



USHER 1F
COLLABORATIVE



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ANNUAL REPORT

2024



Some of the amazing staff and volunteers who make the work we do possible.

2024

YEAR IN REVIEW

In 2024, Usher 1F Collaborative reached exciting milestones in the areas of the organization’s growth, fundraising, and research. This year marked our eleventh year since the founding of Usher 1F Collaborative. In the past eleven years, we have advanced research for a cure for the vision loss of Usher 1F at a faster than average pace.

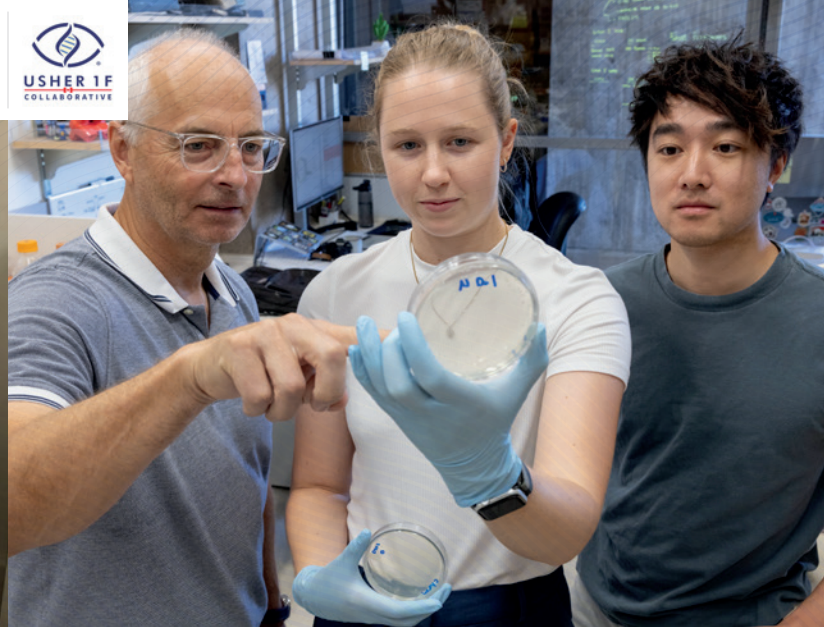
“It is nothing short of remarkable that the Usher 1F Collaborative has been able to make this level of progress and a testament to the team’s ability to identify the most important things to be done and then to fund those projects with a complete focus on translating scientific discoveries to preclinical and eventually clinical development.”

–Frank Gentile, PhD, CEO, Casma Therapeutics, Venture Partner, Third Rock Ventures,
Usher 1F Collaborative board member

This year, we launched our Seeing Forward campaign to raise over \$3 million in three years to accelerate the pace of research even more rapidly. Time is not on the side of those with Usher 1F, and so it is imperative that we bring them a cure as soon as possible.

“My wife Elizabeth and I decided to increase our own donations to maximize funding for researchers who are so close to treatments and a cure. It’s a race against time, and we need everyone’s support.”

–David Shapiro, brother of Dorie Shapiro, Usher 1F patient and Usher 1F Collaborative board member



Usher 1F Harvard team - Maryna Ivanchenko, MD, PhD, and David Corey, PhD

Usher 1F Salk team - Samuel Pfaff, PhD, with Claire Williams and Ryan Hsu

Milestones In **RESEARCH**

Harvard Medical School

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

One of our most exciting projects continues to come from Dr. David Corey's lab at Harvard Medical School. The most advanced treatment is his Usher 1F mini-gene. The Usher 1F gene is too large to fit on the standard delivery vehicle used in retinal gene therapy, a viral vector. The mini-gene contains only those portions of the gene necessary for vision, and it fits on one viral vector. Testing over the past year demonstrated that the mini-gene not only restores hearing in our Usher 1F mouse model but also rescues vision in our Usher 1F zebrafish. Dr. Corey's team has conducted a first round of preclinical testing of the mini-gene, and Dr. Corey has described results thus far as "stunning." He has published two papers describing the success of the mini-gene. In addition, Foundation Fighting Blindness, the most prominent funder of vision research, recognized his Usher 1F work this year, both with an invitation to speak at their annual research conference and with a three-year \$1.2 million grant to help support preclinical testing of the mini-gene over the next three years.

