



Adverum Biotechnologies Announces Publication of Preclinical Long-Term Expression and Efficacy Data in Molecular Therapy

December 3, 2018

Results demonstrate single intravitreal administration of ADVM-022 gene therapy may provide a safe and effective long-term treatment option for wet AMD, potentially decreasing the need for frequent anti-VEGF injections

MENLO PARK, Calif., Dec. 03, 2018 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ophthalmology and rare diseases, today announced the publication of preclinical long-term expression and efficacy data in *Molecular Therapy*, the leading international journal for research on the development of molecular and cellular therapeutics to correct genetic and acquired diseases.

The publication entitled, "Preclinical evaluation of ADVM-022, a novel gene therapy approach to treating wet age-related macular degeneration," reported the following:

- A single intravitreal administration of ADVM-022 in non-human primates (NHPs) provided stable and robust intraocular expression of aflibercept and resulted in high levels of aflibercept within the retina and choroid where wet age-related macular degeneration (wet AMD) occurs
- Robust aflibercept expression in retinal and choroidal tissues obtained at 16 months post-administration demonstrated that cells transduced with ADVM-022 may continue to generate aflibercept for an extended period of time
- In a laser-induced choroidal neovascularization model in NHPs, a single intravitreal administration of ADVM-022 13 months before lasering prevented the occurrence of clinically relevant choroidal neovascularization lesions, similar to animals that received a bolus of intravitreal aflibercept (standard-of-care) at the time of lesioning
- A single intravitreal administration of ADVM-022 may provide a safe and effective long-term treatment option for wet AMD

The full online publication can be accessed at the following link: <https://doi.org/10.1016/j.ymthe.2018.11.003>

Adverum recently received Fast Track Designation from the U.S. Food and Drug Administration (FDA) and announced initiation of a phase 1 trial evaluating ADVM-022 in patients with wet AMD.

About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD

The multi-center, open-label, phase 1, dose-escalation trial is designed to assess the safety and tolerability of a single intravitreal (IVT) administration of ADVM-022 in patients with wet AMD who are responsive to anti-vascular endothelial growth factor (VEGF) treatment. At least six leading retinal centers across the United States are expected to participate in the phase 1 trial. The trial is expected to enroll 18 patients and evaluate three doses of ADVM-022; first dose: 6×10^{11} vg/eye, second dose: 2×10^{12} vg/eye, and third dose: 6×10^{12} vg/eye. Patients will be administered a tapering prophylactic corticosteroid regimen. The primary endpoint of the trial is the safety and tolerability of ADVM-022 at 24 weeks after a single IVT administration. Secondary endpoints include changes in best-corrected visual acuity (BCVA), measurement of central retinal thickness (CRT), as well as mean number of rescue anti-VEGF injections and percentage of patients needing rescue anti-VEGF injections. Each patient enrolled is expected to be followed for a total of two years. For further details about the trial, enrollment and eligibility please contact adverumopticstudy@adverum.com or visit <https://clinicaltrials.gov/ct2/show/NCT03748784>.

About ADVM-022 Gene Therapy Candidate in Wet AMD

Adverum's gene therapy candidate, ADVM-022, utilizes a proprietary vector capsid (AAV.7m8) carrying an aflibercept coding sequence under the control of a proprietary expression cassette and is administered as a single intravitreal administration. AAV.7m8 was discovered by directed evolution and is a variant of AAV2. The vector was designed with the purpose of improved transduction efficiency to the retina when administered intravitreally. ADVM-022 is designed to provide potentially sustained therapeutic levels of aflibercept and to minimize the burden of frequent anti-VEGF injections.

About Wet Age-related Macular Degeneration (Wet AMD)

Age-related macular degeneration (AMD) is a progressive disease affecting the retinal cells in the macula, the region of the eye responsible for central vision. Disease progression results in the death of retinal cells and the gradual loss of vision. Approximately 10% of patients living with AMD have an advanced form of the disease called wet AMD, in which blood vessels begin to invade the cellular space between the layers of cells in the retina. These new blood vessels are often leaky, which results in fluid and blood in the retina and causes vision loss.

Wet AMD is a leading cause of vision loss in subjects over 60 years of age. A significant number of individuals are impacted by this disease, which has a prevalence of approximately 1.2 million individuals in the U.S. The incidence of new cases of wet AMD in the U.S. is approximately 150,000 to 200,000 annually, and this number is expected to grow significantly based on the country's aging population.

The current treatment regimen can be burdensome, as patients generally require intravitreal injections with anti-VEGF proteins every 4-12 weeks.

Compliance with this regimen can be difficult for patients and their caregivers, leading to compliance deficiencies and loss of vision from under dosing of treatment.

About Adverum Biotechnologies, Inc.

Adverum is a clinical-stage gene therapy company targeting unmet medical needs in ophthalmology and rare diseases. Adverum develops gene therapy product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein. Adverum has collaboration agreements with Regeneron Pharmaceuticals to research, develop, and commercialize gene therapy products for ophthalmic diseases and Editas Medicine to explore the delivery of genome editing medicines for the treatment of inherited retinal diseases. Adverum's core capabilities include clinical development, novel vector discovery and in-house manufacturing expertise, specifically in scalable process development, assay development, and current Good Manufacturing Practices quality control. For more information please visit www.adverum.com.

Forward-Looking Statements

Statements contained in this press release regarding events or results that may occur in the future are "forward looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements regarding AAV.7m8 vector designed to have improved transduction efficiency to the retina, a single intravitreal administration of ADV-022 may provide a safe and effective long-term treatment option for wet AMD, Adverum's OPTIC Phase 1 clinical trial of ADV-022, and expected growth in the number of new cases of wet AMD in the U.S., all of which are based on certain assumptions made by Adverum on current conditions, expected future developments and other factors Adverum believes are appropriate in the circumstances. Actual results and timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, the risk of a delay in the enrollment of patients in Adverum's clinical studies or in the manufacturing of products to be used in such clinical studies, as well as risks and uncertainties facing Adverum described more fully in Adverum's periodic reports filed with the Securities and Exchange Commission (SEC), especially under the caption "Risk Factors" in its latest Quarterly Report on Form 10-Q filed with the SEC on November 8, 2018. As a result, you should not place undue reliance on these forward-looking statements. All forward-looking statements contained in this press release speak only as of the date on which they were made. Adverum undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

Investor and Media Inquiries:

Katherine Bock

Vice President Investor Relations & Corporate Communications

Adverum Biotechnologies, Inc.

650-656-9347

kbock@adverum.com

 [ADVERUM_ID_RGB
200x49.jpg](#)

Source: Adverum Biotechnologies, Inc.