Ophthotech Obtains Exclusive Global License to AAV Gene Therapy Program for BEST1 Related Retinal Diseases

April 11, 2019

NEW YORK--(BUSINESS WIRE)--Apr. 11, 2019-- Ophthotech Corporation (NASDAQ: OPHT) announced today that the Company has converted its option and entered into an exclusive global license agreement with the University of Pennsylvania (Penn), including the Perelman School of Medicine at the University of Pennsylvania and the University of Pennsylvania School of Veterinary Medicine, and the University of Florida Research Foundation (UFRF) for rights 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 and other bestrophinopathies, which are diseases related to mutations to the *BEST1* gene.

Best disease, which generally affects individuals in both eyes, is an orphan inherited retinal disease. In October 2018, Ophthotech entered into an exclusive option agreement with Penn and UFRF for rights to negotiate to acquire a license for novel AAV serotype 2 based gene therapy product candidates for the treatment of Best disease. Since that time, Ophthotech has sponsored research at Penn, facilitated by the Penn Center for Innovation (PCI), to conduct preclinical and natural history studies for Best disease and commenced IND-enabling activities. Based on current timelines and subject to regulatory review, the Company expects to initiate a Phase 1/2 clinical trial in the first half of 2021.

Ophthotech estimates that approximately 10,000 individuals in the United States and the five major European markets on a combined basis 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 EMA approved therapy to treat this orphan inherited degenerative retinal disease.

Proof-of-concept studies demonstrating that the AAV-*BEST1* gene therapy product candidate restored the anatomy between photoreceptors and retinal pigment epithelial (RPE) cells in the naturally occurring canine disease model with distinct phenotypic similarities to human bestrophinopathies were published in March 2018 in the journal *Proceedings of the National Academy of Sciences*(PNAS), titled: "*BEST1* gene therapy corrects a diffuse retina-wide microdetachment modulated by light exposure."

About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of transformative gene therapies and novel therapeutics to treat retinal diseases, with a focus on orphan and age related indications. 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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