

# **Ophthotech Reports Fourth Quarter and Full Year 2014 Financial and Operating Results**

- Conference Call and Webcast Today, February 23, at 5:00 p.m. ET -

NEW YORK--(BUSINESS WIRE)-- Ophthotech Corporation (Nasdaq:OPHT) today announced financial results for the fourth quarter and full year ended December 31, 2014 and provided an update on the Company's business and product development programs. Today's conference call includes Pravin Dugel, M.D., Managing Partner of Retinal Consultants of Arizona and Clinical Professor, the University of Southern California Eye Institute, Keck School of Medicine, who will discuss initial Fovista<sup>®</sup> data from his Investigator Sponsored Trial that was featured at the 12<sup>th</sup> Annual Bascom-Palmer Eye Institute Angiogenesis, Exudation, and Degeneration Meeting in Miami, Florida that took place on February 7, 2015.

### **Product Pipeline Highlights**

- Ophthotech is on track with respect to the execution of its pivotal Fovista<sup>®</sup> Phase 3 program, evaluating the safety and efficacy of Fovista<sup>®</sup> administered in combination with anti-vascular endothelial growth factor (anti-VEGF) therapy for the treatment of wet age-related macular degeneration (AMD). The Company expects to have initial, top-line data from the Phase 3 program available in 2016.
- Ophthotech has expanded the clinical program for its lead product candidate Fovista<sup>®</sup> beyond its pivotal Phase 3 program in wet AMD, and is advancing its second product candidate Zimura<sup>TM</sup>, an inhibitor of complement factor C5, in both dry AMD and wet AMD.
  - Fovista<sup>®</sup> Expansion Program
    - Ophthotech commenced a study in August 2014 investigating the potential of Fovista<sup>®</sup> in combination with anti-VEGF therapy in reducing subretinal fibrosis in wet AMD patients.
    - The Company recently initiated its treatment burden reduction program to investigate the potential of Fovista<sup>®</sup> combination therapy in reducing the treatment frequency associated with anti-VEGF monotherapy in wet AMD patients.
    - Ophthotech has initiated the planning process for a Fovista<sup>®</sup> combination therapy trial in monotherapy anti-VEGF resistant (failure) patients with wet AMD, which is expected to commence this year.
  - <u>Zimura<sup>™</sup> Program</u>: Ophthotech has initiated a clinical trial of Zimura<sup>™</sup> with an¥IEGF therapy for patients with polypoidal choroidal vasculopathy, a variant of wet AMD. The Company expects to advance Zimura<sup>™</sup> to a Phase 2/3 clinical trial for treatment of geographic atrophy, a form of dry AMD, in the second half of 2015.

### **Research and Licensing Highlights**

In November 2014, Ophthotech entered into an exclusive research and option agreement with AVEO Pharmaceuticals to license tivozanib, a small molecule vascular endothelial growth factor tyrosine kinase inhibitor, for the treatment of non-oncologic conditions of the eye. Ophthotech is solely responsible for the ocular formulation and development of this compound, and plans

to focus on a sustained release formulation for the compound to be used in combination with Fovista<sup>®</sup> as a treatment for the maintenance phase of wet AMD therapy.

### **Translational Scientific Advisory Board**

Ophthotech today announced the formation of a Translational Scientific Advisory Board. Joining this board are Dr. Rakesh Jain, Andrew Werk Cook Professor of Tumor Biology at the Harvard Medical School, and Director of the Edwin L. Steele Laboratory for Tumor Biology at the Massachusetts General Hospital and Dr. David Cheresh, Distinguished Professor in the Department of Pathology, UC San Diego School of Medicine and previously Professor at The Scripps Research Institute in the Department of Immunology.

#### **Recent Corporate Highlights**

• In October 2014, Ophthotech received an initial \$50 million enrollment-based milestone payment from Novartis Pharma AG related to the potential \$130 million total enrollment-based milestones under its ex-US licensing and commercialization

agreement with Novartis entered into in May 2014.

