November 8, 2017

Ophthotech Reports Third Quarter 2017 Financial and Operating Results

(Conference Call and Webcast Today, November 8, 2017, at 8:00 a.m. ET)

- Complement C5 Inhibitor, Zimura®, on Track to Have Four Ophthalmic Clinical Programs Ongoing by Year End -

NEW YORK--(BUSINESS WIRE)-- Ophthotech Corporation (Nasdaq: OPHT) today announced financial and operating results for the third quarter ended September 30, 2017 and provided a business update.

"We are very excited to provide an update on the expansion of our age related and orphan retina programs with our complement C5 inhibitor, Zimura," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "Scientific literature continues to strengthen our belief in the potential role of complement C5 inhibition in the treatment of retinal diseases. We have progressed in all of our clinical programs by initiating new trials and modifying a current clinical trial. We remain on track to have four trials ongoing by the end of the year."

Zimura® Complement Factor C5 Inhibitor Program

- ***Geographic Atrophy, a severe form of Dry Age-related Macular Degeneration***: Ophthotech has modified its on-going Zimura (avacincaptad pegol) clinical trial for the treatment of geographic atrophy (GA) secondary to dry age related macular degeneration (AMD). This on-going randomized, double-masked, sham controlled Phase 2b clinical trial is designed to assess the safety and efficacy of Zimura monotherapy in patients with GA. The Company has modified the design of the trial to accelerate the anticipated timeline for obtaining top-line data. The Company has reduced the number of patients it plans to enroll in this trial to approximately 200 and has shortened the time point for attaining the primary efficacy endpoint to 12 months. The number of sites has been expanded within the United States and globally to expedite enrollment. The primary efficacy endpoint is the mean rate of change in GA over 12 months. Patients will be treated and monitored for 18 months. The modified study design incorporates patients that were already enrolled in the study prior to these modifications. A range of Zimura dosing regimens will also be assessed. The Company submitted a modified Phase 2b clinical trial protocol to the U.S. Food and Drug Administration (FDA) early in the fourth quarter of 2017. Initial, top-line data is expected to be available during the second half of 2019.
- ***Wet Age-related Macular Degeneration***: During the third quarter of 2017, the Company initiated a new dose-ranging, open-label Phase 2a clinical trial of Zimura in combination with Lucentis® in patients with wet AMD who have not been previously treated with any anti-VEGF agents. Approximately 60 patients will be enrolled and treated for a duration of 6 months. Based on the anticipated enrollment rate, the Company expects initial top-line data from this trial to be available by the end of 2018.
- ***Idiopathic Polypoidal Choroidal Vasculopathy***: The Company plans to initiate before year end an open-label Phase 2a clinical trial evaluating Zimura in combination with Eylea® for the treatment of idiopathic polypoidal choroidal vasculopathy (IPCV) in treatment experienced patients. Approximately 20 patients will be enrolled and treated for a duration of 9 months. Initial top-line data is expected to be available during the second half of 2019.

Ophthalmic Orphan Disease Program

- ***Autosomal Recessive Stargardt Disease***: Ophthotech remains on track to initiate a Phase 2b randomized, double masked, sham controlled clinical trial in autosomal recessive Stargardt disease (STGD1) before the end of this year. This trial will assess the safety and efficacy of Zimura monotherapy in patients with STGD1, an inherited orphan retinal disease causing vision loss during childhood or adolescence. There are currently no FDA or EMA approved treatments available for STGD1 and it remains a significant unmet medical need. The Company expects to enroll approximately 120 patients in this trial, making it one of the largest interventional clinical trials in Stargardt disease to date. The Company plans to use an anatomic endpoint as measured by spectral domain optical coherence tomography (SD-OCT) as the primary endpoint, which will be assessed at 18 months. Initial top-line data is expected to be available in 2020.

Third Quarter 2017 Financial Highlights

- **Cash Position:** As of September 30, 2017, the Company had $180.2 million in cash and cash equivalents. The

Company expects a 2017 year end cash balance of between $155 million and $165 million, excluding any potential business development activities, and including the approximately $5 million to $7 million that remains committed to implementing a reduction in personnel and winding-down the Fovista® (pegpleranib) in combination with Eylea® or Avastin® clinical trial.

