UW MEDICINE EYE INSTITUTE AND THE HEAR SEE HOPE FOUNDATION:

Restoring Vision Loss Caused by Usher Syndrome

"Advancing vision research is so important to us, because two of our sons — Conner and Dalton — have Usher syndrome. And, we want to be able to tell them they will not go blind."

– Lane and Todd McKittrick, Co-founders, Hear See Hope Foundation

IT IS ESTIMATED THAT BETWEEN 30,000 AND 50,000 PEOPLE in the United States suffer from Usher syndrome, a genetic disorder that causes deafness and blindness. Children with Usher syndrome are born with, or may develop, hearing loss. Then, as these children get older, they develop night blindness and their visual field narrows, creating "tunnel vision." There are currently no treatments for Usher syndrome, but stem cell therapy holds great promise for restoring sight and benefiting patients worldwide.

The UW Medicine Eye Institute, the only academic Eye Institute in the five-state WWAMI region (Washington, Wyoming, Alaska, Montana and Idaho), is at the frontier of developing new techniques to restore vision loss utilizing stem cell therapy. Building on a depth of research expertise, clinicians and scientists at our institute have partnered with the Hear See Hope Foundation to develop treatments for vision loss caused by Usher syndrome. We welcome your partnership to advance this innovative research.

Advancing Stem Cell Therapy

Jennifer Chao, M.D., Ph.D., assistant professor in UW Medicine's Department of Ophthalmology, utilizes a special technique to create undifferentiated stem cells from a patient's own skin or blood cells. These stem cells, called induced pluripotent stem cells (iPSCs), contain a person's entire genome and can be programmed to become almost any other cell in the body. Dr. Chao, in collaboration with Tom Reh, Ph.D., professor in the Department of Biological Structure, converts these iPSCs into retinal cells that are genetically identical to those found in the patient's eye.

Once the iPSCs have been grown into millions of copies of patient-specific retinal cells, high-throughput screening technology tests thousands of compounds on the cells in an effort to determine which of these compounds help the cells to survive. This process, called "cell rescue," is a groundbreaking technique that could help identify already FDA-approved drugs capable of stopping or slowing the progression of vision loss in patients with Usher syndrome or other retinal degenerative diseases.

While animal models exist for many other degenerative retinal diseases, this is not the case for Usher syndrome. This means that traditional basic research techniques used to test hypotheses in animal models cannot be performed for Usher syndrome. However, new techniques are being developed at UW Medicine to produce improved patient-derived cell culture models of Usher syndrome and other degenerative diseases to accelerate research.

With UW Medicine's streamlined approach, and our strong partnership with the Hear See Hope Foundation, we believe an effective cure will soon be made available to patients.

A Partnership with a Vision

Hear See Hope Foundation
The Hear See Hope Foundation exists to support Usher syndrome research and awareness. Through excellent focus, we can create, communicate and gain knowledge of this currently incurable retinal disorder.
hearseehope.com

UW Medicine Eye Institute
The UW Medicine Eye Institute is one of the nation's premier eye and vision science centers for clinical care, research and teaching. The Eye Institute is dedicated to the mission of eliminating suffering from eye disease.
ophthalmology.washington.edu

The Potential of Cell Rescue — From Concept to Cure

In 2013, the Chao Lab performed the first high-throughput screening of stem cells from patients with age-related macular degeneration (AMD). This proof-of-concept study demonstrated promising results: multiple compounds were found to have therapeutic effects, facilitating cell rescue. We are now in the process of understanding the biological mechanisms of the cell rescue, as well as the dosing of potential therapeutic compounds.

In 2015, with support from the Hear See Hope Foundation, the UW Medicine Eye Institute will begin using this same method to test stem cells derived from Usher syndrome patients.

Opportunities to Invest

We can accelerate the pace of research aimed at slowing or stopping vision loss in Usher syndrome patients with your philanthropic investment. Support of the UW Medicine Eye Institute will advance:

- Research associated with collecting and growing millions of Usher syndrome patient-specific stem cells, as well as use of the high-throughput screening equipment to test potential treatments. **$500,000**
- New studies of the mechanisms, dosing and bioavailability of potentially therapeutic compounds identified through high-throughput screening. **$500,000**
- Implementation of advanced imaging technology — available only at UW Medicine — for understanding the retinal degeneration caused by Usher syndrome and assessing the effectiveness of new treatments. **$150,000**
- Devoted Usher syndrome research support via part-time or full-time post-doctorate fellows. **$40,000 to 60,000 per year**

Join Us

By investing in our partnership, you can advance groundbreaking research that will bring hope for a cure to the thousands of children and individuals suffering from Usher syndrome worldwide.

To learn more, please contact Abbey Norris, director for philanthropy at UW Medicine, at 206.221.8274 or abbeyn@uw.edu. Thank you for your interest.