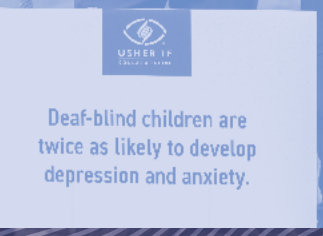


USHER 1F COLLABORATIVE

USHER 1F COLLABORATIVE

ANNUAL REPORT

2025

2025

YEAR IN REVIEW

2025 marked the twelfth year since our founding, a year in which we continued to grow as an organization while accelerating research toward a cure. Most notably, our outreach now extends well beyond the United States, welcoming patients and families from Australia, Canada, Europe, Israel, and South America. Our community now includes thirteen European families from France, Germany, Poland, Spain, Norway, and Sweden, as well as a unique extended family in Uganda, where 16 family members have recently been confirmed to have Usher syndrome type 1F. A map illustrating our expanding global reach is available on our website.

We also strengthened our presence and reputation within the Usher syndrome research community. At the International Symposium on Usher Syndrome, held in the Netherlands in June 2025, Usher 1F was featured in multiple presentations, most notably a presentation by Maryna Ivanchenko, MD, PhD, of the Corey Lab at Harvard Medical School, highlighting the success of our Usher 1F mini-gene in preclinical testing.

Another significant milestone in 2025 was the successful establishment of a dedicated ICD-10 code for Usher syndrome, including subcodes for types 1, 2, and 3. As the worldwide standard for disease diagnosis and the national standard used in U.S. health insurance coverage, this designation provides long-overdue recognition and legitimacy for Usher syndrome patients. It enables improved insurance coverage, more coordinated care, easier identification for research and clinical trials, and a stronger foundation for securing public health funding.

Together, these achievements reflect meaningful progress toward our mission to accelerate the path to a cure for Usher syndrome type 1F.

Maryna Ivanchenko, MD, PhD, presents work on our Usher 1F mini-gene at the International Symposium on Usher Syndrome

Zachary participating in the RUSH1F natural history study

Milestones In

RESEARCH

In 2025, Usher 1F research moved from promising science toward real-world therapeutic readiness

