



REGENXBIO Reports First Quarter 2023 Financial Results and Recent Operational Highlights

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- Continued progress on '5x'25' strategy to advance five AAV Therapeutics from REGENXBIO's internal pipeline and licensed programs into pivotal-stage or commercial products by 2025
- Announced Updates to Eye Care Collaboration with AbbVie
 - Investigational gene therapy renamed ABBV-RGX-314
 - IND sponsorship recently transferred to AbbVie for all ongoing clinical trials
 - Expanding sites and enrollment in ATMOSPHERE[®] and ASCENT[™] pivotal trials using subretinal delivery; now expected to support U.S. and European regulatory submissions in late 2025 through the first half of 2026
- Completed enrollment in expansion cohorts in both Phase II AAVIATE[®] and ALTITUDE[®] trials of ABBV-RGX-314 using in-office suprachoroidal delivery, expect interim trial updates in the second half of 2023
- AFFINITY DUCHENNE[™] Phase I/II trial of RGX-202 continues to be on track to report initial trial data in the second half of 2023
- CAMPSITE[™] clinical trial of RGX-121 for the treatment of MPS II remains on track to file BLA in 2024 using the accelerated approval pathway
- \$474 million in cash, cash equivalents and marketable securities as of March 31, 2023; operational runway into 2025
- Conference call Wednesday, May 3rd at 4:30 p.m. ET

ROCKVILLE, Md., May 3, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the first quarter ended March 31, 2023, and recent operational highlights.

"REGENXBIO is well-positioned for a transformative year, with multiple clinical milestones and updates anticipated in 2023. Our goal in partnering our core expertise in gene therapy with AbbVie's leadership in eye care and their global infrastructure was to expand and advance the reach of our retinal programs for patients in need of a potential one-time gene therapy, and we are pleased to progress this program with a vision of benefiting patients worldwide. Based on the encouraging safety and clinical profiles, we believe ABBV-RGX-314 has the potential to be a highly differentiated product for millions of patients," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We also continue to make tremendous strides in our rare disease pipeline, advancing clinical trials in Duchenne, Hunter and Hurler syndromes and Batten disease, and we anticipate more updates across each of these programs in 2023. Overall, with an active pipeline of clinical-stage products, we are making excellent progress advancing our '5x'25' strategy to bring leading, ground-breaking AAV Therapeutics to patients in need."

AbbVie Eye Care Collaboration Update

REGENXBIO today announced updates from its eye care collaboration with AbbVie to develop and commercialize a potential one-time gene therapy for the treatment of chronic retinal diseases. The updates include the transfer of the Investigational New Drug (IND) applications to AbbVie for all ongoing clinical trials and the expansion of the ATMOSPHERE[®] and ASCENT[™] pivotal trials for the treatment of patients with wet age-related macular degeneration (wet AMD) using subretinal delivery to support new global registration plans. As part of the IND transfers, the investigational gene therapy RGX-314 has been renamed ABBV-RGX-314.

As outlined in the original collaboration agreement, beginning in 2023, AbbVie has been responsible for the majority of all development expenses for ABBV-RGX-314. The transfer of the INDs enables AbbVie to serve as lead regulatory party in the U.S. and globally. AbbVie will lead the clinical development and commercialization of ABBV-RGX-314 globally. AbbVie and REGENXBIO will continue to collaborate on additional trials of ABBV-RGX-314.

ABBV-RGX-314 is currently being evaluated in nine ongoing clinical trials in the U.S. and Canada, 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 diabetic retinopathy (DR), and two corresponding Long-term Follow-up studies, all utilizing in-office suprachoroidal delivery.

The pivotal program utilizing subretinal delivery is now expected to add new sites in Europe, Japan, and Israel to advance global development plans. Additional clinical trial applications will be submitted to the European Medicines Agency (EMA) and the Pharmaceuticals and Medical Devices Agency in Japan. The ATMOSPHERE and ASCENT trials are expected to expand enrollment to approximately 540 and 660 patients, respectively. As

prospectively allowed in the pivotal trial protocols, the enrollment expansion is also expected to support the increase in power of primary and secondary endpoints to enable additional global regulatory submissions and labelling options. The new global site plans and expanded enrollment targets are expected to support regulatory submissions with the U.S. Food and Drug Agency (FDA) and the EMA in late 2025 through the first half of 2026.

