Ophthotech Reports Fourth Quarter and Full Year 2018 Financial and Operating Results

February 26, 2019

- Conference Call and Webcast Today, February 26, 2019, at 8:00 a.m. ET -

NEW YORK--(BUSINESS WIRE)--Feb. 26, 2019-- Ophthotech Corporation (Nasdaq: OPHT) today announced financial and operating results for the fourth quarter and full year ended December 31, 2018 and provided a business update.

“2018 was a significant year for transforming the Company by executing multiple business development transactions that resulted in the addition of four gene therapy research and development programs targeting orphan inherited retinal diseases and an age-related therapeutic development program to our retina portfolio,” stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. “We welcomed Versant Ventures as a major shareholder of Ophthotech through the acquisition of Inception 4 and expanded our Board of Directors by adding three industry leaders. We look forward to topline data from our Phase 2b clinical trial of Zimura for the treatment of geographic atrophy secondary to dry age-related macular degeneration in the fourth quarter of 2019. We are committed to advancing and potentially expanding our pipeline of therapies for retinal diseases and creating value for our shareholders.”

Gene Therapy Program Highlights of 2018

In 2018, the Company initiated multiple innovative gene therapy collaborations to discover and develop next-generation therapies for the treatment of inherited retinal diseases.

- In June 2018, the Company entered into an exclusive global license agreement with the University of Florida Research Foundation (UFRF), Incorporated and the Trustees of the University of Pennsylvania (Penn) for rights to develop and commercialize a novel adeno-associated virus (AAV) gene therapy product candidate for the treatment of rhodopsin-mediated autosomal dominant retinitis pigmentosa (RHO-adRP), an orphan monogenic retinal disease. The novel AAV gene therapy construct is designed to knock down toxic rhodopsin and deliver a transgene for healthy rhodopsin via a single AAV vector in a mutation independent manner. The Company expects to initiate a Phase 1/2 clinical trial in RHO-adRP in 2020.
    - In August, proof-of-concept study results of this product candidate in a naturally occurring canine disease model were published in the prestigious journal *Proceedings of the National Academy of Sciences of the USA*. This publication entitled: “Mutation-independent Rhodopsin Gene Therapy by Knockdown and Replacement with a Single AAV vector” was published by scientists at Penn and University of Florida.
- In October 2018, the Company entered into its second series of gene therapy agreements with Penn and UFRF, including an exclusive option agreement for rights to negotiate to acquire an exclusive global license to develop and commercialize novel AAV gene therapy product candidates for the treatment of diseases impacted by the bestrophin, or BEST1, gene. The Company expects to initiate a Phase 1/2 clinical trial in 2021.
- In February 2018, the Company entered into a series of sponsored research agreements with the University of Massachusetts Medical School (UMMS) and its Horae Gene Therapy Center. The research seeks to utilize a minigene strategy to create novel AAV gene therapy product candidates for Leber Congenital Amaurosis type 10 due to CEP290 mutations (the most common type of LCA), and autosomal recessive Stargardt disease due to ABCA4 mutations, both of which are orphan inherited retinal diseases, and to evaluate different AAV gene delivery methods for application in the eye.

Therapeutic Program Highlights of 2018

Complement Factor C5 Inhibitor Program: Zimura®

- In October 2018, the Company completed patient enrollment for its ongoing randomized, double-masked, sham controlled, multi-center Phase 2b clinical trial of Zimura for the treatment of geographic atrophy secondary to dry AMD. The Company expects initial top-line data for this trial to be available in the fourth quarter of 2019.
- Patient enrollment in the Phase 2b randomized, double-masked, sham-controlled, multi-center clinical trial assessing the efficacy and safety of Zimura in patients with autosomal recessive Stargardt disease (STGD1) is completed and initial top-line data is expected to be available in the second half of 2020.

Corporate Highlights of 2018

- In October 2018, Ophthotech acquired Inception 4, Inc., a privately held company backed by Versant Ventures. Ophthotech gained worldwide development and commercialization rights to Inception 4’s small molecule inhibitors of HtrA1 (high temperature requirement A serine peptidase 1 protein). In addition, Versant Ventures agreed to help Ophthotech identify opportunities to expand the pipeline. Ophthotech obtained approximately $6.1 million in cash through its acquisition of Inception 4 and issued approximately 5.2 million shares to the shareholders of Inception 4. After giving effect to the transaction, funds affiliated with Versant Ventures own approximately 12.5% of the outstanding shares of Ophthotech’s

common stock.

- During 2018, the Company expanded its Board of Directors by adding leading industry experts: Adrienne L. Graves, Ph.D., former Chief Executive Officer of Santen, Inc., and Jane Pritchett Henderson, Chief Financial Officer of Turnstone Biologics and former Chief Financial Officer and Senior Vice President of Corporate Development at Voyager Therapeutics. Effective January 1, 2019, Calvin W. Roberts, M.D., Senior Vice President and Chief Medical Officer, Eye Care at Bausch Health Companies and Clinical Professor of Ophthalmology at Weill Medical College of Cornell University was elected to Ophthotech's Board of Directors.

