



10th ANNIVERSARY
ANNUAL REPORT

2023



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

2023

YEAR IN REVIEW

In 2023, Usher 1F Collaborative reached exciting milestones in the areas of the organization’s growth, fundraising, and research. This year marked the Ten-Year Anniversary of the founding of Usher 1F Collaborative, and its achievements were celebrated by our worldwide network of families, donors, and scientists. Working together, we remain laser-focused on our mission—to fund medical research to find an effective treatment to save or restore the vision of those with Usher syndrome type 1F. The exciting advancements in 2023 brought us closer than ever to reaching our shared goal.



Milestones In

ORGANIZATIONAL GROWTH

2023 marked ten years since the founding of Usher 1F Collaborative. When the organization was founded in 2013, there was no network of Usher 1F families. Through networking and our online presence, we now have 70 individuals in over 50 families working together in our quest for a cure.

In October, over 100 people, including much of the Usher 1F Collaborative board, gathered in Morristown, NJ, to celebrate the Ten-Year Anniversary and our achievements toward a cure for the vision loss of Usher 1F. Guests enjoyed a cocktail reception and the debut of the film, *Seeing Forward*, which follows our journey for a cure, focusing on courageous young adults and children who live their lives to the fullest despite shouldering the weight of vision loss from Usher 1F.

Due to our large presence in Canada, in 2023 we launched Usher 1F Collaborative Canada, an official tax-exempt charitable foundation. This allows us to expand our fundraising efforts in Canada, better serving all our families, including those who live in Toronto, Ottawa, and Newfoundland.





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10TH ANNIVERSARY
\$10 MILLION RAISED
OCTOBER, 10TH 2023

Milestones In

FUNDRAISING

With the increased organizational infrastructure, Usher 1F Collaborative reached new heights in fundraising. With staff and board members working together in both our US and Canadian foundations, we raised USD \$781,551 in 2023. Years ago, we set forth an initiative, *Ten to Cure*, which was a goal to have \$10 million invested in Usher 1F research in ten years. We are proud to report that we exceeded that goal, and there is currently more than \$12.6 million invested in a cure.



OPEN ACCESS
ARVO Annual Meeting Abstract | June 2023
'Mini-PCDH15b' gene therapy rescue visual deficits in a zebrafish retinopathy model of Usher syndrome type 1F
Maryna Ivanchenko; Alex J Klein; Daniel M Hathaway; Jennifer B Phillips; Kevin T Booth; Corey Goldstein; ...

ORIGINAL ARTICLE | VOLUME 31, ISSUE 8, P2439-2453, AUGUST 02, 2023
Rescue of hearing by adenine base editing in a humanized mouse model of Usher syndrome type 1F
Cole W. Peters • Killian S. Hanlon • Maryna V. Ivanchenko • ... Benjamin P. Kleinstiver • Artur A. Indzhykulkar • David P. Corey • Show all authors

Molecular Therapy

Volume 31, Issue 12, 6 December 2023, Pages 3490-3501

Original Article
Dual AAV-based PCDH15 gene therapy achieves sustained rescue of visual function in a mouse model of Usher syndrome 1F

Toward a Therapy for a Rare Genetic Disease
Researchers design "mini gene" therapy for severe syndrome that causes blindness and deafness
By CATHERINE CARUSO | April 26, 2023 | Research
7 min read

Researchers take key step toward developing a gene therapy for Usher syndrome type 1F
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Reviewed
Reviewed by Emily Henderson, B.Sc.
Apr 26 2023

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Article | Open access | Published: 26 April 2023
Mini-PCDH15 gene therapy rescues hearing in a mouse model of Usher syndrome type 1F

Milestones In RESEARCH

2023 brought exciting progress in the research funded by Usher 1F Collaborative.

David Corey Lab at Harvard Medical School

- ▶ In April, Maryna Ivanchenko, MD, PhD, presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting. In her presentation, she shared the exciting results that the "mini gene" developed to treat Usher 1F rescued vision in a zebrafish model.
- ▶ In August, results were published of a second strategy utilizing base editing to successfully restore hearing in an Usher 1F mouse model.
- ▶ In November, published results revealed a third strategy—gene therapy employing dual vectors—also showed promising results in restoring vision in multiple retinal models, including human retinal organoids and non-human primate retina.
- ▶ The lab continued its collaboration with the Samuel Pfaff Lab at the Salk Institute for Biological Studies, on a promising gene therapy approach called RNA end-joining (REJ) technology.