Also as previously announced, under the collaboration, REGENXBIO will continue to lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead manufacturing of ABBV-RGX-314 for commercial supply outside the U.S. Additionally, cGMP material produced by REGENXBIO's proprietary NAVXpress™ platform process has been incorporated into the ongoing clinical trials, including the ATMOSPHERE and ASCENT pivotal trials. REGENXBIO shall participate in U.S. commercialization efforts as provided under a mutually agreed upon commercialization plan. REGENXBIO and AbbVie will share equally in profits from net sales of ABBV-RGX-314 in the U.S. and AbbVie will pay REGENXBIO tiered royalties on net sales outside the U.S.

Program Highlights and Milestones

ABBV-RGX-314: ABBV-RGX-314 is an investigational one-time AAV Therapeutic being developed in collaboration with AbbVie for the treatment of wet AMD, DR and other additional chronic retinal conditions. 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 Subretinal Delivery for the Treatment of Wet AMD**
 - Ahead of global expansion, U.S. enrollment is ongoing in ATMOSPHERE and ASCENT. Material from REGENXBIO's NAVXpress platform process has been incorporated in the pivotal trials and is expected to be produced at REGENXBIO's Manufacturing Innovation Center for future commercialization of RGX-314.
 - REGENXBIO also announced today that a Fellow Eye Treatment study has also been initiated as part of the pivotal program using subretinal delivery. This study will evaluate the safety, efficacy, and immunogenicity of subretinal ABBV-RGX-314 administration in the fellow eye of patients having bilateral disease from ATMOSPHERE and ASCENT who previously received a subretinal injection of ABBV-RGX-314. Data from patients enrolled in this study are expected to further support global regulatory submission plans.
 - In February 2023, interim data was presented from the Phase II bridging study demonstrating that ABBV-RGX-314 manufactured using REGENXBIO's NAVXpress platform process, material from which has been incorporated into the pivotal trials, was well tolerated and exhibited a similar clinical profile to the initial adherent cell culture process used in the Phase I/IIa trial. Patients in the two high dose cohorts also demonstrated stable to improved BCVA and CRT, and meaningful reductions in anti-VEGF burden, with a majority of subjects injection-free. The Phase II pharmacodynamic study is designed to evaluate the same dose levels being used in the two pivotal trials.
- **ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD**
 - REGENXBIO has completed enrollment of Cohort 6 in the Phase II AAVIATE® trial. Cohort 6 (dose level 3) incorporated short-course prophylactic ocular steroids following ABBV-RGX-314 administration to evaluate the ability to prevent or reduce the occurrence of the mild to moderate intraocular inflammation seen in previous cohorts. Patients were enrolled in Cohort 6 regardless of neutralizing antibody (NAb) status.
 - REGENXBIO expects to report additional interim trial data from the Phase II AAVIATE trial, including initial data from Cohort 6, in the second half of 2023.
- **ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of DR**
 - REGENXBIO has completed enrollment in Cohorts 4 and 5 (dose level 3) in the Phase II ALTITUDE® trial. In these cohorts, patients were stratified by Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS) levels and all received short-course prophylactic ocular steroids following ABBV-RGX-314 administration.
 - REGENXBIO expects to report additional interim trial data, including initial data from the third dose level, in the second half of 2023.

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

- The Phase I/II AFFINITY DUCHENNE™ trial continues to recruit patients and is expected to use commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center in the clinical trial.
- In April, REGENXBIO announced the U.S. Food and Drug Administration (FDA) granted Fast Track Designation to RGX-202 for the treatment of Duchenne. Fast Track designation aims to facilitate the development and expedite the review of new therapeutics to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs.
- REGENXBIO is also recruiting patients in the AFFINITY BEYOND™ trial, an observational screening study to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.
- REGENXBIO continues to expect to report initial data from the Phase I/II AFFINITY DUCHENNE trial in the second half of 2023.

RGX-121: RGX-121 is an investigational one-time AAV Therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase (I2S) enzyme.

