REGENXBIO Presents Interim Data from Phase II Bridging Study Evaluating the Clinical Performance of RGX-314 Using the NAVXpress™ Manufacturing Platform Process

February 11, 2023 1:40 PM EST

- *RGX-314 produced by the NAVXpress platform process has been well-tolerated and demonstrated a similar clinical profile to the initial adherent cell culture process*
- *NAVXpress platform process is incorporated in the two ongoing pivotal trials and is expected to be used for future commercialization of RGX-314; the two pivotal trials are expected to support BLA submission in 2024*
- *Company to host live webcast with wet AMD Key Opinion Leaders to discuss new interim Phase II bridging study data, today, Saturday, February 11, at 11:30 a.m. ET*

ROCKVILLE, Md., Feb. 11, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced results from a Phase II bridging study evaluating the pharmacodynamics, safety and efficacy of RGX-314, a potential one-time therapy delivered subretinally using cGMP material produced by the company's NAVXpress ™ bioreactor platform process. The data is being presented at the Angiogenesis, Exudation, and Degeneration 2023 Conference by Charles C. Wykoff, M.D., PhD., Director of Research at Retina Consultants of Texas; Chairman of Research, Retina Consultants of America; and Deputy Chair of Ophthalmology for the Blanton Eye Institute, Houston Methodist Hospital.

"The interim results observed in the Phase II bridging study show a similar clinical profile between our manufacturing processes. We believe our approach, focused on early product quality and process control, allows us to efficiently transition from clinical trials to commercial readiness," said Curran Simpson, Chief Operating Officer of REGENXBIO. "This update provides validation of our plans for the NAVXpress platform process to support the production of RGX-314 in anticipation of future commercialization."

"There is a significant need for treatment options that can reduce the burden of frequent injections for wet AMD patients while maintaining optimal function and anatomic outcomes," said Dr. Wykoff. "The clinical profile of RGX-314 manufactured using the commercial-scale process is encouraging, as is the potential of a one-time therapy for the treatment of wet AMD."

Interim Data Summary from the Phase II Bridging Study of RGX-314 using Subretinal Delivery

The Phase II bridging study is designed to evaluate RGX-314 using subretinal delivery across two dose levels ($6.4x10^{10}$ GC/eye and $1.3x10^{11}$ GC/eye) in 60 patients with wet AMD. At each dose level, patients are assigned into two cohorts, with half of the patients at each dose level receiving RGX-314 produced by the NAVXpress platform process, and the other half receiving RGX-314 produced by the adherent cell culture manufacturing process that was used in the Phase I/IIa trial of RGX-314 for the treatment of wet AMD. The primary endpoint of the study is RGX-314 target protein concentration in the eye at Month 6. Secondary endpoints include safety and tolerability, change from baseline in Best Corrected Visual Acuity (BCVA), change in central retinal thickness (CRT) and need for supplemental anti-VEGF injections.

As of November 14, 2022, RGX-314 was well tolerated across 46 patients dosed in cohorts at both dose levels. Five SAEs were reported, none of which were considered related to RGX-314. In the high dose cohorts, all common treatment emergent adverse events (TEAEs) through 6 months in the study eye were mild or moderate and included post-operative conjunctival hemorrhage, post-operative inflammation and retinal pigmentary changes.

The two high dose cohorts have fully enrolled and completed six month visit assessments. Data presented at Angiogenesis highlighted the results from these cohorts (n=30). In these cohorts, target protein concentrations in the eye were similar between the manufacturing processes. 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.

Pivotal Trials for the Treatment of RGX-314 for wet AMD using Subretinal Delivery

To support future commercialization of RGX-314, the cGMP material produced by the Company's NAVXpress platform process has been incorporated in the ongoing pivotal trials, ATMOSPHERE® and ASCENT ™, for the treatment of wet AMD using RGX-314 delivered subretinally. These pivotal trials are multi-center, randomized, active-controlled trials to evaluate the efficacy and safety of a single-administration of RGX-314 using subretinal delivery versus standard of care in patients with wet AMD. The two pivotal trials are designed to evaluate the same dose levels being used in the Phase II bridging study.

"The RGX-314 program is central to our '5x'25' strategy to have five AAV Therapeutics either on the market or in late-stage development by 2025. Our NAVXpress platform process is producing cGMP material at the REGENXBIO Manufacturing Innovation Center and has supported the advancement of several of our on-going clinical programs," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are one of only a few gene therapy companies with a cGMP facility capable of producing at scales of up to 2,000 liters, which we believe represents a key differentiator for REGENXBIO that we expect to use across our ongoing clinical trials to support accelerating the development of our AAV Therapeutics, including RGX-314."

A live discussion of the Phase II bridging study data with Dr. Wykoff and Dr. Peter Kaiser, Chaney Family Endowed Chair in Ophthalmology Research and Professor of Ophthalmology, Cleveland Clinic Lerner College of Medicine and Cole Eye Institute, and Dr. Steve Pakola, Chief Medical Officer of REGENXBIO will stream on the "Investors" events page of the REGENXBIO website at 11:30 a.m. ET. The presentation is available on the

"Presentations and Publications" section of the REGENXBIO website at www.regenxbio.com.

About RGX-314
RGX-314, being developed in collaboration with AbbVie, is being investigated as a potential one-time treatment for wet AMD, diabetic retinopathy, and other chronic retinal conditions. RGX-314 consists of the NAV® AAV8 vector, which encodes an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). RGX-314 is believed to inhibit the VEGF pathway by which new, leaky blood vessels grow and contribute to the accumulation of fluid in the retina.

REGENXBIO is advancing research in two separate routes of administration of RGX-314 to the eye, through a standardized subretinal delivery procedure as well as delivery to the suprachoroidal space. REGENXBIO has licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. to deliver gene therapy treatments to the suprachoroidal space of the eye.

About Wet AMD
Wet AMD is characterized by loss of vision due to new, leaky blood vessel formation in the retina. Wet AMD is a significant cause of vision loss in the United States, Europe and Japan, with up to 2 million people living with wet AMD in these geographies alone. Current anti-VEGF therapies have significantly changed the landscape for treatment of wet AMD, becoming the standard of care due to their ability to prevent progression of vision loss in the majority of patients. These therapies, however, require life-long repeated intraocular injections to maintain efficacy. Due to the burden of treatment, it is difficult for patients to adhere to frequent injections, which can lead to a decline in vision over time.

About NAVXpress ™
NAVXpress is REGENXBIO's proprietary, high-yielding manufacturing platform process for NAV vector production that can be applied across multiple AAV Therapeutics. The suspension-based manufacturing platform process has demonstrated robust scalability from bench-scale to 2,000 liter batches with consistent yield and product purity demonstrated via comparability studies.

The NAVXpress platform is used at the REGENXBIO Manufacturing Innovation Center, a state-of-the-art facility built to meet global clinical and commercial regulatory standards. It includes two independent bulk drug substance production suites, a final drug product suite and integrated quality control labs. 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.

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, AAV9, and AAVrh10. 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 "5 x '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 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, 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, 2021, 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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