Adverum Biotechnologies Reports First Quarter 2019 Financial Results and Provides Corporate Update

May 8, 2019

MENLO PARK, Calif., May 08, 2019 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases, today reported financial results for the first quarter ended March 31, 2019 and provided a corporate update.

“Our primary focus is on advancing ADVM-022, our novel gene therapy designed as a single-administration treatment for wet AMD,” said Leone Patterson, chief executive officer of Adverum Biotechnologies. “For ADVM-022, we presented new 30-month preclinical safety and expression data at ASGCT and second eye preclinical safety data at ARVO. We look forward to presenting the first clinical data from the OPTIC phase 1 trial for ADVM-022 in the second half of this year. Beyond wet AMD, we continue to evaluate other ocular VEGF-related indications for this novel gene therapy to treat additional patients with ocular disease.

Ms. Patterson continued, “In addition, we recently welcomed Dr. Aaron Osborne as our new chief medical officer and Thomas Leung as our new chief financial officer as we assemble a leadership team with deep industry experience and commitment to developing novel gene therapies for patients. At the board level, we recently appointed three new members who broaden the set of financial, operational, and development experiences and can add valuable insights as we execute our development plans for our pipeline of gene therapies.”

Recent Program Updates

- At the ASGCT 22nd Annual Meeting in May 2019, Adverum presented new 30-month safety data on ADVM-022 (AAV.7m8-aflibercept) in wet age-related macular degeneration (wet AMD) from a preclinical study. Data presented in an oral presentation showed a single intravitreal (IVT) injection of ADVM-022 (2 × 10^12 vg/eye) was safely administered and provided long-term, sustained delivery of aflibercept up to 30 months at therapeutic levels in non-human primate (NHP) eyes. Long-term intraocular expression of aflibercept did not affect the retina structure or function and long-term suppression of VEGF activity did not cause retinal atrophy.

- At the ARVO 2019 Annual Meeting in April 2019, Adverum presented for the first time preclinical data on dosing the second eye with a single IVT injection of ADVM-022. Data showed ADVM-022 was safely administered to the contralateral eye, two months after administering to the first eye, in NHPs. Findings from this study could prove valuable when designing AAV-mediated gene therapy protocols to treat wet AMD patients with pre-existing vector immunity, or those with bi-lateral disease.

- In April 2019, Adverum announced the completion of enrollment and dosing of patients (n=6) in the first cohort in the OPTIC phase 1 clinical trial of ADVM-022 in patients with wet AMD. The independent data monitoring committee (DMC) determined that enrollment and dosing of patients in the second cohort could proceed. This was based on a review of the preliminary safety data from the first cohort of patients, which has shown no serious adverse events (SAEs) or dose-limiting toxicities (DLTs) following a single intravitreal injection of ADVM-022 at the initial trial dose of 6 x 10^11 vg/eye. No patient has experienced an SAE, with a follow up period of up to five months.

- In early April 2019, Adverum received a notification from the FDA requesting additional chemistry, manufacturing, and controls (CMC) information and requirements on the ADVM-022 manufacturing process and placing Adverum’s IND application for ADVM-022 for the treatment of wet AMD on clinical hold. Adverum subsequently submitted its response and should the agency decide to lift the clinical hold, Adverum expects to resume dosing patients in the OPTIC trial.

Recent Corporate Updates

- In May 2019, Adverum announced the appointment of Rekha Hemrajani, M.B.A., James Scopa, J.D., M.B.A., and Mark Lupher, Ph.D. to its board of directors and the appointment of current board member Patrick Machado, J.D. as board chair.

- In April 2019, Aaron Osborne, MBBS MRCOphth joined Adverum as chief medical officer and Thomas Leung joined as chief financial officer.

