Adverum Biotechnologies to Present 30-month Preclinical Safety and Expression Data for ADVM-022 Gene Therapy in Wet AMD at the ASGCT 22nd Annual Meeting

May 2, 2019

- Oral Presentation Today at 10:45 am ET at the ASGCT 22nd Annual Meeting -

- ADVM-022 Was Demonstrated to be Safe and Well Tolerated, Provided Long-term, Sustained Aflibercept Expression out to 30 Months Following a Single Intravitreal Injection -

- Long-term Aflibercept Expression and VEGF Suppression Did Not Affect Retina Structure or Function -

MENLO PARK, Calif., May 02, 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 new long-term safety and expression data out to 30 months following a single intravitreal injection of ADVM-022 in a preclinical study in wet age-related macular degeneration (wet AMD).

The data are being presented today in an oral presentation by Szilard Kiss, M.D., director of clinical research in the Department of Ophthalmology at Weill Cornell Medical College, at the American Society of Gene and Cell Therapy (ASGCT) 22nd Annual Meeting in Washington, D.C.

Dr. Kiss commented, “Wet AMD is an aggressive ocular disease that requires many patients to receive anti-VEGF intravitreal injections every 4 to 8 weeks to maintain their vision. Compliance can be challenging, and underdosing can lead to vision loss. The preclinical data on ADVM-022 demonstrate long-term safety and aflibercept expression following a single intravitreal injection of this novel gene therapy. We are eager to continue to assess ADVM-022 as this treatment approach has the potential to improve real-world visual outcomes for patients living with wet AMD.”

“Today’s presentation adds to the growing body of long-term preclinical data showing ADVM-022’s safety, sustained expression, and efficacy following a single intravitreal injection,” said Mehdi Gasmi, Ph.D., president and chief scientific officer of Adverum Biotechnologies. “We are excited to share these new data showing that ADVM-022 induced long-term, sustained expression of aflibercept up to 30 months at therapeutic levels, and did not affect the structural integrity of the retina or its function.”

Highlights from the oral presentation include:

- A single intravitreal injection of ADVM-022 at $2x10^{12}$ vg induced long-term, sustained levels of aflibercept expression up to 30 months at clinically efficacious levels in non-human primates (NHPs). The aflibercept levels measured in the retina at 30 months post ADVM-022 injection matched the aflibercept levels measured 21-28 days post-bolus of recombinant protein injection, and were within the duration of action.
- ADVM-022 was administered safely and was well-tolerated up to 30 months post-dose.
    - The retina structure was normal. Long-term expression of aflibercept and VEGF suppression at therapeutic levels did not affect the retina structure. There were no signs of geographic (RPE) atrophy or retinal thinning evaluated by OCT out to 30 months.
    - The retina continued to function properly. Electroretinography (ERG) responses following dosing with ADVM-022 were within normal range of ERG responses expected for treatment naïve NHPs.
    - ADVM-022 was well tolerated, as assessed by ophthalmic slit lamp exams out to 30 months.
    - No serious adverse events (SAEs) occurred, with follow-up of up to 30 months.

Oral Presentation at the ASGCT 22nd Annual Meeting
Title: Ocular safety of long-term suppression of VEGF by intravitreally-administered gene therapy, ADVM-022, in non-human primates
Abstract Number: 931
Session Title: Neurosensory Diseases
Date: May 2, 2019
Time: 10:45 – 11:00 am ET
Location: Washington Hilton, Jefferson Room, Washington, D.C.
Speaker: Szilard Kiss, M.D., director of clinical research in the Department of Ophthalmology at Weill Cornell Medical College

About ADVM-022 Gene Therapy Candidate for Wet AMD
Adverum’s gene therapy candidate for wet AMD, 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 administration. ADVM-022 is designed to provide sustained therapeutic levels of aflibercept, minimize the burden of frequent anti-VEGF injections, and improve real-world vision outcomes for patients with wet AMD.

About Wet Age-related Macular Degeneration (Wet AMD)
Age-related macular degeneration (AMD) is a progressive disease affecting the retinal cells in the macula, the region of the eye responsible for central vision. Disease progression results in the death of retinal cells and the gradual loss of vision. Wet AMD is an advanced form of AMD, affecting

approximately 10% of patients living with AMD. 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 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 fully maintain vision gains. Compliance with this regimen can be difficult for patients, caregivers, and healthcare systems, leading to suboptimal dosing and loss of vision from undertreatment.

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. As a leader in ophthalmic gene therapy, Adverum has collaboration agreements with Regeneron Pharmaceuticals and Editas Medicine. 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.

Forward-looking Statements
Statements contained in this press release regarding results or events 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 ADVM-022 potential to improve real-world visual outcomes for patients living with wet AMD and to demonstrate long-term durability and safety. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, the risk that Adverum's resources will not be sufficient for Adverum to conduct or continue planned development programs and planned clinical trials, and the risk of a delay in the enrollment of patients in Adverum's clinical studies or in the manufacturing of products to be used in such clinical studies. Risks and uncertainties facing Adverum are described more fully under the caption "Risk Factors" in Adverum's Annual Report on Form 8-K filed with the SEC on March 6, 2019. 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, except as may be required by applicable law.

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