Monte Westerfield Lab at the University of Oregon Institute of Neuroscience

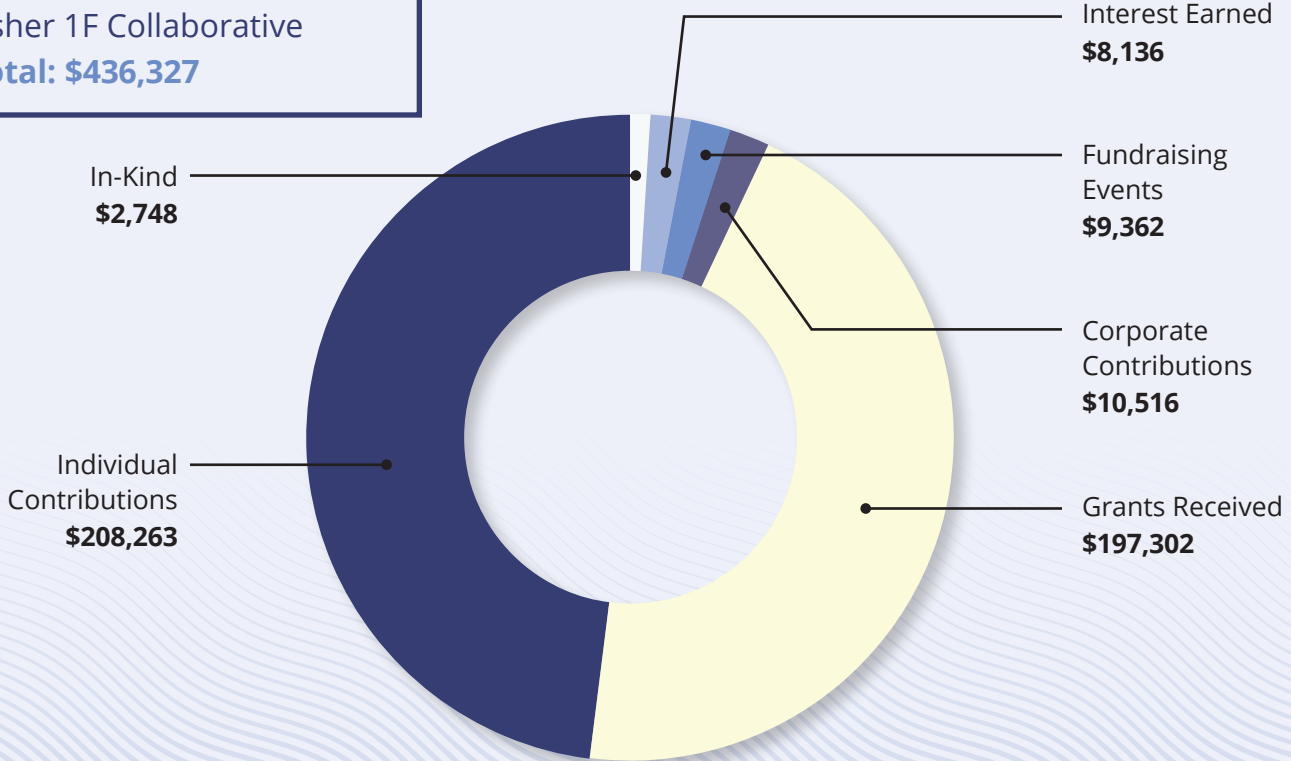
- ▶ In December, published results revealed that a potent antioxidant compound, Hexafluoro, improved visual function and slowed photoreceptor cell death in the Usher 1F zebrafish model.

