Adverum Biotechnologies Provides 2019 Outlook

January 7, 2019

Advancing Novel Gene Therapy ADVM-022 for Wet AMD in OPTIC Phase 1 Clinical Trial

MENLO PARK, Calif., Jan. 06, 2019 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ophthalmology and rare diseases, today reviewed recent progress and provided an outlook for 2019.

“For our lead gene therapy ADVM-022 for the treatment of wet AMD, we are building off of last year’s momentum to execute our ongoing OPTIC phase 1 clinical trial,” said Leone Patterson, chief executive officer of Adverum Biotechnologies. “In a very short period, we have dosed our first patient in the OPTIC phase 1 trial, published long-term preclinical efficacy data in a leading scientific journal, and received Fast Track designation for ADVM-022. With this key groundwork complete, this year our primary focus is on advancing this gene therapy for an initial indication in wet AMD and evaluating additional anti-VEGF indications to pursue. We have sufficient cash to fund operations at least through the first half of 2020, including interim data from the three cohorts in the OPTIC trial. Our team is excited to be working on developing this novel single intravitreal injection therapy for patients.”

Key Accomplishments for 2018

- In December 2018, long-term preclinical expression and efficacy data on ADVM-022 in wet age-related macular degeneration (wet AMD) were published in *Molecular Therapy*, a leading peer-reviewed scientific journal. The data in this publication combined with two year preclinical expression data presented in October 2018 at the European Society of Gene and Cell Therapy (ESGCT) showed the following:
    - A single intravitreal injection of ADVM-022 in non-human primates (NHPs) at dose ranges of 2 x 10^{11} vg/eye to 2 x 10^{12} vg/eye provided stable intraocular expression of aflibercept at levels comparable with the levels measured in aflibercept recombinant protein-injected eyes approximately 3 to 4 weeks post-dose in all of the following: vitreous humor, aqueous humor, retina and choroid
    - A single intravitreal injection of ADVM-022 provided robust and durable expression of aflibercept, sustained for approximately two years post-dose in NHPs
    - In a laser-induced choroidal neovascularization model in NHPs, a single intravitreal injection of ADVM-022 13 months before lasering prevented the occurrence of clinically relevant choroidal neovascularization lesions, similar to animals that received a bolus of intravitreal aflibercept (standard-of-care) at the time of lesioning
    - A single intravitreal injection of ADVM-022 delivering a continuous supply of aflibercept may provide an effective long-term treatment option and prevent further vision loss for patients with wet AMD
    - The full online publication can be accessed at the following link: https://doi.org/10.1016/j.ymthe.2018.11.003.
- In November 2018, Adverum dosed the first patient in the OPTIC phase 1 trial evaluating a single intravitreal injection of ADVM-022 for patients with wet AMD. ADVM-022 (AAV.7m8-aflibercept) is designed to provide long-lasting therapy without the need of chronic or frequent anti-VEGF injections
- In September 2018, Adverum received Fast Track designation for ADVM-022 in wet AMD from the U.S. Food and Drug Administration (FDA). Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Despite the availability of anti-VEGF therapies, patients with wet AMD still have a significant burden from the frequency of injections and undertreatment may lead to vision loss
- In late August 2018, Adverum announced that the IND application for ADVM-022 in patients went active

2019 Outlook - Planned Pipeline Milestones
ADVM-022 for wet AMD

- Provide an update on enrollment from the OPTIC phase 1 clinical trial in the first half of 2019
- Provide interim data on the three cohorts from the OPTIC phase 1 clinical trial by the first quarter of 2020

Rare Disease Program

- Provide an update on rare disease program’s preclinical development plan in the first half of 2019

Financial Guidance
Adverum’s cash, cash equivalents and marketable securities were $217.9 million as of September 30, 2018. Adverum expects this quarter-end cash

position to fund operations at least through the first half of 2020.

Upcoming Events

- Adverum plans to participate in the following upcoming conferences:
    - J.P. Morgan's 37 th Annual Healthcare Conference in San Francisco January 7-10, 2019. CEO Leone Patterson will present on Thursday, January 10 at 8:00 am PT
    - Leerink's 8 th Annual Global Healthcare Conference in New York, February 27-March 1, 2019
    - Cowen's 39 th Annual Health Care Conference in Boston, March 11-13, 2019

About Adverum Biotechnologies, Inc.
Adverum is a clinical-stage gene therapy company targeting unmet medical needs in ophthalmology and rare diseases. Adverum develops gene therapy 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, novel vector discovery and in-house manufacturing expertise, specifically in scalable process development, assay development, and current Good Manufacturing Practices quality control. 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 for advancing ADVM-022 and the statements under the headings "2019 Outlook - Planned Pipeline Milestones," "Financial Guidance" and "Upcoming Events", 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 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 Form 10-Q filed with the SEC on November 8, 2018, particularly 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.

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