

# Ophthotech Reports Fourth Quarter and Full Year 2013 Financial and Operating Results

- Conference Call and Webcast Today, February 27, at 8:00 a.m. ET -

**New York, NY, February 27, 2014** – Ophthotech Corporation (Nasdaq: OPHT) today announced financial results for the fourth quarter and full year ended December 31, 2013 and provided an update on the Company's business and product development programs.

### **Recent Corporate Highlights**

- Cash Resources:
  - As of December 31, 2013, the Company had \$210.6 million in cash and cash equivalents.
  - In January 2014, the Company received an additional \$41.7 million from a second tranche payment under the Company's \$125.0 million royalty financing agreement with Novo A/S. A potential third tranche of \$41.7 million under this royalty agreement remains available based upon a further patient enrollment milestone.
  - o In February 2014, Ophthotech completed a follow-on public offering of common stock resulting in net proceeds of approximately \$55.5 million for the Company.
- With the follow-on public offering complete, Ophthotech is expanding the global clinical program for its lead product candidate Fovista<sup>TM</sup> beyond its pivotal Phase 3 program in wet age-related macular degeneration (AMD), and is advancing its second product candidate Zimura<sup>TM</sup>, an inhibitor of complement factor C5 (formerly known as ARC1905), in both dry AMD and wet AMD.
  - o Fovista<sup>™</sup> program expansion: The Company is planning clinical trials of Fovista<sup>™</sup> in combination with anti-VEGF therapy for the treatment of anti-VEGF resistant wet AMD patients and to assess possible reduction of treatment burden in wet AMD therapy. Ophthotech also plans to initiate a clinical trial to investigate the effect of Fovista<sup>™</sup> in potentially inhibiting the formation of subretinal fibrosis in wet AMD patients treated with anti-VEGF therapies. Studies have shown that subretinal fibrosis is associated with severe vision loss in wet AMD patients. These trials are scheduled to commence in 2014. In addition, the National Eye Institute is scheduled to conduct a clinical trial with Fovista<sup>™</sup> in von Hippel-Lindau disease starting in 2014, and the Company is planning to initiate in 2015 a clinical trial of Fovista<sup>™</sup> in proliferative vitreoretinopathy.
  - O Zimura<sup>™</sup> program: The Company is expected to advance to a Phase 2/3 clinical trial for treatment of geographic atrophy, a severe form of dry AMD, in late 2014 or early 2015. In addition, a Phase 2 clinical trial is planned for Zimura<sup>™</sup> and Fovista<sup>™</sup> in combination with anti-VEGF therapy for the treatment of anti-VEGF resistant wet AMD patients believed to have complement mediated inflammation. This trial is scheduled to initiate in 2015.

• In late February, the Company received written confirmation from the European Medicine Agency's Committee for Medicinal Products for Human Use (CHMP) indicating that the CHMP and the Company are now in agreement regarding the Company's trial design and dosing regimens for the Fovista™ Phase 3 program.

"It has been a very busy and exciting time for Ophthotech following our IPO in September," said David Guyer, M.D., Chief Executive Officer of Ophthotech. "Our lead product Fovista™ being developed as a first-in-class anti-PDGF agent for wet AMD combination therapy, is on track as we continue to expect initial, topline data and an NDA filing in 2016. In addition, recent science-driven findings along with net proceeds of approximately \$55.5 million from a public offering in February of this year provide us with the foundation to expand our Fovista™ and Zimura™ clinical programs to address the unmet medical needs in AMD and other ophthalmic diseases and conditions. We are extremely pleased with the progress we have made and look forward to the expansion of our programs with our plan for 10 clinical trials ongoing or initiating in 2014 and 2015 and data expected to begin in 2015."

#### **Financial Results**

Operating expenses for the quarter ended December 31, 2013 were \$20.4 million, with \$15.4 million attributable to research and development. This compares to operating expenses of \$3.5 million and research and development expenses of \$2.0 million for the same period in 2012. The Company reported a net loss for the quarter ended December 31, 2013 of \$20.4 million, or \$0.65 per share.