Zubair Ahmed Lab at the University of Maryland School of Medicine

- ▶ In December, published results detailed sustained rescue of visual function in a mouse model of Usher 1F utilizing a dual AAV-based gene therapy

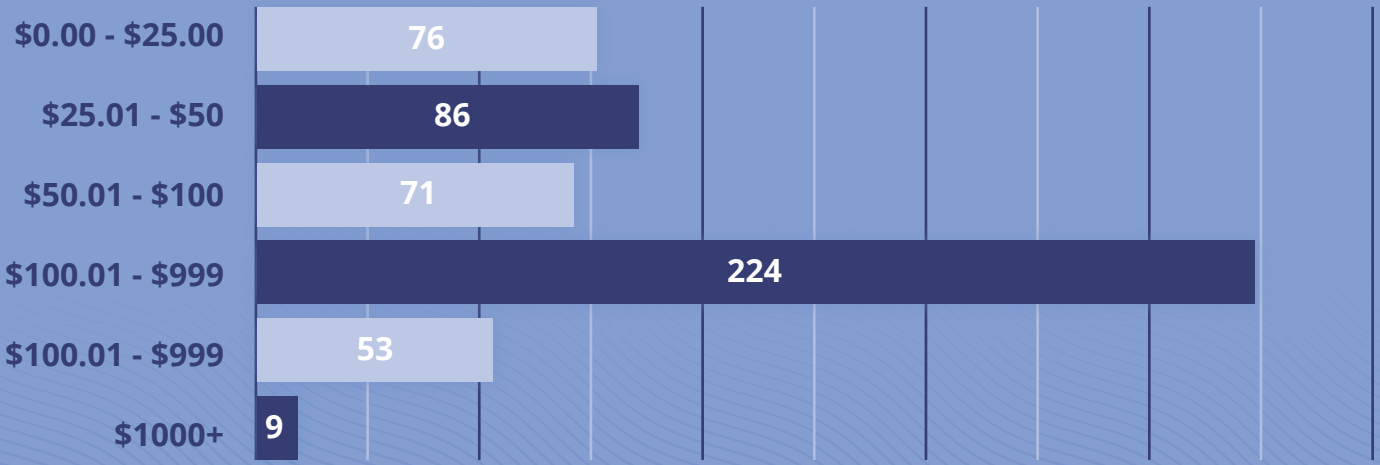
FUNDING RAISED

