AbbVie and REGENXBIO Announce Updates on the ABBV-RGX-314 Clinical Program

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- *Pivotal data evaluating the safety and efficacy of the subretinal delivery of ABBV-RGX-314 in patients with wet age-related macular degeneration (wet AMD) are expected in 2026*
- *AbbVie and REGENXBIO will plan the Phase 3 clinical program of investigational ABBV-RGX-314 in diabetic retinopathy (DR)*

NORTH CHICAGO, Ill. and ROCKVILLE, Md., Jan. 13, 2025 /PRNewswire/ -- AbbVie (NYSE: ABBV) and REGENXBIO Inc. (Nasdaq: RGNX) today announced updates to the ABBV-RGX-314 clinical program.

ABBV-RGX-314 in Wet Age-Related Macular Degeneration (wet AMD), Subretinal Delivery

Data from the ATMOSPHERE® and ASCENT ™ pivotal trials evaluating the safety and efficacy of the subretinal delivery of ABBV-RGX-314 in patients with wet AMD are expected in 2026.

ABBV-RGX-314 in Diabetic Retinopathy (DR), Suprachoroidal Delivery

AbbVie and REGENXBIO will plan a Phase 3 clinical program. The clinical program will utilize the in-office SCS Microinjector® to deliver gene therapy to the suprachoroidal space of the eye.

"Retinal diseases are progressive, with wet AMD and DR among the leading causes of blindness," said Michael Robinson, M.D., vice president, global head of ophthalmology clinical development, AbbVie. "More treatment options are needed to help relieve the current treatment burden of chronic, frequent dosing. We are excited to continue moving closer to our goal of delivering an additional treatment option to patients with wet AMD and DR in hopes of addressing their significant unmet needs."

"ABBV-RGX-314 has the potential to help millions of people living with wet AMD and DR, globally, who are facing these debilitating diseases," said Curran Simpson, president and chief executive officer, REGENXBIO. "Together with AbbVie, we are excited to continue developing ABBV-RGX-314 as the first potential one-time gene therapy for wet AMD and DR."

About ABBV-RGX-314

ABBV-RGX-314 is being investigated as a potential one-time treatment for wet AMD, diabetic retinopathy and potentially other chronic retinal conditions. ABBV-RGX-314 consists of the NAV® AAV8 vector, which encodes an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). ABBV-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]

AbbVie and REGENXBIO are advancing the development of two separate routes of administration of ABBV-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.[2] Wet AMD is a significant cause of vision loss in the United States, Europe and Japan, with up to two 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] Due to the burden of treatment, patients often experience a decline in vision with reduced frequency of treatment over time.[7]

About Diabetic Retinopathy

Diabetic retinopathy (DR) is the leading cause of vision loss in adults between 24 and 75 years of age worldwide.[8] DR affects nearly 10 million people in the United States alone.[9] The spectrum of DR severity ranges from non-proliferative diabetic retinopathy (NPDR) to proliferative diabetic retinopathy (PDR).[4] As DR progresses, a large proportion of patients develop vision threatening complications, including diabetic macular edema (DME) and neovascularization that can lead to blindness.[10] Current treatment options for patients with NPDR typically include "watchful waiting" or anti-VEGF treatment. For patients with PDR, current treatment options include anti-VEGF treatment or retinal laser; surgical treatment may be required for advanced PDR.[2]

About AbbVie

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

ABOUT REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for rare and retinal diseases, including RGX-202 for the treatment of Duchenne, ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, and RGX 121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA® for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.regenxbio.com.

Forward-Looking Statements

Some statements in this news release are, or may be considered, forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995. The words "believe," "expect," "anticipate," "project" and similar expressions and uses of future or conditional verbs, generally identify forward-looking statements. AbbVie cautions that these forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those expressed or implied in the forward-looking statements. Such risks and uncertainties include, but are not limited to, challenges to intellectual property, competition from other products, difficulties inherent in the research and development process, adverse litigation or government action, and changes to laws and regulations applicable to our industry. Additional information about the economic, competitive, governmental, technological and other factors that may affect AbbVie's operations is set forth in Item 1A, "Risk Factors," of AbbVie's 2023 Annual Report on Form 10-K, which has been filed with the Securities and Exchange Commission, as updated by its subsequent Quarterly Reports on Form 10-Q. AbbVie undertakes no obligation, and specifically declines, to release publicly any revisions to forward-looking statements as a result of subsequent events or developments, except as required by law.

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