Adverum Biotechnologies Announces Completion of Patient Dosing in Cohort 4 of the OPTIC Phase 1 Trial of ADVM-022 Single Injection Gene Therapy for Wet AMD

July 6, 2020

-- Patients in Cohort 4 received ADVM-022 high dose 6x10^{11} vg and prophylactic steroid eye drops --

-- Data from all four cohorts planned by the end of 2020 --

REDWOOD CITY, Calif., July 06, 2020 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today announced the completion of patient dosing in Cohort 4 of OPTIC, a Phase 1 clinical trial, assessing a single intravitreal (IVT) injection of ADVM-022 in patients with wet age-related macular degeneration (AMD). 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 excitement in the retina community around the OPTIC clinical trial and the ADVM-022 program continues to grow,” said Aaron Osborne, MBBS, chief medical officer of Adverum Biotechnologies. “We look forward to presenting data from all four cohorts by the end of this year. Our goal is to deliver a novel one-time intravitreal treatment to patients living with wet AMD as quickly as possible.”

David S. Boyer, M.D., senior partner, Retina-Vitreous Associates Medical Group and adjunct clinical professor of ophthalmology with the University of Southern California/Keck School of Medicine in Los Angeles, California and investigator in OPTIC added, “Patients enrolled in the OPTIC study have previously required frequent and ongoing anti-VEGF injections to avoid losing vision. The long-term durability beyond 1 year seen with a single intravitreal injection of ADVM-022 in Cohort 1 is unprecedented. ADVM-022 has the potential to transform the treatment paradigm and improve real-world vision outcomes for patients with wet AMD.”

About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD
This multi-center, open-label, Phase 1, dose-ranging 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 Cohort 1 (n=6) and Cohort 4 (n=9), patients received ADVM-022 at the high dose of 6 x 10^{11} vg/eye. In Cohort 2 (n=6) and Cohort 3 (n=9), patients received ADVM-022 at the low dose of 2 x 10^{11} vg/eye. Patients in Cohorts 3 and 4 received six weeks of prophylactic steroid eye drops rather than 13 days of oral steroids which were used in Cohorts 1 and 2. The primary endpoint of the trial is the safety and tolerability of ADVM-022 after a single IVT administration. Secondary endpoints include changes in best-corrected visual acuity (BCVA), measurement of central retinal thickness (CRT), as well as the need for anti-VEGF rescue injections. Each patient enrolled will be followed for a total of two years.

Eleven leading retinal centers across the United States (U.S.) are participating 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 ADVM-022 Gene Therapy
ADVM-022 utilizes a proprietary vector capsid, AAV.7m8, carrying an aflibercept coding sequence under the control of a proprietary expression cassette. ADVM-022 is administered as a one-time intravitreal injection (IVT), designed to deliver long-term efficacy and reduce the burden of frequent anti-VEGF injections, optimize patient compliance and improve vision outcomes for patients with wet age-related macular degeneration (wet AMD) and diabetic macular edema (DME).

In recognition of the need for new treatment options for wet AMD, the U.S. Food and Drug Administration granted Fast Track designation for ADVM-022 for the treatment of wet AMD. Adverum is currently evaluating ADVM-022 in the OPTIC Phase 1 clinical trial in patients with wet AMD and the INFINITY Phase 2 trial in patients with DME.

About Wet Age-related Macular Degeneration (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. In patients with wet AMD, an aggressive form of 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 eye injections every 4-12 weeks in order to maintain vision. Compliance with this regimen can be difficult for patients, caregivers, and healthcare systems, leading to undertreatment and resulting in loss of vision.

About Adverum Biotechnologies
Adverum Biotechnologies (Nasdaq: ADVM) is a clinical-stage gene therapy company targeting unmet medical needs in serious ocular and rare diseases. Adverum is advancing the clinical development of its novel gene therapy candidate, ADVM-022, as a one-time, intravitreal injection for the treatment of patients with wet age-related macular degeneration and diabetic macular edema. For more information, please visit www.adverum.com.

Forward-looking Statements
Statements contained in this press release regarding the 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: the potential for ADVM-022 in treating patients with wet AMD and DME; and Adverum’s expectations that it will present data from all four cohorts of the OPTIC Phase 1 trial for ADVM-022 in wet AMD by the end of this year. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include risks inherent to, without limitation: Adverum’s novel technology, which makes it difficult to predict the time and cost of product candidate development and obtaining regulatory approval; the results of early clinical trials not always being predictive of future results; the potential for future complications or side effects in connection with use of ADVM-022; obtaining regulatory approval for gene therapy product candidates; enrolling patients in clinical trials; reliance on third parties for conducting the OPTIC and INFINITY trials and vector production; the effects of the COVID-19 pandemic on the company’s operations and on the company’s ongoing clinical trials; and ability to fund operations through completion of the OPTIC and INFINITY trials and thereafter. Risks and uncertainties facing Adverum are described more fully in Adverum’s Form 10-Q filed with the SEC on May 28, 2020 under the heading “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.

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