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Regeneron and Avalanche Biotechnologies Announce Collaboration to Develop Next-Generation Gene Therapy Products in Ophthalmology

TARRYTOWN, N.Y. and MENLO PARK, Calif., May 5, 2014 /PRNewswire/ -- Regeneron Pharmaceuticals, Inc. (NASDAQ: **REGN**) and Avalanche Biotechnologies, Inc., today announced the formation of a broad collaboration to discover, develop and commercialize novel gene therapy products for the treatment of ophthalmologic diseases. The collaboration covers novel gene therapy vectors and proprietary molecules, discovered jointly by Avalanche and Regeneron, and developed using the Avalanche Ocular BioFactory™, an adeno-associated virus (AAV)-based, proprietary, next-generation platform for the discovery and development of gene therapy vectors for ophthalmology.

Under the terms of the agreement, Avalanche will receive an upfront cash payment, contingent payments of up to $640 million upon achievement of certain development and regulatory milestones, plus a royalty on worldwide net sales of collaboration products. The collaboration covers up to eight distinct therapeutic targets, and Regeneron will have exclusive worldwide rights for each product it moves forward in clinical development. In addition, Avalanche has the option to share in development costs and profits for products directed toward two collaboration therapeutic targets selected by Avalanche.

As part of the agreement, Regeneron has a time-limited right of first negotiation for certain rights to AVA-101, Avalanche's gene therapy product targeting vascular endothelial growth factor (VEGF) currently under development for the treatment of wet age-related macular degeneration (AMD), upon completion of the ongoing Phase 2a trial.

"We look forward to the opportunity to collaborate with Avalanche, a leader in the field of next-generation gene therapy technologies," said George D. Yancopoulos, M.D., Ph.D., Chief Scientific Officer of Regeneron and President of Regeneron Laboratories. "This collaboration highlights the commitment by Regeneron to invest in potentially breakthrough therapies that could benefit patients with sight-threatening diseases."

"We are excited to work with Regeneron to discover and develop novel gene therapy medicines for serious eye diseases," said Thomas W. Chalberg, Ph.D., co-founder and Chief Executive Officer of Avalanche Biotechnologies. "The collaboration will bring together Avalanche's novel platform technology with Regeneron's proprietary molecules and research capabilities, with the goal of creating a new class of next-generation biologics in ophthalmology. Regeneron is a terrific partner for their scientific leadership, as well as their product development capabilities and commercialization track-record."

About Regeneron Pharmaceuticals
Regeneron is a leading science-based biopharmaceutical company based in Tarrytown, New York that discovers, invents, develops, manufactures, and commercializes medicines for the treatment of serious medical conditions. Regeneron commercializes medicines for eye diseases, colorectal cancer, and a rare inflammatory condition, and has product candidates in development in other areas of high unmet medical need, including hypercholesterolemia, oncology, rheumatoid arthritis, asthma, and atopic dermatitis. For additional information about the company, please visit www.regeneron.com.

About Avalanche Biotechnologies, Inc.
Founded in 2006, Avalanche Biotechnologies, Inc. is a privately held, clinical-stage biotechnology company that develops technologies and products for sustained delivery of therapeutic proteins. Avalanche's lead product, AVA-101, is currently under development in a Phase 2a trial for wet age-related macular degeneration. Avalanche's Ocular BioFactory™ 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. The company is headquartered in Menlo Park, California. For more information, please visit www.avalanchebiotech.com.

Regeneron Forward-Looking Statements
This news release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron, and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of Regeneron's products, product candidates, and research and clinical programs now underway or planned including without limitation the planned collaborative programs with Avalanche Biotechnologies, Regeneron's translational research and functional biology capabilities, and the planned expansion in the use of human genetics in Regeneron's research process; unforeseen safety issues resulting from the administration of products and product candidates in patients, including any next-generation gene therapy product candidates; the likelihood and timing of possible regulatory approval and commercial launch of Regeneron's product candidates; ongoing regulatory obligations and

oversight impacting Regeneron's research and clinical programs and business, including those relating to any next-generation gene therapy product candidates; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's products and product candidates including any next-generation gene therapy product candidates; competing drugs and product candidates that may be superior to Regeneron's products and product candidates; uncertainty of market acceptance and commercial success of Regeneron's products and product candidates; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; coverage and reimbursement determinations by third-party payers, including Medicare and Medicaid; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its sales or other financial projections or guidance and changes to the assumptions underlying those projections or guidance; the potential for any license or collaboration agreement, including Regeneron's agreements with Sanofi and Bayer HealthCare, to be cancelled or terminated without any further product success; and risks associated with third party intellectual property and pending or future litigation relating thereto. A more complete description of these and other material risks can be found in Regeneron's filings with the United States Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2013. The reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update publicly any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

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