- In November 2014, Ophthotech achieved the final enrollment-based milestone related to the Phase 3 Fovista<sup>®</sup> program under the terms of the Company's \$125 million royalty financing agreement with Novo A/S entered into in May 2013. Achievement of this milestone triggered a payment of \$41.7 million to Ophthotech. The funding of this final tranche resulted in an additional royalty interest to Novo A/S based on worldwide Fovista<sup>®</sup> sales.
- In December 2014, Ophthotech was selected for addition to the NASDAQ Biotechnology Index, designed to track the performance of a set of NASDAQ-listed securities that are classified as either biotechnology or pharmaceutical according to the Industry Classification Benchmark. The listing went into effect on December 22, 2014.

"Last year was truly a transformative one for Ophthotech," said David Guyer, M.D., Chief Executive Officer and Chairman of the

Board of Ophthotech. "Not only did we advance and expand our Fovista<sup>®</sup> and Zimura<sup>™</sup> franchises, but we also continued to explore other opportunities that are based on innovation, such as tivozanib, and that have the potential to address unmet medical needs in AMD and other back-of-the-eye diseases. We executed an ex-US partnership with Novartis and added experienced and talented people to our management team as we continue to build out our commercial capabilities. We are also very pleased to welcome Drs. Jain and Cheresh, two of the most accomplished scientists and key opinion leaders in angiogenesis, to our translational scientific advisory board and we look forward to their contributions. We expect to make great strides in 2015 as we continue to move our Phase 3 Fovista<sup>®</sup> program forward and advance the rest of our product pipeline."

## **Financial Results**

- As of December 31, 2014, the Company had \$463.6 million in cash, cash equivalents, and marketable securities.
- Collaboration revenue under the Company's agreement with Novartis was \$1.7 million and \$41.3 million for the quarter and year ended December 31, 2014. The Company did not have revenue during the comparable periods in 2013.
- Research and development expenses were \$22.2 million for the quarter ended December 31, 2014 compared to \$15.4 million for the same period in 2013. Research and development expenses were \$88.4 million for the year ended December 31, 2014 compared to \$33.2 million for the same period in 2013. The increase in research and development expense in the quarter and year ended December 31, 2014 relates primarily to the Company's Fovista<sup>®</sup> Phase 3 clinical program.
- General and administrative expenses were \$10.7 million for the quarter ended December 31, 2014 compared to \$5.1 million for the same period in 2013. General and administrative expenses were \$33.4 million for the year ended December 31, 2014 compared to \$14.2 million for the same period in 2013. The increased general and administrative expense in the quarter and year ended December 31, 2014 relates primarily to an increase in costs to support the expanded operations and our public company infrastructure, including additional management, corporate staffing, professional services and consulting fees, and increased share-based compensation.
- The Company reported a net loss for the quarter ended December 31, 2014 of \$35.9 million, or (\$1.06) per diluted share, compared to a net loss of \$20.4 million, or (\$0.65) per diluted share for the same period in 2013. The Company reported a net loss for the year ended December 31, 2014 of \$98.2 million, or (\$2.95) per diluted share, compared to a net loss of \$57.0 million, or (\$6.34) per diluted share for the same period in 2013.

# About the Fovista<sup>®</sup> Phase 3 Program

The Fovista<sup>®</sup> Phase 3 program consists of three clinical trials to evaluate the safety and efficacy of Fovista<sup>®</sup> (anti-PDGF) therapy, which Ophthotech is developing for use in combination with anti-VEGF therapy for the treatment of wet age-related macular degeneration. The Company expects to enroll a total of 1,866 patients in the three trials in more than 225 centers worldwide. Ophthotech expects to have initial, top-line data from the Fovista<sup>®</sup> Phase 3 clinical program available in 2016.

