



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

November 8, 2017

MENLO PARK, Calif., Nov. 08, 2017 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq:ADVM), a leading gene therapy company targeting unmet medical needs in serious rare and ocular diseases, today reported financial results for the third quarter ended September 30, 2017 and provided a corporate update.

"We continue to make progress advancing our gene therapy programs to reach our goal of transforming Adverum into a clinical-stage company by the end of this year," said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. "To accomplish this goal, this quarter we plan to begin patient enrollment in ADVANCE, a Phase 1/2 clinical trial for ADVM-043 for alpha-1 antitrypsin deficiency. Looking ahead, we plan to file two INDs with the FDA in the second half of 2018, for ADVM-022 in wet AMD and ADVM-053 in hereditary angioedema. We are well positioned and well capitalized to accelerate the development of our pipeline of novel gene therapies in 2018."

Recent Progress

- For ADVM-043, Adverum's gene therapy product candidate for treating alpha-1 antitrypsin (A1AT) deficiency, the Company plans to begin patient enrollment in the ADVANCE Phase 1/2 trial in the fourth quarter of 2017. Site activation is underway at five leading centers in the United States and Adverum continues to prepare for release of ADVM-043 drug product to the sites to support first patient dosing. In addition, the Company is working closely with the Alpha-1 Foundation to identify potential patients for this trial. This multi-center, open-label, dose-escalation clinical trial plans to evaluate ADVM-043 in three cohorts of patients receiving intravenous administration and one cohort receiving intrapleural administration. The trial is designed to assess the safety and protein expression of ADVM-043, and further details about the study can be found at ClinicalTrials.gov under trial identifier number [NCT02168686](https://clinicaltrials.gov/ct2/show/study/NCT02168686). The Company expects to report preliminary data from this trial in the second half of 2018.
- For ADVM-022, Adverum's intravitreally-administered gene therapy product candidate for the treatment of wet age-related macular degeneration (wAMD), the Company is conducting an ongoing preclinical study to assess the durability of protein expression in non-human primates and expects to report efficacy at 12 months in the first half of 2018. The Company also plans to file an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) in the second half of 2018.
- Also for ADVM-022, Adverum data were presented at the Targeting Ocular Disorders and the Retina Society conferences in September 2017. The additional long-term data continued to demonstrate sustained expression of anti-VEGF protein following a single intravitreal injection of ADVM-022. Pharmacokinetic data on one non-human primate showed sustained expression for 52 weeks. In a separate ongoing study, sustained expression for at least seven months was observed in seven non-human primates.
- For ADVM-053, Adverum's gene therapy product candidate for treating hereditary angioedema (HAE), the Company also plans to file an IND application with the FDA in the second half of 2018.
- In September 2017, Adverum appointed Eric G. Carter, M.D., Ph.D. to its board of directors. Dr. Carter is a pharmaceutical industry executive with over 20 years of global research and development experience in multiple therapeutic areas. Most recently, Dr. Carter served as senior vice president, chief medical officer, and global head of clinical and non-clinical development of Allergan from 2011 until its acquisition by Actavis Pharmaceuticals in 2015.

Upcoming Events

- Adverum plans to attend the following upcoming conferences:
 - Piper Jaffray's 29th Annual Healthcare Conference in New York on November 28, 2017 at 12:10 – 12:30 p.m. ET
 - Barclays' Gene Editing and Gene Therapy Summit in New York on November 30, 2017 at 9:00 – 9:20 a.m. ET
 - Cell Therapy Manufacturing and Gene Therapy Congress in Amsterdam, December 6-7, 2017

Financial Results for the Three Months Ended September 30, 2017

- **Cash, cash equivalents and marketable securities** were \$186.6 million as of September 30, 2017, compared to \$197.4 million as of June 30, 2017 and \$222.2 million as of December 31, 2016. The Company's current cash position is expected to fund the three lead gene therapy programs through the end of 2019 and through the achievement of meaningful clinical data in patients for at least one of the Company's lead programs.

- **Revenues**, consisting of revenue from collaborative research, were \$0.5 million for the three months ended September 30, 2017, compared to \$0.4 million for the same period in 2016.
- **Research and development expenses** were \$10.3 million for the three months ended September 30, 2017, compared to \$8.4 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 ADV-043.
- **General and administrative expenses** were \$4.8 million for the three months ended September 30, 2017, compared to \$6.1 million for the same period in 2016. This decrease was primarily due to lower salary expense, professional fees, and stock-based compensation expenses.
- **Net loss attributable to common stockholders** was \$13.8 million, or \$0.32 per basic and diluted share, for the three months ended September 30, 2017, compared to \$14.3 million, or \$0.35 per basic and diluted share, for the same period in 2016.

About Adverum Biotechnologies, Inc.

Adverum is a leading 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
(Unaudited)
(In thousands)

	September 30, 2017	December 31, 2016
ASSETS		
Current assets:		
Cash, cash equivalents and marketable securities	\$ 186,642	\$ 222,170
Receivable from collaborative partner	-	886
Prepaid expenses and other current assets	2,881	2,218
Total current assets	189,523	225,274
Property and equipment, net	3,347	4,169
Deposits and other long-term assets	340	140
Intangible assets	5,000	5,000
Total assets	\$ 198,210	\$ 234,583

LIABILITIES AND STOCKHOLDERS' EQUITY

Current liabilities:

Accounts payable and accrued liabilities	\$	7,642	\$	7,925
Restructuring liabilities		-		25
Current portion of deferred rent		121		96
Current portion of deferred revenue		1,850		1,850
Total current liabilities		<u>9,613</u>		<u>9,896</u>

Deferred rent, less current portion		257		352
Deferred revenue, less current portion		5,711		7,099
Deferred tax liability		1,250		1,250
Other liabilities		387		386
Total liabilities		<u>17,218</u>		<u>18,983</u>

Stockholders' equity		<u>180,992</u>		<u>215,600</u>
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Total liabilities and stockholders' equity	\$	<u>198,210</u>	\$	<u>234,583</u>
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ADVERUM BIOTECHNOLOGIES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)
(In thousands, except per share amounts)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Collaboration and license revenue	\$ 463	\$ 395	\$ 1,388	\$ 967
Operating expenses:				
Research and development	10,272	8,362	27,825	23,772
General and administrative	4,762	6,146	16,815	19,578
Impairment of goodwill and intangible assets	-	394	-	49,514
Total operating expenses	<u>15,034</u>	<u>14,902</u>	<u>44,640</u>	<u>92,864</u>
Operating loss	(14,571)	(14,507)	(43,252)	(91,897)
Other income (expense), net	742	206	1,894	544
Net loss before income tax benefit	<u>(13,829)</u>	<u>(14,301)</u>	<u>(41,358)</u>	<u>(91,353)</u>
Income tax benefit	-	-	-	-
Net loss attributable to common stockholders	<u>\$ (13,829)</u>	<u>\$ (14,301)</u>	<u>\$ (41,358)</u>	<u>\$ (91,353)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.32)</u>	<u>\$ (0.35)</u>	<u>\$ (0.97)</u>	<u>\$ (2.66)</u>
Weighted-average common shares outstanding, basic and diluted	<u>43,381</u>	<u>41,416</u>	<u>42,849</u>	<u>34,382</u>

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Source: Adverum Biotechnologies, Inc.