



REGENXBIO Announces RGX-314 Data Presentations at Retina Society 54th Annual Scientific Meeting and KOL Conference Call

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- *Interim data for Cohort 1 with six months of follow-up from the RGX-314 Phase II trial for the treatment of wet AMD (AAVIATE[®]) to be presented*
- *REGENXBIO to host conference call and webcast to review the interim data from the AAVIATE trial on Friday, October 1, 2021, at 5:00 p.m. ET featuring wet AMD Key Opinion Leaders, Nikolas London, M.D., and Peter Campochiaro, M.D.*

REGENXBIO Inc. (Nasdaq: RGNX) today announced that data from the RGX-314 clinical trials will be presented in two oral presentations at the Retina Society 54th Annual Scientific Meeting taking place in Chicago, IL, from September 29 to October 2, 2021. RGX-314 is a potential best-in-class, one-time gene therapy for the treatment of wet AMD. The presentations will include interim results at six months of follow-up for patients in Cohort 1 (dose level: 2.5×10^{11} genome copies per eye (GC/eye)) of the Phase II AAVIATE[®] trial in patients with wet age-related macular degeneration (wet AMD). This trial is designed to evaluate the in-office, suprachoroidal delivery of RGX-314.

In connection with the presentation, REGENXBIO will host a webcast and conference call with accompanying slides on Friday, October 1, 2021, at 5:00 p.m. ET. This event will feature Nikolas London, M.D., M.S., F.A.C.S., Partner and Director of Research, Retina Consultants of San Diego, Chief of Ophthalmology, Scripps Memorial Hospital, and Peter Campochiaro, M.D., Director, Retinal Cell and Molecular Laboratory, Professor of Ophthalmology, The Wilmer Eye Institute, The Johns Hopkins University School of Medicine. Conference call details are below.

The Retina Society 54th Annual Scientific Meeting presentations include:

Title: Subretinal Delivery of RGX-314 for Neovascular AMD: End of Study Phase I/IIa Results (Encore Presentation)

Presenter: Allen Ho, M.D., Director of Retina Research at Wills Eye Hospital and Mid Atlantic Retina

Session Title: Wet Age-Related Macular Degeneration II

Date/Time: Friday, October 1, 2021, from 2:41 to 2:47 p.m. CT

Type: Oral presentation

Title: Suprachoroidal Delivery of RGX-314 for Neovascular AMD: Initial Results from the Phase II AAVIATE[®] Study

Presenter: Nikolas London, M.D., M.S., M.S., F.A.C.S., Partner and Director of Research, Retina Consultants of San Diego, Chief of Ophthalmology, Scripps Memorial Hospital

Session Title: Wet Age-Related Macular Degeneration II

Date/Time: Friday, October 1, 2021, from 2:47 to 2:51 p.m. CT

Type: Oral presentation

Conference Call

REGENXBIO will host a webcast and conference call with accompanying slides on Friday, October 1, 2021 at 5:00 p.m. ET. To access a live or recorded webcast of the call and accompanying slides, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international) and enter the passcode 4577338. The recorded webcast will be available for approximately 30 days following the call.

About RGX-314

RGX-314 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 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 in multiple therapeutic areas.

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, 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, 2020 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.

SCS Microinjector® is a trademark of Clearside Biomedical, Inc. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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