



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

November 8, 2018

MENLO PARK, Calif., Nov. 08, 2018 (GLOBE NEWSWIRE) -- Adverum Biotechnologies, Inc. (Nasdaq: ADVM), a clinical-stage gene therapy company targeting unmet medical needs in ophthalmology and rare diseases, today reported financial results for the third quarter ended September 30, 2018 and provided a corporate update.

"2018 has been a year focused on progressing our internal drug development including obtaining an open IND and Fast Track designation for our ADVM-022 wet AMD program," said Leone Patterson, chief executive officer of Adverum Biotechnologies. "I am also pleased to announce the appointment of Mehdi Gasmi, Ph.D. to president and chief scientific officer. Under Mehdi's leadership, the Adverum team worked together to design the Company's first proprietary gene therapy candidate, ADVM-022, designed as a single intravitreal injection treatment for patients living with wet AMD. We look forward to initiating the OPTIC Phase 1 trial, with ADVM-022, this quarter targeting its initial indication, wet AMD. Our team is deeply committed to advancing our technology in ophthalmology and rare diseases to improve the quality of life for patients, and our strong cash position enables us to fund operations at least through the first half of 2020."

"I am very excited about this opportunity and to continue leading the Adverum organization with Leone and the rest of the executive team," said Mehdi Gasmi, Ph.D., president and chief scientific officer of Adverum Biotechnologies. "I am pleased with our recent accomplishments in translating our wet AMD program into the clinic and I look forward to continuing to develop transformational gene therapies to benefit patients living with serious unmet medical needs."

Recent Updates

- In November 2018, appointed Mehdi Gasmi, Ph.D. to president and chief scientific officer. Dr. Gasmi has over 22 years of experience in gene therapy and is an expert in the design, development and manufacturing of lentiviral and recombinant AAV vectors for clinical applications. He has worked at multiple gene therapy organizations, including Chiron (Novartis), Cell Genesys, Ceregene, Genethon, University of California, San Diego and City of Hope
- In November 2018, announced the Company discontinued the development of ADVM-043, an investigational AAVrh.10-based gene therapy for the treatment of A1AT deficiency and announced that the Company will not submit an Investigational New Drug (IND) application for ADVM-053, an AAVrh.10-based gene therapy for the treatment of hereditary angioedema (HAE). The Company plans to conduct additional preclinical studies to determine the best gene therapy candidate to advance for the rare disease programs
- In October 2018, announced appointment of Leone Patterson as chief executive officer and a member of the Board of Directors
- In October 2018, presented long-term, durable preclinical efficacy data on ADVM-022 (AAV.7m8-afibercept) in a model of wet age-related macular degeneration (wet AMD) at European Society of Gene and Cell Therapy (ESGCT) 26th Annual Congress. Data presented demonstrated that a single intravitreal (IVT) administration of ADVM-022 delivering a continuous supply of aflibercept may provide an effective long-term treatment option for neovascularization associated with wet AMD. The data showed a single IVT administration of ADVM-022 provided robust expression of aflibercept at levels on par with current standard of care, sustained for approximately two years post-dose in non-human primates
- In September 2018, received Fast Track designation for ADVM-022 in wet AMD from the U.S. Food and Drug Administration (FDA). Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Despite the availability of anti-VEGF therapies, patients still have a significant unmet need as the frequency of injections is burdensome and undertreatment may lead to vision loss
- In late August 2018, announced that the IND application for ADVM-022 in patients went active

Future Outlook - Planned Pipeline Milestones

Wet AMD Program

- Initiate the OPTIC Phase 1 clinical trial for ADVM-022 in patients with wet AMD in the fourth quarter of 2018

Rare Disease Programs

- Provide an update on rare disease programs in the first half of 2019

Upcoming Events

Adverum plans to attend the following upcoming conferences:

- Piper Jaffray's 30th Annual Healthcare Conference in New York, November 27-28, 2018
- J.P. Morgan's 37th Annual Healthcare Conference in San Francisco, January 7-10, 2019