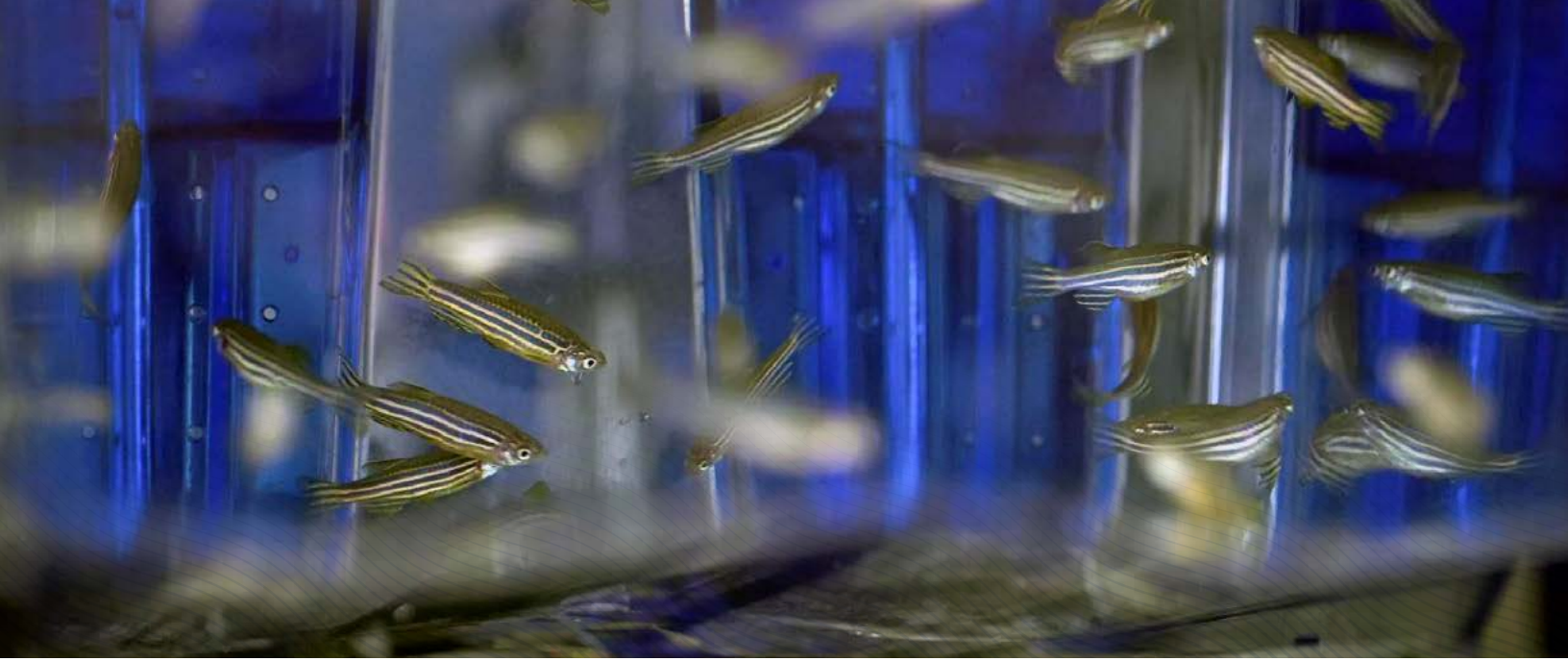
Harvard Medical School

David Corey, PhD, and Maryna Ivanchenko, MD, PhD

The Corey Lab continues to advance promising research toward a gene therapy for Usher syndrome type 1F. In 2025, the team published a peer-reviewed study, *Mini-Pcdh15b Gene Therapy Rescues Visual Deficits in a Zebrafish Model of Usher Syndrome Type 1F*, demonstrating that their Usher 1F mini-gene successfully restored vision in a zebrafish model of the disease. (PCDH15 is the gene associated with Usher 1F.)

At the annual international meeting of the Association for Research in Vision and Ophthalmology (ARVO), Dr. Ivanchenko presented two scientific posters on this work. One showed restoration of vision in an Usher 1F zebrafish model. The second, conducted in partnership with Spark Therapeutics, reported results from preclinical testing in nonhuman primates. These studies showed that delivery of the mini-gene to the retina was safe and well tolerated, achieved strong gene expression, and caused no retinal damage, an important step toward future clinical trials for patients with Usher 1F.

In addition, the Corey Lab has begun testing a second gene therapy approach for Usher 1F in collaboration with **Samuel Pfaff, PhD**, at the **Salk Institute for Biological Studies**. This innovative strategy splits the large Usher 1F gene into two parts and uses a cutting-edge technique called RNA End-Joining to reassemble the gene once inside the eye. Early results have been very promising, and Usher 1F Collaborative is funding further testing of this approach.



Zebrafish at the Westerfield Lab at the University of Oregon Institute of Neuroscience

Milestones In
RESEARCH

The University of Oregon Institute of Neuroscience
Monte Westerfield, PhD, and Jennifer Phillips, PhD

Our University of Oregon research team has developed multiple zebrafish models carrying human Usher 1F mutations that closely mirror the symptoms seen in patients, including hearing loss, balance challenges, and progressive vision loss. These models provide a powerful platform for testing potential therapies.

The current focus of this work is evaluating drug-based treatments that could slow vision loss. In partnership with a lab at Emory University, the team identified a highly promising new compound.

Because repurposing existing FDA-approved drugs could offer the fastest path to patients, we also partnered with **Unravel Biosciences** to conduct a large-scale drug screening effort. Results were received at the end of 2025, and plans are underway to test the most promising candidates in zebrafish models. Identifying a drug that slows vision loss could buy valuable time while gene therapies move toward the clinic.

