# Adverum Biotechnologies Announces Data Presented at the European Society of Gene and Cell Therapy (ESGCT)

October 17, 2018

- *A single intravitreal injection of ADVM-022 in non-human primates (NHP) demonstrated durable expression of aflibercept sustained out to 22 months*
- *In an NHP model, ADVM-022 provided ocular expression of aflibercept at therapeutic levels equivalent to current standard of care*

MENLO PARK, Calif., Oct. 17, 2018 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases, today announced data presented at the ESGCT 26$^{th}$ Annual Congress in Lausanne, Switzerland.

**October 17, 2018 Poster #P223: ADVM-022**

The long-term efficacy data on ADVM-022, a novel gene therapy candidate, in an NHP model of wet age-related macular degeneration (wAMD), was selected as one of the top-scoring abstracts at ESGCT 26$^{th}$ Annual Congress. The data was presented in a lightning talk followed by a poster session.

Abstract Title: Long-term aflibercept expression levels in non-human primates following intravitreal administration of ADVM-022, a potential gene therapy for wet age-related macular degeneration

Highlights include:

- A single intravitreal administration of ADVM-022 delivering a continuous supply of aflibercept may provide an effective long-term treatment option and prevent further vision loss for patients with wAMD
- A single intravitreal administration of ADVM-022 provided robust expression of aflibercept, sustained for approximately two years post-dose in NHPs
- A single intravitreal administration of ADVM-022 in NHPs at dose ranges of $2x10^{11}$ vg/eye to $2x10^{12}$ vg/eye provided stable intraocular expression of aflibercept at levels comparable with the levels measured in aflibercept recombinant protein-injected eyes approximately 3-4 weeks post-dose in all of the following: vitreous humor, aqueous humor, retina and choroid
- Systemic treatment with prophylactic prednisone in order to prevent a potential inflammatory response resulted in a trend towards reduced levels of intraocular aflibercept; the levels were still comparable to those observed 56 days and approximately two years post ADVM-022 dose, within the therapeutic window of aflibercept recombinant protein

“We are very excited to present this robust set of preclinical data at ESGCT,” said Mehdi Gasmi, Ph.D. , chief science and technology officer of Adverum Biotechnologies. “This comprehensive data set in non-human primates demonstrating long term aflibercept expression from a single intravitreal injection of ADVM-022, on par with what can be achieved with current recombinant protein standard of care, is very encouraging and confirms the potential of ADVM-022 to benefit patients suffering with wAMD. The recent FDA Fast Track Designation for ADVM-022 will allow us to accelerate our development plans for this promising new gene therapy approach to treating wAMD. We look forward to initiating the OPTIC Phase 1 study this quarter.”

The multi-center, open-label, Phase 1, dose-escalation OPTIC trial is designed to assess the safety and tolerability of a single intravitreal (IVT) injection of ADVM-022 in patients with wAMD who are responsive to anti-vascular endothelial growth factor (VEGF) treatment. A number of leading retinal centers across the United States are planned to participate in the OPTIC trial.

“While current generation anti-VEGF monotherapies are tremendously valuable in the management of wet AMD, they do have key limitations, including durability. The need for frequent office visits and repeated dosing creates a burden for many patients. Due to this burden, some patients are under-treated and this can lead to suboptimal outcomes,” said Charles C. Wykoff, M.D., Ph.D., Retina Consultants of Houston and Clinical Associate Professor of Ophthalmology at Weill Cornell Medical College, Houston Methodist Hospital. "ADVM-022, a single intravitreal administration of an anti-VEGF therapy with potential long-term durability, represents an exciting opportunity to fundamentally alter how we manage patients with wet AMD."

**October 18, 2018 Poster #P384: ADVM-043**

Poster Title: Preliminary Safety Data from the ADVANCE Phase 1/2 Clinical Study (ADVM-043-01) in Alpha-1 Antitrypsin (A1AT) Deficiency

Due to the close proximity of expected preliminary data in this fourth quarter on patients in Cohorts 1 through 3 in the ADVANCE Phase 1/2 clinical trial of ADVM-043 in A1AT deficiency, a poster will not be presented at this time.

**About Adverum Biotechnologies, Inc.**
Adverum is a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases. Adverum has a robust pipeline that includes product candidates designed to treat rare diseases alpha-1 antitrypsin (A1AT) deficiency and hereditary angioedema (HAE) as well as wet age-related macular degeneration (wAMD). Leveraging a next-generation adeno-associated virus (AAV)-based directed evolution platform, Adverum generates product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein. Adverum has collaboration agreements with Regeneron Pharmaceuticals to research, develop, and commercialize gene therapy products for ophthalmic diseases and Editas Medicine to explore the delivery of genome editing medicines for the treatment of inherited retinal diseases. Adverum's core capabilities include clinical development and in-house manufacturing expertise, specifically in process development and assay development. 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 the potential and benefits that ADVM-022 may have to patients with wAMD, the recent FDA Fast Track Designation for ADVM-022 that may allow Adverum to accelerate its development plans for treating wAMD, the timing of initiating the OPTIC Phase 1 trial, the expected conduct of the OPTIC trial for ADVM-022, and the expectation that new cases of wAMD in the U.S. is expected to grow significantly, all of which are based on certain assumptions made by Adverum, expected future developments and other factors Adverum believes are appropriate in the circumstances. Actual results may differ from those set forth in these forward-looking statements as a result of various risks and uncertainties, which include, without limitation, the risk of unexpected delays in the enrollment of patients in Adverum's new OPTIC clinical trial or in the manufacturing of ADVM-022 to be used in the OPTIC clinical trial, as well as the risks and uncertainties facing Adverum described more fully in Adverum's periodic reports filed with the Securities and Exchange Commission (SEC), especially under the caption "Risk Factors" in its latest Quarterly Report on Form 10-Q filed with the SEC on August 8, 2018. 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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