In Memorium, with Gratitude: Abraham Samuel "Sam" Shiff

In November 2017, a check appeared in our mailbox for $1000 from Abraham S. Shiff. In the memo section of the check, he had written

CONTRIBUTION
NO PUBLIC ANNOUNCEMENT
SOLICITED BY DR. SEEWALD

Dr. Seewald is our board member, Julian Seewald. According to Julian, Sam Shiff was a single man in his 70s, a member of his synagogue. The first week of December 2017, another $1000 check appeared. This continued for 68 months without fail. Sam Shiff never missed a month. Each check was dated the first of the month, and each one had the same lines written in uppercase in the memo section of the check. Thus, while we thanked him profusely personally, we could not publicly acknowledge his contributions that totaled $68,000 toward Usher 1F research.

Sadly, on July 26th, Sam Shiff passed away in his sleep. While we could not acknowledge his contributions during his lifetime, we would like to thank and honor him now.

Mr. Shiff was a bit of an enigma to us. He did not communicate to us directly other than with his donations, although we know that Julian Seewald kept him updated. It has only been since his death that we learned more about this unusual man and scholar. He enjoyed careers in pharmacy, administration, and computer programming and held degrees in Clinical and Hospital Pharmacy, Physiology, Healthcare Administration, Computer Programming, History, and Liberal Arts. Upon retirement in 2006, he became a historian, returning to graduate studies full time, enrolling in the history department at Brooklyn College and in the Master of Liberal Studies program at the Graduate Center, City University of New York, where he studied Shakespeare, writing two master's theses, and William Blake, writing yet another publication on this topic in 2019.

Sam Shiff

With his quiet generosity and caring, seeking no recognition, giving solely to help others, Abraham Samuel Shiff was an exceptional man who will be missed. ◆

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planning to hire an intern that summer, they knew they had to invite him on because he was the only intern who truly understood the significance of their work to restore vision loss. Brendan assisted the lab that summer with a project assessing how well a drug could lower the immune response to stem cell therapeutics that weren't in development elsewhere.

Brendan graduated from Lewis & Clark in 2021 with a bachelor's degree in biochemistry and molecular biology. Rather than going directly to graduate school, he spent two years as a postbaccalaureate fellow in a lab at the National Institutes of Health (NIH), where he gained research skills by helping to test out the ability of custom-built molecular drugs to target very specific cell-surface receptor subtypes.

Brendan's experience at the NIH helped solidify his decision to get a PhD in biomedical sciences because then he could have a voice that would guide the direction of research for a cure for Usher 1F. He applied and was accepted to the University of Iowa and is now in his first year in their biomedical sciences program. In his first rotation, he is working in a lab that researches molecular mechanisms in retinal cells. Upon completion of his PhD, his goal is to be part of a team that helps guide the research for a cure and helps bring it to market.

Brendan is strongly optimistic that a cure for Usher 1F will become a reality. In fact, he has been hinging some aspects of his life on this happening, for example largely avoiding blindness preparation. Because of frustrations at his current stage in life, though, in particular his inability to drive, he is impatient for at least "half a cure," that is a drug that slows vision loss.

Stacey and David recognized early that Brendan was very bright with an incredible capacity to learn. His journey so far has been an exceptional one, and we join them with excitement and anticipation, watching Brendan's continued achievement and impact on Usher 1F research. ◆

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USHER 1F COLLABORATIVE

10·10

10TH ANNIVERSARY

$10 MILLION RAISED

OCTOBER 10TH, 2023

at the Morris Museum in Morristown, NJ, to celebrate ten years of achievements toward a cure for the vision loss of Usher 1F and to thank those who have helped us realize our success. Jared and Rachel Root, board members and co-hosts of the event and parents of Zachary, who has Usher 1F, opened the program by recognizing the board members and staff who were present and recognizing all who have supported this effort. "Let's applaud and celebrate how far we have come together. Let's applaud all the dedication, time, and love. Let's applaud the generosity. Let us applaud you."