The Salk Institute for Biological Studies

Samuel Pfaff, PhD

Our newest exciting research is at the Samuel Pfaff Lab at The Salk Institute. Dr. Pfaff's team has developed a cutting-edge means of delivering large genes, RNA End Joining (REJ). While splitting large genes into two parts and having them recombine once in the eye is not new, REJ is a new approach that is demonstrating significantly higher efficacy. Usher 1F Collaborative funded a joint research proposal in July 2024 for a collaboration between Drs. Corey and Pfaff, a true dream team. Dr. Pfaff's lab should complete production of the split gene vectors no later the middle of 2025 and will then transfer them to Dr. Corey for testing in his Usher 1F mouse model.



Jennifer Phillips, PhD, and Monte Westerfield, PhD, University of Oregon Institute of Neuroscience, studying our Usher 1F zebrafish.

Milestones In **RESEARCH**

The University of Oregon Institute of Neuroscience

Monte Westerfield, PhD, and Jennifer Phillips, PhD

Their previous studies of zebrafish Usher syndrome models showed that degeneration of retinal and inner ear cells is due to particular types of cellular stress. They, thus, first started screening compounds that are known to modulate these types of cellular stress. Their preliminary screen identified a candidate compound that significantly reduces retinal degeneration and improves visual function in the Usher 1F models. These studies provide preclinical evidence for the tractability of this compound as an intervention to improve retinal cell function and survival in those with Usher 1F.

The team is also beginning to work with a privately funded clinical stage pharmaceutical company to conduct pre-Investigational New Drug (IND) studies that will support FDA approval for a clinical trial to test their lead compound as a therapeutic for Usher syndrome type 1F.

Natural History Study

Our Natural History Study, RUSH1F, in partnership with Foundation Fighting Blindness, has completed year three testing of over half of the patients with a few patients already having completed their year four visits. This study will provide critical data to measure efficacy in a future clinical trial.



Diego, who has Usher 1F, at his annual vision exam.

Milestones In **FUNDRAISING**



Seeing Forward

In 2024, Usher 1F Collaborative launched *Seeing Forward*, a 3-year major gift fundraising initiative to meet our next bold goals on a rapid path toward a cure. As science is reaching a critical tipping point, *Seeing Forward* donors will help the Collaborative to increase speed as well as to derisk the development process for new drugs and therapies to treat Usher 1F. Our donors rallied around this new initiative, in 2024 contributing more than \$819,000 toward our \$3.015 million goal. Each donation brings us closer to a cure, making a profound impact on the future for Usher 1F families.



Usher 1F Collaborative Golf, Tennis & Pickleball Outing

Usher 1F Collaborative held its first Golf, Tennis & Pickleball Outing in West Caldwell, NJ. The event also featured cards and Mahjong, as well as a dinner reception with live music and an auction. More than 250 guests attended and raised \$274,000 for Usher 1F research. Some attendees were longtime Collaborative supporters, and others learned about Usher 1F for the first time. The outing was such a resounding success we plan to make it an annual event.

