Uploaded to the VFC Website

2016

This Document has been provided to you courtesy of Veterans-For-Change!

Feel free to pass to any veteran who might be able to use this information!

For thousands more files like this and hundreds of links to useful information, and hundreds of "Frequently Asked Questions, please go to:

Veterans-For-Change

If Veterans don't help Veterans, who will?

Note: VFC is not liable for source information in this document, it is merely provided as a courtesy to our members & subscribers.

Study shows children with rare eye disease have greatest benefit from gene therapy

Published on April 18, 2016 at 1:54 PM

Scientists at Oregon Health & Science University's Casey Eye Institute and Baylor College of Medicine's Cullen Eye Institute published findings from a two-year Phase I clinical trial in the journal *Ophthalmology*, which showed that children had the greatest benefit from gene therapy for treatment of Leber congenital amaurosis (LCA) or severe early childhood onset retinal degeneration (SECORD). Importantly, 9 of the 12 participants experienced improvement in visual function. LCA and SECORD are related inherited retinal degenerative diseases that cause severe loss of vision in infancy due to mutations in the gene RPE65.

"While other studies have shown similar results, an important finding from this study is that the young patients saw the greatest benefit in treatment. In addition, this study for the first time demonstrates improvement in visual field by using visual field modeling and assessment," said Timothy Stout, M. D., Ph.D., M.B.A., a senior author and study surgeon who previously worked at OHSU Casey Eye Institute and is now director of the Baylor Cullen Eye Institute.

The study included 8 adults and 4 children ages 6 to 39, and showed that treatment for LCA and SECORD delivered by subretinal injection in the poorer-seeing eye, was safe, the primary outcome for Phase I clinical trials. Nine out of 12 patients experienced an improvement in visual acuity (sharpness of vision) or an improvement in visual field.

In order to more accurately measure visual field, the total area in which objects can be seen (both central and side vision), Richard Weleber, M.D. of OHSU's Casey Eye Institute, senior author and professor of ophthalmology in the OHSU School of Medicine, Casey Eye Institute developed a novel quantitative visual field analytic tool, called Visual Field Modeling and Assessment.

"I am very optimistic about these study findings," said Weleber. "Gene replacement therapy has proven to be the most promising method to halt progression of childhood blindness due to single gene defects. The eye is a perfect platform for gene therapy because we can treat one eye to see how it responds compared to the other eye."

Gene therapy is a new approach to treating rare genetic diseases and has shown a lot of promise in treating rare genetic eye diseases such as LCA due to mutations of the RPE65 gene. For this study, surgeons used a viral vector to insert a normal copy of the mutated gene (RPE65) into the retinal cells of the patient's eye to restore retinal function.

OHSU Casey Eye Institute's gene therapy program is world-renowned, with leading experts currently overseeing five gene therapy trials, the most retinal gene therapy clinical trials being conducted at a single institution. Patients travel from all over the world to participate in these groundbreaking studies targeting severe inherited blinding disorders.

"Treatments for childhood blindness have profound effects because these children have their whole lives ahead of them," said David Wilson, M.D., director of the OHSU Casey Eye Institute, chair of the Department of Ophthalmology in the OHSU School of Medicine and study co-author. "Demonstrating the effectiveness of gene therapy in the eye will have broad implications for the rest of medicine as well."

Source:
Oregon Health & Science University