The debut of the film, *Seeing Forward*, follows our journey for a cure, focusing on a number of courageous young adults and Zachary Root, who are living their lives to the fullest despite shouldering the weight of vision loss from Usher 1F. All desperately desire a cure. In the film, Dr. Corey emphasizes the collaborative nature of the research that has led to current successes, "We need to talk to each other and work as a collaborative, not just as individual laboratories." Board member and biopharma venture capitalist, Frank Gentile, PhD, spoke about why Usher 1F Collaborative is uniquely positioned for success, "... to ensure that a product can get developed, we need to be great stewards of your money."

Following the screening of *Seeing Forward*, Usher 1F Ambassador Dorie Shapiro and Tzila Seewald-Russell, daughter of board member Julian Seewald, spoke about their lives with Usher 1F. Dorie talked about her determination to travel the world while she still has her vision. "I've taken in the sights with an intensity and appreciation I've never felt before. Each breathtaking landscape, historic site, and natural wonder has become an opportunity to capture the world's beauty with my remaining vision." Tzila talked about her struggles as a parent with Usher 1F, as well as her hope for the future. "I want to see my children grow up and get married." Both spoke of their gratitude to the supporters who have stood by us throughout the past ten years, helping to make their dream of a cure for Usher 1F a reality. Added Dorie, "Their unwavering support has been a source of inspiration and motivation for me."

Rachel and Jared Root

Tzila Seewald-Russell and Dorie Shapiro

Christian Guardino performed during the 10th anniversary celebration.

American Idol and *America's Got Talent* contestant, singer Christian Guardino performed at our celebration of the first decade of our journey. Christian is a recipient of retinitis pigmentosa, Leber's Congenital Amaurosis, Christian received gene therapy at age 13 that saved and restored much of his vision. He spoke of marveling at what he saw for the first time and what his vision has meant for his life. Christian's journey reminded all of us that our quest for a cure is within reach.

Usher 1F Collaborative co-founders, Melissa and Elliot Chaikof, closed the program, speaking of their awe and excitement of our progress to date, their hopes for the future, and the need for continued support to realize our collective dream for a cure. ◆

USHER 1F COLLABORATIVE

10th ANNIVERSARY

NEWSLETTER

Fall 2023

Usher 1F Collaborative board members and staff

Usher 1F Collaborative: Reflecting on and Celebrating Ten Years of Achievements

By Melissa and Elliot Chaikof, Usher 1F Collaborative cofounders

As the parents of two daughters with Usher 1F, ten years ago, we decided we could not sit back and wait for someone else to do something while the loss of their vision progressed. We hired attorneys, formed Usher 1F Collaborative, applied for 501c3 tax exempt nonprofit status, deposited $2000 into a bank account, had our daughter design our first website, and reached out to all our family and friends for help. We very much hoped we could make a difference, but we had no idea of all that we would achieve in only ten years.

Cofounders Elliot and Melissa Chaikof.

IF YOU BUILD IT, THEY WILL COME

We started our work knowing one other family with Usher 1F, but over the years, initially through networking and then through our online presence, many more have joined us. We now have 70 individuals in over 50 families working together in our quest for a cure.

SUCCESS BREEDS SUCCESS

More families working together meant increased fundraising, and greater fundraising has led to more funded research. We began funding one research lab at the University of Oregon Institute of Neuroscience to create a zebrafish model so that we would have a tool on which to test potential treatments. Over the past ten years, we have grown from funding one laboratory to funding nine academic research teams, initially casting a wide net and now honing in on those therapies most likely to successfully transition from the lab to the clinic.

