



## Adverum Biotechnologies Reports Fourth Quarter 2018 Financial Results and Provides Corporate Update

March 6, 2019

### Advancing Novel Gene Therapy ADVM-022 for Wet AMD in OPTIC Phase 1 Clinical Trial

MENLO PARK, Calif., March 06, 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 fourth quarter ended December 31, 2018 and provided a corporate update.

"Over the past year our lead gene therapy, ADVM-022 for the treatment of wet AMD, has gained significant momentum," said Leone Patterson, chief executive officer of Adverum Biotechnologies. "We are encouraged by having started our OPTIC phase 1 clinical trial evaluating ADVM-022 for the treatment of wet AMD as well as by the growing body of preclinical evidence on this novel, proprietary intravitreal injection gene therapy for wet AMD. We are on track to provide an update on OPTIC trial enrollment in the first half of 2019 and report interim data by the first quarter of 2020. In addition, we are evaluating other ocular VEGF-related indications for this gene therapy. Our team is committed to advancing ADVM-022 and looks forward to the potential of bringing this unique single intravitreal injection gene therapy to patients living with wet AMD."

#### Recent Updates

- Adverum is on track to provide an enrollment update in the first half of 2019 on the OPTIC phase 1 clinical trial of ADVM-022 (AAV.7m8-aflibercept) in patients with wet age-related macular degeneration (wet AMD). Adverum announced the first patient was dosed in cohort 1 in the OPTIC study in mid-November 2018. ADVM-022 is designed to be administered as a single intravitreal injection that provides long-lasting therapy without the need for chronic or frequent anti-VEGF injections
- In February 2019, long-term preclinical data was presented at the 2019 Angiogenesis, Exudation, and Degeneration conference. The data demonstrated that the normal non-human primate retinal structure is maintained for up to 21 months after a single intravitreal injection of  $2 \times 10^{12}$  vg of ADVM-022, which continuously delivers the anti-VEGF molecule aflibercept. The results presented were measured by optical coherent tomography (OCT) and were presented by David M. Brown, M.D., Clinical Professor of Ophthalmology at Baylor College of Medicine, vice-chair for research at the Blanton Eye Institute, Houston Methodist Hospital, and partner at Retina Consultants of Houston
- In December 2018, long-term preclinical expression and efficacy data on ADVM-022 in wet AMD were published in [Molecular Therapy](#), the leading peer-reviewed scientific journal for gene and cell therapy. Robust aflibercept expression was detected in the retinal and choroidal tissue obtained 16 months after a single intravitreal injection of ADVM-022 in non-human primates (NHPs) and in a laser-induced choroidal neovascularization (CNV) model in NHPs; a single intravitreal injection of ADVM-022 13 months before laser treatment provided the same level of protection against clinically relevant CNV lesions as a bolus of aflibercept (a standard-of-care) at the time of lesioning

#### Future Outlook - Planned Milestones

##### ADVM-022 for Wet AMD

- Provide an update on enrollment from the OPTIC phase 1 clinical trial in the first half of 2019
- Provide interim data from the OPTIC phase 1 clinical trial by the first quarter of 2020

##### Rare Disease Program

- Provide an update on Adverum's A1AT deficiency and hereditary angioedema programs in the first half of 2019

##### Manufacturing Capabilities

- Occupy the 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 conferences:
  - Cowen's 39<sup>th</sup> Annual Health Care Conference in Boston, MA, March 11-13, 2019
  - Retina World Congress in Ft. Lauderdale, FL, March 21-24, 2019

- Alliance for Regenerative Medicine's Cell & Gene Meeting on the Mediterranean in Barcelona, Spain, April 23-24, 2019
- ARVO Annual Meeting in Vancouver, B.C., April 28-May 2, 2019 poster presentation:
  - Abstract Title: Immunological response and durability of expression following sequential intravitreal administration of AAV2.7m8 gene therapy to the contralateral eye in non-human primates
  - Poster Session: April 30, 2019 from 8:45am to 10:30am PT (Session Number: 325, Posterboard Number: 2910-A0097)
- American Society of Gene and Cell Therapy (ASGCT) 22<sup>nd</sup> Annual Meeting in Washington, D.C., April 29-May 2, 2019