Usher 1F Collaborative
Total: \$436,327



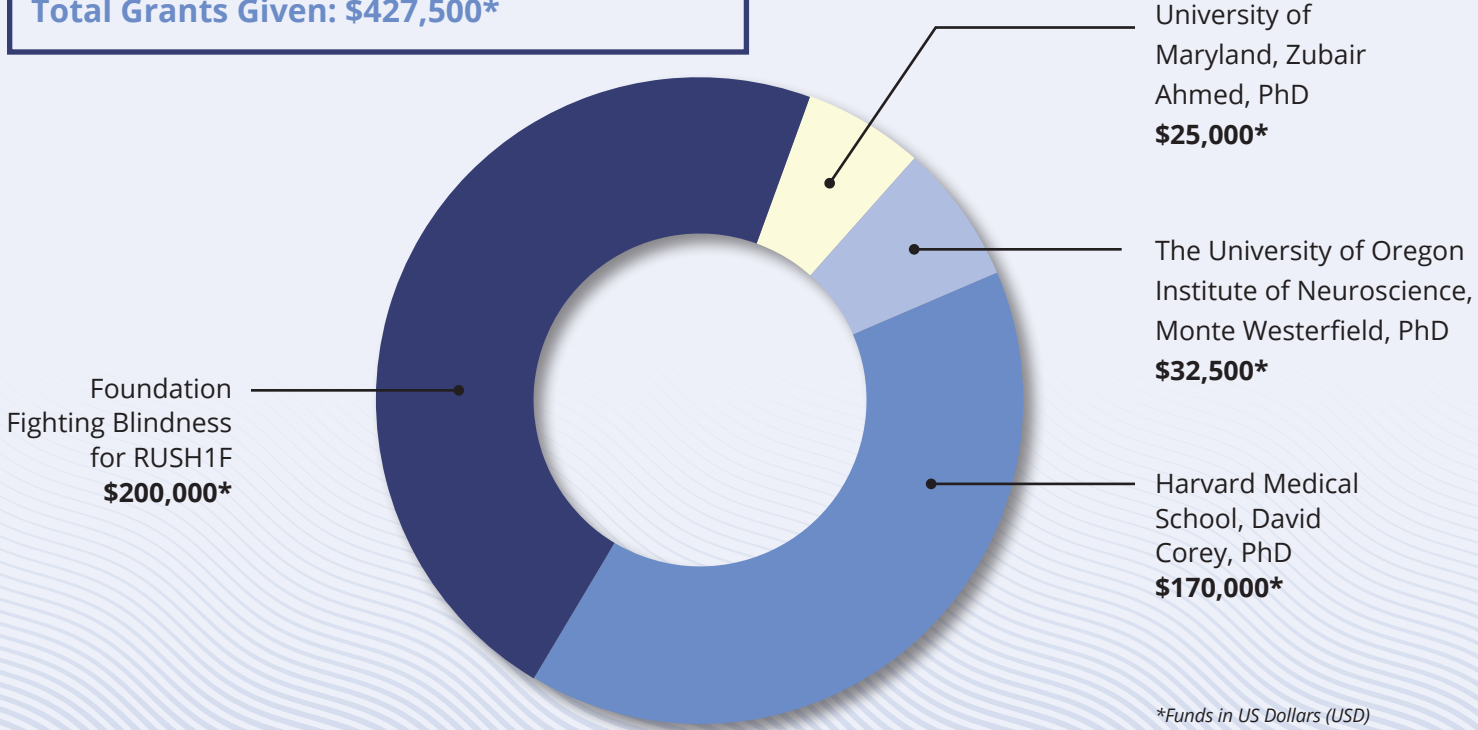
GIVING BREAKDOWN

Usher 1F Collaborative
Total Number of Gifts: 519



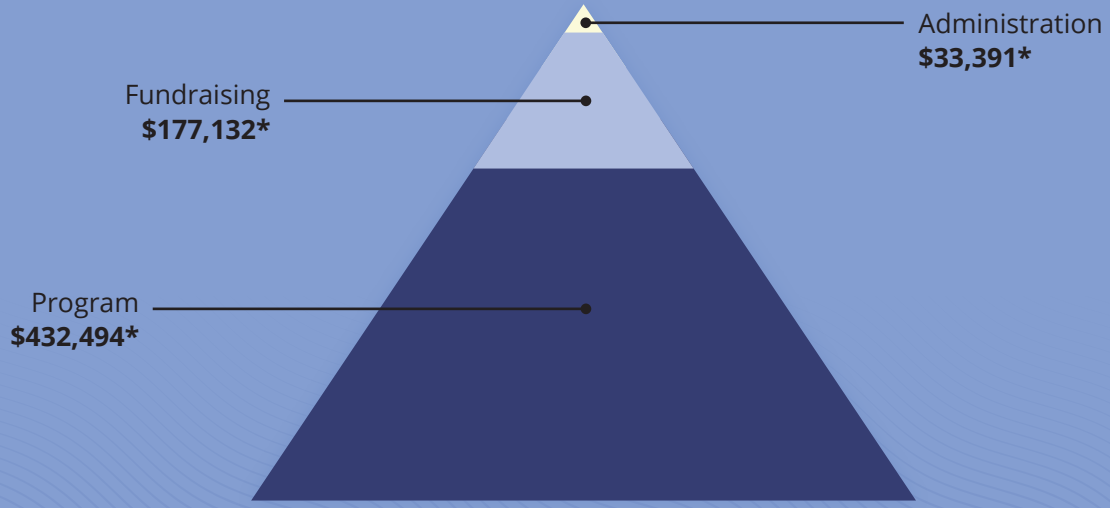
TOTAL GRANT DISTRIBUTION

Usher 1F Collaborative
Total Grants Given: \$427,500*