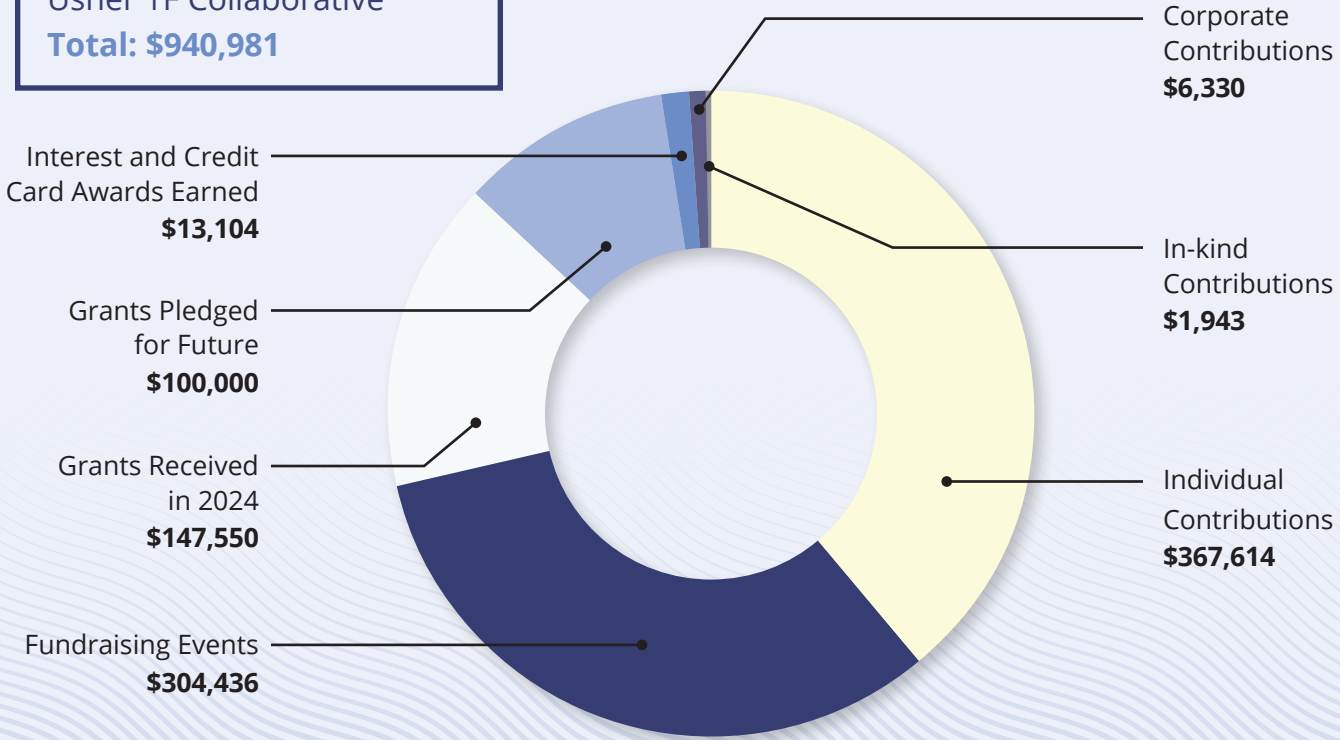


Laurie and Gary Shapiro Memorial Fund

The Laurie and Gary Shapiro Memorial Fund was established in 2024 to honor the Shapiros' legacy of tirelessly supporting Usher 1F Collaborative and its goal to treat the disease. In 2003 when Laurie and Gary learned that their daughter, Dorie, had Usher 1F, they became determined to help her and countless others find a cure. Tragically, Dorie and her brother David lost their beloved parents far too young. In 2024, Dorie partnered with fellow board member Margi Levitt to host a Giving Tuesday event in the Shapiros' community in Arizona. Guests were moved to collectively contribute \$42,000 toward the fund. In all, donors contributed more than \$141,000 to the Shapiro Memorial Fund in 2024, which supports the *Seeing Forward* initiative. The Shapiros' legacy endures through the generosity of a committed community.

