

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 Institut de la Vision in Paris, France. 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 Stargardt disease gene therapy clinical trial possible.

Research Team Is Creating Patch Derived from Stem Cells to Restore Vision

David Gamm, MD, PhD, at the University of Wisconsin–Madison, is leading a team of experts to create a patch composed of retinal cells derived from stem cells as a vision-restoring therapy for people with macular conditions such as Stargardt disease. The company Opsis Therapeutics was recently formed to advance the therapy into and through a clinical trial and out to the patients who need it. The patch consists of two layers. One layer will serve as a precursor to vision-enabling photoreceptors. Once transplanted, the stem cells of this layer

will mature into photoreceptors. The other layer will consist of mature retinal pigment epithelial (RPE) cells, which provide waste disposal and nutrition for photoreceptors. A thin plastic film will serve as a structural backbone for the patch. A biodegradable gel will protect the cells and hold the layers together. The Foundation Fighting Blindness currently funds the project and has funded Dr. Gamm for more than a decade to advance this therapeutic approach.

Clinical Trial of Stem-Cell Derived Therapy Launched for Stargardt Disease

Ocata Therapeutics (formerly Advanced Cell Technology) has conducted human studies of retinal pigment epithelial (RPE) cells derived from human stem 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. Ocata's Phase I/II clinical trial evaluated the treatment in people with Stargardt disease and dry AMD. Early results were encouraging; vision improvements were reported for some participants. The studies

Stargardt Disease: Research Advances, Continued

were conducted at multiple sites in the U.S. and the U.K. The pharmaceutical company Astellas recently acquired Ocata. Details of a Phase III trial have not yet been announced. The Foundation funded several research efforts that made this potential therapy possible.

Acucela Launches Clinical Trial of Drug for Stargardt Disease

Emixustat, an oral drug which originally targeted the dry form of age-related macular degeneration (AMD), is moving into a Phase II clinical trial for Stargardt disease. While the drug did not meet desired outcomes in the AMD clinical trial, experts believe it may work well for people with Stargardt disease. 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.

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

The Foundation Fighting Blindness Clinical Research Institute is conducting a \$6 million natural history study for people affected by Stargardt disease. Known as ProgSTAR, the study has three primary goals: 1) Determine the best outcome measures to accelerate evaluation of emerging treatments in clinical trials, 2) better understand disease progression for selecting future clinical trial participants, and 3) identify potential participants for forthcoming clinical trials. The study has enrolled 365 patients in 10 international clinical centers.