Operating expenses for the year ended December 31, 2013 were \$47.4 million, with \$33.2 million attributable to research and development. This compares to operating expenses of \$13.7 million and research and development expenses of \$6.8 million for 2012. The Company reported a net loss for the year ended December 31, 2013 of \$57.0 million, or \$6.34 per share.

# 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 drugs 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 and to have initial, topline 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 product development programs and provide a general business update. The call is scheduled for February 27 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-715-1391 (USA) or 913-981-5556 (International), passcode 7375299 shortly before 8:00 a.m. Eastern Time. A replay of the call will be available from approximately two hours following the live call for two weeks. The replay number is 888-203-1112 (USA) or 719-457-0820 (International), passcode 7375299. The audio webcast can be accessed at: www.ophthotech.com.

#### **About Ophthotech Corporation**

Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the eye, with a focus on developing innovative therapies for age-related macular degeneration (AMD). Ophthotech's most advanced product candidate, Fovista™ anti-PDGF therapy, is in Phase 3 clinical trials for use in combination with anti-VEGF drugs that represent the standard of care for the treatment of wet AMD. The Company's second product candidate Zimura™, an inhibitor of complement factor C5, is being developed for the treatment of dry and wet forms of AMD. For more information, please visit <a href="https://www.ophthotech.com">www.ophthotech.com</a>.

## **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," "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 conduct of the Fovista Phase 3 clinical program, including obtaining initial, top-line data from the Fovista Phase 3 clinical program and seeking marketing approval for Fovista, the potential of Fovista as a wet AMD combination therapy, the initiation of additional clinical trials for Fovista and Zimura and obtaining data from these additional planned trials. 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 express 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 quarterly 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.

#### Ophthotech Corporation (A Development Stage Entity) Selected Financial Data (in thousands, except per share data)

	Three Months Ended December 31,				Years Ended December 31,				Period from January 5, 2007 (Inception) to December 31,		
Statement of Operations Data:	2013		2012		2013		2012		2013		
Costs and expenses:					(Ui	naudited)					
Research and development	\$	15,379	\$	1,998	S	33,215	S	6,792	\$	108,107	
General and administrative	4	5,065	4	1,548	4	14,210	-	6,889	4	41,559	
Total costs and expenses		20,444		3,546		47,425		13,681		149,666	
Loss from operations		(20,444)		(3,546)		(47,425)		(13,681)		(149,666)	
Interest expense, net		-		(251)		(1,454)		(507)		(1,482)	
Loss on extinguishment of debt		-		-		(1,091)		-		(1,091)	
Other gain (loss)		56		(33)		(1,175)		(374)		(1,546)	
Change in fair value related to investor rights liability		-		-		-		-		683	
Net loss before income tax benefit		(20,388)		(3,830)		(51,145)		(14,562)		(153,102)	
Income tax benefit		-		-		-		-		1,327	
Net loss		(20,388)		(3,830)		(51,145)		(14,562)		(151,775)	
Add: accretion of preferred stock dividends		-		(1,776)		(5,891)		(7,063)		(33,046)	
Net loss attributable to common stockholders	\$	(20,388)	\$	(5,606)	\$	(57,036)	\$	(21,625)	\$	(184,821)	
Net loss attributable to common stockholders per share:											
Basic and diluted	\$	(0.65)	\$	(3.81)	\$	(6.34)	\$	(14.89)			
Weighted average common shares outstanding: Basic and diluted		31,355		1,470		9,003		1,452			

	As of December 31,				
	 2013		2012		
	 (Unaudited)				
Balance sheet data:					
Cash and cash equivalents	\$ 210,596	\$	4,304		
Total assets	\$ 217,682	\$	4,879		
Royalty purchase liability	\$ 41,667	\$	-		
Total liabilities	\$ 47,962	\$	14,410		
Preferred stock	\$ -	\$	113,939		
Additional paid-in capital	\$ 352,739	\$	-		
Deficit accumulated during the development stage	\$ (183,050)	\$	(126,471)		
Total stockholders' equity (deficit)	\$ 169,720	\$	(123,470)		