Adverum Biotechnologies Doses First Patient in Cohort 4 of OPTIC Phase 1 Clinical Trial of ADVM-022 Intravitreal Gene Therapy for Wet AMD

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Patients in Cohort 4 of OPTIC will Receive a Single ADVM-022 Dose of 6 x 10 ^11 vg/eye

REDWOOD CITY, Calif., April 27, 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 that the first patient was dosed in Cohort 4 of the ongoing OPTIC Phase 1 clinical trial for ADVM-022 for the treatment of wet age-related macular degeneration (AMD). Patients in Cohort 4 (n=9) are receiving a single intravitreal injection of gene therapy candidate ADVM-022 at a dose of 6 x 10 ^11 vg/eye (same as Cohort 1) and are receiving steroid eye drop prophylaxis for six weeks (same as Cohort 3).

David S. Boyer, M.D., senior partner, Retina-Vitreous Associates Medical Group and adjunct professor of ophthalmology with the University of Southern California/Keck School of Medicine in Los Angeles said, “The current standard-of-care requires patients with wet AMD to receive frequent anti-VEGF injections to maintain their vision. A one-time treatment such as ADVM-022, which, similar to standard of care, is administered as an in-office intravitreal injection, could transform the treatment paradigm for wet AMD, particularly at this time when it is more important than ever to reduce the need for frequent injections and clinic visits. The data demonstrated in OPTIC have been positive and underscore the potential of ADVM-022 to be a long-lasting treatment option for patients.”

“We are pleased to have enrolled our first patient in Cohort 4, furthering our execution of the OPTIC trial,” said Aaron Osborne, MBBS, chief medical officer of Adverum. “Patients in this Cohort are receiving the higher dose of ADVM-022, which has demonstrated outstanding efficacy and durability in Cohort 1, as has been presented. We believe that utilizing the higher dose of ADVM-022 with the use of steroid eye drop prophylaxis, will further support that our gene therapy candidate, ADVM-022, has the potential to be an important treatment option for patients living with wet AMD. Additionally, I’m grateful for the continued support of our clinical trial sites as we all manage through the global pandemic. Due to COVID-19, sites quickly implemented extra safety precautions for patients and their staff, allowing us to proceed with enrollment. It’s a pleasure to partner with investigators in OPTIC who share our commitment to develop a novel single-administration approach for treating patients with wet AMD and we look forward to sharing data from all four cohorts later this year.”

About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD
The 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, patients (n=6) received ADVM-022 at a higher dose of 6 x 10^11 vg/eye and in Cohort 2, patients (n=6) received ADVM-022 at a lower dose of 2 x 10^11 vg/eye. In Cohort 3, patients (n=9) also received a dose of 2 x 10^11 vg/eye and in Cohort 4, patients (n=9) are receiving a dose of 6 x 10^11 vg/eye. Patients in Cohorts 3 and 4 receive prophylactic steroid eye drops instead 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.

Ten 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 propriety 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, designed to deliver long-term efficacy and reduce the burden of frequent anti-VEGF injections, optimize patient compliance and improve vision outcomes for wet AMD and diabetic retinopathy patients.

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 this disease.

Adverum is currently evaluating ADVM-022 in the OPTIC study, a Phase 1 clinical trial in patients 50 years and older with wet AMD. Additionally, Adverum plans to initiate a Phase 1/2 clinical trial of ADVM-022 for the treatment of diabetic retinopathy in the second half of 2020.

About Adverum Biotechnologies, Inc.
Adverum Biotechnologies (Nasdaq: ADVM) is a clinical-stage gene therapy company targeting unmet medical needs in serious ocular and rare diseases. Adverum is evaluating its novel gene therapy candidate, ADVM-022, as a one-time, intravitreal injection for the treatment of its lead indication, wet age-related macular degeneration. 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 plans for advancing ADVM-022; the potential benefits of ADVM-022: the expected timing of submitting an IND for diabetic retinopathy, 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 achieve any of these in a timely manner, or at all, or otherwise carry out the intentions or meet the expectations

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 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 trial and vector production; and ability to fund operations through completion of the OPTIC trial and thereafter. Risks and uncertainties facing Adverum are described more fully in Adverum's Form 10-K filed with the SEC on March 12, 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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