REGENXBIO Announces Initiation of Second Pivotal Trial in RGX-314 Clinical Program for the Treatment of Wet AMD Using Subretinal Delivery

January 10, 2022 12:05 PM EST

ROCKVILLE, Md., Jan. 10, 2022 /PRNewswire/ --

- *ASCENT™, a Phase III clinical trial conducted in partnership with AbbVie, is expected to enroll patients in the United States and Canada*
- *Pivotal trials expected to support BLA submission for RGX-314 in 2024*

REGENXBIO Inc. (Nasdaq: RGNX) today announced the initiation of ASCENT™, the second of two Phase III pivotal trials to evaluate the efficacy and safety of subretinal delivery of RGX-314 in patients with wet age-related macular degeneration (wet AMD). ASCENT, the first trial to be initiated by REGENXBIO under the eye care collaboration with AbbVie, is currently active and screening patients. RGX-314 is being investigated as a potential one-time gene therapy for the treatment of wet AMD.

A Biologics License Application (BLA) is expected to be submitted to the United States Food and Drug Administration (FDA) in 2024 based on two pivotal trials, ASCENT and the ongoing ATMOSPHERE trial.

"The initiation of ASCENT is an important milestone for the pivotal program for subretinal delivery of RGX-314 in patients with wet AMD, and it is the first trial to be started under our partnership with AbbVie," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "ASCENT is designed similarly to our ongoing ATMOSPHERE trial, and key design elements for both pivotal studies are based on the positive long-term data from our dose-escalation Phase I/IIa trial of RGX-314. We look forward to advancing both trials to support our goal of a BLA filing in 2024."

"The initiation of this Phase III study, a first under our collaboration with REGENXBIO, is an important advancement in our continued pursuit of innovative treatments for patients living with difficult-to-treat retinal diseases, visual impairment, and devastating vision loss," said Michael Robinson, MD, vice president, clinical development, ophthalmology, AbbVie. "We look forward to identifying the full potential of RGX-314 as part of our commitment to advancing vision care."

ASCENT is a multi-center, randomized, active-controlled trial evaluating the efficacy and safety of subretinal delivery of RGX-314 across two dose arms, $6.4x10^{10}$ genomic copies per eye (GC/eye) and $1.3x10^{11}$ GC/eye, versus intravitreal injections of aflibercept, per label instructions. The primary endpoint of the trial is non-inferiority to aflibercept based on the change from baseline in Best Corrected Visual Acuity (BCVA) at one year. The trial will enroll approximately 465 patients across the two dose arms and the aflibercept control arm.

About RGX-314

REGENXBIO is investigating RGX-314 in collaboration with AbbVie as a potential one-time treatment for wet AMD, diabetic retinopathy, and other chronic retinal conditions. RGX-314 includes the NAV AAV8 vector containing a gene encoding for a monoclonal 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[1].

Two separate routes of administration of RGX-314 to the eye are being evaluated, including 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[2]. 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[3]. 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[4]. These therapies, however, require life-long repeated intraocular injections to maintain efficacy[5,6] and patients often experience a decline in vision with reduced frequency of treatment over time[7].

About ASCENT™

ASCENT is a multi-center, randomized, active-controlled trial to evaluate the efficacy and safety of a single administration of RGX-314 versus standard of care in patients with wet AMD. The trial is designed to enroll 465 patients at a 1:1:1 ratio across two RGX-314 dose arms ($6.4x10^{10}$ genomic copies per eye (GC/eye) and $1.3x10^{11}$ GC/eye delivered subretinally) and an active control arm of bi-monthly intravitreal injections of aflibercept (0.5 mg/eye), per label instructions. The primary endpoint of the trial is non-inferiority to aflibercept based on change from baseline in Best Corrected Visual Acuity (BCVA) at one year. Secondary endpoints of the trial include safety and tolerability, change in central retinal thickness (CRT) and need for supplemental anti-VEGF injections in the treatment arms. Patient selection criteria will include patients with wet AMD who are responsive to anti-VEGF treatment and will be independent of preexisting neutralizing antibody status. Patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314. The trial will be conducted at approximately 70 clinical sites across the United States and Canada. REGENXBIO and partner AbbVie are collaborating and sharing costs on this trial.

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.

About AbbVie
AbbVie's mission is to discover and deliver innovative medicines that solve serious health issues today and address the medical challenges of tomorrow. We strive to have a remarkable impact on people's lives across several key therapeutic areas: immunology, oncology, neuroscience, eye care, virology, women's health and gastroenterology, in addition to products and services across its Allergan Aesthetics portfolio. For more information about AbbVie, please visit us at www.abbvie.com. Follow @abbvie on Twitter, Facebook, Instagram, YouTube and LinkedIn.

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