



g sequence for PCDH15 is too large for AAV;
of some EC domains might preserve function



	EC3	EC4	EC5	EC6	EC7	EC8	EC9	EC10	EC11	MAD12
22	X				X	X				
		X	X	X	X	X				
	X				X	X				
	X	X	X	X	X	X				

USHER 1F
COLLABORATIVE

ANNUAL REPORT

2021

SIGHT.
SOUND.
SWEAT.

The Year in Review



Melissa Chaikof with her daughters Rachel and Jessica

2021 was a year of many successes, not just in our fundraising but, more importantly, in our research progress. Thanks to our very generous donors, our hardworking board, Usher 1F families and friends, and our creative Usher 1F Cereal Challenge, we realized a total of \$600,000 to invest in vision-rescuing research. This research now includes ten gene and drug therapies at eight research labs, including at least one gene therapy that will likely be ready to move from the lab to preparation for testing in humans in three to four years, as well as a drug therapy nearing readiness for testing in humans.

2021 also saw the launch of RUSH1F, our natural history study in partnership with Foundation Fighting Blindness. The importance of this study cannot be overstated. Investigators at ten clinical centers around the world will track the progression of vision loss in the absence of treatment in 40 patients with Usher 1F over a period of four years. This data will then be used to determine efficacy in a future clinical trial. Because Usher 1F is so rare, it is not possible to conduct a traditional clinical trial with a portion of the patients receiving a placebo. RUSH1F will, essentially, provide this control group in advance. Without this study, we could not have a clinical trial nor attract the interest of biopharma companies.

We continue to benefit from our Chan Zuckerberg Initiative (CZI) Rare As One Project grant, both from the opportunities the funding has afforded us and from the training and connections from which we have benefitted. These include webinars with expert

speakers, in-depth training on fundraising and nonprofit accounting, as well as the opportunity to network, compare notes, exchange ideas, and learn from the other 29 grantees.

Our international scientific research conference was a major benefit from our CZI grant, enabling us to bring together investigators to share ideas and foster collaborations. This talented pool of presenters focus their research on Usher 1F, other similar types of Usher syndrome, as well as on the development of relevant scientific approaches and tools. While COVID forced us to hold a virtual conference, over 300 persons registered from locations not just in the US but from all over the world. While we missed the personal face-to-face interactions more readily facilitated by an in-person conference, holding it online enabled more people to join us to benefit from the wealth of valuable information presented.

Usher 1F Collaborative is uniquely positioned with our exemplary combination of skillsets to attract and fund the best scientists to achieve our mission. We leverage the world's best brains, current science, cutting-edge engineering, and new technology to find a cure for this debilitating genetic disease. Our researchers work collaboratively and are making tremendous strides. Our board has a world-renowned physician and scientist (also an Usher 1F parent), a biopharma venture capitalist, a drug developer, motivated parents and friends of affected families, and others in the corporate world. This winning combination allows us to access the best researchers and effectively understand and evaluate the science.

We are harnessing science and breaking barriers in our quest for a cure.

We are eager to see the research progress that 2022 will bring and invite you to follow along on our journey toward our dream of vision for a lifetime.

Melissa K. Chaikof

Board President
Usher 1F Collaborative

FEBRUARY

The Usher 1F Cereal Challenge

On February 13th, the first participants donned blindfolds and felt around their kitchens, attempting to gather a bowl, cereal, milk, and a spoon and subsequently prepare the classic breakfast meal that is also 9-year-old Zachary Root's favorite food.

With a little help from his parents and his dad's college friends, Zachary's Usher 1F Cereal Challenge took off! With over \$150,000 raised and over one thousand videos, people all over the world accepted the challenge!

We were even more excited when some of the most famous cereal brands and their iconic mascots accepted the challenge. Not only did Kellogg's donate \$20,000, but also we saw Snap, Crackle, and Pop blindfolded on Instagram, and we saw their friends Tony the Tiger, Toucan Sam, and the Apple Jacks team attempt to prepare a bowl of cereal as well. Post Cereals donated \$1000 and accepted the challenge, gifting Zachary with a one-of-a-kind personalized box of Fruity Pebbles.

In addition to these icons from the world of cereal, we also saw many celebrities accept the challenge.