EXPENDITURES BY FUNCTIONAL AREA

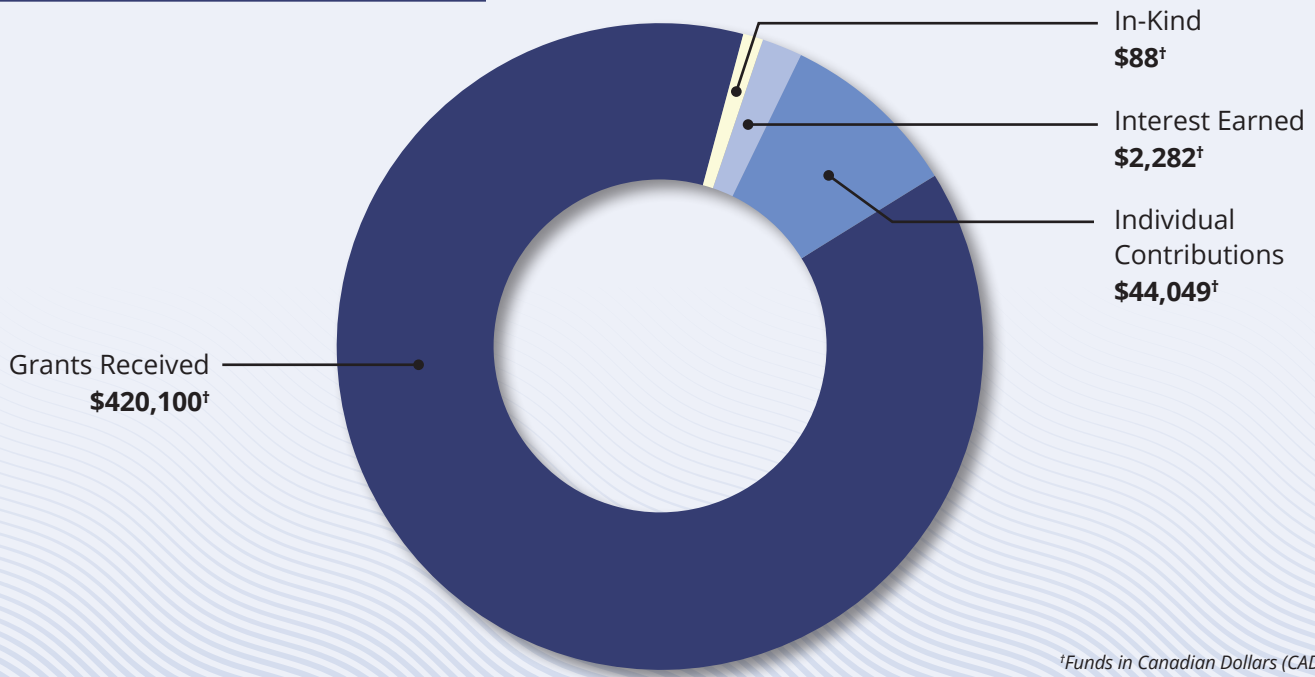
Usher 1F Collaborative
Total: \$643,017*



Total assets as of 12/31/23
\$801,100*

FUNDING RAISED

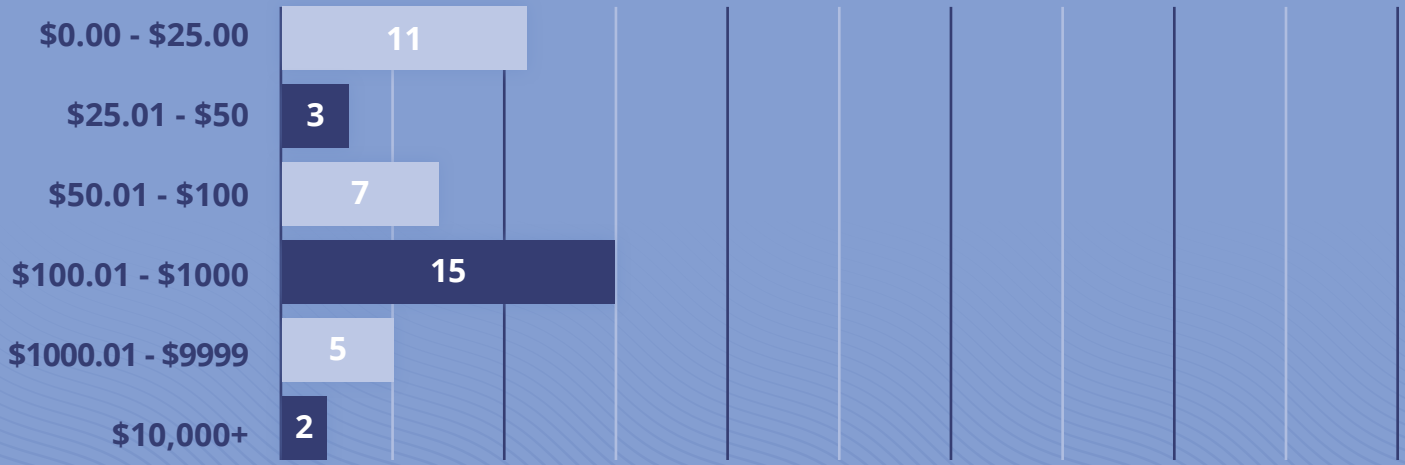
Usher 1F Collaborative Canada
Total: \$466,519[†]



