Wills Eye Hospital Treats 1st U.S. Patient in New Gene Therapy RESCUE Trial

*Research Study and Treatment for early symptoms of genetic eye disease offers hope for wider group of patients with vision loss*

Wills Eye patient, David Esteves and his wife, Maria

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Philadelphia, PA- March 1, 2016 - [Wills Eye Hospital](https://www.willseye.org) today announced it has treated the first RESCUE trial patient in the United States enrolled in an FDA-approved gene therapy vision research study. This represents a significant new
chapter in the potentially wider use of gene therapy for treating, curing or even reversing certain cases of vision loss. The hope for the 50 year old patient, who has a vision-threatening genetic disease called Leber’s Hereditary Optic Neuropathy (LHON) is that his eye-injection will correct the defective gene that is causing his severe vision loss and either stops the progression of the disease or reverses the damage and improves his sight.

Leber’s Hereditary Optic Neuropathy is a rare, genetic eye disease, passed on by the mother, affecting 35,000 patients worldwide. It causes the optic nerve to atrophy resulting in sudden but painless central vision loss in predominantly young men. Symptoms include quick onset of severe blurry vision. Because the patients are generally in good health and seeing well up until the first symptoms occur, the fast onset of vision problems can make it a mystery to the patient; prolonging an accurate diagnosis and early treatment.

Although the trial, referred to as the RESCUE and REVERSE studies, is for this rare disease, if successful, it opens the door for future patients with other daunting eye diseases where no cures presently exist. In gene therapy, doctors deliver healthy “working copies” of a mutated gene into the patient’s DNA. Vision related disorders are considered ideal candidates for gene therapy because the genetic material can be injected right into the eye and in a sense, “re-wire” or lower the patient’s risk for getting the disease. The RESCUE study targets patients within the first six months of symptoms. Patients in the REVERSE study receive treatment within 6-12 months of symptoms. The Wills Eye patient is the first to be injected in the RESCUE study.

“Hopefully, in this trial, we’ll be able to prevent visual loss if we treat people early enough in the process,” said trial investigator, Dr. Mark Moster, a neuro-ophthalmologist at Wills Eye Hospital.

GenSight Biologics S.A. is a clinical stage biotechnology company based in Paris discovering and developing novel therapies for neurodegenerative retinal diseases. The product, GS010, is administered by a single intravitreal injection to the eye – for the trial, only one eye will be treated with GS010, while the other eye will receive a “sham” injection. “GenSight is thrilled to have participation of Wills Eye Hospital as we enter the final stages of development for GS010. Dr. Mark Moster and Dr. Julia Haller’s expertise will be invaluable as GenSight attempts to show the ability of GS010 to stop and potentially restore the brutal
vision loss caused by LHON, said - Bernard Gilly, PhD, Chairman and Chief Executive Officer of GenSight Biologics.

Wills Eye Hospital consistently ranks as one of America’s top ophthalmology centers in US News & World Report’s annual Best Hospitals rankings each year. Established in 1832 as the nation’s first hospital specializing solely in eye care, Wills Eye is a premier training site for all levels of medical education. Its resident and post-graduate training programs are among the most competitive in the country. Wills provides the full range of primary and subspecialty of eye care for improving and preserving sight, including cataract, cornea, retina, emergency care, glaucoma, neuro-ophthalmology, ocular oncology, oculoplastics, pathology, pediatric ophthalmology, ocular genetics and refractive surgery. Twitter: @wills_eye

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