Adverum Biotechnologies Receives Fast Track Designation for ADVM-022 Gene Therapy for the Treatment of wAMD

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- *ADVM-022 is a unique single-administration gene therapy delivered intravitreally for the treatment of wAMD*
- *OPTIC Phase 1 clinical trial initiation expected in 4Q18*

MENLO PARK, Calif., Sept. 19, 2018 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases, today announced the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ADVM-022. ADVM-022 is a novel gene therapy candidate for the treatment of wet age-related macular degeneration (wAMD).

"The FDA's Fast Track designation is an important recognition of our ADVM-022 gene therapy program," said Leone Patterson, interim president and chief executive officer of Adverum Biotechnologies. "We look forward to working with the FDA and benefiting from the potential expedited development and regulatory path offered by their Fast Track program."

A Fast Track designation is intended to facilitate the development and expedite the review of drugs and biologics to treat serious conditions and fill unmet medical needs. The designation enables more frequent communication with the FDA throughout a product candidate's development and review process. The designation also provides eligibility for Priority Review and Accelerated Approval, which may potentially result in a shorter FDA review process.

About the OPTIC Phase 1 Trial of ADVM-022 in wAMD
The multi-center, open-label, Phase 1, dose-escalation trial is designed to assess the safety and tolerability of a single intravitreal (IVT) injection of ADVM-022 in patients with wAMD who are responsive to anti-vascular endothelial growth factor (VEGF) treatment. A number of leading retinal centers across the United States are planned to participate in the Phase 1 trial. The trial is expected to enroll 18 patients and will evaluate three doses of ADVM-022; first dose: 6 x 10^11 vg/eye, second dose: 2 x 10^12 vg/eye, and third dose: 6 x 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 injection. Secondary endpoints include changes in best-corrected visual acuity (BCVA) at 24 weeks, measurement of central retinal thickness (CRT), and percent of patients needing rescue aflibercept injections. Each patient enrolled will be followed for a total of two years.

About ADVM-022 Gene Therapy Candidate
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 injection. Excess VEGF activity can lead to wAMD progression and vision loss and current anti-VEGF therapies need to be administered frequently to patients (every 4-12 weeks). Reduced compliance with the current approved regimen is associated with decreased vision. Treatment with ADVM-022 is designed to provide potentially sustained therapeutic levels of aflibercept and to minimize the burden of frequent anti-VEGF injections.

In May 2018, long-term preclinical efficacy data on ADVM-022 was presented at the American Society of Gene & Cell Therapy 21st Annual Meeting. In this preclinical study in non-human primate models of wAMD, the efficacy of ADVM-022 at 13 months post-administration was consistent with earlier reported data, demonstrating that single intravitreal injection of ADVM-022 was found to be safe and statistically significant ($p<0.0001$) in preventing the development of Grade IV lesions compared to the untreated vehicle control group. ADVM-022 induced long-term efficacy that was comparable to aflibercept, an anti- VEGF standard-of-care therapy. In this preclinical study, ADVM-022 was well-tolerated, with no serious adverse events.

In the same preclinical study, ADVM-022 induced sustained intraocular expression of aflibercept for up to 16 months following a single intravitreal injection. Robust levels of aflibercept protein were detected up to 16 months in aqueous and vitreous humor and, more importantly, in retina and choroid tissues, where neovascularization occurs in wAMD.

About Wet Age-related Macular Degeneration (wAMD)
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 advance form of the disease called wAMD, 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.

wAMD 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 wAMD 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 underdosing of treatment.

About Adverum Biotechnologies, Inc.
Adverum is a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases. Adverum has a robust pipeline

that includes product candidates designed to treat rare diseases alpha-1 antitrypsin (A1AT) deficiency and hereditary angioedema (HAE) as well as wet age-related macular degeneration (wAMD). Leveraging a next-generation adeno-associated virus (AAV)-based directed evolution platform, Adverum generates 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 and in-house manufacturing expertise, specifically in process development and assay development. For more information please visit www.adverum.com.

Forward-Looking Statements

Statements contained in this press release regarding matters 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 Adverum's expectations regarding the new OPTIC clinical trial, including expected timing and structure, the expected benefits of receiving fast track designation, the benefits ADVM-022 may have to patients with wAMD, and the expectation that new cases of wAMD in the U.S. is expected to grow significantly, all of which are based on certain assumptions made by Adverum, expected future developments and other factors Adverum believes are appropriate in the circumstances. Actual results may differ from those set forth in these forward-looking statements as a result of various risks and uncertainties, which include, without limitation, the risk of unexpected delays in the enrollment of patients in Adverum's new OPTIC clinical trial or in the manufacturing of ADVM-022 to be used in the OPTIC clinical trial, as well as the 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 Adverum's latest Quarterly Report on Form 10-Q filed with the SEC on August 8, 2018. 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.

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