[†]Funds in Canadian Dollars (CAD)

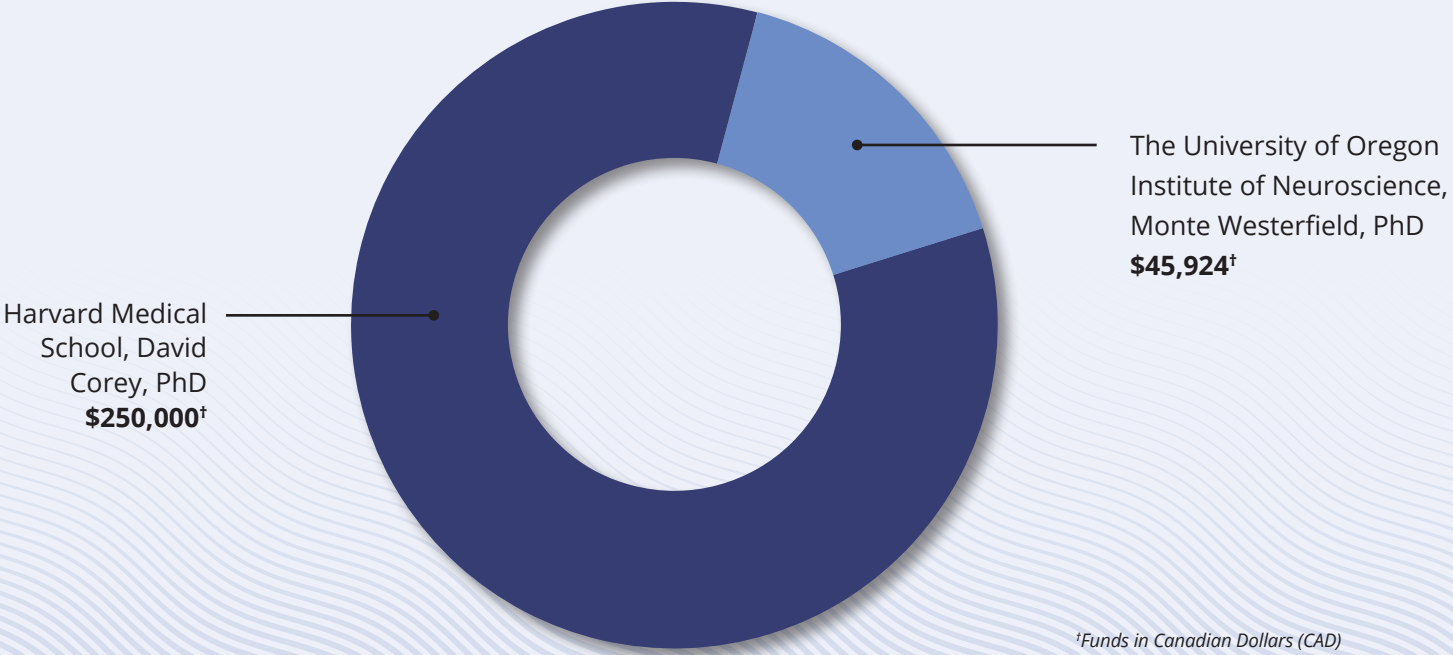
GIVING BREAKDOWN

Usher 1F Collaborative Canada
Total Number of Gifts: 43



TOTAL GRANT DISTRIBUTION

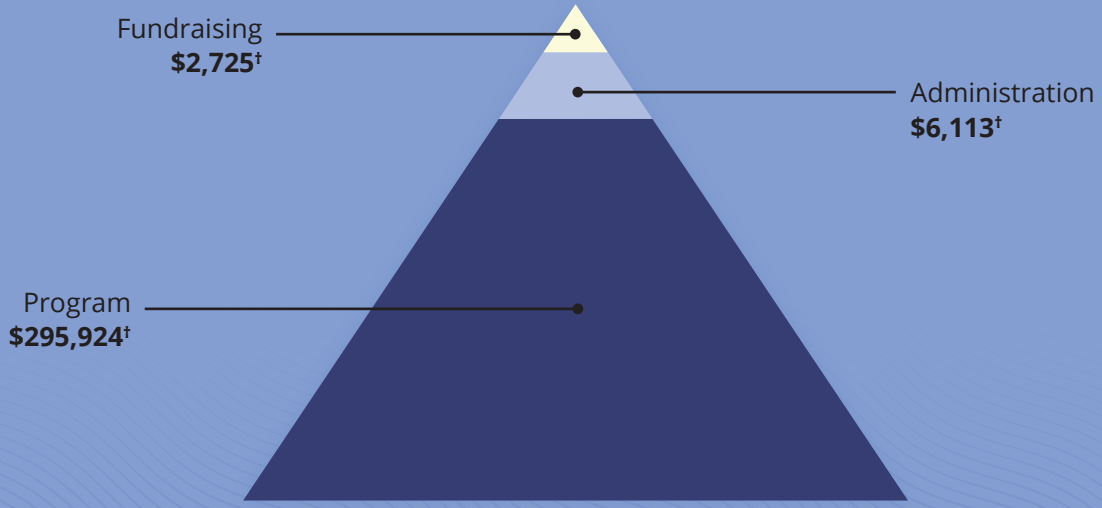
