Scientific Data for Novel Adeno-Associated Virus Gene Therapy in Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa Published in Proceedings of the National Academy of Sciences

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- Preclinical Proof-of-Concept Results in a Large Animal Model Published Online Ahead of Print in PNAS -

NEW YORK--(BUSINESS WIRE)--Aug. 22, 2018-- Ophthotech Corporation (NASDAQ: OPHT) announced today that scientists at the University of Pennsylvania (Penn) and University of Florida published proof-of-concept study results on an adeno-associated virus (AAV) gene therapy product candidate for the treatment of rhodopsin-mediated autosomal dominant retinitis pigmentosa (RHO-adRP) licensed by Ophthotech. Their work conducted in a naturally occurring canine model of the disease was published online ahead of print in the journal *Proceedings of the National Academy of Sciences of the USA*(PNAS). In June 2018, Ophthotech announced that it entered into an exclusive global license agreement with the University of Florida Research Foundation and Penn for rights to develop and commercialize this novel AAV gene therapy product candidate for the treatment of RHO-adRP. 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, pursuant to which Ophthotech and Penn plan to conduct additional preclinical studies of the RHO-adRP product candidate and a natural history study in RHO-adRP patients.

This publication is entitled: “Mutation-independent Rhodopsin Gene Therapy by Knockdown and Replacement with a Single AAV vector” by Artur V. Cideciyan, Raghavi Sudharsan, Valérie L. Dufour, Michael T. Massengill, Simone Iwabe, Malgorzata Swider, Brianna Lisi, Alexander Sumaroka, Luis Felipe Marinho, Tatyana Appelbaum, Brian Rossmiller, William W. Hauswirth, Samuel G. Jacobson, Alfred S. Lewin, Gustavo D. Aguirre, and William A. Beltran. *PNAS* 2018.

RHO-adRP is an orphan monogenic inherited retinal disease that is characterized by progressive and severe loss of vision, and is caused by more than 150 different mutations in the *RHO* gene. The construct for the RHO-adRP product candidate combines a transgene expressing a highly efficient, novel short hairpin RNA (shRNA) designed to target and knock-down endogenous rhodopsin (*RHO*) in a mutation-independent manner with a human *RHO* replacement transgene made resistant to RNA interference, in a single AAV2/5 vector. This construct was tested in a naturally-occurring canine disease model of RHO-adRP by investigators at Penn, resulting in a complete suppression of the endogenous *RHO* RNA while the human *RHO* replacement transgene resulted in up to 30% of normal RHO protein levels. Long term (over 8 months) anatomic and functional preservation was demonstrated with retinal imaging and electrophysiology. Ophthotech believes these results further confirm the therapeutic benefit of a similar knock-down and replacement approach that was tested in mice by investigators at the University of Florida, the results of which were previously published in *Human Gene Therapy* in 2012.

“Our prior research indicated that the canine animal model may provide useful predictive data when developing treatment strategies for patients with degenerative retinal diseases,” stated Professor William A. Beltran, DVM, PhD, Director of the Division of Experimental Retinal Therapies, Department of Clinical Sciences & Advanced Medicine, School of Veterinary Medicine, University of Pennsylvania. “We are honored to have our preclinical research in AAV therapy for RHO-adRP published in *PNAS*.”

“We are excited to collaborate with prominent scientists in the field of degenerative retinal diseases at the University of Pennsylvania and University of Florida and congratulate them on the strength and elegance of this publication,” stated Kourous A. Rezaei, M.D., Chief Medical Officer of Ophthotech. “We are intrigued by these proof-of-concept results in the canine disease model which further support the potential therapeutic impact of this AAV gene therapy product in patients with RHO-adRP. We are commencing IND-enabling activities, and based on current timelines and subject to regulatory review, we expect to initiate a Phase 1/2 clinical trial for the treatment of RHO-adRP in 2020.”

About RHO-adRP

RHO-adRP is an orphan monogenic inherited retinal disease that is characterized by progressive and severe loss of vision. Ophthotech estimates that there are approximately 11,000 individuals in the United States and the five major European markets with RHO-adRP. There is currently no U.S. Food and Drug Administration 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 utility of its product candidates. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech’s preclinical and 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 regulatory matters, need for additional financing 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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