REGENXBIO to Present Interim Phase I/IIa Trial Update for RGX-314 for the Treatment of Wet AMD at the American Academy of Ophthalmology 2019 Annual Meeting in October

August 27, 2019 11:00 AM EDT

- Dr. Jeffrey Heier to present the data at the late breaking developments session during the Retina Subspecialty day program
- Interim update to include six-month results from all five cohorts in the Phase I/IIa trial
- REGENXBIO remains on track to initiate a Phase IIb trial for wet AMD in late 2019

ROCKVILLE, Md., Aug. 27, 2019 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that an interim trial update from the RGX-314 Phase I/IIa clinical trial for the treatment of wet age-related macular degeneration (wet AMD) will be reported during a presentation at the Retina Subspecialty Day program of the American Academy of Ophthalmology (AAO) 2019 Annual Meeting in San Francisco, CA. The results will be presented by Dr. Jeffrey S. Heier, M.D., Co-President and Director of Retina Research at Ophthalmic Consultants of Boston, and primary investigator for the trial. Dr. Heier will present the interim results that include data for all five dose cohorts.

"We are excited by the previously announced positive interim data from the Phase I/IIa RGX-314 trial and the potential of NAV gene therapy as a one-time treatment for wet AMD, and we look forward to the presentation of data from all five dose cohorts from the Phase I/IIa clinical trial in wet AMD at the upcoming AAO meeting," said Steve Pakola, M.D., Senior Vice President and Chief Medical Officer of REGENXBIO.

Details of the presentation are as follows:

Title: Results of Cohorts 1-5 for the RGX-314 Phase I/IIa Study of Gene Therapy for Neovascular wAMD
Presenter: Jeffrey S. Heier, M.D., Co-President and Director of Retina Research at Ophthalmic Consultants of Boston
Session date/time: Friday, October 11, 2019, 4:18 p.m. PT / 7:18 p.m. ET
Session title: RET11 – Section VIII: Late Breaking Developments, Part 1
Room: Moscone Center, WEST 3002

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," "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, 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, 2018, 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. 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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