Usher 1F Collaborative Canada
Total Grants Given: \$295,924.[†]



[†]Funds in Canadian Dollars (CAD)

EXPENDITURES BY FUNCTIONAL AREA

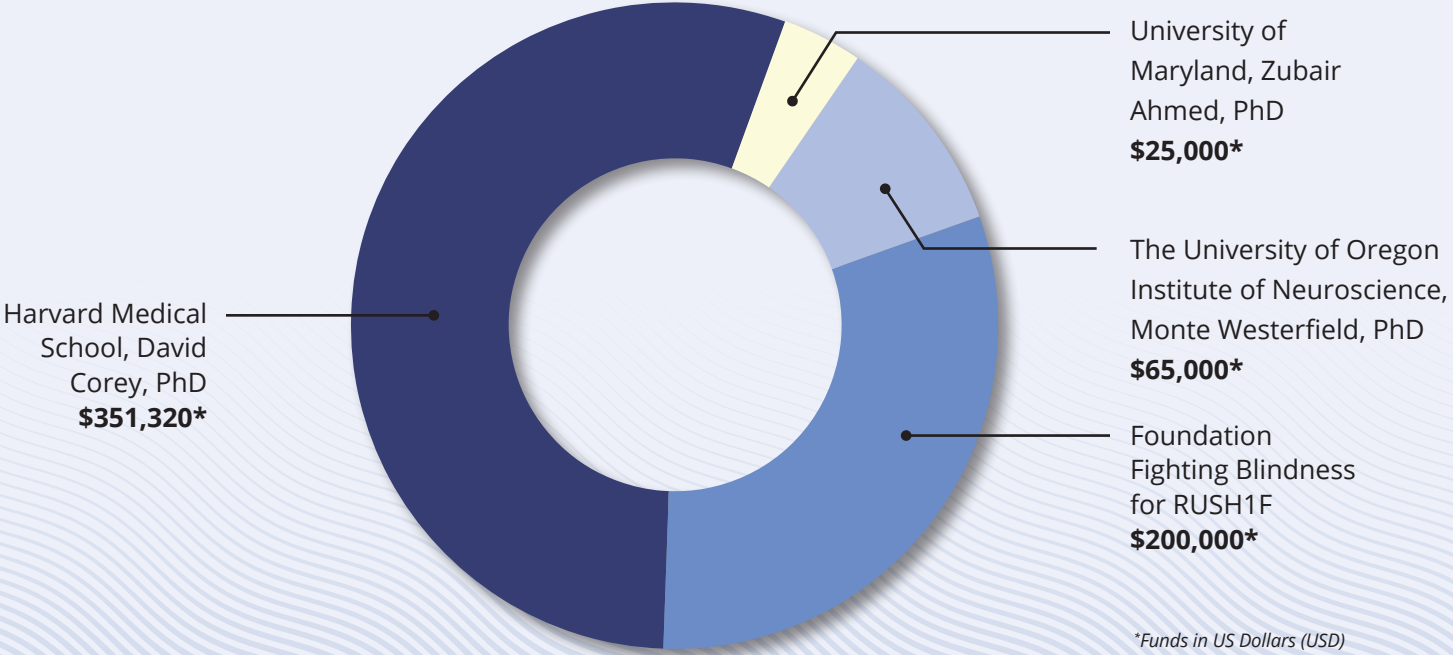
Usher 1F Collaborative Canada
Total: \$304,762[†]



