Adverum Biotechnologies Reports Additional Clinical Data from First Cohort of OPTIC Phase 1 Trial of ADVM-022 Intravitreal Gene Therapy for Wet AMD at the Atlantic Coast Retina Club Macula 20/20 Annual Meeting

January 11, 2020

--44 week median follow up for patients (n=6)--
--Zero anti-VEGF rescue injections required following intravitreal ADVM-022; First patient has reached 52-weeks post treatment--
--Vision remains stable and anatomical improvements maintained--

MENLO PARK, Calif., Jan. 11, 2020 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today announced clinical data for the first cohort of patients (n=6) in the OPTIC phase 1 clinical trial of ADVM-022, the company's intravitreal injection gene therapy, in treatment-experienced patients with wet age-related macular degeneration (wet AMD). The data are being presented today by Charles C. Wykoff M.D., Ph.D., director of research, Retina Consultants of Houston, at the Atlantic Coast Retina Club Macula 20/20 Annual Meeting in New York, NY.

A copy of the presentation is available on the Adverum corporate website under Events and Presentations in the Investors section, available here.

In October 2019, Adverum presented data from the first cohort in OPTIC at a median 34-week time point (28-44 week range). Today, additional data for the first cohort are being presented, including efficacy and safety data, with a median follow up of 44 weeks at a range of 40-52 weeks, and included:

- Zero rescue injections for any patient. Additionally, the first patient treated in OPTIC has reached 52 weeks post ADVM-022 administration.
- Vision was maintained as demonstrated by stable mean Best Corrected Visual Acuity (BCVA) compared to baseline.
- Anatomical improvements were maintained as assessed by optical coherence tomography (OCT) and central subfield thickness (CST) measurements, compared to baseline.

As of December 1, 2019, ADVM-022 continues to be well-tolerated in the first cohort with no drug-related or procedure-related serious adverse events (SAEs), no drug-related systemic adverse events and no adverse events meeting the criteria for dose-limiting toxicities (DLTs). Low-grade inflammation was reported in all six patients and was generally mild to moderate and responsive to steroid eye drops. One ocular SAE, a retinal detachment, that was not related to ADVM-022 or the administration procedure was reported.

OPTIC Phase 1 Clinical Trial Data from Cohort 1 (n=6)

Results Following a Single ADVM-022 Dose:	
Median follow-up (weeks)	44
Minimum/Maximum follow-up (weeks)	40-52
Follow-up cutoff date	December 1, 2019
Rescue Injections:	
Number of patients requiring anti-VEGF rescue injections	0 patients
Mean number of anti-VEGF rescue injections	0 injections
Change in BCVA[1]:	
Mean (ETDRS letters)[3]	-1.0
Minimum/Maximum (ETDRS letters)	-7.0 / +7.0
Change in CRT[2]:	
Mean (mm)[3]	-25.5
Minimum/Maximum (mm)[3]	-117 / +32
Safety:	

Dose-limiting toxicities (DLTs)	0
Serious adverse events (SAEs)[4]	1

[1] Best corrected visual acuity (BCVA) as measured by Early Treatment Diabetic Retinopathy Study (ETDRS) (i.e., sight charts)

[2] Central retinal thickness (CRT), also referred to as central subfield thickness (CST) assessed using Optical Coherence Tomography (OCT) imaging and measured by an independent Central Reading Center

[3] BCVA and CST values for patient with retinal detachment (unrelated to study treatment) used last observations prior to detachment

[4] This event was deemed unrelated to ADVM-022 or any study procedure

“These longer-term follow-up data demonstrate that patients in this first cohort of OPTIC are achieving sustained benefits from ADVM-022, a one-time intravitreal therapy, and have not required any anti-VEGF rescue injections through a median of 44 weeks while demonstrating impressive anatomic improvements,” said Charles C. Wykoff M.D., Ph.D., director of research, Retina Consultants of Houston and associate professor of clinical ophthalmology, Blanton Eye Institute, Houston Methodist Hospital and Weill Cornell Medical College, Houston Texas. “With a median follow-up period of 44 weeks, ADVM-022 continues to control wet AMD disease activity in all 6 patients and the low-grade intraocular inflammation appears manageable with steroid eyedrops. Based on the data to date, ADVM-022 has the potential to be a meaningful and potentially transformative treatment for patients with wet AMD.”