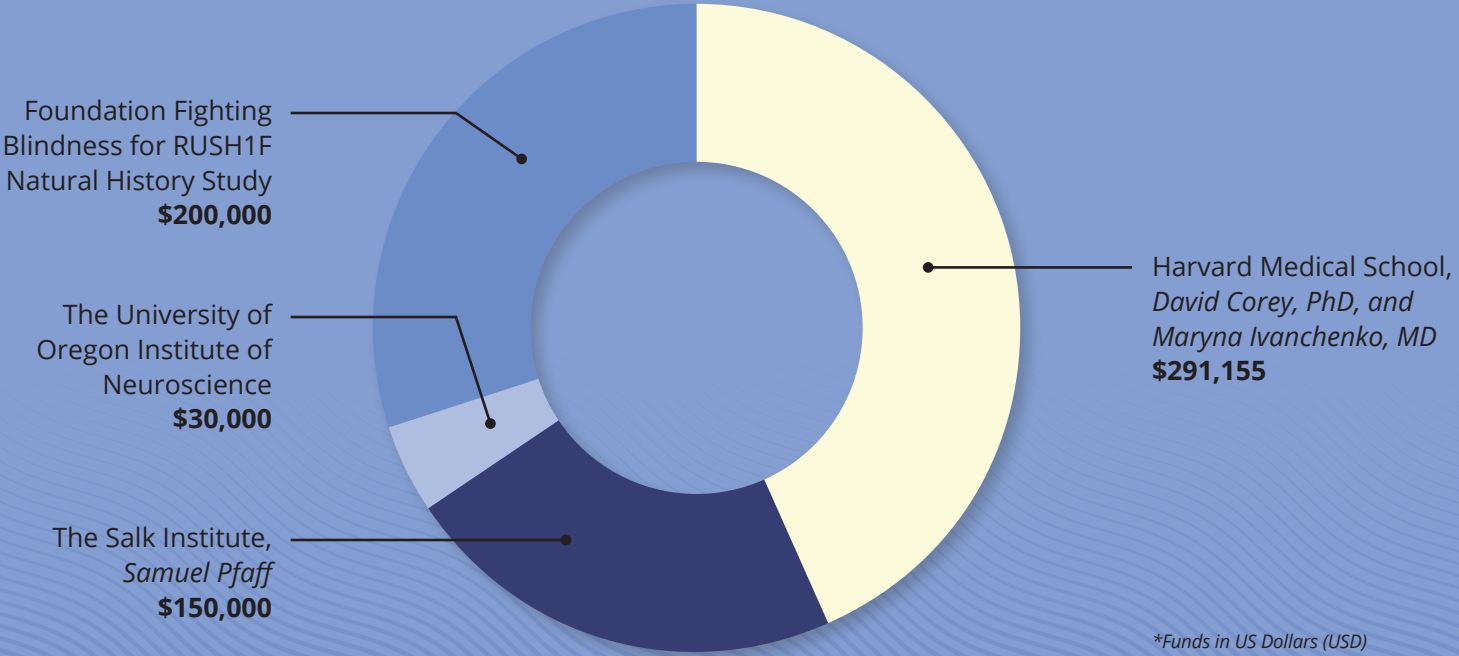
FUNDING RAISED

Usher 1F Collaborative
Total: \$940,981



RESEARCH GRANTS AWARDED

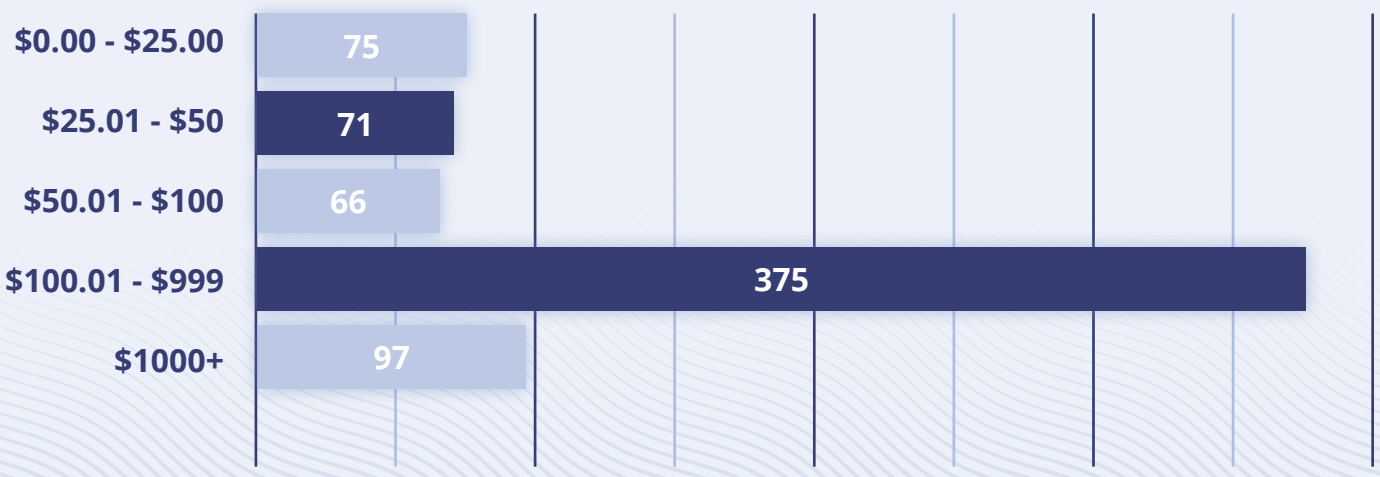
Usher 1F Collaborative
Total Grants Given: \$671,155



*Funds in US Dollars (USD)

