



Ophthotech Reports Third Quarter 2018 Financial and Operating Results

October 31, 2018

- Conference Call and Webcast to Discuss Q3 2018 Results and Two Pipeline Deals Announced Today -

(Conference Call: Today, October 31, 2018, at 8:00 a.m. ET)

NEW YORK--(BUSINESS WIRE)--Oct. 31, 2018-- [Ophthotech Corporation](#) (Nasdaq:OPHT) today announced financial and operating results for the third quarter ended September 30, 2018 and provided a business update.

"During 2018 Ophthotech made significant progress in bringing together a diversified pipeline of therapeutic and gene therapy programs for the treatment of retinal diseases," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "Today, we announced two transactions that add compelling opportunities to our existing pipeline of retinal programs, and through the acquisition of Inception 4, Inc. we are excited to welcome Versant Ventures as a major shareholder of Ophthotech. We are on track to provide topline data from our Zimura program in wet age-related macular degeneration by the end of this year, followed by topline data in geographic atrophy secondary to dry AMD in the second half of 2019 and in Stargardt disease in 2020. We look forward to advancing and expanding our pipeline of age-related and orphan retinal diseases and creating value for our shareholders."

Corporate Highlights

The following announcements will be discussed during today's conference call/webcast (see full detailed press releases issued earlier today and call in information below).

- As announced today, Ophthotech has acquired Inception 4, Inc., a privately held company backed by Versant Ventures, expanding Ophthotech's therapeutic pipeline in age-related retinal indications. Through this acquisition, Ophthotech gains worldwide development and commercialization rights to Inception 4's small molecule inhibitors of HtrA1 (high temperature requirement A serine peptidase 1 protein). As a major new investor with substantial geographic reach, Versant Ventures has agreed to help Ophthotech identify exceptional opportunities to expand the pipeline. As a result of the closing of the acquisition, Ophthotech obtained approximately \$6.1 million in cash through Inception 4. As upfront consideration in the transaction, Ophthotech agreed to issue approximately 5.2 million shares to the shareholders of Inception 4. After giving effect to the transaction, Versant Ventures, through its affiliated investment funds, owns approximately 12.5% of the outstanding shares of Ophthotech's common stock. In addition, Inception 4 equity holders will be entitled to receive post-closing payments upon the achievement of certain clinical and marketing approval milestones in certain AMD indications.
- Ophthotech also announced today that it expanded its gene therapy pipeline with a novel product candidate to treat Best vitelliform macular dystrophy, also known as Best disease. The Company entered into its second series of gene therapy agreements with the University of Pennsylvania and the University of Florida, including an exclusive option agreement for rights to negotiate to acquire an exclusive global license to develop and commercialize novel adeno-associated virus (AAV) gene therapy product candidates for the treatment of Best disease.

Therapeutic Program Highlights

Complement Factor C5 Inhibitor Program: Zimura®

- **Wet Age-related Macular Degeneration:** The Company expects initial top-line data by the end of this year from its randomized, dose-ranging, open-label, uncontrolled, multi-center Phase 2a clinical trial of Zimura® (avacincaptad pegol) in combination with the anti-vascular endothelial growth factor (anti-VEGF) agent Lucentis® (ranibizumab) in patients with wet AMD who have not been previously treated with anti-VEGF therapies. This trial is designed to assess the safety of Zimura combination therapy at different dosages and to detect a potential efficacy signal at month six. The Company completed patient recruitment for this trial in April 2018.
- **Geographic Atrophy, an Advanced Form of Dry Age-related Macular Degeneration:** 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.
- **Autosomal Recessive Stargardt Disease:** 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 currently on-going. Initial top-line data is expected to be available in 2020.

Gene Therapy Program Highlights

- **Rhodopsin-mediated Autosomal Dominant Retinitis Pigmentosa:** In August 2018, Ophthotech announced that proof-of-concept study results of its adeno-associated virus (AAV) gene therapy product candidate for the treatment of rhodopsin-mediated autosomal dominant retinitis pigmentosa (RHO-adRP) in a naturally occurring canine model were published in the journal *Proceedings of the National Academy of Sciences of the USA* (PNAS). Ophthotech obtained a license for rights to develop and commercialize this AAV gene therapy product candidate in June 2018. This publication entitled: "Mutation-independent Rhodopsin Gene Therapy by Knockdown and Replacement with a Single AAV vector" was published by scientists at the University of Pennsylvania and University of Florida. Based on current timelines and subject to regulatory review, Ophthotech expects to initiate a Phase 1/2 clinical trial in RHO-adRP in 2020.

