



## Adverum Biotechnologies Reports Fourth Quarter and Full Year 2016 Financial Results and Provides Update

March 6, 2017

MENLO PARK, Calif., March 06, 2017 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq:ADVM), a gene therapy company committed to discovering and developing novel medicines for patients suffering from diseases with few or burdensome treatment options, today reported financial results for the fourth quarter and year ended December 31, 2016 and provided a corporate update.

"We are laser-focused on advancing our three lead gene therapy programs, with a goal of becoming a clinical-stage company by the end of this year," said Amber Salzman, Ph.D., president and chief executive officer of Adverum Biotechnologies. "Adverum has a robust pipeline of gene therapies, a platform of industry-leading technology and capabilities, and an experienced leadership team with expertise in developing gene therapies. We are excited to be doing this important work to develop potential new therapies for patients living with wet AMD and rare diseases alpha-1 antitrypsin (A1AT) deficiency and hereditary angioedema. Looking ahead this year, we are executing our plans to initiate patient enrollment during the fourth quarter in a Phase 1/2 clinical trial for ADVM-043 in patients with A1AT deficiency. Importantly, we believe our cash is sufficient to fund the development of our three lead programs through the end of 2019."

### Recent Highlights

- In February 2017, Adverum was notified by Regeneron that it is extending the term of the companies' research collaboration by an additional three years, to May 1, 2020. The companies' research collaboration and license agreement, entered into in May 2014 for an initial period of three years, was created to discover, develop and commercialize novel gene therapy products for the treatment of ophthalmologic diseases. The collaboration covers up to eight distinct ocular therapeutic targets.
- At the European Society of Gene and Cell Therapy (ESGCT) meeting in October 2016, Adverum presented new preclinical data from its gene therapy candidates ADVM-022 (AAV.7m8-afiberccept) and ADVM-032 (AAV.7m8-ranibizumab) for the treatment of wet age-related macular degeneration (wAMD). Administered through a single, intravitreal injection, ADVM-022 and ADVM-032 demonstrated compelling proof-of-concept of these vectors' anti-angiogenic effect which was comparable to the standard-of-care anti-VEGF therapies in a laser-induced choroidal neovascularization model of wAMD in non-human primates.
- In August 2016, Adverum and Editas Medicine, Inc. announced a collaboration to explore the delivery of genome editing medicines to treat up to five inherited retinal diseases. This collaboration leverages Adverum's AAV vector platform technology and expertise to deliver Editas' leading CRISPR-based genome editing technologies to create a series of novel therapies for the treatment of inherited retinal diseases.

### 2017 Outlook

- Adverum has selected ADVM-022 to advance as an intravitreal injection gene therapy product candidate for wAMD. The Company met with the FDA in February 2017 and plans to initiate investigational new drug (IND)-enabling toxicology and biodistribution studies in the first half of 2017 to support a planned IND filing. The Company continues to review data for ADVM-032, its backup gene therapy product candidate for wAMD.
- For ADVM-043, Adverum's gene therapy product candidate for treating alpha-1 antitrypsin (A1AT) deficiency, the Company is upgrading ADVM-043's manufacturing to a commercial-grade baculovirus-based process and plans to transfer this process to a contract manufacturing organization for the production of clinical materials. The Company is planning to engage with the FDA to review development plans under an open IND in the first half of 2017. Adverum plans to initiate a toxicology study using ADVM-043 made in the baculovirus system in the first half of 2017 and to start enrolling patients for a Phase 1/2 trial in the fourth quarter of 2017.
- For ADVM-053, Adverum's gene therapy product candidate for treating hereditary angioedema (HAE), the Company plans to initiate IND-enabling toxicology studies and engage with the FDA in the first half of 2017. In addition, the Company plans to transfer its manufacturing process to a contract manufacturing organization and prepare to file an IND in order to move forward with a planned Phase 1/2 trial.
- Adverum's cash and cash equivalents of \$222.2 million as of December 31, 2016 are 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.
- Adverum plans to present at the following upcoming conferences:
  - Cowen and Company 37th Annual Health Care Conference today, March 6, 2017, at 2:40 – 3:10 pm ET

- 29<sup>th</sup> Annual ROTH Conference on Monday, March 13, 2017 at 7:30 – 8:00 am PT
- World Orphan Drug Congress USA 2017 on Thursday, April 20, 2017 at 3:10 pm ET

#### Financial Results for the Three Months Ended December 31, 2016

- **Cash, cash equivalents and marketable securities** were \$222.2 million as of December 31, 2016, compared to \$231.3 million as of September 30, 2016 and \$259.1 million as of December 31, 2015.
- **Revenues**, consisting of revenue from collaborative research, were \$0.5 million for the three months ended December 31, 2016, compared to \$1.0 million for the same period in 2015. This decrease was primarily due to the recognition of an event under a collaboration agreement for the three months ended December 31, 2015.
- **Research and development expenses** were \$7.9 million for the three months ended December 31, 2016, compared to \$7.2 million for the same period in 2015. This increase was primarily attributable to clinical material production and laboratory expenses for the Company's three lead programs, partially offset by a decrease in stock-based compensation expense.
- **General and administrative expenses** were \$4.8 million for the three months ended December 31, 2016, compared to \$5.4 million for the same period in 2015. This reduction was primarily due to a decrease in stock-based compensation expense.
- **Impairment of goodwill and intangible assets** was \$11.2 million for the three months ended December 31, 2016 and related to an impairment of intangible assets recorded in connection with the Annapurna transaction that closed in May 2016. This non-cash charge was the result of the Company's in-process research and development (IPR&D) asset assessment that was based on the Company's analysis of the fair value of intangible assets at December 31, 2016.
- **Income tax benefit** was \$0.8 million for the three months ended December 31, 2016 and related to the change in the deferred tax liabilities balance due to the intangible assets impairment recognized in the same quarter.
- **Net loss attributable to common stockholders** was \$22.4 million or \$0.54 per basic and diluted share, for the three months ended December 31, 2016, compared to \$14.1 million, or \$0.55 per basic and diluted share, for the same period in 2015.

