Adverum Biotechnologies Announces Long-term Preclinical Efficacy Data on ADVM-022 Gene Therapy in Wet AMD

May 1, 2018

-- 13-Month Data Show Efficacy and Durability of Protein Expression following a Single Intravitreal Administration of ADVM-022 --

-- Data to be Presented in a Poster Session on May 17, 2018 at the ASGCT 21st Annual Conference --

MENLO PARK, Calif., May 01, 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 long-term preclinical efficacy data on ADVM-022 in a non-human primate model of wet age-related macular degeneration (wAMD). In this preclinical study, the efficacy of ADVM-022 at 13 months post-administration was consistent with earlier reported data, demonstrating that 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. ADVM-022 induced long-term efficacy that was comparable to aflibercept, an anti-Vascular Endothelial Growth Factor (VEGF) standard-of-care therapy. ADVM-022 was well tolerated, with no serious adverse events.

The data will be presented in a poster presentation on May 17, 2018 at the ASGCT 21st Annual Meeting in Chicago, IL.

“We continue to be encouraged by the efficacy and sustained protein levels we are seeing a year post a single intravitreal injection of ADVM-022,” said Mehdi Gasmi, Ph.D, chief science and technology officer of Adverum Biotechnologies. “These durable efficacy data support our plans to advance ADVM-022 into the clinic and we remain on track to submit an Investigational New Drug (IND) Application for ADVM-022 in the second half of this year. We are excited to be working on a gene therapy candidate that has demonstrated the potential to alleviate the treatment burden of frequent injections for patients living with wet AMD.”

“ADVM-022 represents a novel approach to treating wAMD with a gene therapy administered as a single intravitreal injection,” said Szilard Kiss, M.D., director of clinical research in the Department of Ophthalmology at Weill Cornell Medical College. “Intravitreal injections of aflibercept are a routine office practice for retinal specialists, and a single-administration treatment with durable efficacy would greatly improve the way this degenerative disease is treated.”

About the ADVM-022 Preclinical Study
The long-term efficacy of ADVM-022 was evaluated in the industry-standard laser-induced choroidal neovascularization (CNV) model in non-human primates (NHPs). NHPs received a single intravitreal injection of either ADVM-022 (n=4, 100 µL, ~$2x10^{12}$ vg/eye, bilaterally) or vehicle (n=4, 100µL, bilaterally) 12.5 months prior to lasering of the macular region of the retina to induce VEGF upregulation and CNV. As a positive control, a separate group of animals received bilateral intravitreal injections of aflibercept recombinant protein (n=4, 30 µL, 1.2 mg/eye), an anti-VEGF standard-of-care therapy, at the time of lasering. Clinically-relevant Grade IV lesions were evaluated at two and four weeks post lesioning and results were as follows:

	Efficacy Data Incidence of Grade IV Lesion		
	ADVM-022 (13 Months) (n=4)	Aflibercept (at Lasering) (n=4)	Vehicle Control (13 months) (n=4)
2 Weeks Post Lesioning	0%[1,2]	2.8%[1,2]	42.8%[1]
4 Weeks Post Lesioning	6.3%[1,2]	4.5%[1,2]	40.3%[1]

[1] *$p<0.0001$ vs vehicle*

[2] *$p=0.4$ and 0.7 between ADVM-022 and aflibercept groups at 2 and 4 weeks, respectively*

Three additional animals that did not undergo laser treatment and that received the same intravitreal ADVM-022 injection showed stable vitreous levels of aflibercept at approximately 3 µg/mL 13 months post vector administration.

ASGCT Poster Session
Poster Title: AAV.7m8-aflibercept Provides Long-term Protection in a Non-human Primate Model of Wet Macular Degeneration Over One Year Post Intravitreal Vector Administration (#554)
Session Title: Neurologic Diseases (Including Ophthalmic and Auditory Diseases) II
Time: Thursday, May 17, 2018, 5:15-7:15 pm CT
Location: Hilton Chicago, Stevens Salon C & D

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. VEGF overexpression can lead to wAMD progression and vision loss. 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 SEC. 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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