Future Outlook - Planned Milestones

ADVM-022 for Wet AMD

- Report 24-week primary and secondary outcomes from the first cohort of patients in the OPTIC phase 1 clinical trial at a scientific meeting in the second half of 2019

Rare Disease Programs

- Provide an update on Adverum’s A1AT deficiency and hereditary angioedema programs by midyear 2019

Manufacturing Capabilities

- Occupy Adverum’s new facility in Redwood City by the end of this year. This facility will serve as Adverum’s new corporate headquarters and includes the expansion of its in-house process development capabilities to the 1000 liter scale

Upcoming Events

- Adverum plans to participate in the following upcoming conference:
  - American Society of Virology (ASV) 2019 Annual Meeting in Minneapolis, MN, July 20-24, 2019 oral presentation:
    - Abstract Title: Analysis of the structural differences between AAV2.7m8 and its parental capsid AAV2 by Cryo-EM
    - Program Number: W5-2
    - Session Title: Oncolytic Viruses
    - Date: July 20, 2019
    - Time: 7:00 pm CT
    - Location: University of Minnesota, Moos 2-690

Financial Results for the Three Months Ended March 31, 2019

- **Cash, cash equivalents and short-term investments** were $189.5 million as of March 31, 2019, compared to $205.1 million as of December 31, 2018. Adverum expects this quarter-end cash position to fund operations into 2021.

- **Revenue**, consisting of revenue from collaborative research, was $0.0 million the three months ended March 31, 2019, compared to $0.2 million for the same period in 2018. Revenue decreased as no research activities were performed under collaboration agreements for the three month period in 2019.

- **Research and development expenses** were $10.1 million for the three months ended March 31, 2019, compared to $12.8 million for the same period in 2018. Research and development expenses decreased due to the discontinuation of Adverum's ADVANCE phase 1 clinical trial for ADVM-043, partially offset by an increase related to the ongoing OPTIC phase 1 clinical trial for ADVM-022.

- **General and administrative expenses** were $5.6 million for the three months ended March 31, 2019, compared to $5.4 million for the same period in 2018. General and administrative expenses increased primarily due to higher professional expenses.

- **Net loss attributable to common stockholders** was $14.5 million, or $0.23 per basic and diluted share, for the three months ended March 31, 2019, compared to $17.2 million, or $0.30 per basic and diluted share, for the same period in 2018.

About Adverum Biotechnologies, Inc.
Adverum is a clinical-stage gene therapy company targeting unmet medical needs in ocular and rare diseases. Adverum develops gene therapy product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein. As a leader in ophthalmic gene therapy, Adverum has collaboration agreements with Regeneron Pharmaceuticals and Editas Medicine. 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, statements regarding the expected timing of reporting first clinical data for the OPTIC trial, the statements under the heading "Future Outlook - Planned Milestones," and Adverum's expectations that its current cash position will fund its operations into 2021, 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 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 that the FDA's clinical hold may not be lifted in a timely manner or at all, and 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. Risks and uncertainties facing Adverum are described more fully in Adverum's Form 10-K filed with the SEC on March 6, 2019, 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.

ADVERUM BIOTECHNOLOGIES, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands)
(Unaudited)

	March 31,	December 31,

	2019	2018
ASSETS		
Current assets:		
Cash and cash equivalents	$ 144,107	$ 154,949
Short-term investments	45,381	50,130
Prepaid expenses and other current assets	3,649	3,675
Total current assets	193,137	208,754
Operating lease right to use asset	22,592	—
Property and equipment, net	4,145	3,586
Restricted cash	999	999
Deposits and other long-term assets	174	156
Total assets	$ 221,047	$ 213,495
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,752	$ 1,707
Accrued expenses and other current liabilities	5,032	8,784
Lease liability, current portion	3,826	–
Deferred rent, current portion	—	228
Total current liabilities	10,610	10,719
Deferred rent, less current portion	—	1,366
Lease liability, less current portion	22,078	—
Other non-current liabilities	216	243
Total liabilities	32,904	12,328
Stockholders’ equity	188,143	201,167
Total liabilities and stockholders’ equity	$ 221,047	$ 213,495

ADVERUM BIOTECHNOLOGIES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except per share amounts)
(Unaudited)

	Three Months Ended March 31,	
	2019	2018
Collaboration and license revenue	$ -	$ 216
Operating expenses:		
Research and development	10,131	12,794

General and administrative	5,576	5,368
Total operating expenses	15,707	18,162
Operating loss	(15,707)	(17,946)
Other income (expense), net	1,218	746
Net loss	$ (14,489)	$ (17,200)
Net loss per share, basic and diluted	$ (0.23)	$ (0.30)
Weighted-average common shares outstanding, basic and diluted	63,125	57,420

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Source: Adverum Biotechnologies, Inc.