Ophthotech Announces Results from Phase 2a Safety Trial of Zimura® in Combination with Lucentis® in Wet Age-Related Macular Degeneration

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NEW YORK--(BUSINESS WIRE)--Nov. 12, 2018-- Ophthotech Corporation (NASDAQ:OPHT) today announced the results from its Phase 2a safety trial of Zimura® (avacincaptad pegol), the Company's complement factor C5 inhibitor, in patients with wet age-related macular degeneration (AMD). This trial was designed to evaluate the safety of different dosage regimens of Zimura combination therapy in wet AMD and was the first in human trial assessing the safety of Zimura 4mg dose in combination with Lucentis® (ranibizumab) 0.5mg, an anti-vascular endothelial growth factor (anti-VEGF). Various dosing regimens of Zimura were administered in combination with Lucentis in patients with wet AMD who have not been previously treated with anti-VEGF drugs.

Based on a preliminary analysis of the safety data from this trial, Zimura combination therapy was generally well tolerated after six months of treatment. The most frequently reported ocular adverse events were related to the injection procedure. No adverse events were attributed to Zimura combination therapy.

"The current Phase 2a trial further reinforced the previously observed safety profile of intravitreal Zimura administered in combination with Lucentis 0.5mg in patients with wet AMD who have not had any previous anti-VEGF treatment," stated Kourous A. Rezaei, M.D., Chief Medical Officer of Ophthotech. "This uncontrolled clinical trial with a small sample size was not designed to detect a statistically significant difference between Zimura dose groups or to evaluate the efficacy of Zimura combination therapy with statistical significance. Interestingly, similar to the corresponding group of patients in our previously completed Phase 1/2a trial, 60% of patients who had received monthly Zimura 2mg in combination with Lucentis 0.5mg gained greater than or equal to three lines of vision, or 15 Early Treatment of Diabetic Retinopathy Study (ETDRS) letters, defined as significant visual gain. Although, these findings may be intriguing, based on the totality of the data, at this time we will focus our resources on the efficient execution of our other ongoing Zimura clinical programs in geographic atrophy secondary to dry AMD and autosomal recessive Stargardt disease, our gene therapy programs in rhodopsin mediated autosomal dominant retinitis pigmentosa and Best disease, our minigene programs in LCA 10 and autosomal recessive Stargardt disease and our recently acquired HtrA1 inhibitor program in dry AMD."

Phase 2a Clinical Trial Key Design Features and Findings (safety population):

Sixty-four patients were enrolled and treated in this randomized, dose-ranging, open-label, multi-center Phase 2a clinical trial designed to assess the safety of various Zimura dosing regimens in combination with anti-VEGF therapy at month 6 for the treatment of wet AMD. The trial enrolled treatment-naïve patients and was designed to allow an assessment of available safety measures, including visual acuity. Patients were enrolled in four groups:

- In Group 1, 10 patients were administered monthly combination therapy consisting of Lucentis 0.5mg followed by Zimura 4mg two days later. In this group, the mean change in visual acuity from baseline at month 6 was 9.0 ETDRS letters with a median of 7.0 letters. 40% of patients gained greater than or equal to three lines of vision, or 15 ETDRS letters, defined as significant visual gain.
- In Group 2, 10 patients were administered monthly combination therapy consisting of Lucentis 0.5mg and Zimura 2mg on the same day, which was the same dosing regimen as the best-performing group from the previously completed Phase 1/2a clinical trial. In this group, the mean change in visual acuity from baseline at month 6 was 10.2 ETDRS letters with a median of 16.0 letters. 60% of patients gained greater than or equal to three lines of vision, or 15 ETDRS letters, defined as significant visual gain.
- In Group 3, during the induction phase (Day 1 – Month 2), 22 patients were administered Lucentis 0.5mg followed by Zimura 2mg on the same day followed by Zimura 2mg fourteen days later. During a subsequent maintenance phase (Month 3 – Month 5), patients were administered Lucentis 0.5mg followed by Zimura 2mg on the same day. In this group, the mean change in visual acuity from baseline at month 6 was 10.7 ETDRS letters with a median of 10.0 letters. 40.9% of patients gained greater than or equal to three lines of vision, or 15 ETDRS letters, defined as significant visual gain.
- In Group 4, during the induction phase (Day 1 – Month 2) 22 patients were administered Lucentis 0.5mg followed by Zimura 2mg on the same day followed by Zimura 2mg fourteen days later. During a subsequent maintenance phase (Month 3 – Month 5) patients were administered Zimura 2mg followed two days later by Lucentis 0.5mg and Zimura 2mg. In this group, the mean change in visual acuity from baseline at month 6 was 9.9 ETDRS letters with a median of 11.0 letters. 18.2% of patients gained greater than or equal to three lines of vision, or 15 ETDRS letters, defined as significant visual gain.

Previously, Ophthotech completed a multicenter, ascending dose and parallel group, open-label, first in human Phase 1/2a clinical trial to evaluate the safety, tolerability and pharmacokinetic profile of Zimura given in combination with Lucentis 0.5mg in patients with wet AMD. This trial included a group of patients that received the same dosing and schedule regimen as in Group 2 in the current Phase 2a trial. In the treatment-naïve patients who had received all six monthly Zimura 2mg injections in combination with Lucentis 0.5mg (n=15) in the Phase 1/2a trial, the mean change in visual acuity from baseline at month 6 was 15.3 ETDRS letters with a median of 16.0 letters and 60% of patients had gained greater than or equal to three lines of vision, or 15 ETDRS letters, defined as significant visual gain. Although one needs to be cautious in making comparisons, these results in this earlier Phase 1/2a clinical trial were better than what is generally observed with anti-VEGF monotherapy. All doses in this earlier trial were well tolerated with no adverse events considered to be related to the study drug.

“Our goal is to be a leader in drug development for retinal diseases and create value for our shareholders,” stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. “We look forward to continue advancing and expanding our deep portfolio of novel therapeutics and gene therapy programs in age-related and orphan retinal diseases.”

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 its future development plans for Zimura and its other programs and product candidates, projected use of its cash resources and future cash balances, the timing, progress and results of clinical trials and other research and development activities, the potential utility of its product candidates, its expectations with respect to the financial impacts and benefits to Ophthotech of the acquisition of Inception 4, and the potential for its business development strategy, including its collaborative gene therapy research programs and any potential in-license or acquisition opportunities. 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 and development 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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