

Avalanche Biotechnologies, Inc. Reports First Quarter 2015 Financial Results

May 13, 2015

MENLO PARK, Calif., May 13, 2015 (GLOBE NEWSWIRE) -- Avalanche Biotechnologies, Inc. (Nasdaq:AAVL), a clinical-stage biopharmaceutical company committed to improving or preserving the sight of people suffering from blinding eye diseases with an unmet medical need, today reported financial results for the first quarter ended March 31, 2015.

"The start of 2015 marked important milestones for Avalanche as we continue to advance our pipeline and innovate in next-generation vector technology," said Thomas W. Chalberg, Jr., Ph.D., founder and chief executive officer. "We're proud to collaborate with the University of Washington and vision scientists Drs. Jay and Maureen Neitz as we develop AVA-322 and AVA-323, our gene therapy product candidates for red-green color blindness. Combined with our anticipated mid-year announcement of 12-month topline data from the Phase 2a trial and 36-month follow-up from the Phase 1 trial of AVA-101 – our lead product candidate – and initiation of a Phase 2b trial, we're looking forward to continued progress in our work to transform the treatment landscape for patients with sight-threatening eye diseases."

Recent Business Highlights

- In January 2015, Avalanche completed a public offering of 2,369,375 shares of its common stock, which included 359,918 shares the Company issued pursuant to the underwriters' exercise of their option to purchase additional shares. The Company received net proceeds of approximately \$130.6 million, after underwriting discounts, commissions and offering expenses.
- On March 25, 2015, Avalanche announced an exclusive license agreement with the University of Washington in Seattle to develop products based on Avalanche's proprietary Ocular BioFactory™ Platform for the treatment of color vision deficiency, commonly known as red-green color blindness. Currently, there are no approved treatment options.
- Avalanche presented updates on its clinical programs in development as well as advancements in next-generation vector technology at its Analyst and Investor Day in New York City in March 2015.

First Quarter 2015 Financial Results

- Cash, cash equivalents and marketable securities as of March 31, 2015 was \$290.1 million, compared to \$159.4 million as of December 31, 2014. In January 2015, Avalanche raised net proceeds of approximately \$130.6 million in a public offering of common stock.
- **Revenues**, consisting of revenue from collaborative research, was \$0.2 million for the quarter ended March 31, 2015, compared to \$30,000 for the quarter ended March 31, 2014.
- Research and development expenses were \$5.6 million for the quarter ended March 31, 2015, compared to \$0.9 million for the quarter ended March 31, 2014.
- **General and administrative expenses** were \$4.1 million for the quarter ended March 31, 2015, compared to \$0.7 million for the quarter ended March 31, 2014.
- **Net loss** was \$9.5 million, or \$0.38 per basic and diluted share, for the quarter ended March 31, 2015, compared to a net loss of \$1.7 million, or \$0.45 per basic and diluted share, for the quarter ended March 31, 2014.

About Avalanche Biotechnologies, Inc.

Avalanche is a biopharmaceutical company committed to improving or preserving the sight of people suffering from blinding eye diseases with an unmet medical need. Avalanche's proprietary Ocular BioFactoryTM is a platform for discovering and developing novel medicines with the potential to offer life-changing therapeutic benefit. Avalanche's lead product candidate, AVA-101, is in mid-stage clinical development for patients with wet age-related macular degeneration. For more information, please visit www.avalanchebiotech.com.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Avalanche's plans, potential opportunities, expectations, projections, goals, objectives, milestones, strategies, product pipeline, clinical studies, collaborations, product development and the 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, including Avalanche's expectations regarding its ability to advance its AVA-101, AVA-322 and AVA-323 product candidates, initiate its Phase 2b and Phase 3 clinical trials with respect to AVA-101, and improve outcomes for patients suffering from blinding eye diseases. Such forward-looking statements involve substantial risks and uncertainties that could cause our product development program, 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 development

process, including the regulatory approval process, the timing of our regulatory filings and other matters that could affect the availability or commercial potential of our product candidates. Avalanche undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties relating to the business of Avalanche, see our Annual Report on Form 10-K for the year ended December 31, 2014, filed with the Securities and Exchange Commission on March 5, 2015, and our subsequent periodic reports filed with the Securities and Exchange

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

| | March 31, 2015 | December 31, 2014 |
|---|-------------------|-------------------|
| ASSETS | (unaudited) | |
| Current assets: | | |
| Cash, cash equivalents and marketable securities | \$ 290,125 | \$ 159,404 |
| Prepaid expenses and other current assets | 1,007 | 874 |
| Total current assets | 291,132 | 160,278 |
| Property and equipment, net | 1,421 | 1,085 |
| Deposits and other long-term assets | 138 | 543 |
| Total assets | \$ 292,691 | \$ 161,906 |
| LIABILITIES AND STOCKHOLDERS' EQUITY Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 5,315 | \$ 4,658 |
| Current portion of deferred revenue | 2,313 | 813 |
| Total current liabilities | 7,628 | 5,471 |
| Deferred revenue, less current portion | 4,943 | 6,646 |
| Other liabilities | 440 | 306 |
| Total liabilities | 13,011 | 12,423 |
| Stockholders' equity | 279,680 | 149,483 |
| Total liabilities and stockholders' equity | \$ 292,691 | \$ 161,906 |

AVALANCHE BIOTECHNOLOGIES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(In thousands, except per share amounts)

| | Three Months Ended March 31, | | |
|---|------------------------------|------------|--|
| | 2015 | 2014 | |
| | (unaudited) | | |
| Collaboration and license revenue | \$ 203 | \$ 30 | |
| Operating expenses: | | | |
| Research and development | 5,621 | 910 | |
| General and administrative | 4,143 | 726 | |
| Total operating expenses | 9,764 | 1,636 | |
| Operating loss | (9,561) | (1,606) | |
| Other income (expense), net | 52 | (57) | |
| Net loss | \$ (9,509) | \$ (1,663) | |
| Net loss per share attributable to common stockholders, basic and diluted | \$ (0.38) | \$ (0.45) | |
| Weighted-average common shares outstanding, basic and diluted | 24,887 | 3,673 | |

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Avalanche Biotechnologies Logo

Avalanche Biotechnologies, Inc.