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 is being developed by Oxford BioMedica, a biopharmaceutical company in the U.K. which also has gene therapy products for wet age-related macular degeneration (AMD) and Usher syndrome in clinical development. The Foundation Fighting Blindness funded many of the pivotal lab studies that are making Oxford's gene therapy clinical trials possible.

Stem Cell Clinical Trial Launched for Stargardt Disease

Advanced Cell Technology (ACT) is conducting human studies of a retinal degenerative disease treatment derived from human stem cells. The Phase I/II clinical trials are evaluating the treatment in people with Stargardt disease and dry AMD. Early results are 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. ACT'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 ACT'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 is giving $2 million to 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 is launching 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 will recruit approximately 300 patients from 10 international clinical centers.

The Foundation publishes frequent updates on the latest advancements in research and clinical trials for Stargardt disease and similar diseases.

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