REGENXBIO Highlights AAV Pipeline with Interim Results from Retinal and Duchenne Programs at its Virtual Investor Day on July 11, 2023

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-Interim results from Phase II AAVIATE and ALTITUDE trials of dose level 3 cohorts with short-course prophylactic steroid eye drops resulted in zero cases of intraocular inflammation

-Initial safety results from Cohort 1 of the AFFINITY DUCHENNE study support well-tolerated profile to date; initial efficacy data to be presented at World Muscle Society meeting in October

-A new program in Duchenne with preclinical data using innovative science to produce near full-length wild-type dystrophin; expects to file IND to the FDA in the first half of 2025

-Detailed updates will be presented at its Virtual Investor Day, today at 8:30 a.m. ET

ROCKVILLE, Md., July 11, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) will announce today new interim data from the Phase II AAVIATE® and ALTITUDE® trials of ABBV-RGX-314 suprachoroidal delivery in wet AMD and diabetic retinopathy (DR). The company will also provide a comprehensive Duchenne program overview including initial safety data for RGX-202 and a new exon 53 program.

REGENXBIO will host a virtual Investor Day today at 8:30 a.m. ET to discuss these updates. The webcast and slides of the presentation can be accessed in the Investors section of REGENXBIO's website at www.regenxbio.com.

"We continue to perform at a high level as we execute on our mission of improving lives through the curative potential of gene therapy," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Today, we are pleased to share an update on REGENXBIO's pipeline of AAV-based gene therapies with a focus on our retinal and Duchenne programs. The safety and tolerability data presented today for the high-dose cohorts of our in-office wet AMD and DR programs is encouraging and further validate the potential of the suprachoroidal space for AAV delivery to treat chronic retinal diseases."

Mr. Mills continued, "I am also thrilled to share an update on our Duchenne program, from our neuromuscular franchise, which includes progress on RGX-202 and our new exon-skipping program that we have initiated. We continue to develop highly differentiated, transformational treatments with an unwavering commitment to advance therapies designed to prevent ambulation loss and improve the quality of life in boys worldwide, regardless of age, who suffer from Duchenne."

"I believe we have a clear and definable path to achieve our '5 by '25' vision," said Mr. Mills. "We are advancing a strong pipeline of AAV-based therapeutic candidates in diseases representing large current market opportunities and that address significant unmet needs for millions of patients."

Key Investor Day Highlights

Retinal Program Update

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

Interim data to be presented today from the Phase II AAVIATE & ALTITUDE trials demonstrated that ABBV-RGX-314 suprachoroidal delivery administered to patients in cohorts at dose level 3 ($1.0x10^{12}$ genome copies per eye) with short-course (seven-week) prophylactic topical steroid eye drops (N=39) resulted in zero cases of intraocular inflammation. Time of post-administration follow up ranged from six weeks to six months.

- ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD
  - Patients in Cohort 6 (dose level 3, N=20) in the Phase II AAVIATE trial all received short-course prophylactic ocular steroids following ABBV-RGX-314 administration, to evaluate the ability to prevent or reduce the occurrence of mild to moderate intraocular inflammation seen in previous cohorts.
  - As of June 12, 2023, ABBV-RGX-314 was reported to be well tolerated in 20 patients from Cohort 6, with no drug-related serious adverse events. Time of post-administration follow up ranged from six weeks to six months.
  - Short-course of ocular steroid prophylaxis meaningfully reduced the occurrence of mild to moderate intraocular inflammation seen in previous cohorts. In all 10 patients who received the short-course (seven-week) prophylactic topical steroid eye drops there were zero cases of intraocular inflammation.

    - REGENXBIO expects to report additional interim data from the Phase II AAVIATE trial, including full 6-month results from Cohorts 5 and 6, at the Hawaiian Eye and Retina meeting being held January 13-19, 2024.
- ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of DR
    - Patients in Cohorts 4 and 5 (dose level 3, N=29) in the Phase II ALTITUDE trial were stratified by Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS) levels, and all received short-course (seven-week) prophylactic topical steroid eye drops following ABBV-RGX-314 administration.
    - As of June 12, 2023, ABBV-RGX-314 was reported to be well tolerated in 29 patients from Cohorts 4 and 5, with no drug-related serious adverse events. Time of post-administration follow up ranged from 12 weeks to six months.
    - There were zero cases of intraocular inflammation.
    - REGENXBIO expects to report additional interim data from the Phase II ALTITUDE trial at the American Academy of Ophthalmology meeting being held November 3-6, 2023.

Duchenne Program Update

RGX-202 is an investigational one-time AAV therapeutic for 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.

REGENXBIO will present interim safety data from Cohort 1 of the ongoing Phase I/II AFFINITY DUCHENNE™ trial, which continues to recruit patients (aged 4 to 11 years) and is using commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center.

- As of July 6, 2023, RGX-202 was reported to be well-tolerated in two patients dosed to date, aged 4 and 10 years, with no drug-related serious adverse events. Time of post-administration follow up was 45 days and more than three months.
- REGENXBIO will share additional interim data of the AFFINITY DUCHENNE trial, including longer-term safety and microdystrophin protein expression levels in muscle at three months, at the World Muscle Society Congress being held October 3-7, 2023.

New Exon Skipping Program: REGENXBIO will also announce today the development of a potential one-time gene therapy for Duchenne, which is based on a novel exon-skipping construct. REGENXBIO scientists have demonstrated that AAV-mediated exon skipping by the expression of antisense ribonucleic acid (AS RNA) sequences has the potential to restore high and sustained levels of near full-length dystrophin in Duchenne patients with relevant mutations in the *DMD* gene.

- REGENXBIO's new candidate is derived and characterized from the company's NAV Technology Platform, designed to use a muscle-specific capsid and deliver three AS RNAs targeting exon 53 of the *DMD* gene.
- Proof of concept data from preclinical studies in the hDMDdel52/mdx mouse model of Duchenne demonstrated over 90% exon 53 skipping efficiency, broad and robust (up to 50% of expected wild-type dystrophin levels were restored) expression of near full-length dystrophin in skeletal and heart muscle, and improvements in muscle histopathology, with durability up to three months, which is the longest timepoint measured to date.
- REGENXBIO estimates that nearly 50% of Duchenne patients have mutations of the *DMD* gene that are amenable to exon skipping and can potentially be addressed with its AAV-mediated exon skipping platform.
- The company is initiating Investigational New Drug (IND) application-enabling studies and expects to submit an IND to the FDA in the first half of 2025.

Virtual Investor Day Webcast Information
The virtual event will take place on Tuesday, July 11, 2023, at 8:30 a.m. Eastern Time. The live webcast and presentation slides can be accessed in the Investors section of REGENXBIO's website at www.regenxbio.com. An archived replay of the webcast will be available in the Investors section of the website for approximately 30 days following the presentation.

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 its internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

Forward-Looking Statement

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 and clinical trials. 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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.

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