Adverum Biotechnologies Provides 2018 Outlook

January 4, 2018

Accelerating Pipeline Development in 2018 after Transforming into Clinical-stage Company in 2017

MENLO PARK, Calif., Jan. 04, 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 reviewed recent progress and provided an outlook for 2018.

"In 2017, our newly-assembled team achieved our stated goal of transforming Adverum into a clinical-stage company," said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. "We were focused on our corporate milestones, and last month we began patient enrollment in ADVANCE, our Phase 1/2 clinical trial of ADVM-043 in alpha-1 antitrypsin deficiency. We expect to report preliminary data from the ADVANCE trial in the second half of 2018. In addition, we are accelerating the development of our pipeline of gene therapies and plan to submit two investigational new drug applications with the FDA in the second half of 2018, for ADVM-022 in wet AMD and ADVM-053 in hereditary angioedema. With our platform of industry-leading technology and experienced leadership team, we are well positioned to advance our pipeline of novel gene therapies in 2018."

Key Accomplishments for 2017

- Dosed the first patient in the ADVANCE Phase 1/2 trial for ADVM-043 in alpha-1 antitrypsin (A1AT) deficiency. The primary endpoint is safety and tolerability, and secondary endpoints include changes in plasma concentrations of both total and M-specific A1AT levels.
- Demonstrated long-term sustained expression of anti-VEGF protein in non-human primates following a single intravitreal injection of ADVM-022 for the treatment of wet age-related macular degeneration (wAMD).
- Appointed three highly-experienced new board members: Eric G. Carter, M.D., Ph.D., Richard N. Spivey, Pharm.D., Ph.D., and Patrick Machado, J.D.
- Appointed Athena Countouriotis, M.D. as senior vice president and chief medical officer.

2018 Outlook - Planned Pipeline Milestones
ADVM-043 for A1AT Deficiency

- Report preliminary data from the ADVANCE Phase 1/2 clinical trial in the second half of 2018.

ADVM-022 for wAMD

- Report 12-month efficacy data in non-human primates in the first half of 2018.
- Complete Investigational New Drug (IND)-enabling preclinical studies.
- Submit an IND application to the U.S. Food and Drug Administration (FDA) in the second half of 2018.

ADVM-053 for Hereditary Angioedema (HAE)

- Complete IND-enabling preclinical studies.
- Submit an IND application to the FDA in the second half of 2018.

Financial Guidance
Adverum's cash, cash equivalents and marketable securities were $186.6 million as of September 30, 2017. The Company's current cash position is expected to fund the three lead gene therapy programs through the end of 2019 and through the achievement of meaningful clinical data in patients for at least one of the Company's lead programs.

Upcoming Events

- Adverum plans to participate in the following upcoming conferences:
  - ARM's Cell & Gene Therapies State of the Industry Briefing in San Francisco on January 8, 2018. Amber Salzman, Ph.D., president and chief executive officer of Adverum, will participate on the Gene Therapy: The Outlook for 2018 panel at 9:05 – 9:50 a.m. PT
  - Cowen 38th Annual Health Care Conference in Boston, March 12 – 14, 2018
  - Cowen 17th Annual Life Sciences Winter Meeting in Colorado, March 20 – 23, 2018

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.

Contact for Adverum:
Leone Patterson
Chief Financial Officer
650-665-7222
lpatterson@adverum.com

Primary Logo

Source: Adverum Biotechnologies, Inc.