#### Financial Results for the Twelve Months Ended December 31, 2016

- **Revenues**, consisting of revenue from collaborative research, were \$1.5 million for the year ended December 31, 2016, compared to \$2.3 million for 2015. This decrease was primarily due to the recognition of an event in 2015 under a collaboration agreement.
- **Research and development expenses** were \$31.7 million for the year ended December 31, 2016, compared to \$25.5 million for 2015. This increase was primarily attributable to increases in stock-based compensation expense and in clinical material production, license fees and laboratory expenses for the Company's three lead programs.
- **General and administrative expenses** were \$24.4 million for the year ended December 31, 2016, compared to \$22.1 million for 2015. This increase was primarily due to Annapurna acquisition-related expenses, partially offset by a decrease in stock-based compensation expense.
- **Impairment of goodwill and intangible assets** was \$60.7 million for the year ended December 31, 2016 and related to a non-cash goodwill and intangible assets impairment charges recorded in connection with the Annapurna transaction that closed in May 2016.
- **Income tax benefit** was \$0.8 million for the year ended December 31, 2016 and related to the change in the deferred tax liabilities balance due to intangible assets impairment recognized in the fourth quarter of 2016.
- **Net loss attributable to common stockholders** was \$113.7 million or \$3.14 per basic and diluted share, for the year ended December 31, 2016, compared to \$47.5 million, or \$1.86 per basic and diluted share, for 2015. The increase in net loss was primarily due to the non-cash impairment charges of \$60.7 million. The non-cash charges have no effect on the Company's current cash balance or operating cash flows.

#### About Adverum Biotechnologies, Inc.

Adverum is a gene therapy company committed to discovering and developing novel medicines that can offer life-changing benefits to patients living with rare diseases or diseases of the eye who currently have limited or burdensome treatment options. Adverum has a robust pipeline that includes product candidates designed to treat wet age-related macular degeneration (wAMD) and rare diseases alpha-1 antitrypsin (A1AT) deficiency and hereditary angioedema (HAE). Leveraging a next-generation adeno-associated virus (AAV)-based directed evolution platform, the Company 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](http://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, and the risk that Adverum will not be able to successfully develop or commercialize any of its product candidates. 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)

	<b>December 31, 2016</b>	<b>December 31, 2015</b>
<b>ASSETS</b>		
Current assets:		
Cash, cash equivalents and marketable securities	\$ 222,170	\$ 259,080
Receivable from collaborative partner	886	449
Prepaid expenses and other current assets	2,218	1,463
Total current assets	225,274	260,992
Property and equipment, net	4,169	3,187
Deposits and other long-term assets	140	140
Intangible assets	5,000	-
Total assets	<b>\$ 234,583</b>	<b>\$ 264,319</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 7,925	\$ 4,612
Restructuring liabilities	25	1,013
Current portion of deferred rent	96	66
Current portion of deferred revenue	1,850	883
Total current liabilities	9,896	6,574
Deferred rent, less current portion	352	447
Deferred revenue, less current portion	7,099	4,706
Deferred tax liability	1,250	-
Other liabilities	386	-
Total liabilities	18,983	11,727
Stockholders' equity	215,600	252,592
Total liabilities and stockholders' equity	<b>\$ 234,583</b>	<b>\$ 264,319</b>

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

	Three Months Ended December 31,		Year Ended December 31,	
	2016	2015	2016	2015
Collaboration and license revenue	\$ 488	\$ 960	\$ 1,455	\$ 2,319
Operating expenses:				
Research and development	7,898	7,192	31,670	25,462
General and administrative	4,777	5,374	24,355	22,107
Impairment of goodwill and intangible assets	11,200	-	60,714	-
Restructuring charges	-	2,573	-	2,573
Total operating expenses	<u>23,875</u>	<u>15,139</u>	<u>116,739</u>	<u>50,142</u>
Operating loss	(23,387)	(14,179)	(115,284)	(47,823)
Other income (expense), net	<u>218</u>	<u>85</u>	<u>762</u>	<u>370</u>
Net loss before income tax benefit	(23,169)	(14,094)	(114,522)	(47,453)
Income tax benefit	<u>775</u>	<u>-</u>	<u>775</u>	<u>-</u>
Net loss attributable to common stockholders	<u>\$ (22,394)</u>	<u>\$ (14,094)</u>	<u>\$ (113,747)</u>	<u>\$ (47,453)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.54)</u>	<u>\$ (0.55)</u>	<u>\$ (3.14)</u>	<u>\$ (1.86)</u>
Weighted-average common shares outstanding, outstanding, basic and diluted	<u>41,758</u>	<u>25,777</u>	<u>36,246</u>	<u>25,479</u>

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