

# Usher 2020 Foundation

## Bringing Hope thru Science



For over a decade, Usher 2020 Foundation has been funding research to help bring treatments to those losing their vision because of Usher syndrome and Retinitis Pigmentosa (RP). Our work has led to a successful stage 2 study of a medication to slow the loss of central vision in those with RP, and today, Usher 2020 is the only patient-advocacy organization to support a multi-locational Clinical Trial to study the efficacy of N-Acetylcysteine, also known as NAC, in slowing the degeneration of sight caused by RP. We strongly believe this will result in a standard of care for people with RP, and the first hope for maintaining vision.

### *Science isn't Our Goal*

At Usher 2020, we fund research that will lead to treatments for patients. Over the years, we have:

- Created a **novel vector for the delivery of gene therapy** treatments to the retina.
- Created the **first-ever large animal model of Usher syndrome** that allows the study of treatments in a model that more closely resembles the human eye.
- Created a **retinal organoid from Usher patients** to study the disease mechanisms.
- Co-founded **Odylia Therapeutics**, a non-profit biotech working to bring rare disease treatments to patients.
- Continue the work of a **Natural History Study of those with Usher syndrome** to understand the progression of the disease and the possibility of effective treatments.
- Collaborate on the study of a **gene therapy to slow the progression of vision loss** in a large animal model with the intention of going to a clinical trial by 2025.

### *Never Lose Sight*

We won't stop until we have a treatment to help those with Usher syndrome maintain their vision. For more information, contact us at [info@usher2020.org](mailto:info@usher2020.org). See our website at [www.usher2020.org](http://www.usher2020.org).

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