Natural History Study

Our natural history study, RUSH1F, conducted in partnership with **Foundation Fighting Blindness**, is entering its final year. Some participants have already completed their final study visits, and final data analysis is expected in 2027. This study will provide essential information on the natural progression of vision loss needed to measure treatment effectiveness in future clinical trials.

Golf players enjoying our annual event in New Jersey

Milestones In
FUNDRAISING

Powered by the dedication of our most loyal supporters and an expanding community of new donors, Usher 1F Collaborative achieved a successful fundraising year in 2025. Through the combined efforts of our United States and Canadian foundations, we raised USD$924,598, advancing critical research in every funded lab and bringing a cure closer to reality.

Usher 1F Collaborative – United States

By the close of 2025, the *Seeing Forward* campaign reached an important milestone—its halfway point. Launched in June 2024, *Seeing Forward* is a three-year, $3 million major-gifts initiative designed to accelerate progress toward a cure. As of year-end, the campaign had raised $1,646,000, achieving 55% of its overall goal.

Our primary fundraising anchor once again was the annual Golf, Tennis & Pickleball Outing, featuring Cards and Mahjong, held in June at Mountain Ridge Country Club in West Caldwell, New Jersey. The event generated more than $320,000 in support of sight-saving Usher 1F research.

The Shapiro Fund, established in memory of Laurie and Gary Shapiro, also served as a powerful catalyst for fundraising. Created to honor the Shapiros' enduring legacy of unwavering support for the Usher 1F community and the mission to end the disease, the fund inspired generous giving. Momentum was further accelerated by a Giving Tuesday matching pledge, prompting an outpouring of donations in tribute to their family.

Canadian board member, Nicolas Forte, top right, and his family with Catch the Ace Chair Doug Anthony, bottom right

Milestones In
FUNDRAISING

Usher 1F Collaborative – Canada

In 2025, Usher 1F Collaborative Canada launched *Illuminating the Future*, a $100,000 targeted fundraising initiative. Following the identification of a Canadian scientist capable of screening existing drugs for potential repurposing to slow vision loss in individuals with Usher 1F, the campaign was created to fully fund this promising research project.

Our efforts gained new momentum through the Catch the Ace lottery. Usher 1F Collaborative Canada was selected by the Kin Club of Russell as a beneficiary of the program, allowing residents across Ontario to purchase weekly lottery tickets and direct their support to a charity of their choice. By the conclusion of the lottery series, $45,000 CAD was raised to advance Usher 1F research. We are pleased to share that Usher 1F Collaborative Canada has been selected once again as a beneficiary for the next round, beginning in 2026.

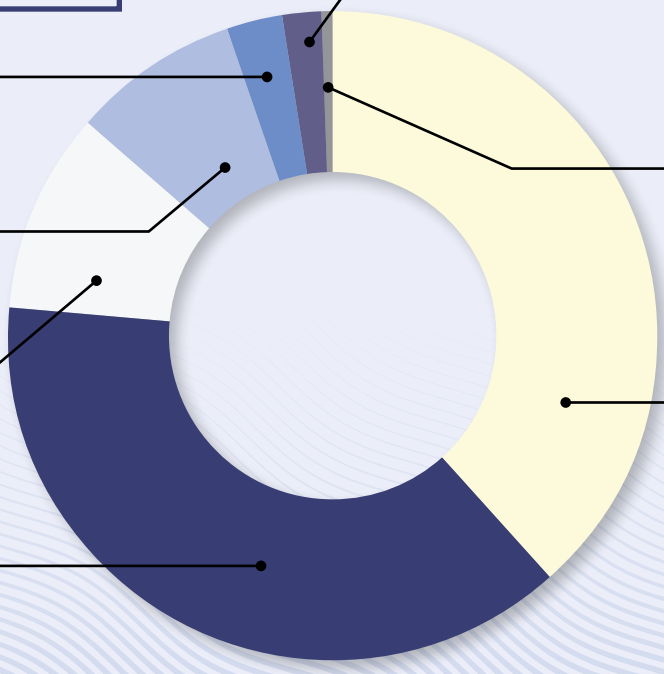
Usher 1F Collaborative – United States and Canada

Both our United States and Canadian foundations closed 2025 with a strong finish through successful Giving Tuesday and Year End Giving campaigns. Most notably, our donor community expanded in a powerful new way to include families from around the world. For the first time, families in Spain, France, and Germany created personal fundraising pages for Usher 1F, inspiring generosity and drawing support from across the globe.

