Adverum Biotechnologies Begins Patient Enrollment in the ADVANCE Phase 1/2 Clinical Trial for A1AT Deficiency

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-- Patients to Receive Single-administration of Gene Therapy Candidate ADVM-043 --
-- Company Expects to Report Preliminary Data in the Second Half of 2018 --

MENLO PARK, Calif., Dec. 05, 2017 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq:ADVM), a leading gene therapy company targeting unmet medical needs in serious rare and ocular diseases, today announced the initiation of patient enrollment in the ADVANCE Phase 1/2 clinical trial of ADVM-043 in patients with alpha-1 antitrypsin (A1AT) deficiency. The ADVANCE clinical trial is designed to evaluate the safety and protein expression following a single administration of ADVM-043, Adverum's novel gene therapy candidate.

"Our team has been laser-focused on initiating the ADVANCE clinical trial, which is an important milestone that transforms Adverum into a clinical-stage company," said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. "We are thrilled to collaborate with The Alpha-1 Foundation and gain from their expertise and insight into patient needs. Given their reach and robust Patient Registry, we are grateful for their support in patient recruitment and look forward to working together to develop a new treatment option for patients living with A1AT deficiency."

"Current approved therapy for A1AT deficiency patients with lung disease is weekly IV plasma-derived protein replacement," said Henry Moehring, chief executive officer of the Alpha-1 Foundation. "Until new and innovative therapies become available, the weekly infusions unfortunately are lifelong for patients."

"We are excited to see the ADVANCE clinical trial open for patient enrollment to be able to evaluate ADVM-043 as a novel, single-administration approach for treating A1AT deficiency," added Jean-Marc Quach, chief executive officer of The Alpha-1 Project (TAP), a wholly-owned subsidiary of the Alpha-1 Foundation.

About the ADVANCE Phase 1/2 Clinical Trial of ADVM-043 for A1AT deficiency
The ADVANCE Phase 1/2 clinical trial is a multi-center, open-label, dose-escalation study of ADVM-043 in patients with A1AT deficiency. The study will include up to 20 patients across up to four dosing cohorts of up to 5 patients each. The first cohort will receive an intravenous (IV) low dose of ADVM-043 of 8E13 total vg (equivalent to approximately 1E12 vg/kg based on an 80-kg patient). The next two cohorts will receive an intermediate IV dose or high IV dose, with the fourth cohort potentially evaluating intrapleural (IP) delivery of ADVM-043.

The study will be conducted at 5 leading centers in the United States. The primary endpoint is safety and tolerability and secondary endpoints include changes in plasma concentrations of both total and M-specific A1AT levels. The Company expects to report preliminary data from this trial in the second half of 2018.

Additional information about this clinical trial can be found at ClinicalTrials.gov under trial identifier number NCT02168686.

About ADVM-043
ADVM-043 (AAVrh.10-A1AT) is a gene therapy candidate that has the potential to induce stable, long-term A1AT protein following a single administration. In a preclinical proof-of-concept study, ADVM-043 demonstrated robust protein expression above therapeutic levels in mice following either IV or IP administration. In another study in non-human primates, evidence of stable long-term expression of hA1AT mRNA was observed out to one year following IP administration of ADVM-043.

About Alpha-1 Antitrypsin (A1AT) Deficiency
A1AT deficiency is a fairly common orphan disease impacting approximately 100,000 individuals in the United States. It is estimated that 20 million people carry the gene for the disease, which unfortunately can be passed to their children. A1AT deficiency is associated with premature emphysema. The disease is caused by mutations in the SERPINA1 gene, resulting in very low levels of A1AT. The current standard-of-care treatment for patients with A1AT deficiency can be challenging, with weekly IV infusions of an alpha-1 proteinase inhibitor. The current treatment regimen can result in underdosing and lead to worsening lung function.

About Adverum Biotechnologies, Inc.
Adverum is a leading 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 that are not historical facts 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 plans related to Adverum's product candidates and clinical studies and the therapeutic and commercial potential of its product candidates, 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 plans or product or clinical development 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 that Adverum's resources will not be sufficient for Adverum to conduct or continue planned development programs and planned clinical trials, 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, the risk that Adverum will not be able to successfully develop or commercialize any of its product candidates and the risk that Adverum will be delayed in receiving or fail to receive required regulatory approvals. Risks and uncertainties facing Adverum are described more fully 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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Primary Logo

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