



Avalanche Biotechnologies and the University of Washington Enter Into Exclusive License Agreement to Develop Gene Therapy Medicines to Treat Color Blindness

March 25, 2015

Vision Experts Jay and Maureen Neitz Join Company's Scientific Advisory Board

Company Launches www.colorvisionawareness.com to Provide More Information for People with Color Blindness

MENLO PARK, Calif. and SEATTLE, March 25, 2015 (GLOBE NEWSWIRE) -- Avalanche Biotechnologies (Nasdaq:AAVL) today announced that it has entered into an exclusive license agreement with the University of Washington (UW) in Seattle to develop products based on Avalanche's proprietary Ocular BioFactory™ Platform for the treatment of color vision deficiency (CVD), commonly known as red-green color blindness.

Avalanche also announced today, outside of the scope of the license agreement, that Drs. Jay and Maureen Neitz, faculty in the UW's Department of Ophthalmology and CVD experts, have joined its Scientific Advisory Board. They will be technical advisors to the company on the science of vision.

"This agreement with the University of Washington and world-renowned vision scientists Drs. Jay and Maureen Neitz will help us further advance our goal of developing therapeutic products for the millions of people who suffer from CVD," said Thomas W. Chalberg, Jr., Ph.D., founder and chief executive officer of Avalanche Biotechnologies. "Our proprietary technology enables us to target the retina through intravitreal adeno-associated virus delivery, presenting, for the first time, the opportunity to pursue previously untreatable ophthalmic conditions such as CVD."

Avalanche will build on gene therapy research conducted by the Neitz research team at the UW. They used gene therapy to confer color vision in two adult male squirrel monkeys that had been colorblind since birth.¹ This groundbreaking work demonstrating proof-of-concept for treating CVD was published in the journal *Nature*.

"Avalanche is performing pioneering research that has the potential to cure CVD," said Jay Neitz, Ph.D., professor of ophthalmology at the UW. "CVD is just one of the many cone diseases that could be treated with Avalanche's technology."

In addition, today Avalanche launched www.colorvisionawareness.com, a disease awareness website designed to help people suffering from color blindness better understand their condition. The website provides the opportunity for people to sign up to be among the first to receive information about potential research study opportunities or therapies for color blindness that become available.

About CVD and Avalanche's Targeted Development Program

Color vision deficiency (CVD), also known as red-green color blindness, is among the most common genetic diseases. CVD affects approximately 8 percent of males and 0.5 percent of females, or more than 10 million people in the U.S. alone.² CVD is a visual impairment that impacts many aspects of everyday life, resulting in limitations in professional choices, compromised health and safety, and the inability to perform many activities of daily living.³

Photopigments in the retina are crucial for perceiving color. People with normal color perception have three different types of photopigments. These photopigments are tuned to perceive either long wavelengths (red), middle wavelengths (green) or short wavelengths (blue), referred to as L-, M- and S-opsin. The most common forms of CVD are due to genetic defects that lead to missing either the L-opsin (protan defects) or the M-opsin (deutan defects). Affected individuals have trouble distinguishing between red and green and between colors that contain red or green hues.

Avalanche has two drug candidates targeting these areas. AVA-322 carries the gene for L-opsin and is being developed for the treatment of protan defects. AVA-323 carries the gene for M-opsin and is being developed for the treatment of deutan defects.

About Avalanche Biotechnologies, Inc.

Avalanche is a biopharmaceutical company committed to improving or preserving the sight of people suffering from blinding eye diseases with a clear unmet medical need. Avalanche's proprietary Ocular BioFactory™ 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, 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. Statements regarding the projected size of the CVD patient population are also forward-looking statements. 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 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 Commission.

¹ Mancuso, K. et al. Gene therapy for red–green colour blindness in adult primates. *Nature*. 2009; 461:784-787.

² Sharpe LT, et al. Opsin genes, photopigments, color vision and color blindness. Gegenfurtner KR, Sharpe LT (eds.) *Color Vision*. Cambridge UP: Cambridge, 1999.

³ Cole, B. The handicap of abnormal colour vision. *Clin Exp Optom*. 2004; 87: 4-5:258–275.

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