Adverum Biotechnologies Announces Completion of Dosing of First Cohort of Patients in the ADVANCE Phase 1/2 Clinical Trial of ADVM-043 Gene Therapy for A1AT Deficiency

February 26, 2018

-- Data Monitoring Committee Recommends Proceeding to the Second Cohort --

-- Company Expects to Report Preliminary Data in the Second Half of 2018 --

MENLO PARK, Calif., Feb. 26, 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 the completion of dosing and evaluation of patients (n=2) in the first cohort of the ADVANCE Phase 1/2 clinical trial for alpha-1 antitrypsin (A1AT) deficiency. Patients were treated with a single administration of ADVM-043 at a dose of ~1E12 vg/kg (8E13 total vg). Based on a review of the preliminary safety data, the independent data monitoring committee (DMC) has recommended proceeding to the second cohort of patients, which is open for enrollment.

“We are excited to advance to the second cohort of patients with A1AT deficiency in this clinical trial,” said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. “We are pleased by the physician and patient interest in the ADVANCE trial and we are eager to assess ADVM-043’s safety and tolerability, as well as protein expression, in patients. We continue to work closely with the Alpha 1 Foundation and the ADVANCE trial continues on track and we expect to report preliminary data in the second half of 2018.”

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 (n=2) received an intravenous (IV) low dose of ADVM-043 of ~1E12 vg/kg (8E13 total vg). The second cohort will receive an IV intermediate dose of ~5E12 vg/kg (4E14 total vg) and the third cohort will receive an IV high dose of ~1.5E13 vg/kg (1.2E15 total vg). A potential fourth cohort may be opened to evaluate an 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. Adverum 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 designed as a potential single-administration treatment to induce stable, long-term A1AT protein expression. In a preclinical proof-of-concept study, ADVM-043 demonstrated robust protein expression above therapeutic levels in mice following either intravenous (IV) or intrapleural (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 an orphan disease impacting approximately 100,000 individuals in the United States. The disease is caused by genetic mutations resulting in very low levels of A1AT. A1AT deficiency is associated with premature emphysema. The market for A1AT deficiency therapy world-wide was estimated at approximately $1.2 billion in 2016. The current standard of care for this disease includes weekly intravenous infusions of an alpha-1 proteinase inhibitor, at an estimated cost of approximately $100,000 annually per patient. The current treatment regimen can result in underdosing and lead to worsening lung function.

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.

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, clinical studies, regulatory filings 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 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 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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