Odylia Therapeutics is a nonprofit (501c3) biotech working to accelerate the development of treatments for people with rare diseases. We work to change the way therapies are brought from the lab to patients regardless of prevalence or commercial interest. Odylia can change the lives of those with rare diseases by de-risking the most promising therapeutic approaches and streamlining drug development through strategic partnerships.

Usher Syndrome Type 1C (USH1C)

Usher Syndrome, the most common genetic form of deaf-blindness, is broken down clinically into three major types based on disease severity, age of disease onset, and causative genetic mutation. Usher Syndrome types 1 and 2 account for the highest number of cases and are known to be caused by mutations in a number of different genes. The focus of Odylia's gene therapy program is Usher Syndrome type 1C (USH1C).

- Usher Syndrome Type 1C- progressive loss of vision begins in childhood
- Vision loss caused by Retinitis Pigmentosa first presents with night blindness then progresses to tunnel vision, eventually leading to blindness
- There is currently no cure for Usher Syndrome Type 1C

USH1C is characterized by deafness at birth, vestibular (balance) problems, and prepubertal onset of Retinitis Pigmentosa. While Usher Syndrome type 1 can be caused by mutations in several different genes, USH1C is caused by inheritance of mutations in both copies of the USH1C gene. The USH1C gene encodes the protein Harmonin, which is an anchoring and scaffolding protein known to be important for normal development of cochlear hair cells and hearing. While it is known that mutations in this gene also cause vision and vestibular dysfunction, the underlying molecular changes remain poorly understood.

Gene Therapy for USH1C

To address the lack of treatments for this disease, Odylia is developing a gene therapy to prevent vision loss caused by mutations in the USH1C gene. In collaboration with researchers at Johannes Gutenberg-University Mainz, Technical University Munich, Academy of Sciences of the Czech Republic, and the University of Tübingen, Odylia is testing the therapeutic potential of a subretinal gene therapy in a genetic model of Usher Syndrome Type 1C. By teaming up with the Usher 2020 Foundation and the FAUN Foundation, Odylia is providing hope to those affected by USH1C.

Together we can prevent vision loss

Patients diagnosed with USH1C and their families may like additional information. Odylia has a webpage devoted to the USH1C program. Additionally, periodic emails are sent with updates. You can direct patients to our website to find out more about the program and the latest advancements. Visit the Research & Services section on Odylia.org. To sign up for updates and newsletters, visit the Get Involved section of our website or email info@odylia.org.