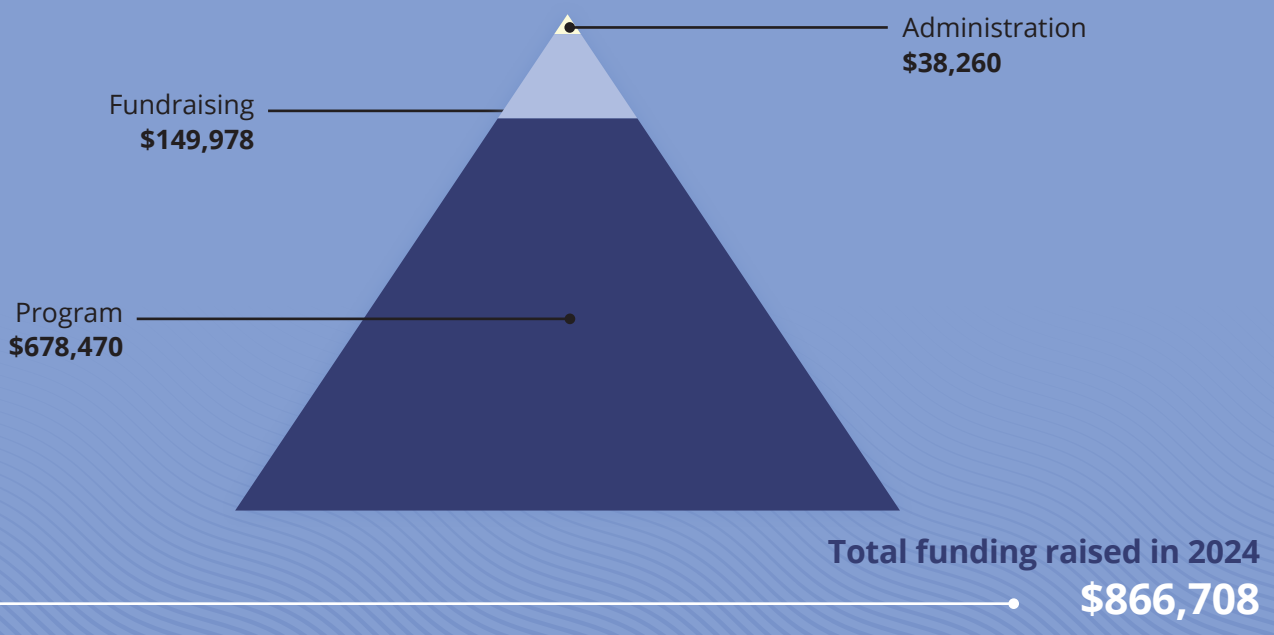
GIVING BREAKDOWN

Usher 1F Collaborative
Total Number of Gifts: 684



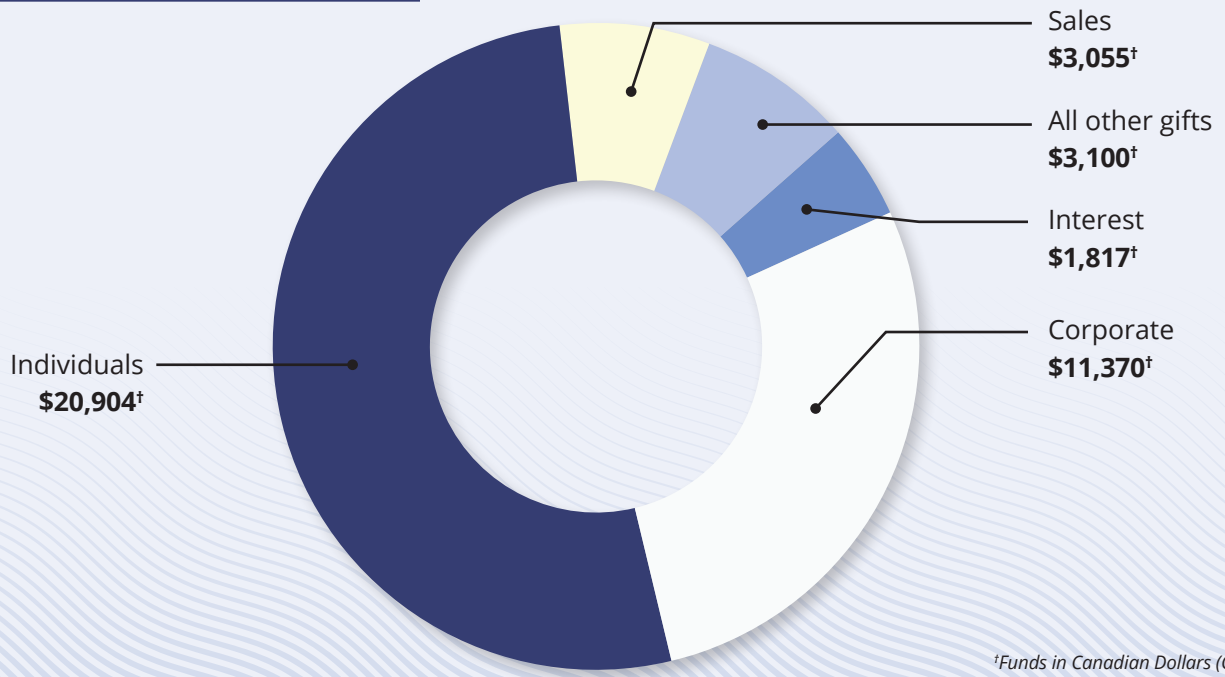
EXPENDITURES BY FUNCTIONAL AREA

Usher 1F Collaborative
Total: \$866,708



FUNDING RAISED

