



Adverum Biotechnologies Reports Second Quarter 2018 Financial Results and Provides Corporate Update

August 8, 2018

MENLO PARK, Calif., Aug. 08, 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 reported financial results for the second quarter ended June 30, 2018 and provided a corporate update.

"We continue to execute on our clinical, regulatory and manufacturing plans and remain on track to deliver on our goals in the second half of this year for our three lead gene therapies," said Leone Patterson, interim president and chief executive officer of Adverum Biotechnologies. "In addition to our positive clinical updates announced last week, we are expanding our manufacturing capabilities at the 2000 liter scale and have secured a new facility to occupy in the second half of next year. We believe these accomplishments position us well for clinical momentum across our pipeline of gene therapies in 2019."

Recent Progress

- In late July 2018, Adverum submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) to evaluate ADVM-022 in patients with wet age-related macular degeneration (wAMD).
- In July 2018, Adverum completed the dosing and evaluation of patients (n=2) in Cohort 2 of the [ADVANCE Phase 1/2 trial](#) for ADVM-043 in alpha-1 antitrypsin (A1AT) deficiency. Based on a review of the preliminary safety information, the independent data monitoring committee (DMC) recommended proceeding to the next dose level, and the first patient was dosed in Cohort 3 in late July 2018.
- In June 2018, Adverum signed a lease on a new facility in Redwood City, California. This facility, which will be occupied in the second half of 2019, will serve as Adverum's new corporate headquarters and will include over 80,000 square feet of office, manufacturing, and laboratory space. This facility will expand Adverum's manufacturing capabilities, enabling the scale up of its manufacturing process at the 2000 liter scale, as well as offering the opportunity for future cGMP manufacturing of clinical trial material.
- In May 2018, Adverum presented new long-term preclinical data on ADVM-022 gene therapy in a non-human primate model of wAMD in a poster at the American Society of Gene & Cell Therapy (ASGCT) 21st Annual Meeting. The data showed that after 13 months, a single intravitreal injection of ADVM-022 was found to be safe and statistically significant ($p < 0.0001$) in preventing the development of Grade IV lesions compared to the vehicle control group. The efficacy at 13 months was consistent with earlier-reported data, demonstrating that ADVM-022 induced long-term efficacy that was comparable to aflibercept, an FDA-approved anti-Vascular Endothelial Growth Factor (VEGF) for wAMD. ADVM-022 was well tolerated, with no serious adverse events.

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

- Report preliminary data from patients in Cohorts 1 through 3 in the ADVANCE Phase 1/2 clinical trial of ADVM-043 in A1AT deficiency patients by the end of this year.

ADVM-053 for Hereditary Angioedema (HAE)

- Complete ongoing IND-enabling preclinical studies.
- Submit an IND application to the FDA for ADVM-053 in HAE in the fourth quarter of 2018.

Upcoming Events

Adverum plans to attend the following upcoming conferences:

- Citi's 13th Annual Healthcare Conference in Boston, September 5-6, 2018
- Cantor Fitzgerald's Annual Global Healthcare Conference in New York, October 1-3, 2018
- Cell & Gene's Meeting on the Mesa in La Jolla, October 3-5, 2018
- European Society of Cell & Gene Therapy in Lausanne, Switzerland, October 16-19, 2018
- Piper Jaffray's 30th Annual Healthcare Conference in New York, November 27-28, 2018

Financial Results for the Three Months Ended June 30, 2018

- **Cash, cash equivalents and marketable securities** were \$234.9 million as of June 30, 2018, compared to \$247.0 million as of March 31, 2018, and \$190.5 million as of December 31, 2017. Adverum expects this quarter-end cash position to fund the three lead gene therapy programs into 2020, including preliminary clinical data for at least two of these programs, and through the initial stage of scaling up manufacturing capabilities.
- **Revenues**, consisting of revenue from collaborative research, were \$0.5 million for each of the three months ended June 30, 2018 and 2017.
- **Research and development expenses** were \$11.2 million for the three months ended June 30, 2018, compared to \$8.5 million for the same period in 2017. This increase was primarily due to an overall increase in research and development activities, including clinical trial expenses for ADVM-043, clinical trial initiation expenses for ADVM-022, and material production costs for ADVM-022 and ADVM-053 for planned clinical trials.
- **General and administrative expenses** were \$9.2 million for the three months ended June 30, 2018, compared to \$4.1 million for the same period in 2017. This increase was primarily due to severance-related expenses, predominantly stock-based compensation expenses from the departure of the former chief executive officer, as well as other compensation benefit expenses and professional service fees.
- **Net loss attributable to common stockholders** was \$18.8 million, or \$0.30 per basic and diluted share, for the three months ended June 30, 2018, compared to \$11.4 million, or \$0.27 per basic and diluted share, for the same period in 2017.

