



Avalanche Biotechnologies, Inc. Reports Second Quarter 2015 Financial Results and Provides Update on AVA-101 Program

August 13, 2015

MENLO PARK, Calif., Aug. 13, 2015 (GLOBE NEWSWIRE) -- Avalanche Biotechnologies, Inc. (Nasdaq:AAVL), a biopharmaceutical company committed to improving or preserving the sight of people suffering from serious eye diseases with an unmet medical need, today reported financial results for the second quarter ended June 30, 2015.

The company also reported that after further analyses of results from a previously reported Phase 2a trial of AVA-101 for the potential treatment of wet age-related macular degeneration (wet AMD), it will not initiate a Phase 2b clinical trial in the second half of 2015.

Instead, Avalanche will conduct additional preclinical studies to investigate optimal dose and delivery of AVA-101 and AVA-201 versus standard of care anti-VEGF protein therapy to select the best gene therapy product candidate for wet AMD to advance back into the clinic. This decision was based on more detailed analyses of the Phase 2a clinical trial data, which included individual subject-level assessment of anatomic and functional outcomes, treatability of the Phase 2a subjects and product administration.

"These analyses gave us more information about some of the factors, including dosing and administration variability, that may have contributed to the Phase 2a study results," said Hans Hull, interim chief executive officer of Avalanche. "We are carefully focusing our resources and leveraging our scientific expertise to develop what we believe could be a transformative treatment for wet AMD that will help patients better manage this devastating disease."

Avalanche remains on track to complete candidate selection for AVA-201 by the end of 2015 and preclinical studies for AVA-322 and AVA-323 are ongoing. The company will also continue to develop its next generation vector technology to enable new therapies for diseases with high unmet need.

Second Quarter 2015 Financial Results

- **Cash, cash equivalents and marketable securities as of June 30, 2015 was \$279.6 million**, compared to \$159.4 million as of December 31, 2014. The Company does not expect the burn rate recognized over the past two quarters to change substantially over the next 18 months.
- **Revenues**, consisting of revenue from collaborative research, was \$203,000 for the quarter ended June 30, 2015, compared to \$135,000 for the quarter ended June 30, 2014.
- **Research and development expenses** were \$5.1 million for the quarter ended June 30, 2015, compared to \$3.1 million for the quarter ended June 30, 2014.
- **General and administrative expenses** were \$5.0 million for the quarter ended June 30, 2015, compared to \$1.5 million for the quarter ended June 30, 2014.
- **Net loss attributable to common stockholders** was \$9.8 million, or \$0.38 per basic and diluted share, for the quarter ended June 30, 2015, compared to a net loss attributable to common stockholders of \$8.3 million, or \$2.27 per basic and diluted share, for the quarter ended June 30, 2014.

About Avalanche Biotechnologies, Inc.

Avalanche is a biopharmaceutical company committed to improving or preserving the sight of people suffering from serious eye diseases with an unmet medical need. We are leveraging our next-generation Ocular BioFactory™ gene therapy platform to discover and develop novel medicines with the potential to offer life-changing therapeutic benefit. 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, 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. Such forward-looking statements involve substantial risks and uncertainties that could cause our product development program, 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 product development process, the uncertainties inherent in 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 Commission.

CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands)

| | <u>June 30,</u> <u>2015</u> | <u>December 31,</u> <u>2014</u> |
|--|--------------------------------|------------------------------------|
| ASSETS | <i>(unaudited)</i> | |
| Current assets: | | |
| Cash, cash equivalents and marketable securities | \$ 279,640 | \$ 159,404 |
| Prepaid expenses and other current assets | <u>992</u> | <u>874</u> |
| Total current assets | 280,632 | 160,278 |
| Property and equipment, net | 2,945 | 1,085 |
| Deposits and other long-term assets | <u>138</u> | <u>543</u> |
| Total assets | <u>\$ 283,715</u> | <u>\$ 161,906</u> |

LIABILITIES AND STOCKHOLDERS' EQUITY

| | | |
|--|-----------------------|-----------------------|
| Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 5,500 | \$ 4,658 |
| Current portion of deferred rent | 51 | -- |
| Current portion of deferred revenue | <u>2,313</u> | <u>813</u> |
| Total current liabilities | 7,864 | 5,471 |
| Deferred revenue, less current portion | 4,740 | 6,646 |
| Other liabilities | <u>485</u> | <u>306</u> |
| Total liabilities | 13,089 | 12,423 |
| Stockholders' equity | <u>270,626</u> | <u>149,483</u> |
| Total liabilities and stockholders' equity | <u>\$ 283,715</u> | <u>\$ 161,906</u> |

AVALANCHE BIOTECHNOLOGIES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited)

(In thousands, except per share amounts)

| | |
|--|--|
| <u>Three Months Ended June</u> <u>30,</u> | <u>Six Months Ended June</u> <u>30,</u> |
|--|--|

| | 2015 | 2014 | 2015 | 2014 |
|--|------------|------------|-------------|------------|
| Collaboration and license revenue | \$ 203 | \$ 135 | \$ 406 | \$ 165 |
| Operating expenses: | | | | |
| Research and development | 5,126 | 3,094 | 10,747 | 4,004 |
| General and administrative | 4,959 | 1,494 | 9,102 | 2,220 |
| Total operating expenses | 10,085 | 4,588 | 19,849 | 6,224 |
| Operating loss | (9,882) | (4,453) | (19,443) | (6,059) |
| Other income (expense), net | 116 | (641) | 168 | (698) |
| Net loss | (9,766) | (5,094) | (19,275) | (6,757) |
| Deemed dividend | -- | (3,230) | -- | (3,230) |
| Net loss attributable to common stockholders | \$ (9,766) | \$ (8,324) | \$ (19,275) | \$ (9,987) |
| Net loss per share attributable to common stockholders, basic and diluted | \$ (0.38) | \$ (2.27) | \$ (0.76) | \$ (2.72) |
| Weighted-average common shares outstanding, outstanding, basic and diluted | 25,555 | 3,673 | 25,223 | 3,673 |

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