Adverum Biotechnologies Reports Continued Momentum with OPTIC Trial for ADVM-022 Intravitreal Gene Therapy in Wet AMD and Provides 2020 Outlook

January 12, 2020

-- OPTIC phase 1 trial progressing with plans to complete patient dosing in cohort three, begin enrollment in cohort four in 1Q20 --

-- IND application planned for ADVM-022 in diabetic retinopathy in 1H20; Patient enrollment targeted for 2H20 --

MENLO PARK, Calif., Jan. 12, 2020 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today reviewed recent business and development progress and provided an outlook for 2020.

“In 2019, we made significant progress advancing our lead gene therapy candidate ADVM-022 in the ongoing OPTIC phase 1 dose-ranging clinical trial in patients with wet age-related macular degeneration (AMD),” said Leone Patterson, president and chief executive officer of Adverum Biotechnologies. “Patients with wet AMD typically require frequent ocular anti-VEGF injections to maintain their vision. Yesterday, additional data were presented from the first cohort of patients in OPTIC, which demonstrated zero anti-VEGF rescue injections required following a single intravitreal injection of ADVM-022 and with median follow up of 44 weeks, ADVM-022 treatment was safe and well-tolerated. This year, we look forward to presenting additional data from all four cohorts of patients in OPTIC. Additionally, we plan to submit an IND for ADVM-022 in a second indication, diabetic retinopathy, and begin enrolling patients in a new clinical trial. We believe ADVM-022 has the potential to be a paradigm-changing treatment for patients with wet AMD and for patients with diabetic retinopathy.”

Recent Data Presented for First Cohort of OPTIC Phase 1 Clinical Trial (n=6)
On January 11, 2020, Adverum presented median 44-week (range of 40-52 weeks) data at the Atlantic Coast Retina Club and Macula 20/20. These data demonstrated that patients in the first cohort of OPTIC achieved vision maintenance and improvements in retinal anatomy with zero anti-VEGF rescue injections required for any patient. Additionally, the first patient treated in OPTIC has reached 52 weeks post ADVM-022 administration. ADVM-022 has been safe and well-tolerated, with no dose-limiting toxicities through the latest time point at December 1, 2019 and inflammation has been manageable with topical eye drops.

OPTIC Phase 1 Trial Execution
After completing patient enrollment in the first and second cohorts, Adverum dosed the first patient in the third cohort (n=9, dose of 2x10^11 vg/eye) in October 2019. In the third and fourth cohorts, Adverum plans to use prophylactic steroid eye drops instead of prophylactic oral steroids to manage inflammation.

2020 Outlook

First quarter of 2020:

- Move to new corporate headquarters in Redwood City, CA, allowing for the expansion of the company’s in-house process development capabilities to the 1,000-liter production scale
- Complete patient dosing in the third cohort, begin enrollment in the fourth cohort, and determine if additional cohorts are needed in the OPTIC trial

First half of 2020:

- Present longer-term data from the first cohort of patients in OPTIC
- Present 24-week data from the second cohort of patients in OPTIC
- Submit an investigational new drug application for ADVM-022 in diabetic retinopathy, a key VEGF-driven cause of vision loss among working-age adults

Second half of 2020:

- Present longer-term data from the first cohort and second cohorts of patients in OPTIC trial
- Present clinical data from the third and fourth cohorts of patients in the OPTIC trial
- Begin enrolling patients in a planned phase 1/2 clinical trial for ADVM-022 in diabetic retinopathy to expand Adverum’s clinical development pipeline

Upcoming Events
Adverum plans to participate in the following upcoming conferences:

- J.P. Morgan’s 38 th Annual Healthcare Conference in San Francisco on January 15, 2020 at 2:30 pm PST
- Angiogenesis, Exudation, and Degeneration 2020 in Miami on February 8, 2020 at 2:44 pm EST. Dr. David Boyer will

present the 24-week data from the second cohort for the first time as well as an update from the first cohort of patients in the OPTIC trial. A KOL event and simultaneous webcast to discuss the data presented with management and an expert panel of retina specialists will take place on February 9, 2020 at 10:00 am EST

- SVB Leerink's 9 th Annual Global Healthcare Conference in New York on February 25, 2020 at 9 am EST
- Cowen's 40 th Annual Health Care Conference in Boston from March 2-4, 2020

About Adverum Biotechnologies, Inc.
Adverum Biotechnologies (Nasdaq: ADVM) is a clinical-stage gene therapy company targeting unmet medical needs for serious ocular and rare diseases. Adverum is evaluating its novel gene therapy candidate, ADVM-022, as a one-time, intravitreal injection for the treatment of its lead indication, wet age-related macular degeneration. For more information, please visit www.adverum.com.

Forward-looking Statements
Statements contained in this press release regarding 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 report additional clinical data for ADVM-022 from the OPTIC trial and to advance ADVM-022, including Adverum's plans to submit an Investigational New Drug Application for ADVM-022 for the treatment of diabetic retinopathy to the U.S. Food and Drug Administration in the first half of 2020, and the potential benefits of ADVM-022, 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 achieve any of these in a timely manner, or at all, or otherwise carry out the intentions or meet the expectations 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 risks inherent to, without limitation: Adverum's novel technology, which makes it difficult to predict the time and cost of product candidate development and obtaining regulatory approval; the results of early clinical trials not always being predictive of future results; the potential for future complications or side effects in connection with use of ADVM-022; obtaining regulatory approval for gene therapy product candidates; enrolling patients in clinical trials; reliance on third parties for conducting the OPTIC trial and vector production; and ability to fund operations through completion of the OPTIC trial and thereafter. Risks and uncertainties facing Adverum are described more fully in Adverum's Form 10-Q filed with the SEC on November 7, 2019 under the heading "Risk Factors." 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.

Investor and Media Inquiries:

Investors:
Myesha Lacy
Adverum Biotechnologies
mlacy@adverum.com
1-650-304-3892

Media:
Cherilyn Cecchini, M.D.
LifeSci Communications
ccecchini@lifescicomms.com
1-646-876-5196

ADVERUM_ID_RGB 200x49.jpg

Source: Adverum Biotechnologies, Inc.