



Ophthotech Enters into Gene Therapy Agreements with the University of Florida and the University of Pennsylvania

June 7, 2018

- Expands Gene Therapy Portfolio with a Novel Mutation Independent Product Candidate for Autosomal Dominant Retinitis Pigmentosa -

- Phase 1/2 Clinical Trial Expected to Initiate in Early 2020 -

NEW YORK--(BUSINESS WIRE)--Jun. 7, 2018-- Ophthotech Corporation (NASDAQ: OPHT) announced today that it has entered into an exclusive global license agreement with the University of Florida Research Foundation and the University of Pennsylvania (Penn) to develop and commercialize a novel adeno-associated virus (AAV) gene therapy product for the treatment of rhodopsin-mediated autosomal dominant retinitis pigmentosa (RHO-adRP), an orphan monogenic disease that is characterized by progressive and severe loss of vision leading to blindness. Preclinical anatomical and functional proof-of-concept studies have demonstrated promising results in a canine disease model. In addition to the exclusive license agreement, Ophthotech and Penn have also entered into a master sponsored research agreement, facilitated by the Penn Center for Innovation (PCI), pursuant to which Ophthotech and Penn plan to conduct preclinical and natural history studies. In parallel with the sponsored research, Ophthotech plans to commence IND-enabling activities. Based on current timelines, Ophthotech expects to initiate a Phase 1/2 clinical trial in early 2020.

"The scientific elegance of this novel gene therapy product is its design to knock down the expression of the mutant rhodopsin while delivering the replacement functional rhodopsin with a single AAV vector, restoring normal protein expression in preclinical studies" stated Kourous A. Rezaei, M.D., Chief Medical Officer of Ophthotech. "Collaborating with the eminent scientists at the University of Florida and the University of Pennsylvania reinforces Ophthotech's commitment to build a gene therapy pipeline for treatment of retinal diseases based on cutting edge technology."

As part of this collaboration, Ophthotech will work in partnership with internationally renowned scientists in gene therapy for orphan retinal diseases.

- Investigators at the University of Florida include Professor Alfred S. Lewin, PhD, Program Director, Molecular Genetics and Microbiology and Professor William W. Hauswirth, PhD, Maida and Morris Rybaczki Eminent Scholar Chair in Ophthalmic Sciences.
- Investigators at the University of Pennsylvania include Professor William A. Beltran, DVM, PhD, Director of the Division of Experimental Retinal Therapies, Department of Clinical Sciences and Advanced Medicine, School of Veterinary Medicine, and Professor Gustavo D. Aguirre, School of Veterinary Medicine, and from Penn's Perelman School of Medicine and Scheie Eye Institute, Professor Samuel G. Jacobson, MD, PhD, Director of the Center for Hereditary Retinal Degenerations, and Professor Artur V. Cideciyan, PhD.

"The results of our gene therapy experiments in the canine model of RHO-adRP whose disease closely mimics that in humans have been encouraging," stated Professor Beltran.

"Orphan retinal degenerative diseases are potentially devastating to patients, who do not have any treatment options available to them," stated Professor Lewin. "Gene therapy presents a compelling scientific approach for treating and potentially curing a wide range of retinal diseases."

"This agreement highlights another step in our deep commitment to build a promising gene therapy portfolio through collaborations with leading academic institutions and innovative biotech companies to create value for our shareholders," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "We continue to focus our efforts on developing treatment options for patients with retinal diseases."

It is estimated that there are approximately 11,000 RHO-adRP individuals in the United States and the five major European markets. There is currently no U.S. FDA or European Medicines Agency approved therapy to treat this orphan inherited retinal disease.

About Ophthotech Corporation

Ophthotech is a science-driven biopharmaceutical company specializing in the development of novel therapies to treat ophthalmic diseases, with a focus on age-related and orphan retinal diseases. For more information, please visit www.ophthotech.com.

Forward-looking Statements

Any statements in this press release about Ophthotech's future expectations, plans and prospects constitute forward-looking statements for purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995. Forward-looking statements include any statements about Ophthotech's strategy, future operations and future expectations and plans and prospects for Ophthotech, and any other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "goal," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. In this press release, Ophthotech's forward-looking statements include statements about the implementation of its strategic plan, the timing, progress and results of clinical trials and other research and development activities, and the potential for its business development strategy, including any potential in-license or acquisition opportunities. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech's 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, those related to the initiation and the conduct and design of research programs and clinical trials, availability of data from these programs, expectations for manufacturing activities and regulatory matters, need for additional financing and negotiation and consummation of in-license and/or acquisition transactions and other factors discussed in the "Risk Factors" section contained in the quarterly and annual reports that Ophthotech files with the Securities and Exchange Commission. Any forward-looking statements represent Ophthotech's views only as of the date of this press release. Ophthotech anticipates that subsequent events and developments will cause its views to change. While Ophthotech may elect to update these forward-looking statements at some point in the future, Ophthotech specifically disclaims any obligation to do so except as required by law.

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