2019 Operational Update

As of December 31, 2018, the Company had $131.2 million in cash and cash equivalents. The Company estimates its year end 2019 cash and cash equivalents will range between $80 million and $85 million based on its current 2019 business plan, including expansion of the Company's gene therapy research and development programs, expansion of its HtrA1 development program, and the continuation of its clinical development programs for Zimura® (avacincaptad pegol sodium). This estimate does not reflect any additional expenditures resulting from the potential in-licensing or acquisition of additional product candidates or technologies or associated development that the Company may pursue.

2018 Financial Highlights

- **Gain on Extinguishment of Royalty Purchase Liability:** The Company recognized a gain on extinguishment of its royalty purchase liability of $125 million for the quarter and year ended December 31, 2018, due to the December 2018 termination of the Company's royalty purchase and sale agreement with Novo Holdings A/S. The termination of the agreement relieved the Company of any obligation to pay Novo Holdings A/S future product royalties on sales of certain platelet derived growth factor (PDGF) antagonists and did not impact the Company's cash balance.
- **Revenues:** The Company did not have any collaboration revenue for the quarters ended December 31, 2018 and 2017 or during the year ended December 31, 2018, compared to $210 million for the year ended December 31, 2017. Collaboration revenue decreased for 2018 as compared to 2017 as the Company had recognized all deferred revenue associated with the completion of the Company's licensing and commercialization agreement with Novartis Pharma AG during the third quarter of 2017 and had no collaboration revenue in 2018.
- **R&D Expenses:** Research and development expenses were $16.1 million for the quarter ended December 31, 2018, compared to $7.9 million for the same period in 2017. Research and development expenses increased primarily due to $6.9 million in charges associated with the Company's October 2018 acquisition of Inception 4 and its HtrA1 inhibitor program and increases in costs associated with the Company's ongoing Zimura and gene therapy development programs. For the year ended December 31, 2018, research and development expenses were $41.7 million compared to $66.3 million for 2017. Research and development expenses decreased primarily due to decreases in expenses related to the discontinuation of the Company's Fovista Phase 3 clinical program and decreases in costs associated with the Company's 2017 reduction in personnel program partially offset by charges associated with the Company's October 2018 acquisition of Inception 4 and its HtrA1 inhibitor program and increases in costs associated with the Company's ongoing Zimura and gene therapy development programs.

- **G&A Expenses:** General and administrative expenses were $5.7 million for the quarter ended December 31, 2018, compared to $6.9 million for the same period in 2017. For the year ended December 31, 2018, general and administrative expenses were $23.6 million compared to $35.7 million for 2017. General and administrative expenses decreased primarily due to decreases in costs to support the Company's operations and infrastructure and decreases in costs associated with its 2017 reduction in personnel program, which included facilities lease termination expenses incurred during the first quarter of 2017.
- **Net Income:** The Company reported net income for the quarter ended December 31, 2018 of $104.1 million, or $2.62 per diluted share, compared to net loss of $9.5 million, or ($0.26) per diluted share, for the same period in 2017. For the year ended December 31, 2018, the Company reported net income of $63.1 million, or $1.70 per diluted share, compared to net income of $114.2 million, or $3.17 per diluted share, for the same period in 2017.

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 February 26, 2019 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-224-1005 (USA) or 323-994-2093 (International), passcode 6840989. 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 6840989.

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, Ophthotech's projected use of cash and cash balances, the timing, progress and results of clinical trials and other research and development activities, the potential utility of its product candidates 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 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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Ophthotech Corporation
Selected Financial Data (unaudited)
(in thousands, except per share data)

	Three months ended December 31,		Year ended December 31,	
	2018	**2017**	**2018**	**2017**
Statements of Operations Data:				
Collaboration revenue	$ -	$ -	$ -	$ 209,977
Operating expenses:				
Research and development	16,128	7,946	41,737	66,289
General and administrative	5,667	6,913	23,612	35,683
Total operating expenses	21,795	14,859	65,349	101,972
Income (loss) from operations	(21,795)	(14,859)	(65,349)	108,005
Interest income	677	409	2,389	1,522
Gain on extinguishment of Royalty Purchase Liability	125,000	-	125,000	-
Other income (expense)	1	-	(16)	(34)
Income (loss) before income tax benefit	103,883	(14,450)	62,024	109,493
Income tax benefit	(231)	(4,908)	(1,063)	(4,712)
Net income (loss)	$ 104,114	$ (9,542)	$ 63,087	$ 114,205
Net income (loss) per common share:				
Basic	$ 2.62	$ (0.26)	$ 1.70	$ 3.18
Diluted	$ 2.62	$ (0.26)	$ 1.70	$ 3.17
Weighted average common shares outstanding:				
Basic	39,673	36,041	37,061	35,919
Diluted	39,687	36,041	37,088	36,007

	December 31, 2018	December 31, 2017
	(in thousands)	
Balance Sheets Data:		
Cash and cash equivalents	$ 131,201	$ 166,972
Total assets	137,165	175,576
Royalty purchase liability	-	125,000
Total liabilities	13,206	137,535
Additional paid-in capital	545,585	522,759
Accumulated deficit	(421,667)	(484,754)
Total stockholders' equity	$ 123,959	$ 38,041

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