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 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 under the caption "2018 Outlook - Planned Pipeline Milestones," expectations as to the ability of its quarter-end cash position to fund the three lead gene therapy programs into 2020 and through the initial stage of scaling up manufacturing capabilities, and expectations on the timing of occupation of expected benefits to be obtained from its new facilities, 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 plans or product or clinical development goals in a timely manner, or at all, or otherwise be able to 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 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, as well as the risks and uncertainties facing Adverum described more fully in Adverum's periodic reports filed with the Securities and Exchange Commission (SEC), especially under the caption "Risk Factors" in its latest Quarterly Report on Form 10-Q filed with the SEC on August 8, 2018. 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)

| | June 30, 2018 | December 31, 2017 |
|---|------------------|----------------------|
| ASSETS | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 155,451 | \$ 70,519 |
| Short-term investments | 79,425 | 119,966 |
| Prepaid expenses and other current assets | 3,046 | 3,256 |
| Total current assets | 237,922 | 193,741 |
| Property and equipment, net | 2,525 | 3,024 |
| Restricted cash | 998 | - |
| Deposits and other long-term assets | 140 | 140 |

| | | |
|---|-------------------|-------------------|
| Intangible assets | 5,000 | 5,000 |
| Total assets | <u>\$ 246,585</u> | <u>\$ 201,905</u> |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 10,475 | \$ 8,695 |
| Current portion of deferred rent | 146 | 129 |
| Current portion of deferred revenue | <u>753</u> | <u>1,850</u> |
| Total current liabilities | 11,374 | 10,674 |
| Deferred rent, less current portion | 146 | 222 |
| Deferred revenue, less current portion | - | 5,250 |
| Deferred tax liability | 1,250 | 1,250 |
| Other non-current liabilities | <u>368</u> | <u>481</u> |
| Total liabilities | 13,138 | 17,877 |
| Stockholders' equity | <u>233,447</u> | <u>184,028</u> |
| Total liabilities and stockholders' equity | <u>\$ 246,585</u> | <u>\$ 201,905</u> |

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

| | <u>Three Months Ended June 30,</u> | | <u>Six Months Ended June 30,</u> | |
|---|------------------------------------|--------------------|----------------------------------|--------------------|
| | <u>2018</u> | <u>2017</u> | <u>2018</u> | <u>2017</u> |
| Collaboration and license revenue | \$ 493 | \$ 463 | \$ 709 | \$ 925 |
| Operating expenses: | | | | |
| Research and development | 11,217 | 8,492 | 24,011 | 17,553 |
| General and administrative | <u>9,179</u> | <u>4,064</u> | <u>14,547</u> | <u>12,053</u> |
| Total operating expenses | <u>20,396</u> | <u>12,556</u> | <u>38,558</u> | <u>29,606</u> |
| Operating loss | (19,903) | (12,093) | (37,849) | (28,681) |
| Other income (expense), net | <u>1,093</u> | <u>663</u> | <u>1,839</u> | <u>1,152</u> |
| Net loss | <u>\$ (18,810)</u> | <u>\$ (11,430)</u> | <u>\$ (36,010)</u> | <u>\$ (27,529)</u> |
| Net loss per share, basic and diluted | <u>\$ (0.30)</u> | <u>\$ (0.27)</u> | <u>\$ (0.60)</u> | <u>\$ (0.65)</u> |
| Weighted-average common shares outstanding, basic and diluted | <u>62,366</u> | <u>43,009</u> | <u>59,907</u> | <u>42,579</u> |

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 [Primary Logo](#)

Source: Adverum Biotechnologies, Inc.