Ophthotech Expands Gene Therapy Pipeline in Orphan Retinal Diseases with Exclusive Option for Novel Product Candidates to Treat Best Vitelliform Macular Dystrophy (Best Disease)

October 31, 2018

- Ophthotech Enters into Second Series of Gene Therapy Agreements with the University of Pennsylvania and the University of Florida for Exclusive Option for Novel Product Candidates to Treat Best Disease -

NEW YORK--(BUSINESS WIRE)--Oct. 31, 2018-- Ophthotech Corporation (NASDAQ:OPHT) announced today that it has entered into an exclusive option agreement with the University of Pennsylvania (Penn) and the University of Florida Research Foundation (UFRF) for rights to negotiate to acquire an exclusive global license to develop and commercialize novel adeno-associated virus (AAV) gene therapy product candidates for the treatment of Best vitelliform macular dystrophy, also known as Best disease. Best disease, which generally affects individuals in both eyes, is an orphan inherited degenerative retinal disease caused by mutations in the *BEST1* gene. Preclinical anatomical proof-of-concept studies conducted by Penn and the University of Florida demonstrated promising results in a canine Best disease model. In addition to the exclusive option agreement, Ophthotech will sponsor research at Penn, facilitated by the Penn Center for Innovation (PCI), and the University of Florida to conduct preclinical and natural history studies of Best disease. Ophthotech plans to commence IND-enabling activities and based on current timelines expects to submit an Investigational New Drug Application (IND) to the U.S. Food and Drug Administration (FDA) by 2021.

“We are excited to strengthen our relationship with the team of distinguished scientists at the University of Pennsylvania and the University of Florida, to expand our gene therapy pipeline by adding rights to obtain the *BEST1* gene therapy product candidates and to build upon our strategy to develop novel treatment options for patients with devastating retinal diseases,” stated Kourous A. Rezaei, M.D., Chief Medical Officer of Ophthotech.

In a scientific publication in the journal *Proceedings of the National Academy of Sciences of the USA*(PNAS), Karina E. Guziewicz, Ph.D., Research Assistant Professor of Ophthalmology, University of Pennsylvania School of Veterinary Medicine, and colleagues presented proof-of-concept studies revealing that a *BEST1* gene therapy product candidate could reverse the vitelliform lesions in the preclinical canine disease model with distinct phenotypic similarities to human Best disease. This publication is entitled: “*BEST1* gene therapy corrects a diffuse retina-wide microdetachment modulated by light exposure” by Karina E. Guziewicz, Artur V. Cideciyan, William A. Beltran, András M. Komáromy, Valerie L. Dufour, Malgorzata Swider, Simone Iwabe, Alexander Sumaroka, Brian T. Kendrick, Gordon Ruthel, Vince A. Chiodo, Elise Héon, William W. Hauswirth, Samuel G. Jacobson, and Gustavo D. Aguirre. *PNAS*February 2018.

“We have developed a preclinical canine disease model with distinct phenotypic similarities to human Best disease. Our AAV based gene therapy achieved a clear and durable reversal of the retinal pathology, including vitelliform lesions and microdetachments, in our canine model,” stated Karina E. Guziewicz, Ph.D., Research Assistant Professor of Ophthalmology, University of Pennsylvania School of Veterinary Medicine.

Ophthotech estimates that approximately 10,000 individuals in the United States and the five major European markets have Best disease. Patients with Best disease develop an egg yolk-like vitelliform lesion in their macular region, which over time leads to macular atrophy and permanent loss of central vision. There is currently no FDA or European Medicines Agency approved therapy to treat this orphan inherited retinal disease.

“Entering into our third gene therapy collaboration with highly accomplished scientific leaders in the field of gene therapy and degenerative retinal diseases reinforces Ophthotech’s commitment to build a gene therapy portfolio in retinal diseases and create value for our shareholders,” stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech.

In June 2018, Ophthotech announced that it had entered into an exclusive global license agreement with UFRF and Penn for rights to develop and commercialize a novel AAV gene therapy product candidate 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. Based on current timelines and subject to regulatory review, Ophthotech expects to initiate a RHO-adRP Phase 1/2 clinical trial in 2020.

In February 2018, Ophthotech announced that it had entered into a collaboration with the University of Massachusetts Medical School to investigate novel gene therapies for retinal diseases utilizing a “minigene” therapy approach as well as novel gene delivery methods.

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 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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Source: Ophthotech Corporation

Investors
Kathy Galante
Ophthotech Corporation
Vice President, Investor Relations and Corporate Communications
212-845-8231
kathy.galante@ophthotech.com
or
Media
Alex Van Rees, 973-442-1555 ext. 111
SmithSolve LLC on behalf of Ophthotech Corporation
alex.vanrees@smithsolve.com