Adverum Biotechnologies Doses First Patient in Second Cohort of OPTIC Phase 1 Clinical Trial of ADVM-022 Gene Therapy for Wet AMD

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-- Continued Progress in the OPTIC Phase 1 Clinical Trial

-- Patients Receiving Single Intravitreal Injection of ADVM-022 Dosed at 2 x 10^11 vg/eye

MENLO PARK, Calif., June 10, 2019 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today announced that the first patient was dosed in the second cohort of the ongoing OPTIC phase 1 clinical trial for ADVM-022 for the treatment of wet age-related macular degeneration (wet AMD). Patients are receiving a single intravitreal injection of gene therapy candidate ADVM-022 at a dose of 2 x 10^11 vg/eye.

"We are pleased to report continued progress in the OPTIC trial by dosing the first patient in the second cohort," said Aaron Osborne, MBBS MRCOphth, chief medical officer of Adverum. "We believe ADVM-022 has potential to provide sustained therapeutic benefit with a single intravitreal injection to patients living with wet AMD. In the first cohort of patients, we have seen a robust preliminary anatomical response and no serious adverse events to date. We look forward to completing recruitment of the second cohort in OPTIC, and to presenting the 24 week primary and secondary outcomes from the first cohort of patients at a scientific meeting in the second half of 2019."

"ADVM-022 has the potential to transform the way we treat patients with neovascular AMD," added Arshad M. Khanani, M.D, M.A, trial investigator and director of clinical research at Sierra Eye Associates. "In addition to greatly alleviating treatment burden, I believe that a more consistent treatment could improve long-term vision outcomes for patients in clinical practice. We are excited to be participating in OPTIC."

Eight leading retinal centers across the United States are expected to participate in the OPTIC phase 1 trial for ADVM-022. For more information on the OPTIC phase 1 clinical trial of ADVM-022 in wet AMD, please visit https://clinicaltrials.gov/ct2/show/NCT03748784.

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. In the first cohort, patients (n=6) received ADVM-022 at a dose of 6 x 10^11 vg/eye, and in the second cohort, patients will receive a dose of 2 x 10^11 vg/eye due to the robust preliminary anatomical response observed from patients in the first cohort. 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

Adverum's gene therapy candidate for wet AMD, 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 sustained therapeutic levels of aflibercept, minimize the burden of frequent anti-VEGF injections, and improve real-world vision outcomes for patients with wet AMD.

In September 2018, Adverum received Fast Track designation for ADVM-022 in wet AMD from the FDA.

About Wet Age-related Macular Degeneration (Wet AMD)

Age-related macular degeneration (AMD) is a progressive disease affecting the macula, the region of the retina at the back of the eye responsible for central vision. Disease progression results in the death of retinal cells and loss of vision. Wet AMD is an aggressive form of AMD, affecting around 10-15% of patients living with AMD, but accounting for approximately 90% of severe vision loss due to the disease. In patients with wet AMD, also known as neovascular AMD, abnormal blood vessels grow underneath and into the retina. These abnormal blood vessels leak fluid and blood into and beneath the retina, causing vision loss.

Wet AMD is a leading cause of vision loss in patients over 60 years of age, with a prevalence of approximately 1.2 million individuals in the U.S. and 3 million worldwide. 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 as the country's population ages.

The current standard-of-care therapy for wet AMD is anti-VEGF intravitreal injections. These are effective but typically require long-term eye injections every 4-8 weeks in order to maintain vision gains. Compliance with this regimen can be difficult for patients, caregivers, and healthcare systems, leading to suboptimal dosing and loss of vision from undertreatment.

About Adverum Biotechnologies, Inc.

Adverum is a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases. Adverum develops gene therapy product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein. The company has collaboration

agreements with Regeneron Pharmaceuticals and Editas Medicine. 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 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 plans related to proceeding to dose additional patients in Adverum's OPTIC Phase 1 trial for ADVM-022, the safety of ADVM-022, the potential therapeutic and commercial potential of ADVM-022, the expected timing of completing recruitment of the second cohort in OPTIC and presentation of 24 week primary and secondary outcomes from the first cohort of patients, 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. Adverum may not meet the expectations or projections disclosed in its forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results and the 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 that Adverum's resources will not be sufficient for Adverum to conduct or continue planned development programs and planned clinical trials, 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, the risk that Adverum will not be able to successfully develop or commercialize any of its product candidates and the risk that Adverum will be delayed in receiving or fail to receive required regulatory approvals. Risks and uncertainties facing Adverum are described more fully in Adverum's Quarterly report on Form 10-Q filed with the SEC on May 8, 2019, especially under the caption "Risk Factors." 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, except as required by law.

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