ILLUMINATING THE FUTURE

Canada Invests in Sight and Science

USHER 1F COLLABORATIVE

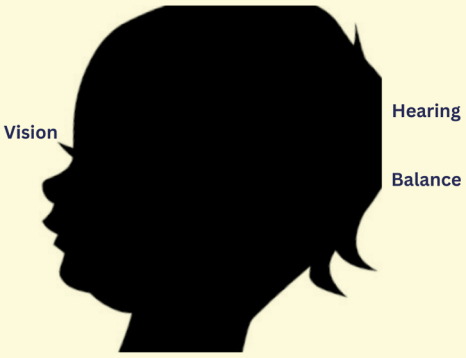
WHAT IS USHER SYNDROME TYPE 1F?

Imagine finding out that your newborn baby is profoundly deaf. You mourn, and then you learn about cochlear implants. A few years later, he/she is thriving, hearing and speaking, attending school with his/her neighborhood friends. His/her balance is off, though, and he/she was late to sit and walk. Your intuition tells you that something is still not right, and genetic testing confirms your fears, revealing a devastating diagnosis, Usher syndrome type 1F. Your child is not only deaf but is also slowly going blind.

Usher Syndrome is the leading cause of inherited deaf-blindness. Type 1 is the most severe, with affected children born profoundly deaf with poor balance. During childhood, they also begin to gradually lose their vision, first with night-blindness followed by ever narrowing tunnel vision, until total blindness results during adulthood.

Approximately 400,000 people worldwide suffer from Usher syndrome, a rare genetic disease, with Type 1 being the most severe. Types 1 and 2 account for over 90% of those with this debilitating combination of disabilities.

Usher Syndrome



Usher syndrome is the most common genetic cause of combined deafness and blindness

Click here for an example of vision loss.

THE UNMET NEED

Currently, there is no cure for Usher Syndrome. While cochlear implants have greatly ameliorated the most major impacts of deafness for those who receive them as babies, there is no cure for blindness. Early identification and educational programs provide support to those affected, but in a world teeming with major technological advances in medicine, this simply isn't good enough. Targeted research is needed to enable a cure.

What is the Current State of Research for a Cure

In 2013, Usher 1F Collaborative in the United States became the first charitable foundation to address this unmet need. In the eleven years since its founding, a total of USD$14 million has been invested in research for a cure, with successes realized in the development in the lab of both gene and drug therapies, though not yet with any advanced to human testing.

In 2022, recognizing the efforts of patients and their families in Canada, as well as the country's wealth of scientific research expertise, Usher 1F Collaborative Canada was founded as a charitable foundation in Canada.

WHY NOW?

If not us, then who will tackle this vital challenge? The stakeholders must lead this. We cannot be reliant solely on government or biopharma. For example, recent cuts in funding to the National Institutes of Health in the U.S., which have set back the field of medical research, are a perfect example of why we need to take charge.

While biopharmaceutical companies in the private sector might expand their investments due to public sector cuts, it is likely they will not focus on rare disease research. This is because the return on investment is low, with fewer opportunities to monetize with a small patient population. This does not diminish the need though, and there is a high likelihood that a therapy for Usher 1F could translate to therapies for blindness in other forms of Usher syndrome and beyond. This presents an opportunity for Canada to step up and take the lead, to change the lives of those with Usher 1F.

Finally, the time is right with the launch of the National Strategy for Drugs for Rare Diseases, first announced in March 2023 and just signed by every province as of April 2025. With all provinces and territories now on board with recognizing the need for treatments for rare diseases and committed to investing in innovation, ***the time to act is now.***

Area of Need

One particular area of need is the screening of existing drugs for efficacy in Usher 1F patients. Our fastest path to the clinic would be to repurpose existing drugs with a proven safety track record. Thus, we are launching a request for proposals across Canada, providing Canadian research scientists with an opportunity to help bring an effective treatment to patients as quickly as possible.

To make this a reality, we must raise a minimum of $100,000 in the next six months in order to launch a request for proposals by the end of 2025.