



## Avalanche Biotechnologies, Inc. Reports Third Quarter 2014 Financial Results

November 12, 2014

MENLO PARK, Calif., Nov. 12, 2014 (GLOBE NEWSWIRE) -- Avalanche Biotechnologies, Inc. (Nasdaq:AAVL), a clinical-stage biotechnology company focused on discovering and developing novel gene therapies to transform the lives of patients with sight-threatening ophthalmic diseases, today reported financial results and operational highlights for the quarter ended September 30, 2014.

"The third quarter of 2014 marked important progress in the history of Avalanche as a leader in the development of gene therapies for diseases of the eye," said Thomas W. Chalberg, Jr., Ph.D., Chief Executive Officer of Avalanche. "The successful completion of our initial public offering substantially strengthened our balance sheet and provides the financial resources to advance the development of our portfolio of gene therapy candidates including AVA-101, which is in a Phase 2a clinical trial for the treatment of patients with wet age-related macular degeneration. Further, we continue to innovate across the full range of our pipeline, including partnered work in rare genetic diseases of the eye and fundamental Avalanche technology advances with the development of novel vectors and methods of administration."

### Recent Business Highlights

- In August 2014, Avalanche completed its initial public offering raising net proceeds of approximately \$106.5 million in its IPO, selling 6,900,000 shares of its common stock, including the full exercise of the underwriters' over-allotment option.
- Concurrent with the completion of the IPO, Avalanche completed a \$10.0 million private placement of common stock to Regeneron Pharmaceuticals, Inc., a collaboration partner of Avalanche.
- In September 2014, Avalanche strengthened its management team with the appointment of Roman G. Rubio, M.D. as Senior Vice President and Head of Translational Medicine.

### Third Quarter 2014 Financial Results

- **Cash** as of September 30, 2014 was \$165.3 million, compared to \$0.6 million as of December 31, 2013.
- **Revenues**, consisting primarily of revenue from collaborative research, was \$0.2 million for the quarter ended September 30, 2014, compared to \$30,000 for the quarter ended September 30, 2013.
- **Research and development (R&D) expenses** were \$5.7 million for the quarter ended September 30, 2014, compared to \$0.6 million for the quarter ended September 30, 2013.
- **General and administrative (G&A) expenses** were \$2.4 million for the quarter ended September 30, 2014, compared to \$0.3 million for the quarter ended September 30, 2013.
- **Net loss attributable to common stockholders** was \$8.3 million for the quarter ended September 30, 2014, compared to a net loss of \$0.8 million for the quarter ended September 30, 2013.

### About Avalanche Biotechnologies, Inc.

Avalanche is a clinical-stage biotechnology company focused on discovering and developing novel gene therapies to transform the lives of patients with sight-threatening ophthalmic diseases. Avalanche's lead product, AVA-101, is currently under development in a Phase 2a clinical trial for wet AMD. Avalanche's Ocular BioFactory(TM) platform technology is a proprietary adeno-associated virus (AAV)-based gene therapy discovery and development technology optimized for ophthalmology that utilizes a directed evolution approach to generate novel drug candidates. For more information please visit [www.avalanchebiotech.com](http://www.avalanchebiotech.com).

### Cautionary Note on Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Avalanche's expectations regarding sufficiency of existing cash to fund operations for projected periods of time, timing of release of additional data for its product candidates, timing of initiation of additional studies for its product candidates, plans regarding ongoing studies for existing programs and potential benefits of its products under development are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our drug candidates. Avalanche undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the company in general, see Avalanche's Quarterly Report on Form 10-Q for the quarter ended June 30, 2014, filed with the Securities and Exchange Commission on September 11, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

**AVALANCHE BIOTECHNOLOGIES, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
(Unaudited)  
(In thousands)

	<b>September 30,</b>	<b>December 31,</b>
	<b>2014</b>	<b>2013</b>
	<u>          </u>	<u>          </u>
<b>ASSETS</b>		
Current assets:		
Cash	\$ 165,329	\$ 564
Accounts receivable	8	8
Prepaid and other current assets	<u>996</u>	<u>250</u>
Total current assets	166,333	822
Property and equipment, net	817	69
Deposits and other long-term assets	<u>89</u>	<u>194</u>
Total assets	<u>\$ 167,239</u>	<u>\$ 1,085</u>
 <b>LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 3,648	\$ 1,162
Current portion of deferred revenue	<u>813</u>	<u>--</u>
Total current liabilities	4,461	1,162
Deferred revenue, less current portion	6,849	--
Convertible preferred stock warrant liability	--	91
Other liabilities	<u>194</u>	<u>50</u>
Total liabilities	11,504	1,303
Series A convertible preferred stock	--	7,992
Stockholders' equity (deficit)	<u>155,735</u>	<u>(8,210)</u>
Total liabilities and stockholders' equity (deficit)	<u>\$ 167,239</u>	<u>\$ 1,085</u>

**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**

(Unaudited)

(In thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Collaboration and license revenue	\$ 204	\$ --	\$ 369	\$ --
Government grant revenue	--	30	--	480
Total revenue	<u>204</u>	<u>30</u>	<u>369</u>	<u>480</u>
Operating expenses:				
Research and development	5,746	550	9,750	1,412
General and administrative	<u>2,398</u>	<u>258</u>	<u>4,618</u>	<u>593</u>
Total operating expenses	<u>8,144</u>	<u>808</u>	<u>14,368</u>	<u>2,005</u>
Operating loss	(7,940)	(778)	(13,999)	(1,525)
Other expense, net	<u>(316)</u>	<u>(35)</u>	<u>(1,014)</u>	<u>(58)</u>
Net loss	(8,256)	(813)	(15,013)	(1,583)
Deemed dividend	--	--	<u>(3,230)</u>	--
Net loss attributable to common stockholders	<u>\$ (8,256)</u>	<u>\$ (813)</u>	<u>\$ (18,243)</u>	<u>\$ (1,583)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.50)</u>	<u>\$ (0.22)</u>	<u>\$ (2.29)</u>	<u>\$ (0.43)</u>
Weighted-average common shares outstanding, basic and diluted	<u>16,394</u>	<u>3,673</u>	<u>7,960</u>	<u>3,673</u>

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