Adverum Biotechnologies Receives Orphan Drug Designation for ADVM-053, a Novel Gene Therapy Candidate for the Treatment of Hereditary Angioedema

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- ADVM-053 is a novel gene therapy candidate designed as a potential single-administration treatment for hereditary angioedema ("HAE")
- Plans to submit an Investigational New Drug (IND) application to the FDA for ADVM-053 in 4Q18

MENLO PARK, Caif. , Aug. 24, 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 that the United States (U.S.) Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to ADVM-053, a preclinical gene therapy candidate being investigated as a potential single-administration treatment which has the potential to provide sustained levels of the C1 esterase inhibitor ("C1EI") protein.

HAE affects approximately 8,000 individuals in the U.S. This disease is caused by a genetic mutation that results in low levels of C1 esterase inhibitor which can be associated with sudden swelling and edema of respiratory airways, gastrointestinal tract, and extremities.

"We are pleased to receive the Orphan Drug Designation for ADVM-053 from the FDA," said Leone Patterson, interim president and chief executive officer of Adverum Biotechnologies. "We are committed to developing effective treatments for patients living with HAE and the support from the FDA will be invaluable towards this goal. We look forward to submitting our IND application in the fourth quarter."

Orphan drug designation is granted by the FDA to novel drugs and biologics, which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. The designation provides incentives for sponsors to develop products for rare diseases, which may include tax credits towards the cost of clinical trials and prescription drug user fee waivers. The orphan drug designation also could entitle Adverum Biotechnologies to a seven-year period of marketing exclusivity in the United States for ADVM-053 should the company receive FDA approval for the treatment of HAE for this product candidate.

About ADVM-053
ADVM-053 (AAVrh.10-C1EI) is designed as a single-administration treatment with the potential to provide sustained expression of the C1 esterase inhibitor protein to eliminate protein level variability and to prevent breakthrough angioedema attacks. In preclinical studies, a single intravenous administration of ADVM-053 increased C1EI protein expression above therapeutic levels and decreased vascular permeability in a mouse model of HAE.

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.

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 Adverum's plans to submit an IND Application for ADVM-053 for HAE in the fourth quarter of 2018, 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 consummate any of these plans or these product, clinical development or regulatory goals in a timely manner, or at all, or otherwise carry out the intentions or meet the expectations or projections 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, without limitation, 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, 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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