FOUNDATION FIGHTING BLINDNESS

STARGARDT DISEASE
RESEARCH ADVANCES

Gene Therapy Clinical Trial Underway for Stargardt Disease

The first-ever gene therapy clinical trial for Stargardt disease is underway at Oregon Health & Science University's (OHSU) Casey Eye Institute in Portland and the Hopital Nationale des Quinze- Vingt in Paris, France. Known as StarGen™, the treatment, which replaces mutated copies of the gene ABCA4 with healthy copies, is being developed by the pharmaceutical company Sanofi. The Foundation Fighting Blindness funded many of the pivotal lab studies that are making the StarGen gene therapy clinical trial possible.

Foundation Partnering with Vision Medicines to Develop Pharmaceutical Therapy

The Foundation and the biopharmaceutical company Vision Medicines are partnering to develop a drug known as VM200 for preserving vision in people with Stargardt disease. The Foundation is investing as much $7.5 million to co-fund VM200 development. Vision Medicines plans to launch a human study of the emerging therapy in 2016. VM200 neutralizes toxic chemicals in the retina that are responsible for progressive retinal damage in Stargardt disease.

Stem Cell Clinical Trial Launched for Stargardt Disease

Ocata Therapeutics (formerly ACT) is conducting human studies of a retinal degenerative disease treatment derived from human stem cells. The Phase I/II clinical trial evaluated the treatment in people with Stargardt disease and dry AMD. Early results were encouraging thus far; vision improvements have been reported for some participants. The studies are taking place at multiple sites in the U.S. and the U.K.

Ocata's treatment involves the transformation of human stem cells into retinal pigment epithelial (RPE) cells. RPE cells degenerate in several retinal conditions, including Stargardt disease. RPE cells provide essential supportive functions for photoreceptors, the cells that provide vision. By placing healthy RPE cells in the retina, researchers believe they can save photoreceptors and slow or halt vision loss. The Foundation Fighting Blindness funded decades of cellular research that is making Ocata's trial possible.

Promising Dry AMD Treatment May Benefit People with Stargardt Disease

An oral medication for the dry form of AMD is in a Phase IIb/III clinical trial after showing positive results for safety and effectiveness in prior clinical and preclinical studies. Developed by Acucela, the drug works by slowing the buildup of toxic waste products that lead to retinal degeneration in a number of retinal conditions, including Stargardt disease.

Gene Therapy Revives Cones Long After They Stop Working

A Foundation-funded research collaboration between the Institut de la Vision in Paris and the Friedrich Miescher Institute in Basel, Switzerland, is developing a gene therapy that revives degenerating cones, enabling them to regain their ability to respond to light and provide vision. The treatment also improves the health of cones and extends their lifespan significantly. Cones are the retinal cells that allow people to see color and fine detail, enabling them to drive, read and see the faces of loved ones. A key benefit of the approach is that it may help people affected by a range of conditions, including Stargardt disease and many forms of retinitis pigmentosa, because it works independently of the underlying disease-causing genetic defect. The collaboration's goal is to move the gene therapy into a clinical trial within three years.

Foundation Commits $2 Million to Development of a Cross-Cutting Drug Treatment

The Foundation Fighting Blindness has invested more than $2 million in MitoChem Therapeutics, a start-up company which, thanks to prior Foundation support, has identified compounds that appear to boost mitochondrial function and show potential for significantly slowing vision loss caused by a variety of retinal degenerations. Mitochondria are the power supplies for all cells. The goal is to determine which compound will work best in people and move it into a clinical trial.

Stargardt Disease Natural History Study Will Help Prepare for Future Clinical Trials

The Foundation Fighting Blindness Clinical Research Institute has launched a natural history study of people affected by Stargardt disease. Known as ProgSTAR, this study has three primary goals: 1) Determine the best outcome measures to accelerate evaluation of emerging treatments, 2) Better understand disease progression for selecting future clinical trial participants, and 3) Identify potential participants for forthcoming clinical trials. The study has enrolled approximately 250 patients in 10 international clinical centers.