Adverum Biotechnologies Provides Update on OPTIC Phase 1 Trial for ADVM-022 in Wet AMD

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FDA Lifts Clinical Hold on Second Cohort to Allow Dose Escalation

Robust Preliminary Anatomical Response from First Cohort Leads Adverum to Begin Dosing Second Cohort at Lower Dose

MENLO PARK, Calif., May 16, 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 U.S. Food and Drug Administration (FDA) has lifted the clinical hold on ADVM-022 for the second cohort in the company's OPTIC Phase 1 trial for wet age-related macular degeneration (wet AMD), allowing dose escalation to 2 x 10^12 vg/eye. This dose would be three times higher than the dose of 6 x 10^11 vg/eye evaluated in the first cohort. However, based on the robust preliminary anatomical response observed to date in the first cohort (n=6), Adverum will begin dosing the second cohort at a lower dose of 2 x 10^11 vg/eye, three times lower than the dose used in the first cohort. The dosing of patients in this second cohort is expected to begin in June 2019. Adverum believes that ADVM-022 at a dose of 6 x 10^11 vg/eye has demonstrated the potential to provide sustained efficacy following a single intravitreal injection.

As previously announced, the independent data monitoring committee (DMC) unanimously voted to proceed with dose escalation per protocol following their review of the safety data from the first cohort. To date, no patient in the first cohort has experienced a serious adverse event (SAE), with the first patient completing the 24-week (6 month) assessment.

While the FDA has lifted the clinical hold on the second cohort, ADVM-022 remains on partial clinical hold for dosing patients in the third cohort, with the highest dose of 6 x 10^12 vg/eye. Adverum currently does not plan to dose at this highest level, based on the robust preliminary anatomical response observed in the first cohort of patients. Adverum will continue to work closely with the FDA to resolve the remaining CMC comments.

"The preliminary results from the first cohort of patients in the OPTIC trial are exciting. We look forward to presenting 24-week primary and secondary outcomes from this cohort of patients at a scientific meeting in the second half of this year," said Leone Patterson, chief executive officer of Adverum. "We are pleased that the FDA and the DMC independently cleared us to dose escalate in the OPTIC trial. Given the encouraging robust anatomical response from patients in the first cohort, we will begin dosing patients in the second cohort at a lower dose of 2 x 10^11 vg/eye, instead of dose escalating as previously planned."

About ADVM-022 Gene Therapy Candidate for Wet AMD

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. For more information on the OPTIC phase 1 clinical trial of ADVM-022 in wet AMD, please visit https://clinicaltrials.gov/ct2/show/NCT03748784.

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 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. Wet AMD is an advanced form of AMD, affecting approximately 10% of patients living with AMD. In patients with wet 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, and the potential therapeutic and commercial potential of its product candidates, 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 consummate any of its clinical development, regulatory or commercialization goals in a timely manner, or at all, or otherwise carry out the intentions or 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 periodic reports filed with the SEC, 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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