2018 Operational Update

As of September 30, 2018, the Company had \$135.2 million in cash and cash equivalents. The Company increased its year end 2018 cash and cash equivalents estimate to range between \$125 million and \$130 million, an increase from the Company's prior estimate of between \$112 million and \$117 million, reflecting the impact of the acquisition of Inception 4, expansion of the Company's gene therapy research and development programs and the continuation of the Company's development programs for Zimura as currently planned. 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

- **Revenues:** The Company did not have any collaboration revenue for the quarter and nine months ended September 30, 2018, compared to \$206.7 million and \$210.0 million for the same periods in 2017. Collaboration revenue decreased due to the completion of the Company's licensing and commercialization agreement with Novartis Pharma AG and the recognition of all associated deferred revenue during the third quarter of 2017.
- **R&D Expenses:** Research and development expenses were \$9.4 million for the quarter ended September 30, 2018, compared to \$10.7 million for the same period in 2017. For the nine months ended September 30, 2018, research and development expenses were \$25.6 million compared to \$58.3 million for 2017. The Company continues to pursue its ongoing Zimura development programs and gene therapy research and development programs. 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.
- **G&A Expenses:** General and administrative expenses were \$6 million for the quarter ended September 30, 2018, compared to \$7.1 million for the same period in 2017. For the nine months ended September 30, 2018, general and administrative expenses were \$17.9 million compared to \$28.8 million for 2017. General and administrative expenses decreased primarily due to decreases in costs to support the Company's reduced operations and infrastructure and decreases in costs associated with its 2017 reduction in personnel, which included facilities lease termination expenses incurred during the first quarter of 2017.
- **Net Income:** The Company reported a net loss for the quarter ended September 30, 2018 of \$14.7 million, or (\$.41) per diluted share, compared to net income of \$189.1 million, or \$5.25 per diluted share, for the same period in 2017. For the nine months ended September 30, 2018, the Company reported a net loss of \$41 million, or (\$1.13) per diluted share, compared to net income of \$123.7 million, or \$3.44 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 for the third quarter of 2018 and to provide a business update. The call is scheduled for October 31, 2018 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-204-4368 (USA) or 323-994-2082 (International), passcode 3714524. 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 3714524.

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 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, 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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Ophthotech Corporation
Selected Financial Data (unaudited)
(in thousands, except per share data)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|----------------------------------|-------------------|---------------------------------|-------------------|
| | 2018 | 2017 | 2018 | 2017 |
| Statements of Operations Data: | | | | |
| Collaboration revenue | \$ - | \$ 206,654 | \$ - | \$ 209,977 |
| Operating expenses: | | | | |
| Research and development | 9,407 | 10,707 | 25,609 | 58,343 |
| General and administrative | 5,968 | 7,059 | 17,945 | 28,770 |
| Total operating expenses | <u>15,375</u> | <u>17,766</u> | <u>43,554</u> | <u>87,113</u> |
| Income (loss) from operations | (15,375) | 188,888 | (43,554) | 122,864 |
| Interest income | 637 | 391 | 1,711 | 1,113 |
| Other expense | (1) | (12) | (17) | (34) |
| Income (loss) before income tax provision (benefit) | (14,739) | 189,267 | (41,860) | 123,943 |
| Income tax provision (benefit) | 6 | 194 | (833) | 196 |
| Net income (loss) | <u>\$ (14,745)</u> | <u>\$ 189,073</u> | <u>\$ (41,027)</u> | <u>\$ 123,747</u> |
| Net income (loss) per common share: | | | | |
| Basic | <u>\$ (0.41)</u> | <u>\$ 5.26</u> | <u>\$ (1.13)</u> | <u>\$ 3.45</u> |
| Diluted | <u>\$ (0.41)</u> | <u>\$ 5.25</u> | <u>\$ (1.13)</u> | <u>\$ 3.44</u> |
| Weighted average common shares outstanding: | | | | |
| Basic | <u>36,202</u> | <u>35,971</u> | <u>36,181</u> | <u>35,878</u> |
| Diluted | <u>36,202</u> | <u>36,047</u> | <u>36,181</u> | <u>35,984</u> |

September 30, 2018 December 31, 2017
(in thousands)

Balance Sheets Data:

| | | |
|----------------------------|------------|------------|
| Cash and cash equivalents | \$ 135,208 | \$ 166,972 |
| Total assets | 140,957 | 175,576 |
| Royalty purchase liability | 125,000 | 125,000 |
| Total liabilities | 135,530 | 137,535 |
| Additional paid-in capital | 531,172 | 522,759 |
| Accumulated deficit | (525,781) | (484,754) |
| Total stockholders' equity | \$ 5,427 | \$ 38,041 |

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Source: Ophthotech Corporation

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