Adverum Announces First Patient Dosed in OPTIC Phase 1 Trial of ADVM-022 Gene Therapy in Wet Age-Related Macular Degeneration

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- *ADVM-022 is the first intravitreally delivered gene therapy utilizing directed evolution AAV.7m8 vector for wet AMD*
- *OPTIC trial to evaluate single administration of ADVM-022 for wet AMD*
- *Interim trial update expected by 1Q20*

MENLO PARK, Calif., Nov. 19, 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 that the first patient was dosed in the OPTIC phase 1 trial evaluating ADVM-022 for patients with wet age-related macular degeneration (wet AMD).

“We are excited to announce the first patient dosed in our OPTIC phase 1 trial assessing a potential single intravitreal administration of ADVM-022 for wet AMD,” said Leone Patterson, chief executive officer of Adverum Biotechnologies. “This milestone is the next step in bringing a new meaningful option to patients, that may provide long-lasting therapy without the need of chronic or frequent anti-VEGF injections. We look forward to providing an interim update of this phase 1 by the first quarter of 2020.”

“While significant advances have been made in the management of wet AMD with the introduction of anti-VEGF therapies, there remains a need to address the treatment burden and suboptimal outcomes patients face from the need of repeated dosing,” said Dr. David S. Boyer, Partner at Retina-Vitreous Associates Medical Group and Clinical Professor of Ophthalmology at University of Southern California/Keck School of Medicine. “I am encouraged by the early data on ADVM-022, the potential to provide long-term durability with a single administration of an anti-VEGF therapy for patients and the ease of intravitreal delivery of ADVM-022, which utilizes the current standard of care delivery method. With these combined, ADVM-022 has the potential to be the next paradigm shift in treating wet AMD.”

At least six leading retinal centers across the United States are expected to participate in the phase 1 trial. For further details about the trial, enrollment and eligibility please contact adverumopticstudy@adverum.com.

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 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 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 will be followed for a total of two years.

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. ADVM-022 is designed to provide potentially sustained therapeutic levels of aflibercept and to minimize the burden of frequent anti-VEGF injections.

In October 2018, Adverum presented long-term preclinical efficacy data in wet AMD, for ADVM-022, at the European Society of Gene & Cell Therapy’s (ESGCT) 26th Annual Congress. Key highlights included:

- A single intravitreal administration of ADVM-022 in NHPs at dose ranges of 2 x 10 ^{11} vg/eye to 2 x 10 ^{12} vg/eye provided stable intraocular expression of aflibercept at levels comparable with the levels measured in aflibercept recombinant protein-injected eyes approximately 3 to 4 weeks post-dose in all of the following: vitreous humor, aqueous humor, retina and choroid
- A single intravitreal administration of ADVM-022 provided robust expression of aflibercept, sustained for approximately two years post-dose in non-human primates (NHPs)

In September 2018, Adverum received Fast Track designation for ADVM-022 in wet AMD from the U.S. Food and Drug Administration (FDA).

In May 2018, long-term preclinical efficacy data in NHP models on ADVM-022 in wet AMD were presented at the American Society of Gene & Cell Therapy (ASGCT) 21st Annual Meeting. Key highlights included:

- The efficacy of ADVM-022 at 13 months post-administration was consistent with earlier reported data, demonstrating that a single intravitreal administration 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. ADVM-022 was well-tolerated, with no serious adverse events

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 advance 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 Adverum's expectation of providing an interim update on the phase 1 trial of ADVM-022 assessing a potential single intravitreal administration for wet AMD, 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.

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