Adverum Biotechnologies Reports Fourth Quarter 2017 Financial Results and Provides Corporate Update

March 6, 2018

MENLO PARK, Calif., March 06, 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 fourth quarter ended December 31, 2017 and provided a corporate update.

"We enter 2018 with significant momentum following a year of critical execution to transform Adverum into a clinical-stage company," said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. "In ADVANCE, our Phase 1/2 clinical trial of ADVM-043 in alpha-1 antitrypsin deficiency, we have completed enrollment in the first dose cohort and have initiated patient enrollment in the second intermediate-dose cohort. We are on track to report preliminary data from this study in the second half of 2018. In addition, we plan to submit two Investigational New Drug Applications with the FDA in the second half of 2018, for ADVM-022 in wet AMD and ADVM-053 in hereditary angioedema. We begin this exciting year of clinical development and regulatory progress in a strong position, funded to execute our three lead programs through the end of 2019 with preliminary clinical data for at least two of these programs."

Recent Progress

- In February 2018, Adverum completed the dosing and evaluation of patients (n=2) in the first cohort 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 second cohort of patients, which is open for enrollment. Adverum has initiated patient enrollment in the second intermediate-dose cohort, which will receive an intermediate dose of ~5E12 vg/kg (equivalent to ~4E14 total vg based on an 80-kg patient). The primary endpoint is safety and tolerability, and secondary endpoints include changes in plasma concentrations of both total and M-specific A1AT levels. Additional information about this clinical trial can be found at ClinicalTrials.gov under trial identifier number NCT02168686.
- In February 2018, Adverum raised $69.0 million in gross proceeds from an underwritten public offering of its common stock.
- In January 2018, Adverum and Editas Medicine, Inc. announced the extension of the companies' collaboration agreement to explore the delivery of genome editing medicines to treat up to five inherited retinal diseases.

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

- Report preliminary data from the ADVANCE Phase 1/2 clinical trial in the second half of 2018.

ADVM-022 for wAMD

- Report 12-month efficacy data in non-human primates in the first half of 2018.
- Complete Investigational New Drug (IND)-enabling preclinical studies.
- Submit an IND Application to the U.S. Food and Drug Administration (FDA) in the second half of 2018.

ADVM-053 for Hereditary Angioedema (HAE)

- Complete IND-enabling preclinical studies.
- Submit an IND Application to the FDA in the second half of 2018.

Upcoming Events

- Adverum plans to attend the following upcoming conferences:
  - Cowen 38th Annual Health Care Conference in Boston on March 12, 2018 at 1:30 pm ET
  - Cowen 17th Annual Life Sciences Winter Meeting in Colorado, March 20-23, 2018
  - ARM Cell & Gene Therapy Investor Day in New York, April 17, 2018
  - ARVO 2018 Annual Meeting in Honolulu, April 29-May 3, 2018
    - Poster titled *"Therapeutic potential and safety of sequential intravitreal dosing to the contralateral eye of novel AAV vectors in non-human primates"* on May 3, 2018, 8:15 – 10:00 am HST
    - Poster titled *"Long-term functional delivery of the human L-opsin cDNA via intravitreal administration of an AAV*

vector in Mongolian gerbils" on May 3, 2018, 8:15 - 10:00 am HST

- 2nd Annual Gene Therapy for Rare Disorders 2018 Meeting in Boston, April 30-May 2, 2018
- ASGCT 21st Annual Meeting in Chicago, May 16-19, 2018

Financial Results for the Three Months Ended December 31, 2017

- **Cash, cash equivalents and marketable securities** were $190.5 million as of December 31, 2017, compared to $186.6 million as of September 30, 2017 and $222.2 million as of December 31, 2016. The year-end cash position, added with approximately $64 million in net proceeds raised in February 2018, is expected to fund the three lead gene therapy programs through the end of 2019, 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 the three months ended December 31, 2017, compared to $0.5 million for the same period in 2016.
- **Research and development expenses** were $12.0 million for the three months ended December 31, 2017, compared to $7.9 million for the same period in 2016. This increase was due to an overall increase in research and development activities for the Company's gene therapy programs, primarily for material production costs for the ADVANCE clinical trial for ADVM-043.
- **General and administrative expenses** were $4.0 million for the three months ended December 31, 2017, compared to $4.8 million for the same period in 2016. This decrease was primarily due to lower legal fees.
- **Net loss attributable to common stockholders** was $14.8 million, or $0.32 per basic and diluted share, for the three months ended December 31, 2017, compared to $22.4 million, or $0.54 per basic and diluted share, for the same period in 2016.
- **Shares of common stock outstanding** were 62.2 million as of February 28, 2018.

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 matters that are not historical facts 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, potential opportunities, expectations, projections, goals, objectives, milestones, strategies, product pipeline, financial condition and results of operations, the sufficiency of its cash, cash equivalents and marketable securities, as well as the advancement of, and anticipated development and regulatory milestones and plans related to Adverum's product candidates and preclinical and clinical studies, and the commercial potential of its product candidates, 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 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 periodic reports filed with the SEC. 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)

	December 31, 2017	December 31, 2016
ASSETS		
Current assets:		
Cash, cash equivalents and marketable securities	$ 70,519	$ 222,170

Short-term investments	119,966	-
Receivable from collaborative partner	-	886
Prepaid expenses and other current assets	3,256	2,218
Total current assets	193,741	225,274
Property and equipment, net	3,024	4,169
Deposits and other long-term assets	140	140
Intangible assets	5,000	5,000
Total assets	$ 201,905	$ 234,583
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 8,695	$ 7,950
Current portion of deferred rent	129	96
Current portion of deferred revenue	1,850	1,850
Total current liabilities	10,674	9,896
Deferred rent, less current portion	222	352
Deferred revenue, less current portion	5,250	7,099
Deferred tax liability	1,250	1,250
Other non-current liabilities	481	386
Total liabilities	17,877	18,983
Stockholders’ equity	184,028	215,600
Total liabilities and stockholders’ equity	$ 201,905	$ 234,583

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

	Three Months Ended December 31,		Year Ended December 31,	
	2017	**2016**	**2017**	**2016**
Collaboration and license revenue	$ 461	$ 488	$ 1,849	$ 1,455
Operating expenses:				
Research and development	12,014	7,898	39,839	31,670
General and administrative	4,042	4,777	20,857	24,355
Impairment of goodwill and intangible assets	-	11,200	-	60,714
Total operating expenses	16,056	23,875	60,696	116,739

Operating loss	(15,595)	(23,387)	(58,847)	(115,284)
Other income (expense), net	806	218	2,700	762
Net loss before income tax benefit	(14,789)	(23,169)	(56,147)	(114,522)
Income tax benefit	-	775	-	775
Net loss attributable to common stockholders	$ (14,789)	$ (22,394)	$ (56,147)	$ (113,747)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.32)	$ (0.54)	$ (1.29)	$ (3.14)
Weighted-average common shares outstanding, outstanding, basic and diluted	46,069	41,758	43,661	36,246

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Primary Logo

Source: Adverum Biotechnologies, Inc.