



## **Adverum Biotechnologies to Present Additional Clinical Data from First Cohort of OPTIC Phase 1 Trial of ADVM-022 Intravitreal Gene Therapy to Treat Wet AMD at the American Academy of Ophthalmology 2019 Annual Meeting**

September 26, 2019

**Company to Host and Webcast a Discussion with Key Opinion Leaders on Saturday, October 12 at 10:30 am PT**

MENLO PARK, Calif., Sept. 26, 2019 (GLOBE NEWSWIRE) -- [Adverum Biotechnologies, Inc.](http://www.adverumbiotech.com) (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today announced it will present additional clinical data for the first cohort of patients (n=6) in the OPTIC phase 1 clinical trial of ADVM-022 intravitreal injection gene therapy in wet age-related macular degeneration (wet AMD) at the Retina Subspecialty Day Program of the American Academy of Ophthalmology (AAO) 2019 Annual Meeting in San Francisco, CA on Friday, October 11, 2019.

### **Key Opinion Leader Event and Webcast:**

The Company will host an event with expert retinal specialists to discuss the OPTIC data presented at AAO. The discussion will be held on Saturday, October 12, 2019, at 10:30 am PT, and will be webcast live and archived on the [Events and Presentations](#) section of Adverum's website.

### **Podium Presentation Details:**

**Event:** 2019 American Academy of Ophthalmology

**Title:** 24-week Results of Phase 1 Study of Intravitreal Gene Therapy with ADVM-022 for Neovascular AMD (OPTIC Trial)

**Abstract:** 30062032

**Section:** Section VIII: Late Breaking Developments, Part I

**Date:** October 11, 2019

**Time:** 4:26 p.m. – 4:31 p.m. PT

**Location:** WEST 3002; Moscone Center, San Francisco, CA

**Speaker:** Szilard Kiss, M.D., Director of Clinical Research in the Department of Ophthalmology at Weill Cornell Medical College

### **About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD**

The multi-center, open-label, phase 1, dose-escalation 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  $6 \times 10^{11}$  vg/eye, and in the second cohort, patients (n=6) received a dose of  $2 \times 10^{11}$  vg/eye due to the robust preliminary anatomical response observed from patients in the first cohort. Patients are administered a tapering prophylactic corticosteroid regimen. 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 mean number of anti-VEGF rescue injections and percentage of patients needing anti-VEGF rescue injections. Each patient enrolled 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**

Adverum's gene therapy candidate for wet AMD and diabetic retinopathy, 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. ADVM-022 is designed to deliver long-term efficacy by significantly reducing the treatment burden of frequent anti-VEGF injections and improving real-world vision outcomes for patients with wet AMD and diabetic retinopathy.

In September 2018, Adverum received Fast Track designation for ADVM-022 for the treatment of wet AMD from the FDA.

### **About Wet Age-related Macular Degeneration (Wet 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. Disease progression results in the death of retinal cells and loss of vision. Wet AMD, also known as neovascular AMD, is an aggressive form of AMD, affecting around 10-15% of patients living with AMD, but accounting for approximately 90% of severe vision loss due to the disease. 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. 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, 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. 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](http://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, and the potential benefits of ADVM-022, 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.

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