FOUNDATION FIGHTING BLINDNESS

RETINITIS PIGMENTOSA RESEARCH ADVANCES

FDA Approves Spark's Vision-Restoring Gene Therapy

Spark Therapeutics' vision-restoring RPE65 gene therapy has received marketing approval from the U.S. Food and Drug Administration, becoming the first gene therapy to gain regulatory approval in the U.S. for the eye or any inherited condition. Known as LUXTURNA™ (voretigene neparvovec), the gene therapy restored vision in a clinical trial for people between the ages of 4 and 44 with Leber congenital amaurosis (LCA) caused by mutations in the gene RPE65. Study participants with severe vision loss reported putting away their navigational canes, seeing stars, being able to read, and recognizing faces of loved ones. Vision restoration has persisted for at least three years. The treatment is also designed to work for people with retinitis pigmentosa (RP) caused by RPE65 mutations. FFB invested about $10 million in more than a decade of lab research that made possible the RPE65 gene therapy clinical trial at the Children's Hospital of Philadelphia (CHOP).

jCyte Stem-Cell Therapy Moves into Phase IIb Clinical Trial for RP

The stem-cell therapy company jCyte is launching a Phase IIb clinical trial of its therapy for people with retinitis pigmentosa (RP). The 85-participant study is being led by Henry Klassen, MD, PhD. The treatment involves intravitreal injection of retinal progenitor cells (RPCs), which are stem cells that have partially developed into the retinal cells that make vision possible. Based on lab studies, researchers believe the treatment can preserve and potentially rescue the patient's existing photoreceptors, thereby saving and possibly restoring vision. Administration of the treatment does not require surgery and can be performed in minutes in an outpatient setting. The RPCs are injected into the vitreous, the gel-like substance in the middle of the eye. Twenty-eight patients were enrolled in the safety-oriented Phase I/IIa trial for the treatment, which began in June 2015. Dr. Klassen says safety results from that trial have been encouraging.

ReNeuron's Stem-Cell Therapy Performs Encouragingly in Phase 2 Clinical Trial

ReNeuron, a stem-cell development company in the United Kingdom, has reported that three patients with retinitis pigmentosa (RP) have been treated with its stem-cell therapy in its Phase 2 clinical trial taking place at Massachusetts

Eye and Ear Infirmary (MEEI). All have shown vision improvements shortly after treatment. The treatment involves the injection of human retinal progenitor cells (hRPCs) — stem cells that have partially developed into photoreceptors — underneath the patient's retina. Based on results from preclinical studies, researchers believe the injected hRPCs will integrate into the retina and fully develop into photoreceptors, replacing those lost to disease, thereby restoring vision. FFB funded previous research that made this trial possible.

Optogenetic Therapy for Advanced RP in Clinical Trial

RetroSense Therapeutics reported that three participants have received injections of its potential optogenetic therapy, known as RST-01, in a Phase I/II clinical trial. The patients were given the lowest dose of RST-01, and no adverse ocular events were observed. The treatment is designed to provide vision to people who are completely blind from conditions such as retinitis pigmentosa and Usher syndrome. The therapy works by bestowing light sensitivity to ganglion cells in patients who have lost all of their photoreceptors. The Foundation funded earlier lab research that helped make this clinical trial possible. In September 2016, RetroSense was acquired by the pharmaceutical company Allergan.

XLRP Gene Therapy Clinical Trial Begins

A 29-year-old British man is the first person to be treated in a gene therapy clinical trial at the University of Oxford (U.K.) for X-linked retinitis pigmentosa (XLRP). The trial is being run by Nightstar, a biopharmaceutical company in the U.K. developing therapies for inherited retinal diseases. As many as 24 patients will be enrolled in the 12-month trial. The Nightstar gene therapy involves injection of healthy copies of RPGR underneath the retina. The RPGR copies are contained in a human-engineered virus — known as an adeno-associated virus or AAV — which is designed to readily penetrate retinal cells to deliver the therapeutic genetic cargo. While the current clinical trial is focused on evaluating safety, researchers hope that a single injection of the XLRP gene therapy will slow or halt vision loss for several years.

Horama Launches Gene Therapy Clinical Trial for RP (PDE6B mutations)

The French biotech Horama reported that three people have been treated in its Phase I/II gene therapy clinical trial for people with retinitis pigmentosa (RP) caused by PDE6B mutations. The three-year trial taking place at University Hospital of Nantes in France will enroll a total of 12 patients.

Company Formed to Advance RP Drug into a Clinical Trial

The Foundation Fighting Blindness Clinical Research Institute (FFB-CRI) announced an investment of up to $7.5 million to advance a promising, emerging drug for retinitis pigmentosa (RP) into and through a Phase II clinical trial. Known as N-acetylcysteine-amide (NACA), the molecule is designed to slow vision loss by protecting retinal cells from oxidative stress. In previous FFB-funded lab studies at Johns Hopkins University, NACA slowed retinal degeneration in rodent models of RP.

SparingVision Formed to Advance Sight-Saving Protein for RP

The development of a vision-saving treatment for people with retinitis pigmentosa (RP) is getting a major boost thanks to the formation of the French biotech SparingVision to move it into a clinical trial and out to the international marketplace. A spin-off of the Institut de la Vision, SparingVision was established to clinically develop and commercialize a protein known as rod-derived cone-viability factor (RdCVF). The emerging therapy performed well in several previous lab studies funded by the Foundation Fighting Blindness. Scientists demonstrated that RdCVF prevented or slowed the degeneration of cones, the cells in the retina that provide central and color vision and enable people to read, drive, and recognize faces. RdCVF is naturally secreted by rods, the retinal cells that provide night and peripheral vision.