



## Ophthotech Targets Gene Therapy to Expand its Orphan and Age-Related Retinal Disease Strategy

February 27, 2018

– Ophthotech collaborates with University of Massachusetts Medical School on novel technology for next-generation gene delivery technology and novel gene therapies for ophthalmic diseases –

– Collaboration is led by Guangping Gao and Hemant Khanna at UMass Medical School's Horae Gene Therapy Center –

NEW YORK--(BUSINESS WIRE)--Feb. 27, 2018-- Ophthotech Corporation (NASDAQ:OPHT) announced today that the Company has initiated an innovative gene therapy research collaboration focused on applying novel gene therapy technology to discover and develop next-generation therapies for the treatment of ocular diseases. As part of Ophthotech's strategy, the Company has entered into a series of sponsored research agreements with the University of Massachusetts Medical School and its Horae Gene Therapy Center to utilize their "minigene" therapy approach and other novel gene delivery technologies to target retinal diseases. The laboratories of Guangping Gao, PhD, professor of molecular genetics & microbiology and director of the Horae Gene Therapy Center; and Hemant Khanna, PhD, associate professor of ophthalmology & visual sciences, will play a key role in this collaboration. Dr. Gao is a pioneer in the development of a novel adeno-associated virus (AAV) family for gene therapy; Dr. Khanna's research focuses on the molecular mechanism of inherited ocular disorders and using this knowledge to develop novel treatment approaches for them. As a condition of each research agreement, UMMS has granted the Company an option to obtain an exclusive license to any patents or patent applications that result from this research.

"Advances in gene therapy technologies have been promising and may provide transformational, next-generation therapies for patients with ophthalmic disease," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "The synergy between Dr. Gao's highly recognized team of experts in gene therapy at the University of Massachusetts Medical School and Ophthotech's clinical expertise has the potential to fulfill our commitment to advance innovative solutions for the treatment of retinal diseases. This is an important step as we execute on our strategy to become a leader in the development of ophthalmic therapies for orphan and larger indications in the back of the eye. We are excited to enter the emerging field of gene therapy as we continue to broaden and advance our ophthalmic pipeline."

"Our goal at the Horae Gene Therapy Center is to develop the next generation of gene delivery technology to provide the most efficient and safe gene transfer to patients," said Dr. Gao.

"Researchers seeking cures to diseases of the eye have led the way in innovation in gene therapy," said Terence R. Flotte, MD, executive deputy chancellor, provost and dean of the school of medicine at the University of Massachusetts Medical School, and a specialist in the field of gene therapy who was the first to use adeno-associated virus as a vehicle to deliver corrective genes in humans. "This collaboration sets the stage for future breakthroughs in this field."

"The translatability of any therapeutic strategy should be based upon sound scientific premise and pass multiple validation steps. Our team of investigators in the Horae Gene Therapy Center and the Department of Ophthalmology & Visual Sciences is in a unique position to fulfill these requirements for developing treatments of retinal degenerative diseases," said Hemant Khanna.

The use of "minigenes" as a novel therapeutic strategy seeks to deliver a shortened but still functional form of a large gene packaged into a standard-size AAV delivery vector commonly used in gene therapy. The "minigene" strategy may offer an innovative solution for diseases that would otherwise be difficult to address through conventional AAV gene replacement therapy where the size of the gene of interest exceeds the transgene packaging capacity of conventional AAV vectors. Research in this newly evolving area of gene therapy is led by Khanna and colleagues in the Horae Gene Therapy Center and was described in a recent journal article in *Human Gene Therapy*, "*Gene Therapy Using a miniCEP290 Fragment Delays Photoreceptor Degeneration in a Mouse Model of Leber Congenital Amaurosis*" by Wei Zhang, Linjing Li, Qin Su, Guangping Gao, and Hemant Khanna, all at the University of Massachusetts Medical School.

The collaboration with UMass Medical School will also focus on developing the next generation of gene therapy vectors to allow novel delivery approaches for treatment of retinal diseases.

"We find the science behind the next generation gene delivery technology and 'minigenes' at UMass Medical School very intriguing," stated Kourous Rezaei, M.D., Chief Medical Officer of Ophthotech. "This is a differentiated approach that would potentially allow the use of AAV vectors for the 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."

### About Ophthotech Corporation

Ophthotech is a biopharmaceutical company specializing in the development of novel therapies for age-related and orphan diseases of the eye. For more information, please visit [www.opthotech.com](http://www.opthotech.com).

### About the University of Massachusetts Medical School

The University of Massachusetts Medical School (UMMS), one of five campuses of the University system, is comprised of the School of Medicine, the Graduate School of Biomedical Sciences, the Graduate School of Nursing, a thriving research enterprise and an innovative public service initiative, Commonwealth Medicine. Its mission is to advance the health of the people of the Commonwealth through pioneering education, research, public service and health care delivery with its clinical partner, UMass Memorial Health Care. In doing so, it has built a reputation as a world-class research institution and as a leader in primary care education. The Medical School attracts more than \$266 million annually in research funding, placing it among the top 50 medical schools in the nation. In 2006, UMMS's Craig C. Mello, PhD, Howard Hughes Medical Institute Investigator and the *Blais University Chair in Molecular Medicine*, was awarded the Nobel Prize in Physiology or Medicine, along with colleague Andrew Z. Fire, PhD, of Stanford University, for their discoveries related to RNA interference (RNAi). The 2013 opening of the Albert Sherman Center ushered in a new era of

biomedical research and education on campus. Designed to maximize collaboration across fields, the Sherman Center is home to scientists pursuing novel research in emerging scientific fields with the goal of translating new discoveries into innovative therapies for human diseases.

### **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, the timing, progress and results of clinical trials and other research and development activities, 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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