#### Financial Results for the Three Months Ended December 31, 2018

- **Cash, cash equivalents and short-term investments** were \$205.1 million as of December 31, 2018, compared to \$217.9 million as of September 30, 2018, and \$190.5 million as of December 31, 2017. Adverum expects this year-end cash position to fund operating expenses and capital expenditures into 2021
- **Revenue**, consisting of revenue from collaborative research, was \$0.1 million the three months ended December 31, 2018, compared to \$0.5 million for the same period in 2017
- **Research and development expenses** were \$11.6 million for the three months ended December 31, 2018, compared to \$12.0 million for the same period in 2017. Research and development expenses remained flat primarily due to a decrease in clinical activities related to the rare disease programs which were offset by increases in clinical activities related to ADVM-022
- **General and administrative expenses** were \$5.2 million for the three months ended December 31, 2018, compared to \$4.0 million for the same period in 2017. This increase was primarily due to higher compensation and benefit and consultant expenses
- **Net loss attributable to common stockholders** was \$15.7 million, or \$0.25 per basic and diluted share, for the three months ended December 31, 2018, compared to \$14.8 million, or \$0.32 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 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](http://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 an update on enrollment and interim data for the OPTIC trial, and the statements under the heading "Future Outlook - Planned Milestones," 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, 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)

	December 31, 2018	December 31, 2017
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 154,949	\$ 70,519
Short-term investments	50,130	119,966
Prepaid expenses and other current assets	3,675	3,256
Total current assets	208,754	193,741

Property and equipment, net	3,586	3,024
Restricted cash	999	-
Deposits and other long-term assets	156	140
Intangible assets	-	5,000
	<u>                    </u>	<u>                    </u>
Total assets	<u>\$ 213,495</u>	<u>\$ 201,905</u>

#### LIABILITIES AND STOCKHOLDERS' EQUITY

##### Current liabilities:

Accounts payable and accrued liabilities	\$ 10,491	\$ 8,695
Current portion of deferred rent	228	129
Current portion of deferred revenue	-	1,850
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Total current liabilities	10,719	10,674
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Deferred rent, less current portion	1,366	222
Deferred revenue, less current portion	-	5,250
Deferred tax liability	-	1,250
Other non-current liabilities	243	481
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Total liabilities	12,328	17,877
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Stockholders' equity	<u>201,167</u>	<u>184,028</u>
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Total liabilities and stockholders' equity	<u>\$ 213,495</u>	<u>\$ 201,905</u>
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**ADVERUM BIOTECHNOLOGIES, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**  
(In thousands, except per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Collaboration and license revenue	\$ 70	\$ 461	\$ 1,612	\$ 1,849
Operating expenses:				
Research and development	11,642	12,014	50,133	39,839
General and administrative	5,187	4,042	24,560	20,857
Impairment of intangible assets	-	-	5,000	-
	<u>16,829</u>	<u>16,056</u>	<u>79,693</u>	<u>60,696</u>
Operating loss	(16,759)	(15,595)	(78,081)	(58,847)
Other income (expense), net	<u>1,100</u>	<u>806</u>	<u>4,204</u>	<u>2,700</u>
Net loss before income tax benefit	(15,659)	(14,789)	(73,877)	(56,147)

Income tax benefit	-	-	1,250	-
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Net loss	\$ (15,659)	\$ (14,789)	\$ (72,627)	\$ (56,147)
	<u>                    </u>	<u>                    </u>	<u>                    </u>	<u>                    </u>
Net loss per share, basic and diluted	\$ (0.25)	\$ (0.32)	\$ (1.18)	\$ (1.29)
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Weighted-average common shares outstanding, basic and diluted	<u>                    </u>	<u>                    </u>	<u>                    </u>	<u>                    </u>
	62,915	46,069	61,375	43,661

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Source: Adverum Biotechnologies, Inc.