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TURNING POINTS

In 2017, we held our first scientific conference in Boston, opening it up to the Harvard community, in particular to the Harvard Stem Cell Institute. One of those who attended was David Corey, PhD, the Bertarelli Professor of Translational Medical Science in the Blavatnik Institute at Harvard Medical School. Dr. Corey had spent years studying the role of the Usher 1F gene in the auditory system. Meeting our daughters inspired Dr. Corey to try to help. "We know so much about this gene, if we don't try to do something for the disease, who else is going to?" Corey said. Fast forward to 2023, and Dr. Corey has created three gene therapies, with one in particular, an Usher 1F mini-gene, showing great promise in restoring hearing in our mouse model and vision in our zebrafish model.

In 2020, the Usher 1F Collaborative was one of the first group of patient-led rare disease organizations to receive a 'Rare As One' Project grant from the Chan Zuckerberg Initiative (CZI) of $600,000 over three years. The purpose of the grant was not to directly fund research but to help us grow as an organization, become financially self-sustaining, and ensure continued funding for research. Three years later, we have done just that, with a full-time Development Manager, Sarah Gauch, consultants whose expertise help us strengthen our work, and numerous trainings from experts CZI recruited on topics ranging from drug development to fundraising. Public recognition of the CZI award has also resulted in press coverage and notice from funders.

SUMMARY OF OUR ACHIEVEMENTS

In ten years, we have grown Usher 1F research from zero laboratories to nine independent investigative groups, with almost $9 million in funded research, resulting in the development and testing of four drug therapies, one stem cell therapy, seven gene therapies, two of which have gained interest from major pharmaceutical companies and one therapy within five years of a clinical trial. We have partnered with Foundation Fighting Blindness to pursue an international study at ten clinical sites, RUSH1F, to characterize the rate of progressive vision loss that will provide data crucial for a future clinical trial.

CELEBRATING OUR SUCCESS AND LAUNCHING OUR NEXT CHAPTER

On October 10th, 2023, over 100 people, including much of the Usher 1F Collaborative Board, gathered

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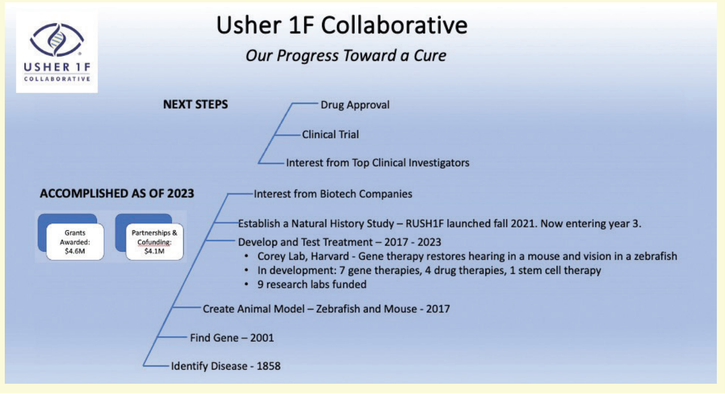
WHAT WE'VE ACCOMPLISHED IN TEN YEARS



Brendan Creemer: Taking Control of His Future

Brendan Creemer, age 24 has Usher 1F, but rather than sitting on the sidelines waiting for scientists to find a cure for his vision, he has taken the bull by the horns to find that cure himself.

Brendan was born in Palo Alto, CA. Through newborn hearing screening, his parents Stacey and David learned immediately that he was profoundly deaf. It was after he received his first cochlear implant at age 12 months that they recognized his vestibular dysfunction resulting in late gross motor milestones. They believed it was related to his deafness and consulted doctors, who felt Brendan likely had Usher syndrome. An electroretinogram (ERG), which measures how well the retina is functioning, confirmed their fears, showing early signs of retinitis pigmentosa (RP), the vision loss component of Usher syndrome.

Brendan was only 22-months-old when he received the diagnosis, and so Stacey and David waited to tell him until he was older. The diagnosis left them devastated, scared, and anxious. Although they searched for information and support, organizations such as Usher 1F Collaborative did not yet exist. Also, genetic testing wasn't yet readily available to identify Brendan's subtype.