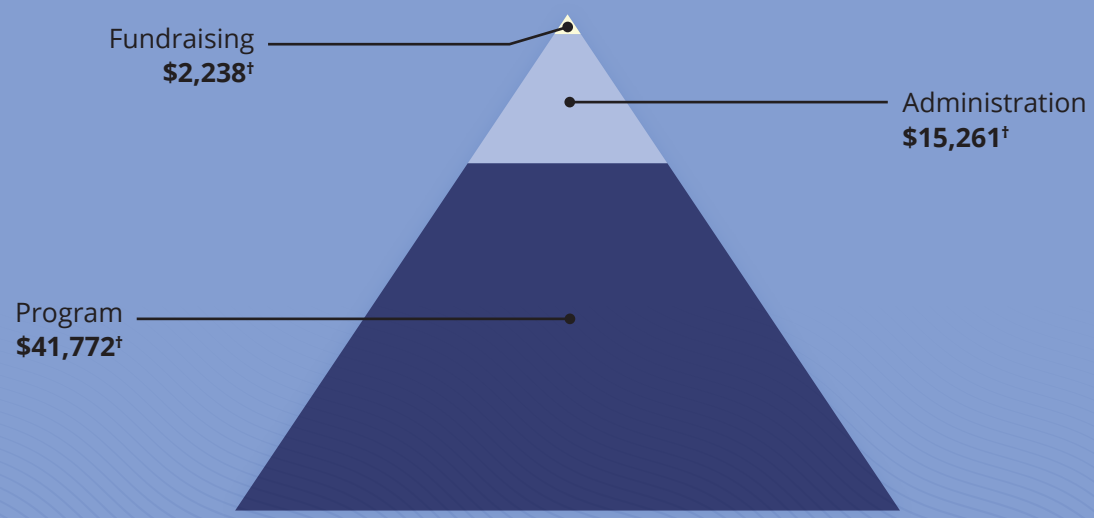
Usher 1F Collaborative Canada
Total: \$40,246[†]



[†]Funds in Canadian Dollars (CAD)

EXPENDITURES BY FUNCTIONAL AREA

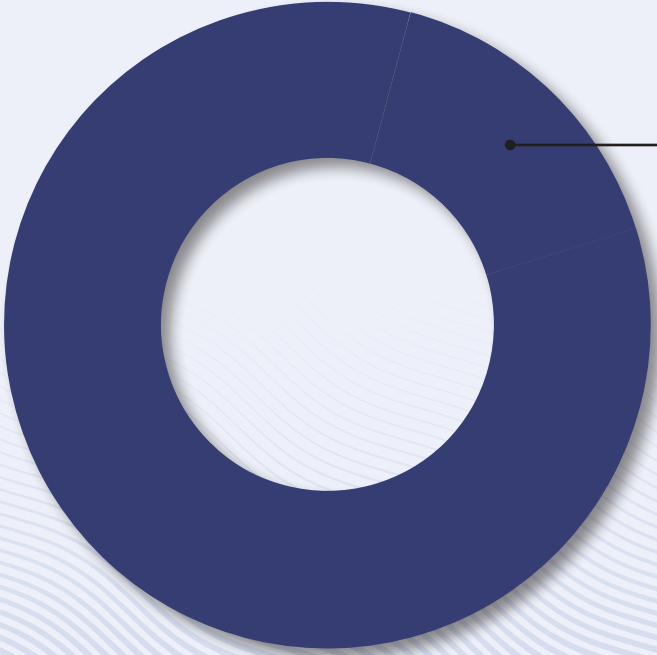
Usher 1F Collaborative Canada
Total: \$59,271[†]