Aaron Osborne, MBBS, chief medical officer of Adverum, added, “These new clinical data are promising as they continue to support the safety, efficacy, and durable clinical profile of ADVM-022 and this therapy’s potential to change the treatment paradigm for patients with wet AMD. Anti-VEGF injections, the current standard of care, carry a significant treatment burden and real-world outcomes data suggest that vision outcomes are suboptimal due to undertreatment. In the first cohort of OPTIC, we continue to see stable vision and anatomical improvements being maintained out to a median of 44 weeks after a single ADVM-022 injection in these difficult-to-treat patients who previously required frequent anti-VEGF injections. We look forward to presenting longer-term data from the first cohort and 24-week data from the second cohort of OPTIC on February 8 at the Angiogenesis, Exudation, and Degeneration 2020 symposium.”

About the OPTIC Phase 1 Trial of ADVM-022 in Wet AMD
The multi-center, open-label, Phase 1, dose-escalation trial is designed to assess the safety and tolerability of a single intravitreal (IVT) administration of ADVM-022 in patients with wet AMD who are responsive to anti-vascular endothelial growth factor (VEGF) treatment. In the first cohort, patients (n=6) received ADVM-022 at a dose of 6 x 10^11 vg/eye and in the second cohort, patients (n=6) received ADVM-022 at a dose of 2 x 10^11 vg/eye. In the third cohort (n=9), patients also are receiving a dose of 2 x 10^11 vg/eye and in the fourth cohort (n=9), patients will receive a dose of 6x10^11 vg/eye. Patients in the third and fourth cohorts will receive prophylactic steroid eye drops instead of oral steroids which were used in the first and second cohorts. The primary endpoint of the trial is the safety and tolerability of ADVM-022 after a single IVT administration. Secondary endpoints include changes in best-corrected visual acuity (BCVA), measurement of central retinal thickness (CRT), as well as mean number of anti-VEGF rescue injections and percentage of patients needing anti-VEGF rescue injections. Each patient enrolled will be followed for a total of two years.

Eight leading retinal centers across the United States (U.S.) are participating in the OPTIC Phase 1 trial for ADVM-022. For more information on the OPTIC Phase 1 clinical trial of ADVM-022 in wet AMD, please visit https://clinicaltrials.gov/ct2/show/NCT03748784.

About ADVM-022 Gene Therapy
ADVM-022 utilizes a propriety vector capsid, AAV.7m8, carrying an aflibercept coding sequence under the control of a proprietary expression cassette. ADVM-022 is administered as a one-time intravitreal injection, designed to deliver long-term efficacy and reduce the burden of frequent anti-VEGF injections, optimize patient compliance and improve vision outcomes for wet AMD and diabetic retinopathy patients.

In recognition of the need for new treatment options for wet AMD, the U.S. Food and Drug Administration granted Fast Track designation for ADVM-022 for the treatment of this disease.

Adverum is currently evaluating ADVM-022 in the OPTIC Study, a Phase 1 clinical trial in patients 50 years and older with wet AMD. Additionally, Adverum 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.

About Wet Age-related Macular Degeneration (Wet AMD)
Age-related macular degeneration (AMD) is a progressive disease affecting the macula, the region of the retina at the back of the eye responsible for central vision. In patients with wet AMD, an aggressive form of AMD, abnormal blood vessels grow underneath and into the retina. These abnormal blood vessels leak fluid and blood into and beneath the retina, causing vision loss.

Wet AMD is a leading cause of vision loss in patients over 60 years of age, with a prevalence of approximately 1.2 million individuals in the U.S. and 3 million worldwide. The incidence of new cases of wet AMD in the U.S. is approximately 150,000 to 200,000 annually, and this number is expected to grow significantly as the country’s population ages.

The current standard-of-care therapy for wet AMD is anti-VEGF intravitreal injections. These are effective but typically require eye injections every 4-12 weeks in order to maintain vision. Compliance with this regimen can be difficult for patients, caregivers, and healthcare systems, leading to undertreatment and resulting in loss of vision.

About Adverum Biotechnologies
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.

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