FUNDING RAISED

Usher 1F Collaborative

Total: $901,680



Interest and Credit Card Rewards Earned
$16,418

In-kind Contributions
$3,525

Fundraising Events
$346,721

Individual Contributions
$343,869

Grants Received
$90,256

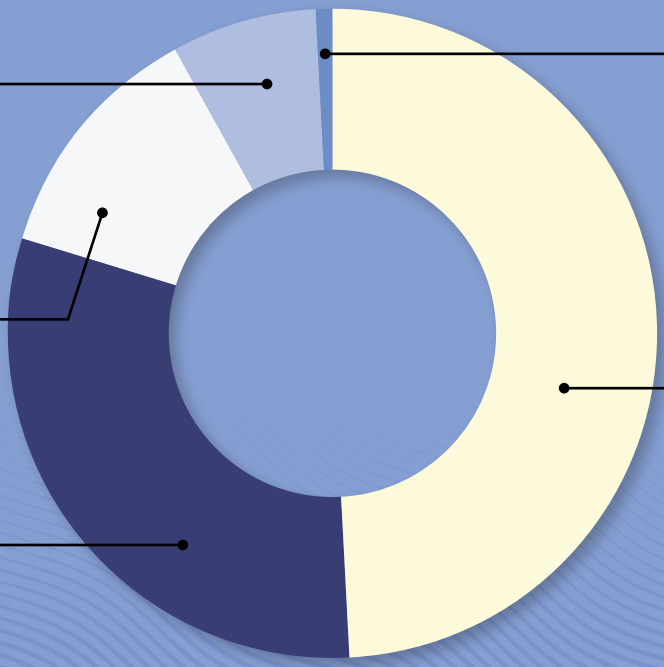
Grants received in 2025 pledged in 2024
$75,000

Corporate Contributions
$25,891

RESEARCH GRANTS AWARDED

Usher 1F Collaborative

Total Grants Given: $650,610



Usher Syndroom for International Symposium on Usher Syndrome
$5,000

Harvard Medical School, *David Corey, PhD, and Maryna Ivanchenko, MD*
$320,000

University of Oregon Institute of Neuroscience, *Monte Westerfield, PhD, and Jennifer Phillips, PhD*
$80,000

Foundation Fighting Blindness for RUSH1F Natural History Study
$200,000

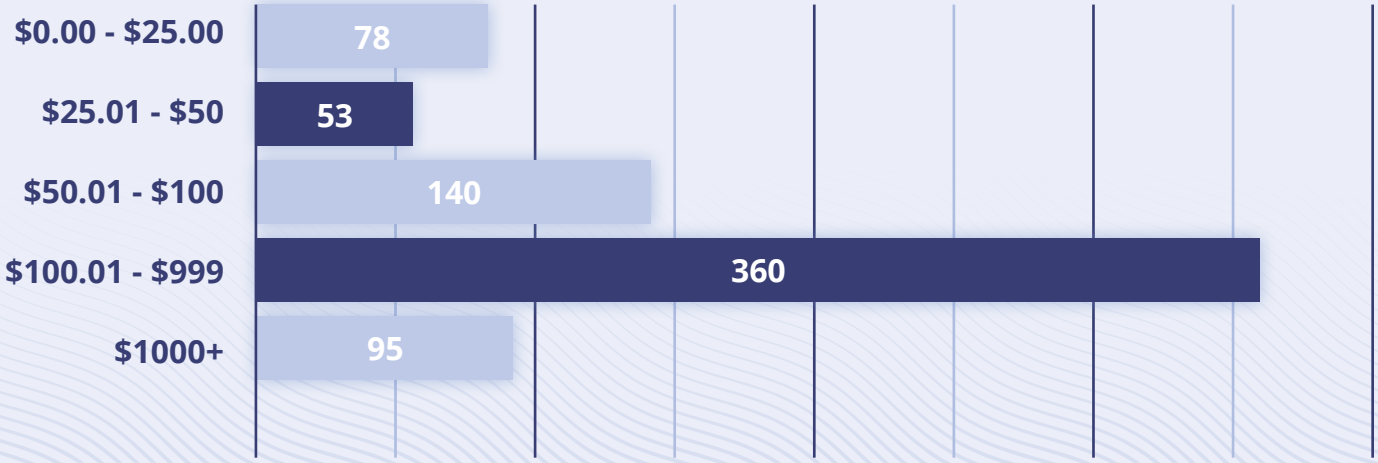
Unravel Biosciences for high throughput drug screen
$45,610

