

# Vision Medicines Announces Exclusive Worldwide Licensing Agreement with Case Western Reserve University for Stargardt Disease Drug Candidate

---

- **Stargardt disease is an orphan disease that leads to legal blindness; affects approximately 100,000 patients in the US, Europe and Japan with no treatments currently on the market**
- **VM200 has the potential to be the first treatment for Stargardt disease; it is planned to advance into a Phase 1/2 program in 2016**

Boston, Mass. -- November 9, 2015 -- Vision Medicines, Inc., a privately-held clinical-stage biopharmaceutical company committed to developing innovative therapies for patients suffering from retinal degeneration diseases, has announced an exclusive worldwide license for VM200 from Case Western Reserve University. VM200 is a preclinical, oral small molecule drug candidate for the treatment of Stargardt disease, an orphan disease that leads to legal blindness.

“We are enthusiastic about the potential for VM200 and have initiated investigational new drug application-enabling studies to advance VM200 into a Phase 1/2 program for Stargardt disease in 2016,” said Chris Varma, PhD, chairman and chief executive officer of Vision Medicines.

Stargardt disease is the most common form of inherited juvenile macular degeneration. There are no current treatments for this disease, and it estimated that a multi-billion dollar market opportunity exists for effective treatment.

Professor Krzysztof Palczewski, PhD, chairman of the Department of Pharmacology in Case Western Reserve’s School of Medicine (SOM) made the discovery that chemicals called primary amines are protective of the retina. His research has been published in Nature Chemical Biology in 2011.

Palczewski later teamed up with William Harte, a chief translational officer at the university’s SOM, to enhance the intellectual property portfolio around this discovery and generate the pre-clinical data package, which led to the identification of VM200.

“This is a fantastic opportunity to combine the world-class visual research of Krzysztof Palczewski with

the experienced team and ophthalmology expertise at Vision Medicines,” said Michael Haag, executive director of Case Western Reserve’s Technology Transfer Office.

“VM200 neutralizes toxic chemicals in the retina that are responsible for progressive retinal damage in Stargardt disease,” said Robert Kim, MD, chief medical officer and head of research and development for Vision Medicines. “It is delivered orally and has the potential to treat both eyes simultaneously in a convenient, non-invasive manner. We believe VM200 has the potential to help prevent Stargardt’s patients from becoming legally blind.”

"Dry age-related macular degeneration (AMD) and inherited retinal diseases, such as Stargardt disease, are areas of great unmet medical need. Through a very stringent diligence process that included world-class experts and involved vetting nearly 50 programs and technologies, we identified and in-licensed drug candidates that we believe have a very high probability of technical success and could be important new treatment options," said Reza Dana, MD, a scientific co-founder and chair of the scientific advisory board of Vision Medicines. Dr. Dana is a professor of Ophthalmology and the Claes Dohlman Chair in Ophthalmology at Harvard Medical School. "Dry AMD and Stargardt disease can both lead to severe vision loss, and our goal is to advance innovative therapies for these patients and to help change their lives, and we feel confident that our programs provide such a path."

In addition, Vision Medicines separately announced that the Foundation Fighting Blindness will co-fund development of VM200 through clinical proof-of-concept and will commit up to \$7.5 million to help advance this novel program. The Foundation Fighting Blindness is currently conducting a natural history study called ProgSTAR for Stargardt disease to gain a better understanding of its progression and how to effectively and quickly measure it in clinical trials. There is a large group of patients who are waiting to be treated, as shown by the rapid enrollment of the 250 patients in the ProgSTAR study. The findings from the ProgSTAR study will help in the development effort of VM200 and in the understanding of this disease.

ProgSTAR’s lead physician investigator, Hendrik Scholl, MD, director of the Retinal Degeneration Clinic and Professor of Ophthalmology at Johns Hopkins Medicine, is a member of Vision Medicines’ scientific advisory board. Vision Medicines has recruited a world-class scientific advisory board including leading

scientists and clinicians specializing in ophthalmology, retinal biology and genetics. A full list is available on the company's website at [www.visionmedicines.com](http://www.visionmedicines.com).

"Vision Medicines is developing promising ocular therapeutics for retinal diseases, including orphan diseases. In addition to VM200, Vision Medicines' pipeline includes VM100, a Phase 2/3-ready drug candidate for the treatment of geographic atrophy, one of the most severe forms of dry age related macular degeneration," said Dr. Varma. "These programs are in the highest growth areas of the ophthalmology marketplace, which we believe is ripe for consolidation. Due to significant inbound business interest, we are in the process of evaluating strategic options."

### **About VM200**

VM200 is an oral small molecule that works by neutralizing toxic chemicals produced during the visual cycle and thereby preventing subsequent retinal damage and cell death. In a preclinical Stargardt disease mouse model, VM200 preserved both retinal structure and function in a dose dependent manner without impairing the vitamin A visual cycle. VM200 has the potential to be used in all stages of Stargardt disease.

### **About Stargardt Disease**

Stargardt disease typically develops during childhood and adolescence. Progressive vision loss associated with Stargardt disease is caused by the death of photoreceptor cells in the central portion of the retina called the macula. The retina is the delicate light-sensing tissue lining the back inside wall of the eye. Photoreceptor cells in the retina provide vision by conveying information from the visual field to the brain. The macula is responsible for sharp central vision for tasks like reading, watching television, and looking at faces.

Decreased central vision is a hallmark of Stargardt disease. Eventually, virtually all patients who suffer from Stargardt disease will lose vision resulting in visual acuity in the range of 20/200 to 20/400, which is legal blindness. The vision loss is irreversible, and cannot be restored with prescription eyeglasses, contact lenses, or refractive surgery.

### **About Vision Medicines**

Vision Medicines, Inc. is a privately held, clinical-stage, biopharmaceutical company committed to

developing innovative therapies for patients suffering from blinding retinal diseases. Founded in 2013, the company is building a differentiated ophthalmology pipeline to treat retinal diseases, including orphan indications for which there are no treatments.

#### About Case Western Reserve University

Case Western Reserve University is one of the country's leading private research institutions. Located in Cleveland, Case offers a unique combination of forward-thinking educational opportunities in an inspiring cultural setting. Case's leading-edge faculty engage in teaching and research in a collaborative, hands-on environment. Case's nationally recognized programs include arts and sciences, dental medicine, engineering, law, management, medicine, nursing and social work. About 4,900 undergraduate and 5,900 graduate students comprise our student body. Visit [case.edu](http://case.edu) to see how Case Western Reserve thinks beyond the possible.

#### About Foundation Fighting Blindness

The [Foundation Fighting Blindness](http://www.foundationfightingblindness.org) is a national non-profit organization driving the research that will lead to preventions, treatments and cures for retinitis pigmentosa, macular degeneration, Usher syndrome and the entire spectrum of retinal degenerative diseases that affect more than 10 million Americans. Since 1971, the Foundation has raised more than \$600 million as the leading non-governmental funder of retinal research. Breakthrough Foundation-funded studies using gene therapy have restored significant vision in children and young adults who were previously blind, paving the way for additional clinical trials to treat a variety of retinal degenerative diseases. With a coveted four-star rating from Charity Navigator, the Foundation also has nearly 50 chapters that provide support, information and resources to affected individuals and their families in communities across the country.

#### Contact:

Jonae Barnes

*Senior Vice President*

Investor Relations and Corporate Communications

Vision Medicines

617-818-2985

[jonae@visionmedicines.com](mailto:jonae@visionmedicines.com)