Total assets as of 12/31/23
\$140,577*

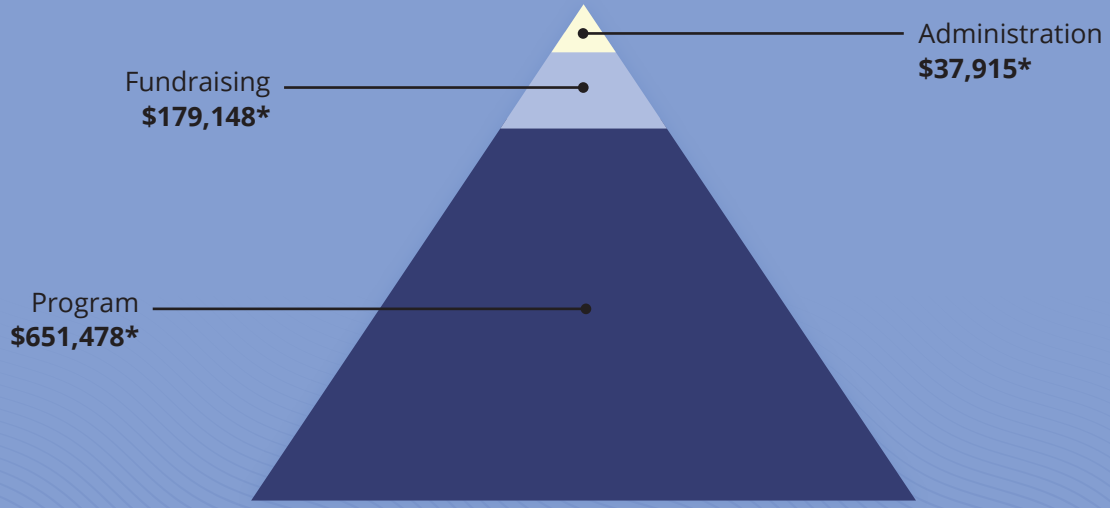
TOTAL COMBINED GRANT DISTRIBUTION

Usher 1F Collaborative and Usher 1F Collaborative Canada
Total Grants Given: \$641,320*



COMBINED EXPENDITURES BY FUNCTIONAL AREA

Usher 1F Collaborative and Usher 1F Collaborative Canada combined
Total: \$868,541*



Total combined assets as of 12/31/23
\$905,126*

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Vice Chair & Trustee
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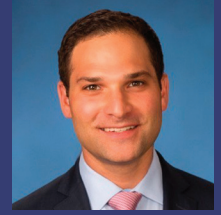
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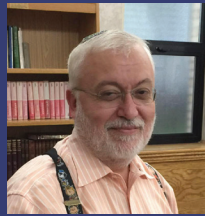
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