Snap, Crackle and Pop take the Usher 1F Cereal Challenge (Photo courtesy of Kellogg's)



WATCH: www.youtube.com/watch?v=huxTaCRHb88
Zachary takes the Usher 1F Cereal Challenge



MAY

Usher 1F International Scientific Research Conference

On May 13th and 14th, 2021, Usher 1F Collaborative hosted a virtual international scientific research conference, *Therapeutic Strategies for Large Protein Coding Genes in Usher Syndrome*. We are grateful to the Chan Zuckerberg Initiative (CZI) for funding the conference through our Rare As One Project grant, as well as to our sponsors, The Ardent Companies, Associated Agencies, I. Halper Paper & Supplies, and Franklin Mutual Insurance. With over 300 attendees and 26 sessions, the event was a major success!

SEPTEMBER

Red Meat Lovers Club

On September 29, 2021, the Red Meat Lovers Club, led by Evan Darnell, the Secretary of Steak, along with Ariane and Michael Duarte, held an amazing, enthusiastic fundraiser at Ariane Kitchen & Bar in Verona, NJ, in support of local resident Zachary Root, who has Usher Syndrome type 1F. Thanks to the generosity of the hosts and guests, the epic “meating” raised \$26,000 for Usher 1F research!

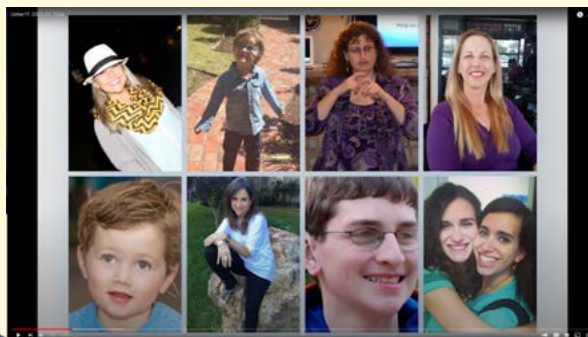


Emily, Rachel, Zachary, and Jared Root at the Red Meat Lovers Club Event

NOVEMBER

Release of Usher 1F Collaborative Film

On Giving Tuesday 2021, we released our new video, illustrating how Usher 1F Collaborative is uniquely positioned with our exemplary combination of skillsets to attract and fund the best scientists to achieve our mission.



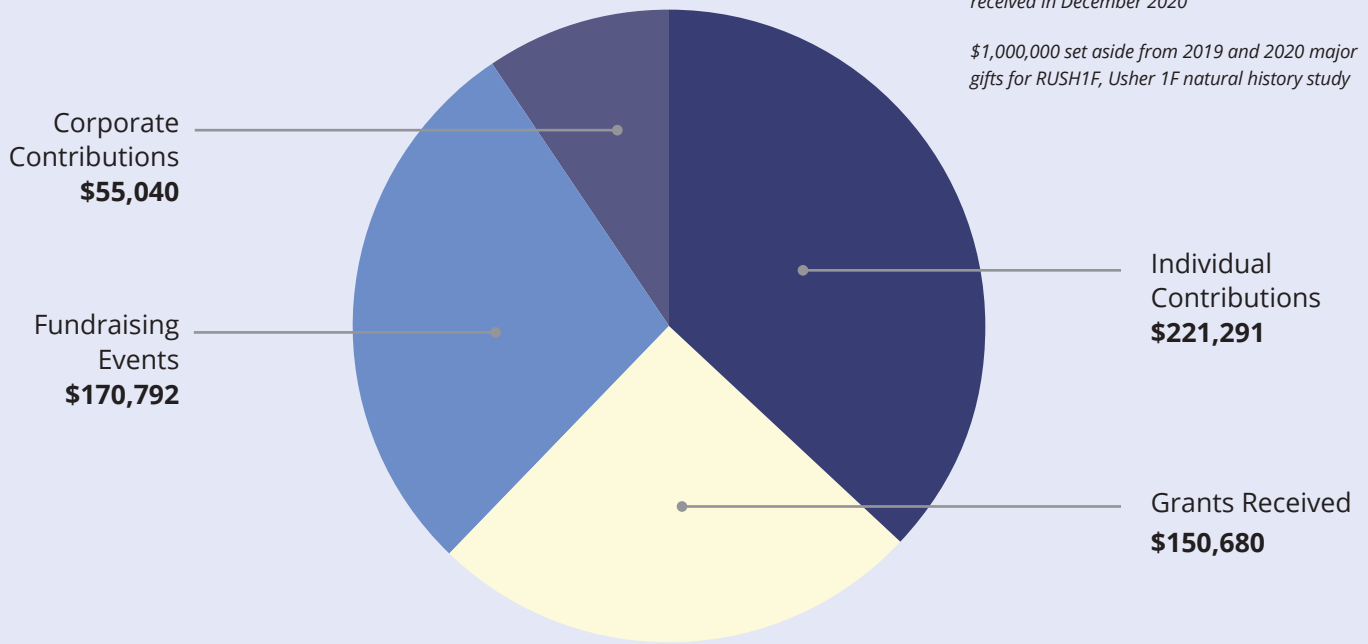
WATCH: www.youtube.com/watch?v=vX8RaNazqQY&t=15s
Usher 1F Giving Tuesday Film: Why We Are Uniquely Positioned to Achieve Our Mission