- The Phase I/II/III CAMPSIITE™ trial of MPS II patients aged 4 months up to 5 years is ongoing and is expected to incorporate material from the NAVXpress platform process manufactured at the REGENXBIO Manufacturing Innovation Center to support the future commercialization of RGX-121.
- In February 2023, REGENXBIO announced additional interim data from the Phase I/II part of the CAMPSIITE trial, demonstrating that RGX-121 continued to be well-tolerated across 15 patients. Patients receiving the pivotal program dose level continued to demonstrate the largest reductions in CSF GAGs, including Heparin Sulfate (HS) and HS D2S6, which approached normal levels at 48 weeks. CSF GAGs have the potential to be considered a surrogate biomarker that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations, including neurodevelopmental deficits. In addition, improvements in neurodevelopmental and daily activity skill acquisition were observed up to three years after RGX-121 administration.
- A Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of five years old is also ongoing.
- CAMPSIITE is expected to complete enrollment of 10 MPS II patients in the first half of 2023 to support a BLA filing in 2024 using the accelerated approval pathway.

RGX-111: RGX-111 is an investigational one-time AAV Therapeutic for the treatment of severe Mucopolysaccharidosis Type I (MPS I), using the NAV AAV9 vector to deliver the α -L-iduronidase (IDUA) gene.

- A Phase I/II trial of RGX-111 for the treatment of MPS I is fully enrolled with follow-up ongoing.
- REGENXBIO has recently completed the manufacture of commercial-scale cGMP material using the NAVXpress platform process at the REGENXBIO Manufacturing Innovation Center to support the continued development of RGX-111.
- In February 2023, REGENXBIO announced additional positive interim data from the Phase I/II trial demonstrating that RGX-111 was well tolerated in eight patients. Biomarker and neurodevelopmental assessments indicated an encouraging CNS profile in patients dosed with RGX-111.
- REGENXBIO expects to complete analytical characterization of the commercial-scale cGMP material and share additional updates on program plans in the second half of 2023.

RGX-181: RGX-181 is an investigational one-time AAV Therapeutic for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, using the NAV AAV9 vector to deliver the TPP1 gene directly to the central nervous system.

- Physician investigators in Brazil continue with follow up for the first child with CLN2 disease dosed with RGX-181 under a single-patient investigator-initiated study.
- REGENXBIO plans to provide a program update by the end of 2023.

RGX-381: RGX-381 is an investigational one-time AAV Therapeutic for the treatment of the ocular manifestations of CLN2 disease using the NAV AAV9 vector to deliver the TPP1 gene directly to the retina.

- The Phase I/II first-in-human, open-label, dose-escalation clinical trial of RGX-381 is on track to initiate in the second quarter of 2023. The trial will evaluate the safety and tolerability, as well as the effect on retinal anatomic and functional outcomes, of the subretinal delivery of RGX-381 for the treatment of ocular manifestations of CLN2 disease.

Operational Updates

- The REGENXBIO Manufacturing Innovation Center in Maryland is fully operational, producing GMP bulk substance lots to support programs using the NAVXpress™ platform process. REGENXBIO is one of only a few gene therapy companies worldwide with a GMP facility capable of production at scales up to 2,000 liters.
 - In 2023, REGENXBIO continues to utilize the facility to produce commercial-scale cGMP material for its entire clinical pipeline and performance qualification lots to support planned regulatory filings for ABBV-RGX-314 and RGX-121.

NAV Technology Licensee Program Highlights

As of March 31, 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 (SMA), is a marketed product utilizing REGENXBIO's NAV AAV9 vector. In April 2023, Novartis AG reported first quarter 2023 global sales of Zolgensma of \$309 million.
- In February 2023, Rocket Pharmaceuticals (Rocket) announced that the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-A501, for the treatment of Danon Disease, that will provide the benefits of added intensive FDA guidance and expedited review through the program's development. The initiation of a Phase II pivotal trial is on track for the second quarter of 2023. As previously disclosed, Rocket anticipates pursuing a single arm, open-label trial with a biomarker-based composite endpoint and a natural history comparator. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In February 2023, Ultragenyx Pharmaceutical Inc. (Ultragenyx) announced that in the Phase III study of DTX401 for Glycogen Storage Disease Type Ia (GSDIa), the last patient has entered the baseline screening period and data readout is expected in the first half of 2024. Ultragenyx also randomized and dosed the first patient in the Phase III study of DTX301 for Ornithine Transcarbamylase (OTC) Deficiency. DTX401 and DTX301 are both being developed as one-time gene therapies utilizing REGENXBIO's NAV AAV8 vector.
- In February 2023, uniQure N.V. announced it expects to submit an investigational new drug application and initiate a Phase I/II clinical study of AMT-260 to treat temporal lobe epilepsy in the second half of 2023. AMT-260 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were \$473.5 million as of March 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 first quarter of 2023.

