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

October 24, 2019

Patients in the Third Cohort of OPTIC will Receive a Single ADVM-022 Dose of 2 x 10 ^11 vg/eye

MENLO PARK, Calif., Oct. 24, 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 third cohort (n=9) of the ongoing OPTIC phase 1 clinical trial for ADVM-022 for the treatment of neovascular or "wet" age-related macular degeneration (wet AMD). Patients in this cohort are receiving a single intravitreal injection of gene therapy candidate ADVM-022 at a dose of 2 x 10 ^11 vg/eye.

"We are excited to report dosing the first patient in the third cohort of OPTIC. This expansion of OPTIC will generate important clinical data to support the further development of ADVM-022," said Aaron Osborne, MBBS, chief medical officer of Adverum. "Based on the recently presented data from the first cohort of OPTIC, which demonstrated a sustained response to a single injection of ADVM-022 out to a median of 34 weeks, with no patient in the first cohort requiring anti-VEGF rescue therapy, we believe that ADVM-022 has the potential to be a transformative treatment option for patients with wet AMD."

Dante Pieramici, M.D., co-director of the California Retina Research Foundation, Managing Partner of The California Retina Consultants and investigator in the OPTIC trial, said, "An intravitreal gene therapy that can significantly reduce the number of injections required to maintain vision would be welcomed by patients with wet AMD as well as their caregivers and physicians. I'm encouraged by the recently presented clinical data from the first cohort of the OPTIC trial showing that the therapy was safe and well tolerated with no rescue injections required in patients who previously required frequent anti-VEGF injections to control their wet AMD."

About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD
The multi-center, open-label, phase 1 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 (n=6) patients received ADVM-022 at a dose of 2 x 10^11 vg/eye. In the third cohort (n=9), patients are receiving ADVM-022 at a dose of 2 x 10^11 vg/eye and in the fourth cohort (n=9), patients will receive ADVM-022 at a dose of 6 x 10^11 vg/eye. Patients in the first and second cohorts received prophylactic oral steroids, while patients in the third and fourth cohorts will receive prophylactic steroid eye drops. The primary endpoint of the trial is the safety and tolerability of ADVM-022 after a single IVT administration. Secondary endpoints include change in best-corrected visual acuity (BCVA), change in central subfield thickness (CST) and macular volume, as well as mean number of anti-VEGF rescue injections and percentage of patients needing anti-VEGF rescue injections. Each patient enrolled in the study will be followed for a total of two years.

Eight leading retinal centers across the United States 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, designed to deliver long-term efficacy, reduce the burden of frequent anti-VEGF injections, optimize patient compliance, and to 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 submit an Investigational New Drug Application for ADVM-022 for the treatment of diabetic retinopathy to the U.S. Food and Drug Administration in the first half of 2020.

About Adverum Biotechnologies, Inc.
Adverum Biotechnologies (Nasdaq: ADVM) is a clinical-stage gene therapy company targeting unmet medical needs for 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-Q filed with the SEC on August 8, 2019 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.

Investor and Media Inquiries:

Investors:
Myesha Lacy
Adverum Biotechnologies
Vice President, Investor Relations and Corporate Communications
mlacy@adverum.com
1-650-304-3892

Media:
Cherilyn Cecchini, M.D.
Account Supervisor
ccecchini@lifescipublicrelations.com
1-646-876-5196

ADVERUM_ID_RGB 200x49.jpg

Source: Adverum Biotechnologies, Inc.