Adverum Biotechnologies Presents Additional Long-term Preclinical Data on ADVM-022 in Wet AMD at ASGCT 21st Annual Meeting

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-- Single Intravitreal Administration of Gene Therapy ADVM-022 Provides Long-term Protection in Nonhuman Primate Model of Wet AMD --

MENLO PARK, Calif., May 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, announced the presentation of additional long-term efficacy data from a preclinical study of ADVM-022 in wet age-related macular degeneration (wAMD) in a poster session today at the ASGCT 21st Annual Meeting in Chicago, IL.

"It is exciting to see that a single administration of ADVM-022 has the potential to provide long-term protection against choroidal neovascularization, which is associated with vision loss in wAMD," said Mehdi Gasmi, Ph.D., chief science and technology officer of Adverum Biotechnologies. "Our poster presentation at this year's ASGCT Annual Meeting includes the most robust set of data to date on ADVM-022. Based on the results from these studies and our ongoing Investigational New Drug-enabling studies, we are on track to submit an IND Application for ADVM-022 in the second half of 2018. We are eager to get ADVM-022 into the clinic to advance this gene therapy that may offer convenient, long-term protection for patients living with wAMD."

Preclinical data from the ADVM-022 poster presentation at ASGCT include:

- After 13 months, a single intravitreal injection of ADVM-022 was found to be safe and statistically significant ($p<0.0001$) in preventing the development of Grade IV lesions compared to the vehicle control group. The efficacy at 13 months was consistent with earlier-reported data, demonstrating that ADVM-022 induced long-term efficacy that was comparable to aflibercept, an anti-Vascular Endothelial Growth Factor (VEGF) standard-of-care therapy. Additionally, mean CNV complex areas were significantly smaller ($p<0.0001$) in the ADVM-022 and aflibercept groups, compared with vehicle, when analyzed by spectral domain optical coherence tomography (SD-OCT).
- ADVM-022 induced sustained intraocular expression of aflibercept for up to 16 months following a single intravitreal injection. Robust levels of aflibercept protein were detected up to 16 months in aqueous and vitreous humor and, more importantly, in retina and choroid tissues, where neovascularization occurs in wAMD.
- A separate pharmacokinetics study revealed that levels of vector-derived aflibercept measured in the vitreous and the retina 56 days post ADVM-022 injection match the levels of aflibercept recombinant protein 3-4 weeks post bolus of protein injection, which is well within the therapeutic window of the standard of care. In addition, the levels of vector-derived aflibercept at 56 days were consistent with those observed in the long-term efficacy study at 16 months. Taken together, these data suggest that a single injection of ADVM-022 administered intravitreally can generate stable levels of aflibercept found to be within the therapeutic window of the standard-of-care recombinant protein.
- ADVM-022 was well tolerated, with no serious adverse events. ADVM-022 had mild effects on aqueous and vitreous cell infiltrates, keratic precipitates, and intraocular pressure (IOP). Treatment-related side effects were limited to mild inflammatory responses and related transient IOP decrease. Longitudinal OCT studies, fundoscopy, and fluorescein angiography did not identify changes in retinal morphology, optic nerve head and vascular integrity.

About ADVM-022 Gene Therapy for wAMD
Adverum's gene therapy candidate 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 injection. Vascular endothelial growth factor (VEGF) activity is associated with wAMD progression and vision loss. Anti-VEGF standard-of-care therapies administered every 4-8 weeks have shown the potential to prevent disease progression and preserve or even improve patients' vision. Treatment with ADVM-022 is designed to minimize the burden of frequent anti-VEGF injections, the current standard-of-care treatment for wAMD.

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.

Adverum's Forward-Looking Statements

Statements contained in this press release regarding Adverum's intention to file an IND application for ADVM-022 in the second half of 2018 and potential for further development of ADVM-022 are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. 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 described in Adverum's periodic reports filed with the Securities and Exchange Commission (SEC), in particular under the caption "Risk Factors" in its Form 10-Q filed with the SEC on May 9, 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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