Revenues: Revenues were \$19.1 million for the three months ended March 31, 2023, compared to \$22.2 million for the three months ended March 31, 2022. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased from \$21.5 million in the first quarter of 2022 to \$16.1

million in the first quarter of 2023.

Research and Development Expenses: Research and development expenses were \$58.5 million for the three months ended March 31, 2023, compared to \$55.6 million for the three months ended March 31, 2022. The increase was primarily attributable to personnel costs as a result of increased headcount and laboratory and facilities costs, driven primarily by the activation of the REGENXBIO Manufacturing Innovation Center in mid-2022.

General and Administrative Expenses: General and administrative expenses were \$22.6 million for the three months ended March 31, 2023, compared to \$22.3 million for the three months ended March 31, 2022.

Net Loss: Net loss was \$66.7 million, or \$1.53 basic and diluted net loss per share, for the three months ended March 31, 2023, compared to a net loss of \$76.7 million, or \$1.79 basic and diluted net loss per share, for the three months ended March 31, 2022.

Financial Guidance

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of \$473.5 million as of March 31, 2023, to fund its operations into 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. 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 "5x25" 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 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.

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

	<u>March 31, 2023</u> <u>December 31, 2022</u>	
Assets		
Current assets		
Cash and cash equivalents	\$ 70,091	\$ 96,952
Marketable securities	261,726	267,690
Accounts receivable	18,861	28,082
Prepaid expenses	15,521	13,900
Other current assets	23,003	9,352
Total current assets	389,202	415,976
Marketable securities	141,709	200,560

Accounts receivable, net	1,300	1,504
Property and equipment, net	141,573	141,685
Operating lease right-of-use assets	63,726	65,116
Restricted cash	2,030	2,030
Other assets	8,290	6,397
Total assets	<u>\$ 747,830</u>	<u>\$ 833,268</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 23,283	\$ 27,213
Accrued expenses and other current liabilities	31,709	46,794
Deferred revenue	1,311	1,829
Operating lease liabilities	6,303	5,997
Liability related to sale of future royalties	49,728	48,601
Total current liabilities	112,334	130,434
Operating lease liabilities	86,992	88,802
Liability related to sale of future royalties	77,382	89,005
Other liabilities	5,983	8,832
Total liabilities	282,691	317,073
Stockholders' equity		
Preferred stock; no shares issued and outstanding at March 31, 2023 and December 31, 2022	—	—
Common stock; 43,465 and 43,299 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively	4	4
Additional paid-in capital	984,986	973,145
Accumulated other comprehensive loss	(11,622)	(15,401)
Accumulated deficit	(508,229)	(441,553)
Total stockholders' equity	465,139	516,195
Total liabilities and stockholders' equity	<u>\$ 747,830</u>	<u>\$ 833,268</u>

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

	<u>Three Months Ended March 31,</u>	
	<u>2023</u>	<u>2022</u>
Revenues		
License and royalty revenue	\$ 19,138	\$ 22,218
Total revenues	19,138	22,218
Operating Expenses		
Cost of revenues	4,112	15,717
Research and development	58,516	55,627
General and administrative	22,634	22,318
Other operating expenses	33	83
Total operating expenses	85,295	93,745
Loss from operations	(66,157)	(71,527)
Other Income (Expense)		
Interest income from licensing	70	94
Investment income	2,166	799
Interest expense	(2,755)	(6,130)
Total other income (expense)	(519)	(5,237)
Loss before income taxes	(66,676)	(76,764)
Income Tax Benefit	—	41
Net loss	<u>\$ (66,676)</u>	<u>\$ (76,723)</u>
Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net	3,779	(9,381)
Total other comprehensive income (loss)	3,779	(9,381)
Comprehensive loss	<u>\$ (62,897)</u>	<u>\$ (86,104)</u>
Net loss per share, basic and diluted	<u>\$ (1.53)</u>	<u>\$ (1.79)</u>

Weighted-average common shares outstanding, basic and diluted 43,451 42,944

Contacts:

Dana Cormack
Corporate Communications
dcormack@regenxbio.com

Investors:

Chris Brinzey, ICR Westwicke
339-970-2843
Chris.brinzey@westwicke.com



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