Total funding raised in 2024
\$59,271*

TOTAL GRANT DISTRIBUTION

Usher 1F Collaborative Canada
Total Grants Given: \$41,772[†]

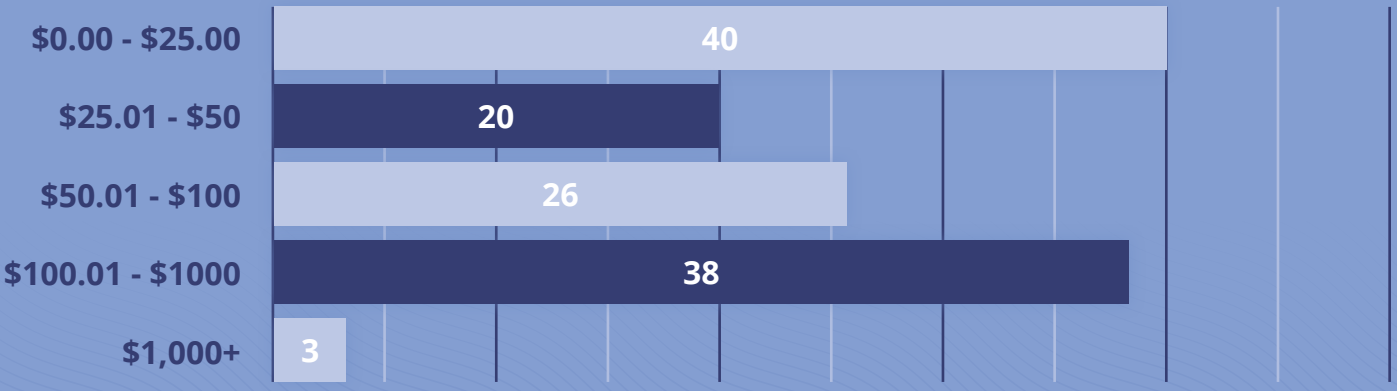


The University of Oregon
 Institute of Neuroscience
\$41,772[†]

[†]Funds in Canadian Dollars (CAD)