Financial Results for the Three Months Ended September 30, 2018

- **Cash, cash equivalents and marketable securities** were \$217.9 million as of September 30, 2018, compared to \$234.9 million as of June 30, 2018 and \$190.5 million as of December 31, 2017. Adverum expects this quarter-end cash position to fund operations at least through the first half of 2020
- **Revenue**, consisting of revenue from collaborative research, was \$0.8 million for the three months ended September 30, 2018 and \$0.5 million for the same period in 2017. The increase was related to research services provided to Editas under Adverum's collaboration agreement
- **Research and development expenses** were \$14.5 million for the three months ended September 30, 2018, compared to \$10.3 million for the same period in 2017. This was primarily due to an overall increase in research and development activities, including clinical trial expenses for ADV-043, clinical trial initiation expenses for ADV-022, and material production costs for the wet AMD and rare disease programs
- **General and administrative expenses** were \$4.8 million for each of the three months ended September 30, 2018 and 2017
- **Impairment of intangible asset** was \$5.0 million for the three months ended September 30, 2018. This non-cash charge was related to the Company's intangible asset for ADV-043 and was due to the Company's recent decision to discontinue its development of ADV-043. The in-process research and development (IPR&D) asset originated from the Annapurna acquisition in May 2016
- **Income tax benefit** was \$1.3 million for the three months ended September 30, 2018 and related to the change in the deferred tax liabilities balance due to the intangible asset impairment charge
- **Net loss attributable to common stockholders** was \$21.0 million, or \$0.34 per basic and diluted share, for the three months ended September 30, 2018, compared to \$13.8 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 ophthalmology and rare diseases. 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 "Future Outlook - Planned Pipeline Milestones," expectations as to the ability of Adverum's quarter-end cash position to fund operations at least through the first half of 2020 and expectations on the timing for initiating the OPTIC clinical trial and the Company's plans to conduct additional preclinical studies to determine the best gene therapy candidate to advance for the rare disease programs, 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 November 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)

	September 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 159,673	\$ 70,519

Short-term investments	58,209	119,966
Prepaid expenses and other current assets	4,292	3,256
Total current assets	222,174	193,741
Property and equipment, net	2,594	3,024
Restricted cash	999	-
Deposits and other long-term assets	140	140
Intangible asset	-	5,000
Total assets	<u>\$ 225,907</u>	<u>\$ 201,905</u>

LIABILITIES AND STOCKHOLDERS' EQUITY

Current liabilities:		
Accounts payable and accrued liabilities	\$ 9,513	\$ 8,695
Current portion of deferred rent	482	129
Current portion of deferred revenue	70	1,850
Total current liabilities	10,065	10,674
Deferred rent, less current portion	102	222
Deferred revenue, less current portion	-	5,250
Deferred tax liability	-	1,250
Other non-current liabilities	187	481
Total liabilities	10,354	17,877
Stockholders' equity	215,553	184,028
Total liabilities and stockholders' equity	<u>\$ 225,907</u>	<u>\$ 201,905</u>

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

	Three Months Ended September		Nine Months Ended September	
	30,		30,	
	2018	2017	2018	2017
Collaboration and license revenue	\$ 833	\$ 463	\$ 1,542	\$ 1,388
Operating expenses:				
Research and development	14,480	10,272	38,491	27,825
General and administrative	4,826	4,762	19,373	16,815
Impairment of intangible asset	5,000	-	5,000	-
Total operating expenses	24,306	15,034	62,864	44,640
Operating loss	(23,473)	(14,571)	(61,322)	(43,252)

Other income (expense), net	<u>1,265</u>	<u>742</u>	<u>3,104</u>	<u>1,894</u>
Net loss before income tax benefit	(22,208)	(13,829)	(58,218)	(41,358)
Income tax benefit	<u>1,250</u>	<u>-</u>	<u>1,250</u>	<u>-</u>
Net loss	<u>\$ (20,958)</u>	<u>\$ (13,829)</u>	<u>\$ (56,968)</u>	<u>\$ (41,358)</u>
Net loss per share, basic and diluted	<u>\$ (0.34)</u>	<u>\$ (0.32)</u>	<u>\$ (0.94)</u>	<u>\$ (0.97)</u>
Weighted-average common shares outstanding, basic and diluted	<u>62,454</u>	<u>43,381</u>	<u>60,856</u>	<u>42,849</u>

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Source: Adverum Biotechnologies, Inc.