FUNDING RAISED

Total: \$597,803

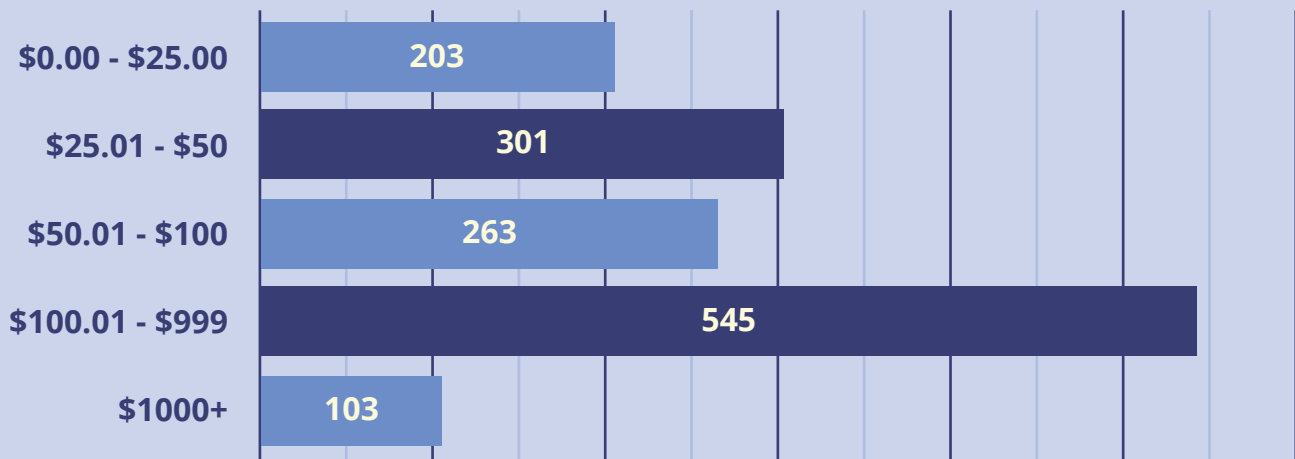
**Notes: \$225,000 in restricted funding for 2021 from the Chan Zuckerberg Rare As One Project received in December 2020*

\$1,000,000 set aside from 2019 and 2020 major gifts for RUSH1F, Usher 1F natural history study



