment of spinal muscular atrophy, is a marketed product utilizing REGENXBIO's NAV AAV9 vector. In October 2023, Novartis AG reported third quarter 2023 global sales of Zolgensma of \$308 million.
- In November 2023, Rocket Pharmaceuticals announced the initiation of the Phase II pivotal trial of RP-A501 for Danon Disease. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In October 2023, Ultragenyx Pharmaceutical Inc. announced plans to provide preliminary Phase III DTX401 data in the first half of 2024 and that the DTX301 Phase III study is expected to complete enrollment in the first half of 2024. DTX401 for the treatment of Glycogen Storage Disease Type Ia and DTX301 for the treatment of Ornithine Transcarbamylase Deficiency are both being developed as one-time gene therapies utilizing REGENXBIO's NAV AAV8 vector.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were \$364.5 million as of September 30, 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 nine months ended September 30, 2023.

Revenues: Revenues were \$28.9 million for the three months ended September 30, 2023, compared to \$26.5 million for the three months ended September 30, 2022. The increase was primarily attributable to Zolgensma royalty revenues, which increased from \$25.2 million for the third quarter of 2022 to \$28.4 million for the third quarter of 2023.

Research and Development Expenses: Research and development expenses were \$58.2 million for the three months ended September 30, 2023, compared to \$63.3 million for the three months ended September 30, 2022. The decrease was 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 was partially offset by an increase in clinical trial expenses for our other lead product candidates.

General and Administrative Expenses: General and administrative expenses were \$23.1 million for the three months ended September 30, 2023, compared to \$20.9 million for the three months ended September 30, 2022. The increase was primarily attributable to personnel-related costs, expenses for professional services and other corporate overhead costs.

Net Loss: Net loss was \$61.9 million, or \$1.41 basic and diluted net loss per share, for the three months ended September 30, 2023, compared to a net loss of \$75.5 million, or \$1.75 basic and diluted net loss per share, for the three months ended September 30, 2022.

FINANCIAL GUIDANCE

As a result of the portfolio prioritization and corporate restructuring, REGENXBIO anticipates total savings of at least \$100 million over the next two years. These anticipated cost savings along with \$365 million in cash, cash equivalents and marketable securities as of September 30, 2023 are now expected to fund 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. EST. A live audio webcast will be available at [HERE](#). Interested parties may also pre-register for the earnings conference call [HERE](#). Once registration is completed, participants will be provided a dial-in number with a personalized conference code to access the call. Those who plan on participating are advised to dial in 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. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8 and AAV9. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a '5x'25' strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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 strategic pipeline prioritization and corporate restructuring plan or other cost-saving measures, and expected charges associated with restructuring and any future cost reduction measures, 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, 2022, 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)

| | <u>September 30, 2023</u> | | <u>December 31, 2022</u> | |
|--|---------------------------|---------|--------------------------|---------|
| Assets | | | | |
| Current assets | | | | |
| Cash and cash equivalents | \$ | 53,045 | \$ | 96,952 |
| Marketable securities | | 248,842 | | 267,690 |
| Accounts receivable | | 28,043 | | 28,082 |
| Prepaid expenses | | 12,561 | | 13,900 |
| Other current assets | | 23,347 | | 9,352 |
| Total current assets | | 365,838 | | 415,976 |
| Marketable securities | | 62,639 | | 200,560 |
| Accounts receivable, net | | 1,078 | | 1,504 |
| Property and equipment, net | | 135,534 | | 141,685 |
| Operating lease right-of-use assets | | 61,773 | | 65,116 |
| Restricted cash | | 2,255 | | 2,030 |
| Other assets | | 4,669 | | 6,397 |
| Total assets | \$ | 633,786 | \$ | 833,268 |
| Liabilities and Stockholders' Equity | | | | |
| Current liabilities | | | | |
| Accounts payable | \$ | 21,859 | \$ | 27,213 |
| Accrued expenses and other current liabilities | | 45,656 | | 46,794 |
| Deferred revenue | | 442 | | 1,829 |
| Operating lease liabilities | | 6,639 | | 5,997 |
| Liability related to sale of future royalties | | 52,750 | | 48,601 |
| Total current liabilities | | 127,346 | | 130,434 |
| Operating lease liabilities | | 84,058 | | 88,802 |
| Liability related to sale of future royalties | | 53,096 | | 89,005 |
| Other liabilities | | 6,186 | | 8,832 |
| Total liabilities | | 270,686 | | 317,073 |
| Stockholders' equity | | | | |

| | | |
|---|-------------------|-------------------|
| Preferred stock; no shares issued and outstanding at September 30, 2023 and December 31, 2022 | — | — |
| Common stock; 43,991 and 43,299 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively | 4 | 4 |
| Additional paid-in capital | 1,012,667 | 973,145 |
| Accumulated other comprehensive loss | (7,413) | (15,401) |
| Accumulated deficit | (642,158) | (441,553) |
| Total stockholders' equity | <u>363,100</u> | <u>516,195</u> |
| Total liabilities and stockholders' equity | <u>\$ 633,786</u> | <u>\$ 833,268</u> |

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

| | Three Months | | Nine Months | |
|---|---------------------|--------------------|---------------------|---------------------|
| | Ended September 30, | | Ended September 30, | |
| | 2023 | 2022 | 2023 | 2022 |
| Revenues | | | | |
| License and royalty revenue | \$ 28,914 | \$ 26,512 | \$ 68,029 | \$ 81,379 |
| Total revenues | 28,914 | 26,512 | 68,029 | 81,379 |
| Operating Expenses | | | | |
| Cost of revenues | 12,388 | 13,094 | 25,975 | 41,762 |
| Research and development | 58,183 | 63,313 | 176,585 | 179,948 |
| General and administrative | 23,083 | 20,921 | 69,415 | 64,071 |
| Other operating expenses | 220 | 229 | 279 | 703 |
| Total operating expenses | <u>93,874</u> | <u>97,557</u> | <u>272,254</u> | <u>286,484</u> |
| Loss from operations | (64,960) | (71,045) | (204,225) | (205,105) |
| Other Income (Expense) | | | | |
| Interest income from licensing | 56 | 18 | 166 | 265 |
| Investment income | 4,660 | 1,497 | 8,953 | 3,357 |
| Interest expense | (1,624) | (5,954) | (5,499) | (18,944) |
| Total other income (expense) | <u>3,092</u> | <u>(4,439)</u> | <u>3,620</u> | <u>(15,322)</u> |
| Loss before income taxes | (61,868) | (75,484) | (200,605) | (220,427) |
| Income Tax Benefit | | | | |
| Net loss | <u>\$ (61,868)</u> | <u>\$ (75,484)</u> | <u>\$ (200,605)</u> | <u>\$ (220,386)</u> |
| Other Comprehensive Income (Loss) | | | | |
| Unrealized gain (loss) on available-for-sale securities, net | 2,685 | (3,493) | 7,988 | (15,687) |
| Total other comprehensive income (loss) | <u>2,685</u> | <u>(3,493)</u> | <u>7,988</u> | <u>(15,687)</u> |
| Comprehensive loss | <u>\$ (59,183)</u> | <u>\$ (78,977)</u> | <u>\$ (192,617)</u> | <u>\$ (236,073)</u> |
| Net loss per share, basic and diluted | <u>\$ (1.41)</u> | <u>\$ (1.75)</u> | <u>\$ (4.60)</u> | <u>\$ (5.11)</u> |
| Weighted-average common shares outstanding, basic and diluted | <u>43,945</u> | <u>43,251</u> | <u>43,644</u> | <u>43,103</u> |

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