Adverum Biotechnologies Provides Clinical Program Update on ADVM-022 Gene Therapy for Wet AMD

April 15, 2019

- Clinical Hold until Additional CMC Information is Reviewed by FDA -
- Independent DMC Review of Safety Data from First Cohort of Six Patients with No SAEs or DLTs -
- Adverum Plans to Present 24-Week Data from First Cohort of Patients at Scientific Meeting in 2H19 -

MENLO PARK, Calif., April 15, 2019 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today provided a program update on ADVM-022, a novel, single-administration gene therapy being evaluated in the OPTIC Phase 1 clinical trial in patients with wet age-related macular degeneration (wet AMD).

The Company has completed enrollment and dosing of patients (n=6) in the first cohort in the OPTIC trial. The independent data monitoring committee (DMC) determined that enrollment and dosing of patients in the second cohort could proceed. This was based on a review of the preliminary safety data from the first cohort of patients, which has shown no serious adverse events (SAEs) or dose-limiting toxicities (DLTs) following a single intravitreal injection of ADVM-022 at the initial trial dose of 6 x 10^11 vg/eye.

Adverum has received a notification from the U.S. Food and Drug Administration (FDA) in early April requesting additional CMC information and requirements on the ADVM-022 manufacturing process. The FDA has placed the Company's IND on clinical hold until the agency has reviewed Adverum's response, which was submitted last week. The Company is working closely with the agency during its review. The Company expects to resume dosing of patients once the FDA review is completed and the clinical hold is lifted.

"We are working with the FDA to resolve this matter as quickly as possible," said Leone Patterson, chief executive officer of Adverum Biotechnologies. "We are deeply committed to the development of our novel gene therapy ADVM-022 for patients with wet AMD. In the OPTIC trial, the DMC reviewed the safety data and unanimously agreed that we could proceed to dosing the second cohort. No patient has experienced an SAE, with a follow up period of up to five months. We look forward to sharing 24-week primary and secondary outcomes from the first cohort of patients at a scientific meeting in the second half of this year."

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 U.S. Food and Drug Administration (FDA).

In February 2019, Adverum presented long-term preclinical data at the 2019 Angiogenesis, Exudation, and Degeneration conference. The data demonstrated that the normal non-human primate (NHP) retinal structure is maintained for up to 21 months after a single IVT injection of 2x10^12 vg of ADVM-022, which continuously delivers the anti-VEGF molecule aflibercept.

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 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 fully 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. As a leader in ophthalmic gene therapy, Adverum 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 that are not historical facts 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. 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.

Investor and Media Inquiries:
Amy Figueroa, CFA
Investor Relations Consultant
afigueroa@adverum.com
650-823-2704

ADVERUM_ID_RGB 200x49.jpg

Source: Adverum Biotechnologies, Inc.