**Funds in US Dollars (USD)*

GIVING BREAKDOWN

Usher 1F Collaborative

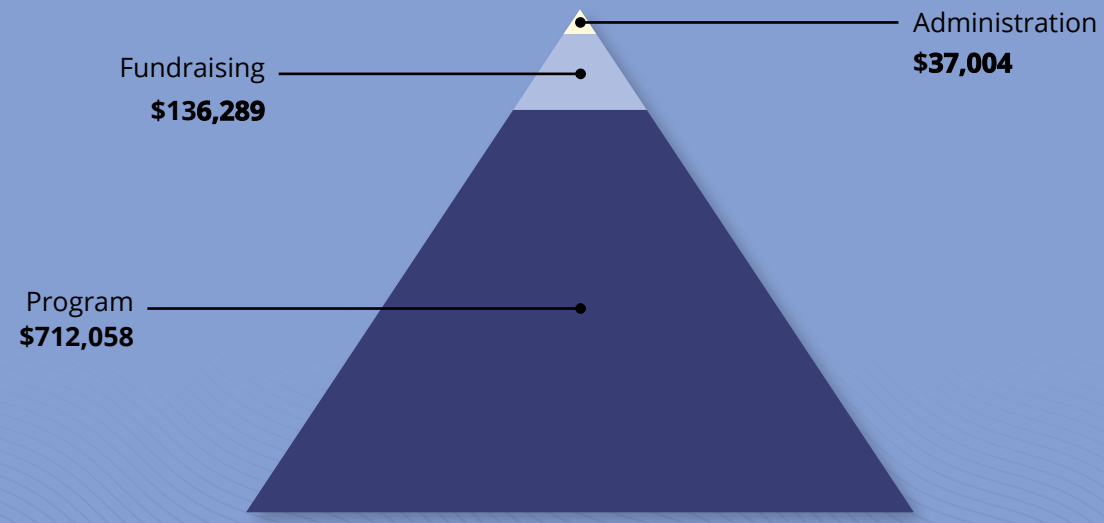
Total Number of Gifts: 726



EXPENDITURES BY FUNCTIONAL AREA

Usher 1F Collaborative

Total: $885,350



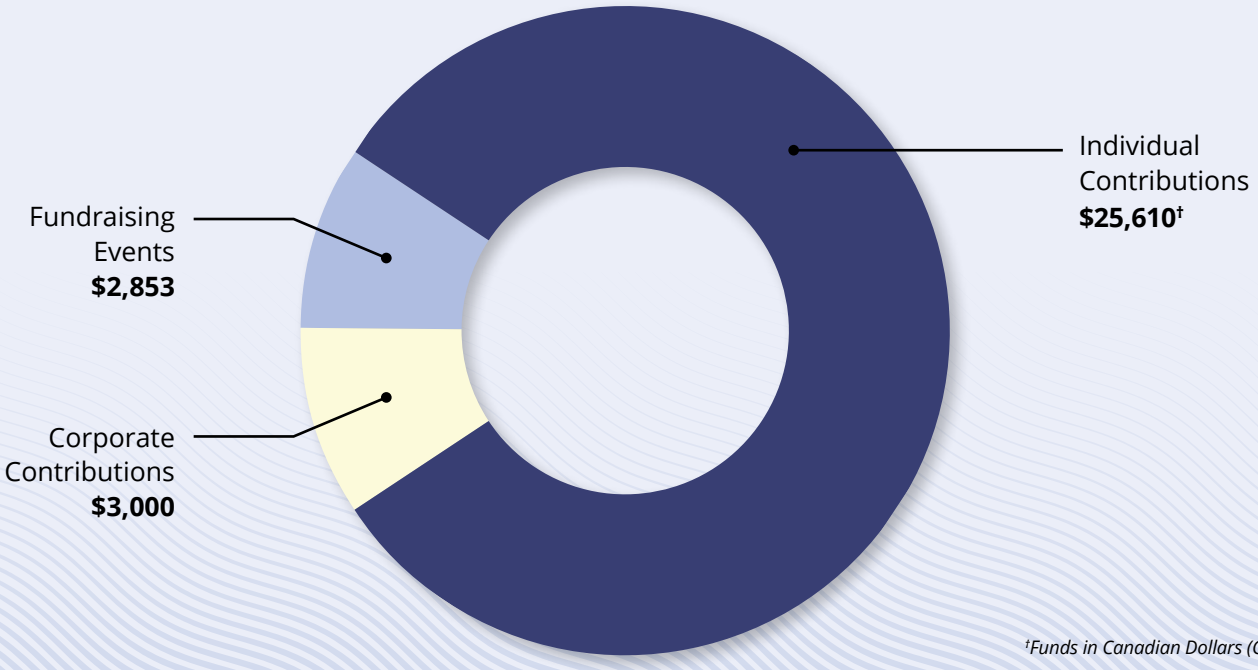
Total expenditures in 2025

