REGENXBIO Announces Additional Positive Interim Phase I Trial Update for RGX-314 for the Treatment of Wet AMD at the American Academy of Ophthalmology 2018 Annual Meeting

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- Sustained protein expression levels at six months reported in Cohort 3
- A higher dose Cohort 4 recently completed dosing
- Company continues plans to proceed to Phase II clinical trial in 2019
- Additional data and program updates are expected in early 2019

ROCKVILLE, Md., Oct. 26, 2018 /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 updated results from the ongoing Phase I study of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD).

The results were presented by Jeffrey Heier, M.D., Co-President and Director of Retina Research at Ophthalmic Consultants of Boston, and primary investigator for the trial, at the Retina Subspecialty Day program of the American Academy of Ophthalmology (AAO) 2018 Annual Meeting.

In August 2018, REGENXBIO presented an interim trial update on the first three dose cohorts, as of July 27, 2018, demonstrating that RGX-314 was well-tolerated and showed dose-dependent protein expression levels and dose-dependent reductions in anti-vascular endothelial growth factor (anti-VEGF) injections, along with maintenance of central retinal thickness and vision. Of the subjects treated at the 6 x 10^10 (Cohort 3) genome copies (GC)/eye dose, 50 percent were free of anti-VEGF injections at six months. A summary of these data can be found **here**.

The interim data update presented today contains new assessments of protein expression levels at six months after administration of RGX-314 for Cohort 3. These new data are summarized below.

Summary of Cohort 3 Intraocular Protein Expression Levels at Six Months

RGX-314 protein expression continues to be detected in all subjects in Cohort 3. At six months, Cohort 3 subjects demonstrated evidence of sustained RGX-314 protein expression levels, as measured from aqueous samples by electrochemiluminescence immunoassay (ECL) after administration of RGX-314 (see Table 1).

Table 1: RGX-314 Protein Expression Levels (ng/ml) of Cohort 3 at One Month and Six Months (N=6)

Visit (Months)	1	6
N	6	6
Mean Protein Level (ng/ml)	160.2	217.8*
Median Protein Level (ng/ml)	93.1	181.7*

*One subject received an anti-VEGF rescue injection 1 month prior to sample.

"Many wet AMD patients require frequent anti-VEGF injections to maintain their vision. The burden of such treatment weighs upon patients, their family and their caregivers. Patients such as these were enrolled in the RGX-314 trial," said Dr. Heier. "The dose-dependent protein expression in the trial, coupled with the sustained expression at six months in Cohort 3 is encouraging. RGX-314 has the potential to provide optimal long-term visual outcomes with a single treatment."

Detailed study findings presented by Dr. Heier at AAO 2018 are available **here**.

Phase I Trial Dose Expansion and Status of Expected Phase II Trial

REGENXBIO recently announced it has completed dosing (six subjects) in the Phase I clinical trial of RGX-314 at the 1.6 x 10^11 GC/eye (Cohort 4) dose. A total of 24 subjects have now been dosed in the trial. REGENXBIO plans to initiate a Phase II clinical trial for RGX-314 in 2019. Final determination of the study design is under way. REGENXBIO expects to provide further information regarding the initial assessments of protein expression levels from Cohort 4, anticipated Phase II clinical trial design and overall plans for the RGX-314 clinical program in early 2019.

"We continue to be encouraged by the RGX-314 interim results and the potential of NAV® gene therapy as a one-time treatment for wet AMD, a leading cause of irreversible blindness and visual impairment in the world," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We extend our thanks to the patients and the investigators who have participated in this trial and contributed to the growing body of evidence to give hope to patients living with wet AMD for a long-lasting solution to their condition."

Seven leading retinal surgery centers across the United States are participating in the Phase I clinical trial of RGX-314. This multi-center, open-label, multiple-cohort, dose-escalation clinical trial is designed to assess the safety and tolerability of RGX-314 as a one-time therapy for patients with previously treated wet AMD. For further details on the trial, enrollment criteria and eligibility, visit **clinicaltrials.gov/ct2/show/NCT03066258**.

About the Phase I Clinical Trial of RGX-314

RGX-314 is currently being evaluated in a Phase I, multi-center, open-label, multiple-cohort, dose-escalation study in adult subjects with wet AMD in the United States. The study has enrolled 24 previously treated wet AMD subjects across four cohorts that are responsive to anti-VEGF therapy and are 50 years of age or older. The study is designed to evaluate four doses of RGX-314 (3 x 10^9 GC/eye, 1 x 10^10 GC/eye, 6 x 10^10 GC/eye, and 1.6 x 10^11 GC/eye). The primary purpose of the clinical study is to evaluate the safety and tolerability of RGX-314 at 24 weeks after a single dose administered by subretinal delivery. Primary endpoints include safety and tolerability and secondary endpoints include ocular examinations, visual acuity, imaging (including spectral domain optical coherence tomography (SD-OCT)) and the need for additional anti-VEGF therapy. Following completion of the primary study period, subjects will enter a follow-up period and will continue to be assessed until week 106 for long-term safety and durability of effect.

About RGX-314

RGX-314 is being developed as a one-time subretinal treatment for wet AMD. It includes the NAV AAV8 vector encoding an antibody fragment which inhibits VEGF, modifying the pathway for formation of new leaky blood vessels which lead to retinal fluid accumulation and vision loss. In preclinical animal models with conditions similar to macular degeneration, significant and dose-dependent reduction of blood vessel growth and prevention of disease progression was observed after a single subretinal dose of RGX-314.

About Wet AMD

Wet AMD is characterized by loss of vision due to new leaky blood vessel formation in the retina. This results in fluid leakage that can manifest in physical changes in the structure of the retina and loss of vision. Wet AMD is a significant cause of vision loss in the United States, Europe and Japan. There may be more than 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 improve vision and retinal fluid in the majority of patients. These therapies, however, require repetitive and inconvenient intraocular injections, typically ranging from every four to eight weeks in frequency, to maintain efficacy. Patients often experience a decline in the initial vision gain from therapy with reduced frequency of treatment over 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, 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, 2017 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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