GIVING BREAKDOWN

Total Number of Gifts: 1427

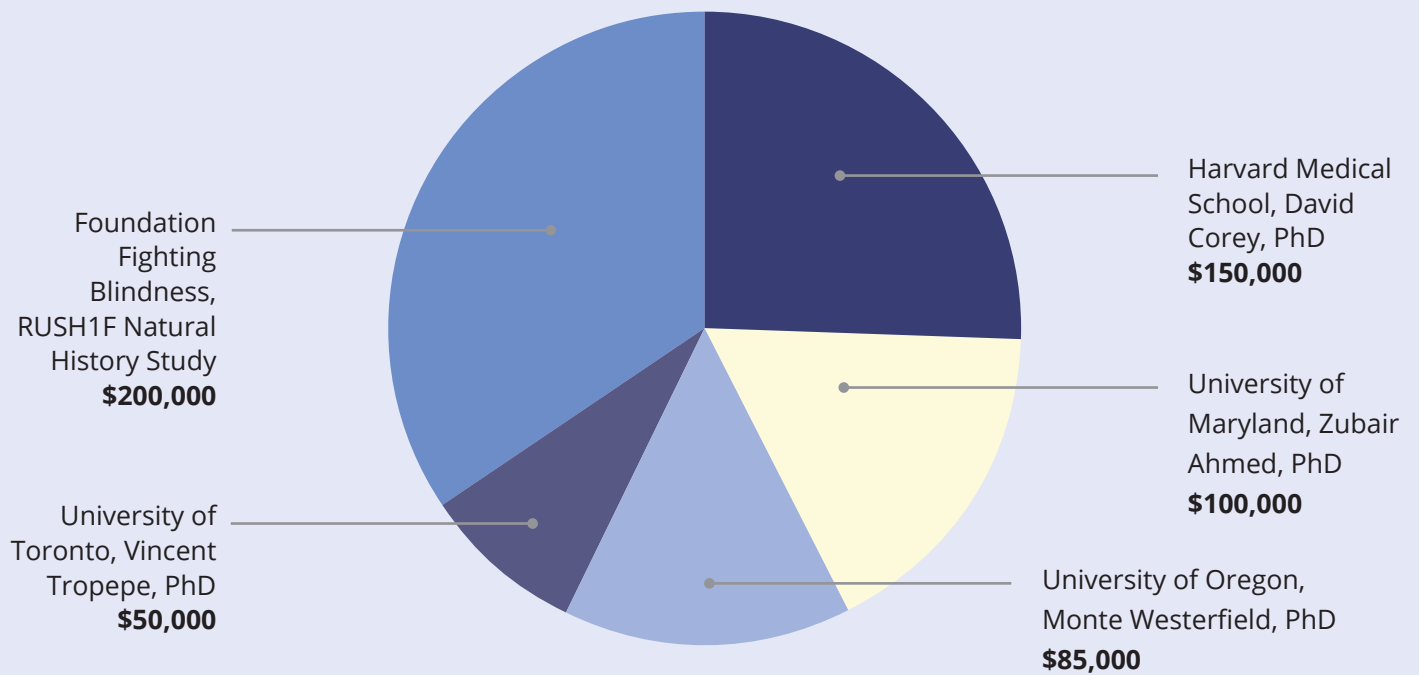


Average Gift Size: \$418

Total Expenditures
\$840,065

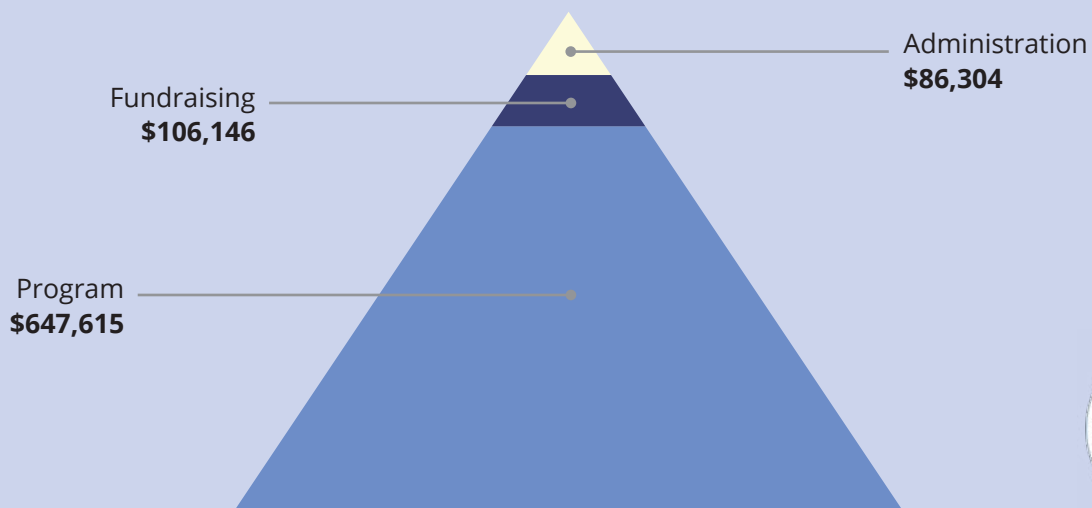
TOTAL GRANT DISTRIBUTION

Total Grants Given: \$585,000



EXPENDITURES BY FUNCTIONAL AREA

Total: \$840,065



Total assets as of 12/31/21
\$1,173,759



Thank you to the following for major gifts in 2021

Apple

The Ardent Companies

Associated Agencies, Inc.

Barry Cohen Homes

Blavatnik Family Foundation

Bochner Eye Institute

Brad Capadona

Leo and Bayla Chaikof

David and Ellen Chaikof

Melissa and Elliot Chaikof

Sheila and Jeremy Chess

David Creemer and Stacey Ashlund

Evan Damast

The Giving Tree Foundation

David and Barbara B. Hirschhorn Foundation

Risa and Daniel Drimmer

Alan and Bunny Fortier

Jennifer and Josh Herz

I. Halper Paper & Supplies

Ideal Fastener Corporation

Kellogg Company

Leo H. Bendit Charitable Foundation

Little Mendelson Foundation

Patricia Nilles

O'Kane Family Foundation

Red Meat Lovers Club

Rosbruch Family Foundation

Sandel Family Foundation

Peter Seligman

David and Elizabeth Shapiro

Carol and David Sidman

Sari and Hal Springer

William S. and Ina Levine Foundation