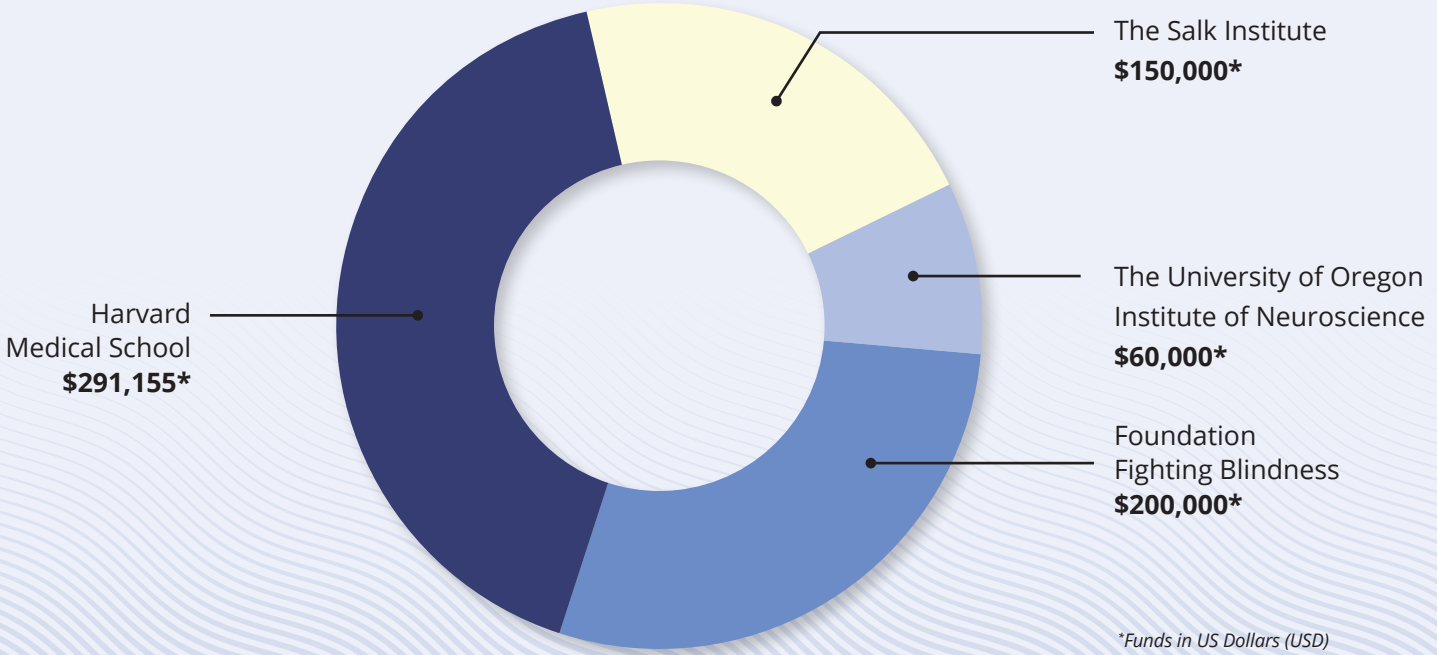
GIVING BREAKDOWN

Usher 1F Collaborative Canada
Total Number of Gifts: 127



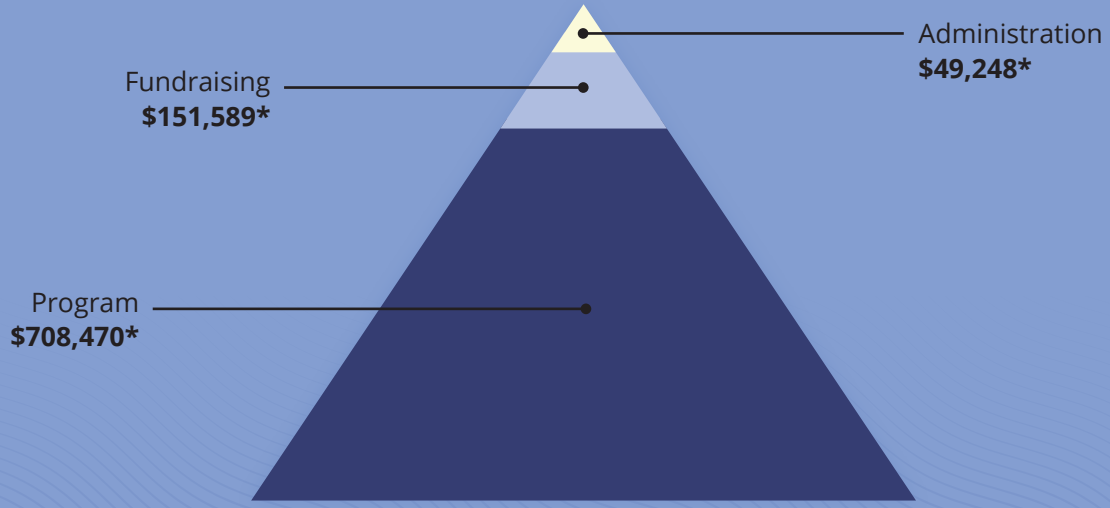
TOTAL COMBINED GRANT DISTRIBUTION

Usher 1F Collaborative and Usher 1F Collaborative Canada
Total Grants Given: \$701,155*



COMBINED EXPENDITURES BY FUNCTIONAL AREA

Usher 1F Collaborative and Usher 1F Collaborative Canada combined
Total: \$909,307 *



Total combined fundraising raised in 2024
\$954,295*

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Chair and Trustee



Elliot Chaikof, MD, PhD
Vice Chair & Trustee
Scientific Oversight Team



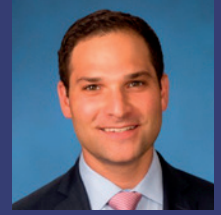
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