## Conference Call/Web Cast Information

Ophthotech will host a conference call/audio web cast to discuss the Company's financial and operating results, its development programs and provide a general business update. The call will also include remarks from Dr. Dugel. The call is scheduled for February 23 at 5:00 p.m. Eastern Time. To participate in this conference call, dial 888-397-5352 (USA) or 719-325-2428 (International), passcode 5289090. A live, listen-only audio web cast of the conference call can be accessed on the Investor Relations section of the Ophthotech website at: <u>www.ophthotech.com</u>. 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 5289090.

## About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics to treat back of the eye

diseases, with a focus on developing innovative therapies for age-related macular degeneration (AMD). Ophthotech's most advanced product candidate, Fovista<sup>®</sup> anti-PDGF therapy, is in Phase 3 clinical trials for use in combination with anti-VEGF therapy that represents the current standard of care for the treatment of wet AMD. Ophthotech's second product candidate, Zimura<sup>™</sup>, an inhibitor of complement factor C5, is being developed for the treatment of geographic atrophy (a form of dry AMC and, in combination with anti-VEGF therapy and, potentially Fovista<sup>®</sup>, for the treatment of wet AMD. For more information, please visit <u>www.ophthotech.com</u>.

### **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. Forwardlooking 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 potential receipt of milestone payments and royalties under its ex-US licensing and commercialization agreement, the conduct of the Fovista<sup>®</sup> Phase 3 clinical program, including obtaining initial, top-line data from the Fovista<sup>®</sup> Phase 3 clinical program and seeking marketing approval for Fovista<sup>®</sup>, the potential of Fovista<sup>®</sup> as a wet AMD combination therapy, the initiation of additional clinical trials for Fovista<sup>®</sup> and Zimura<sup>™</sup>, obtaining data from these additional planned trials, Ophthotech's strategy for the development of, and the potential therapeutic benefit of, tivozanib for the treatment of non-oncologic conditions of the eye. including wet AMD and the potential for related value creation for Ophthotech's stockholders. 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 and expectations for regulatory approvals or other actions and other factors discussed in the "Risk Factors" section contained in the guarterly and annual reports that Ophthotech files with the SEC. 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.

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

	Three Months Ended December 31,				Years Ended December 31,			
		2014		2013	2014		2013	
Statement of operations data:								
Collaboration revenue	\$	1,684	\$	-	\$ 41,259	\$	-	
Costs and expenses:								
Research and development		22,196		15,379	88,385		33,215	
General and administrative		10,656		5,065	 33,387		14,210	
Total costs and expenses		32,852		20,444	 121,772		47,425	
Loss from operations		(31,168)		(20,444)	 (80,513)		(47,425)	
Interest income (expense)		28		-	217		(1,454)	
Loss on extinguishment of debt		-		-	-		(1,091)	
Other gain (loss)		-		56	 -		(1,175)	
Loss before income tax provision		(31,140)		(20,388)	 (80,296)		(51,145)	
Income tax provision		4,731		-	17,892		-	
Net loss		(35,871)		(20,388)	 (98,188)		(51,145)	
Add: accretion of preferred stock dividends		-		-	-		(5,891)	
Net loss attributable to common stockholders	\$	(35,871)	\$	(20,388)	\$ (98,188)	\$	(57,036)	
Net loss attributable to common stockholders per share :								
Basic and diluted Weighted average common shares outstanding:	\$	(1.06)	\$	(0.65)	\$ (2.95)	\$	(6.34)	
Basic and diluted		33,803		31,355	33,258		9,003	

	December 31, 2014		December 31, 2013		
	(in thousands)				
Balance sheet data:					
Cash, cash equivalents, and marketable					
securities	\$	463,560	\$	210,596	
Due from Novartis Pharma, AG	\$	960	\$	-	
Total assets	\$	498,370	\$	217,682	
Royalty purchase liability	\$	125,000	\$	41,667	
Deferred revenue	\$	209,624	\$	-	
Total liabilities	\$	351,249	\$	47,962	
Additional paid-in capital	\$	428,390	\$	352,739	
Accumulated deficit	\$	(281,238)	\$	(183,050)	
Total stockholders' equity	\$	147,121	\$	169,720	

Investors

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