- **Revenues:** Collaboration revenue was $206.7 million for the quarter ended September 30, 2017, compared to $1.7 million for the same period in 2016. For the nine months ended September 30, 2017, collaboration revenue was $210 million, compared to $45.6 million for the same period in 2016. Collaboration revenue increased in both the quarter and nine months ended September 30, 2017 as the Company completed all deliverables required under its licensing and commercialization agreement with Novartis Pharma AG and recognized all associated deferred revenue. This increase in collaboration revenue had no impact on the Company's cash balance.
- **R&D Expenses:** Research and development expenses were $10.7 million for the quarter ended September 30, 2017, compared to $50.9 million for the same period in 2016. For the quarter ended September 30, 2017, research and development expenses included approximately $0.9 million in costs related to the Company's previously announced reduction in personnel. For the nine months ended September 30, 2017, research and development expenses were $58.3 million, compared to $136.9 million for the same period in 2016. For the nine months ended September 30, 2017, research and development expenses included approximately $6.8 million in costs related to the Company's previously announced reduction in personnel. Research and development expenses decreased in both the quarter and nine months ended September 30, 2017 primarily due to a decrease in expenses related to the Company's Fovista Phase 3 clinical program, including a decrease in manufacturing activities.
- **G&A Expenses:** General and administrative expenses were $7.1 million for the quarter ended September 30, 2017, compared to $12.0 million for the same period in 2016. For the quarter ended September 30, 2017, general and administrative expenses included approximately $0.5 million in costs related to the Company's previously announced reduction in personnel. For the nine months ended September 30, 2017, general and administrative expenses were $28.8 million, compared to $37.2 million for the same period in 2016. For the nine months ended September 30, 2017, general and administrative expenses included approximately $5.1 million in costs related to the Company's previously announced reduction in personnel and its termination of facilities leases. General and administrative expenses decreased in both the quarter and nine months ended September 30, 2017 primarily due to a decrease in costs to support the Company's operations and infrastructure.
- **Net Income:** The Company reported net income for the quarter ended September 30, 2017 of $189.1 million, or $5.25 per diluted share, compared to a net loss of $60.9 million, or ($1.71) per diluted share, for the same period in 2016. For the nine months ended September 30, 2017, the Company reported net income of $123.7 million, or $3.44 per diluted share, compared to a net loss of $127.1 million, or ($3.59) per diluted share, for the same period in 2016.

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 November 8, 2017 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 800-239-9838 (USA) or +1 323-794-2551 (International), passcode 7300213. 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 7300213.

About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics for age-related and orphan 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, and the potential for 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, 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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Ophthotech Corporation
Selected Financial Data (unaudited)
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	**2016**	**2017**	**2016**
Statements of Operations Data:				
Collaboration revenue	$ 206,654	$ 1,668	$ 209,977	$ 45,587
Operating expenses:				
Research and development	10,707	50,854	58,343	136,886
General and administrative	7,059	12,024	28,770	37,209
Total operating expenses	17,766	62,878	87,113	174,095
Income (loss) from operations	188,888	(61,210)	122,864	(128,508)
Interest income	391	409	1,113	1,301
Other loss	(12)	(20)	(34)	(88)
Loss before income tax provision	189,267	(60,821)	123,943	(127,295)
Income tax provision (benefit)	194	70	196	(158)
Net income (loss)	$ 189,073	$ (60,891)	$ 123,747	$ (127,137)
Net income (loss) per common share:				
Basic	$ 5.26	$ (1.71)	$ 3.45	$ (3.59)
Diluted	$ 5.25	$ (1.71)	$ 3.44	$ (3.59)
Weighted average common shares outstanding:				
Basic	35,971	35,594	35,878	35,415
Diluted	36,047	35,594	35,984	35,415

	September 30, 2017	December 31, 2016
	(in thousands)	
Balance Sheets Data:		
Cash, cash equivalents, and marketable securities	$ 180,217	$ 289,278
Total assets	182,982	299,630
Deferred revenue	-	209,976
Royalty purchase liability	125,000	125,000
Total liabilities	139,107	394,248
Additional paid-in capital	519,051	504,517
Accumulated deficit	(475,212)	(598,959)
Total stockholders' equity (deficit)	$ 43,875	$ (94,618)

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Investors
Ophthotech Corporation
Kathy Galante, 212-845-8231

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

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