



Ophthotech Reports Second Quarter 2018 Financial and Operating Results

August 1, 2018

(Conference Call and Webcast Today, August 1, 2018, at 8:00 a.m. ET)

NEW YORK--(BUSINESS WIRE)--Aug. 1, 2018-- Ophthotech Corporation (Nasdaq:OPHT) today announced financial and operating results for the second quarter ended June 30, 2018 and provided a business update.

"During the first half of the year, we continued implementing our strategy to broaden and advance our ophthalmic portfolio as we enter the emerging field of gene therapy by securing collaborations with three leading academic institutions, and continued advancing our therapeutic portfolio with Zimura[®]," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "Looking ahead to the remainder of 2018, we expect to report data for our Phase 2a clinical trial for Zimura combination therapy with anti-VEGF in wet-age related macular degeneration (AMD), complete recruitment for our Phase 2b clinical trial for Zimura monotherapy in geographic atrophy secondary to dry AMD and potentially enter into new opportunities to further expand our portfolio in both therapeutics and gene therapies for retinal diseases."

First Half 2018: Key Highlights

Zimura[®] Complement Factor C5 Inhibitor Program

- In April 2018, the Company completed patient recruitment in 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 age-related macular degeneration (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. Data will be evaluated at month six and initial top-line data is expected to be available by the end of 2018.
- Patient recruitment for the Company's ongoing randomized, double-masked, sham controlled, multi-center Phase 2b clinical trial of Zimura for the treatment of geographic atrophy secondary to dry AMD is on track. The Company expects to complete recruitment in the third quarter of this year with initial top-line data expected to be available during the second half of 2019.
- In January 2018, the Company started enrolling patients in a 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). Initial top-line data is expected to be available in 2020.

Gene Therapy Programs

- The Company has initiated an innovative gene therapy program focused on applying novel gene therapy technology to discover and develop new therapies for ocular diseases.
 - In June 2018, the Company entered into an exclusive global license agreement with the University of Florida Research Foundation, Incorporated and the Trustees of the University of Pennsylvania (Penn) for rights to develop and commercialize a novel adeno-associated virus gene therapy product candidate for the treatment of rhodopsin-mediated autosomal dominant retinitis pigmentosa (RHO-adRP), an orphan monogenic disease. The construct for the RHO-adRP product candidate combines a transgene expressing a highly efficient novel short hairpin RNA (shRNA) designed to target and knock-down endogenous rhodopsin (RHO) in a mutation-independent manner with a human RHO replacement transgene made resistant to RNA interference, in a single adeno-associated viral (AAV 2/5) vector. Ophthotech and Penn have also entered into a master sponsored research agreement, facilitated by the Penn Center for Innovation, pursuant to which Ophthotech and Penn plan to conduct natural history studies in RHO-adRP patients and additional preclinical studies. In parallel with the sponsored research, Ophthotech plans to commence IND-enabling activities. Based on current timelines and subject to regulatory review, Ophthotech expects to initiate a Phase 1/2 clinical trial in RHO-adRP in 2020.
 - 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 to utilize their next generation "minigene" therapy approach for the potential treatment of orphan degenerative retinal diseases such as Leber Congenital Amaurosis (LCA) type 10 due to *CEP290* mutations (the most common type of LCA), and autosomal recessive Stargardt disease (STGD1) due to *ABCA4* mutations. Further, the Company and UMMS are also evaluating novel gene delivery methods to target retinal diseases. UMMS has granted Ophthotech an option to obtain an exclusive license to any patent or patent applications that result from this research.

2018 Operational Update

As of June 30, 2018, the Company had \$146 million in cash and cash equivalents. The Company estimates that its year end 2018 cash and cash

equivalents will range between \$112 million and \$117 million based on its current 2018 business plan and planned capital expenditures. This estimate includes continuation of the Company's development programs for Zimura[®] and RHO-adRP gene therapy product candidate and the continuation of the Company's collaborative gene therapy research programs 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 six months ended June 30, 2018, compared to \$1.7 million and \$3.3 million for the same periods in 2017. Collaboration revenue decreased due to the completion of the Company's deliverables under its previous 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 \$8.5 million for the quarter ended June 30, 2018, compared to \$15.7 million for the same period in 2017. For the six months ended June 30, 2018, research and development expenses were \$16.2 million compared to \$47.6 million for 2017. As the Company pursues its ongoing and planned Zimura and gene therapy 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 program.
- **G&A Expenses:** General and administrative expenses were \$6.3 million for the quarter ended June 30, 2018, compared to \$8.6 million for the same period in 2017. For the six months ended June 30, 2018, general and administrative expenses were \$12.0 million compared to \$21.7 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 includes facilities lease termination expenses incurred during the first quarter of 2017.
- **Net Loss:** The Company reported a net loss for the quarter ended June 30, 2018 of \$13.2 million, or (\$0.37) per diluted share, compared to a net loss of \$22.2 million, or (\$0.62) per diluted share, for the same period in 2017. For the six months ended June 30, 2018, the Company reported a net loss of \$26.3 million, or (\$0.73) per diluted share, compared to a net loss of \$65.3 million, or (\$1.82) 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 August 1, 2018 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 800-458-4121 (USA) or 323-794-2597 (International), passcode 3698278. A live, listen-only audio webcast of the conference call can be accessed on the Investor Relations section of the Ophthotech website at: www.opthotech.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 3698278.

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.opthotech.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 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 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Statements of Operations Data:				
Collaboration revenue	\$ -	\$ 1,661	\$ -	\$ 3,323
Operating expenses:				
Research and development	8,516	15,657	16,202	47,636
General and administrative	6,332	8,552	11,977	21,711
Total operating expenses	14,848	24,209	28,179	69,347
Loss from operations	(14,848)	(22,548)	(28,179)	(66,024)
Interest income	602	344	1,075	722
Other expense	-	(1)	(16)	(22)
Loss before income tax provision (benefit)	(14,246)	(22,205)	(27,120)	(65,324)
Income tax provision (benefit)	(1,037)	(1)	(838)	2
Net loss	\$ (13,209)	\$ (22,204)	\$ (26,282)	\$ (65,326)
Net loss per common share:				
Basic and diluted	\$ (0.37)	\$ (0.62)	\$ (0.73)	\$ (1.82)
Weighted average common shares outstanding:				
Basic and diluted	36,188	35,858	36,171	35,831

June 30, 2018 December 31, 2017

(in thousands)

Balance Sheets Data:

Cash, cash equivalents, and marketable securities	\$ 145,991	\$ 166,972
Total assets	151,661	175,576
Royalty purchase liability	125,000	125,000
Total liabilities	134,131	137,535
Additional paid-in capital	528,530	522,759
Accumulated deficit	(511,036)	(484,754)
Total stockholders' equity	\$ 17,530	\$ 38,041

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