$885,350

FUNDING RAISED

Usher 1F Collaborative Canada

Total: $31,463†

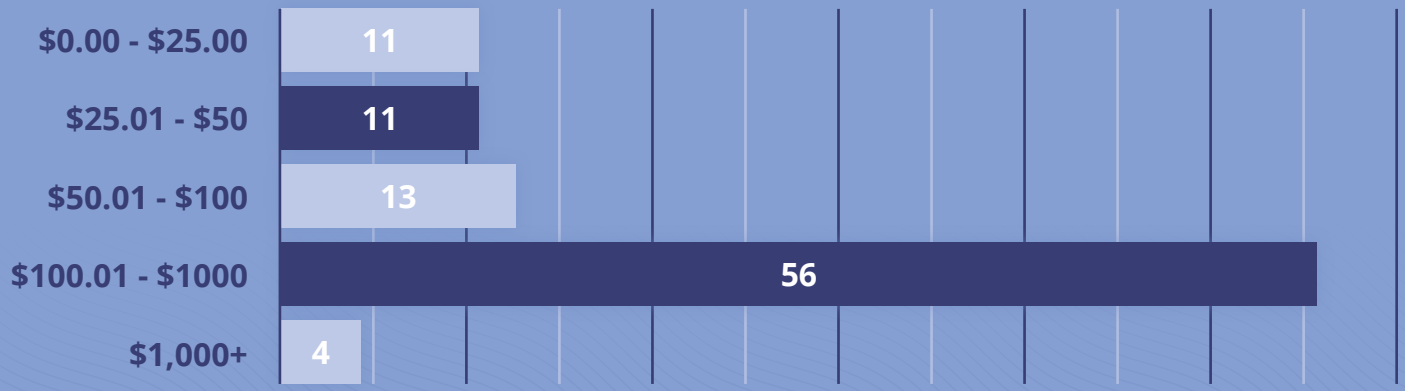


†Funds in Canadian Dollars (CAD)

GIVING BREAKDOWN

Usher 1F Collaborative Canada

Total Number of Gifts: 95



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