July 26, 2017

Ophthotech Expands Focus with Development for Ophthalmic Orphan Diseases

(Conference Call Scheduled for today, July 26, 2017 at 8:00 a.m. ET)

- Stargardt Disease Clinical Trial Planned to Start Before the End of this Year -

- Focus on Multiple Orphan Programs in Retinal Diseases and Continue Age-related Retinal Programs -

- Business Development Efforts Ongoing -

NEW YORK--(BUSINESS WIRE)-- Ophthotech Corporation (Nasdaq:OPHT) today announced that the Company is pursuing a strategy to leverage its clinical experience and retina expertise to identify and develop therapies to treat multiple orphan ophthalmic diseases for which there are limited or no treatment options available. In parallel, the Company continues its ongoing age-related retinal programs and its business development efforts to obtain rights to additional products, product candidates and technologies to treat ophthalmic diseases, particularly those of the back of the eye. Call-in and webcast information is provided below for a discussion of the Company's financial and operating results and a business update.

- Ophthotech's orphan ophthalmic disease strategy will be led by a randomized, controlled clinical trial assessing the efficacy and safety of Zimura® (avacincaptad pegol), the Company's C5 complement inhibitor, for Stargardt disease, a devastating inherited retinal orphan disease causing vision loss during childhood or adolescence for which patients have no approved treatment. This trial is scheduled to start by the end of this year.
- The Company is continuing its programs in age-related eye diseases, including the planned initiation of a Phase 2a clinical trial of Zimura® in combination with anti-VEGF therapy for wet age-related macular degeneration (AMD) and a Phase 2a clinical trial of Zimura® in combination with anti-VEGF therapy for idiopathic polypoidal choroidal vasculopathy. Both of these trials are scheduled to start by the end of this year.
- The Company's Phase 2/3 clinical trial of Zimura® as a monotherapy for the treatment of geographic atrophy, a form of dry AMD, is ongoing. The Company has maintained a limited number of trial sites for this study and will re-assess its strategy for this study following results of a competitor's complement trial for geographic atrophy, which are expected by year end.
- The National Eye Institute is leading a Phase 1/2 clinical trial of the Company's drug candidate, Fovista® (pegpleranib) in combination with anti-VEGF therapy for the treatment of retinal manifestations of the orphan disease Von Hippel-Lindau Syndrome.
- Ophthotech is also planning a Phase 2a clinical trial of Zimura® for intermediate/posterior non-infectious uveitis, a rare inflammatory disease of the back of the eye, and a potential pre-clinical program with Fovista® for retinoblastoma, a rare cancer of the eye in children. These studies are planned to start in 2018.

"We are excited to move the Company forward with a goal of becoming a leader in the development and commercialization of ophthalmic therapeutics for orphan diseases and for larger indications in the back of the eye, such as age-related retinal diseases," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "We believe that we will be well positioned as a company with multiple shots on goal to bring ophthalmic therapeutics to market. We are also continuing our business development efforts with the goal of broadening and advancing our pipeline. We are committed to developing treatments for patients with devastating ophthalmic diseases and to maximizing value for our shareholders."

Supporting the Company's strategy for the development of Zimura® in Stargardt disease is a recently published independent, peer-reviewed paper in the prestigious journal of *Proceedings of National Academy of Science* (PNAS) from a world-class laboratory at the University of California, Los Angeles (UCLA) that highlights the potential role of complement inhibition in addressing the urgent unmet medical need in Stargardt disease. Additionally, independent literature also supports the scientific evidence for the potential role of complement and specifically the membrane attack complex (MAC) in this disease. The clinical safety data for Zimura® from the Company's completed early stage age-related macular degeneration trials provide a basis to proceed directly to a randomized, controlled clinical trial to assess the safety and efficacy of Zimura® in Stargardt disease.

The Company also announced that it has entered into an agreement with the Foundation Fighting Blindness (FFB). FFB is a highly-distinguished organization recognized for its scientific commitment to orphan inherited retinal degenerative diseases with an established network of scientists and a robust patient registry. Ophthotech has engaged FFB to provide the Company with information from its publicly available ProgStar study, the largest natural history study on Stargardt disease to date, which Ophthotech plans to use in the design of its planned clinical trial of Zimura® for Stargardt disease, and to potentially assist with the Company's other orphan degenerative retinal programs.

"We commend Ophthotech for recognizing the underserved patients afflicted with Stargardt disease for whom currently there is no available FDA approved treatment option," stated Patricia Zilliox, Ph.D., FFB's Clinical Research Institute Chief Drug Development Officer. "We are delighted and honored to team up with Ophthotech thereby complementing their expertise in ophthalmic drug development with our experience in studying Stargardt disease."

"We are fortunate to have the opportunity to work closely with the Foundation Fighting Blindness," stated Kourous A. Rezaei, M.D., Senior Vice President of Medical Strategy. "We also intend to work closely with the FDA over the next few months to discuss the regulatory pathway for our Zimura® Stargardt program."

The Company also announced changes to its wet AMD program for Zimura®. The Company believes that supplementing anti-VEGF therapy with an anti-complement such as Zimura® in wet AMD may have the potential to further enhance the efficacy of anti-VEGF monotherapy and decrease unwanted side effects in wet AMD from anti-VEGF drugs. A recent peer reviewed publication from the *Journal of Clinical Investigation* from the prestigious Scripps Research Institute citing the role of anti-VEGF therapy in complement activation supports this thesis. Due to a new study design and updated enrollment criteria, the Company will cease enrollment in its current Phase 2a clinical trial of Zimura® in wet AMD, and initiate a new Phase 2a clinical trial to assess whether it can replicate findings from its previous Phase 1/2a clinical trial. The Company will be assessing a range of dosing regimens before committing to a larger and more costly trial. This trial is scheduled to initiate before the end of the year.

"The opportunities to develop orphan drugs for ophthalmic diseases along with some intriguing new developments regarding the role of complement in anti-VEGF therapy allow us to focus our resources and efforts on science-driven solutions in addressing the unmet need in ophthalmic diseases," stated Mr. Sblendorio. "In addition, we have reviewed a large number of assets and technology platforms over the past few months and are actively continuing to review, in a prudent manner, assets or technology platforms which would fit into our strategic goals in addition to other compelling ophthalmology opportunities."

Conference Call/Web Cast Information

Ophthotech will host a conference call/webcast to discuss the Company's financial and operating results and provide a business update. The call is scheduled for July 26, 2017 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-280-4443 (USA) or 719-457-2603 (International), passcode 8248330. A live, listen-only audio webcast of the conference call can be accessed on the Investor Relations section of the Ophthotech website at: www.ophthotech.com. A replay will be available approximately two hours following the live call for two weeks. The replay number is 888-203-1112 (USA Toll Free), passcode 8248330. A supplemental slide presentation is available in the "Investor" section of the Ophthotech website prior to the start of the call / webcast.

About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics for diseases of the eye. 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, Ophthotech's projected use of cash and cash balances, the timing, progress and results of clinical trials and other development activities, the potential utility or commercialization of any of Ophthotech's product candidates and 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 conduct of clinical trials, availability of data from clinical trials, expectations for regulatory matters and negotiation and consummation of in-license and/or acquisition transactions, 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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