In 2009, when Brendan was ten, his parents told him about his diagnosis. Initially, he was not too bothered. He didn't mind his hearing loss and thought his cochlear implants were "cool," and vision loss seemed to him to be far in the future. He didn't even notice his peripheral vision loss because the progression was slow, and his brain learned how to adapt.

Brendan was 14 when he became fully aware of the implications of his Usher syndrome diagnosis. Usher 1F Collaborative was founded that year, and Stacey wrote his story to be included in the Stories section of the foundation website. Brendan read his story on the site and felt that it portrayed a bleak outcome for his future. At the same time, he was having issues with over accommodation at school. He found all the aides around him overwhelming even though also helpful at times. He didn't like being alienated from other students because of his disabilities. Brendan became angry, realizing none of this would have happened had he not had Usher syndrome. He decided that Usher syndrome was the number one problem in his life and all other problems were irrelevant. He wasn't comfortable with the label, "a person with vision loss" defining him in society. He states, "We are who we choose to be. We should not let society define who we are. The only person who decides who or what I am is me."

Brendan Creemer at the University of Iowa Carver Biomedical Research Building.

Science was always Brendan's favorite subject in school. In his first year of high school, he was "wowed" by a unit on biotechnology in his biology class, in particular how scientists could edit DNA to create new species, for example glowing mice. Up until that point, he had thought this was science fiction. This newfound knowledge coincided with his revelation about his Usher syndrome, energizing him to do whatever it took to find a cure for his condition. After all, if scientists could create mice that glow green, then could they change the genes in retinal cells to reverse blindness? Then he learned that this research was already a reality and resolved that this would be his career path. He would combine his love of science with his personal struggle to find a cure for himself and others. He was not willing to sit back and give up.

Brendan attended Lewis & Clark College in Portland, OR, initially studying biology then changing his major to biochemistry when he realized the curriculum would be better suited for his goal of a career in biomedical research. During the summer following his sophomore year of college, he sought an internship related to his chosen field. Through the Oregon Commission for the Blind, he learned about a research lab at Oregon Health & Science University run by Drs. Martha Neuringer and Trevor McGill. When these scientists learned of Brendan's interests, despite not originally

Brendan Creemer Continued on page 5

Sarah Gauch speaking at the Chan Zuckerberg Initiative Rare as One Annual Conference.

Chan Zuckerberg Initiative Convening

By Sarah Gauch, Usher 1F Collaborative Development Manager

In September, I had the opportunity to represent Usher 1F Collaborative at the Chan Zuckerberg Initiative (CZI) Rare As One Annual Conference. As you may know, Usher 1F Collaborative was selected to be part of the CZI Rare as One network as a Cycle 1 grantee. Recognizing that rare disease patients around the world are building powerful communities and research networks, CZI aims to strengthen these patient organizations by building capacity and promoting collaboration.

CZI requested that Usher 1F Collaborative present our research to the in-person and virtual attendees. I was honored to share details of the innovative gene therapy research conducted in Dr. David Corey's lab, and our exciting results elicited applause from the crowd. Following my presentation, I was a participant on a panel discussion titled "Patients Driving Breakthroughs and Progress in Rare Disease Research, Treatments and Cures." Of a network of 50 worldwide grantees, Usher 1F Collaborative is a recognized leader in patient-driven research.

USHER 1F COLLABORATIVE IS A RECOGNIZED LEADER IN PATIENT-DRIVEN RESEARCH

We remain deeply grateful to CZI for their investment in Usher 1F Collaborative. The opportunity to participate in a network of rare disease patient organizations has made our team stronger and the resources provided by CZI has elevated our organization.

At the conference, a keynote speaker, Mark Hund, CEO of EB Research Partnership, spoke about the difference in how cows and buffalo react to an oncoming storm. As a storm approaches, cows herd together and run away from the storm. Inevitably the weather catches up with the cows so instead of outrunning the storm, they actually run right along with the storm. By contrast, buffalo run directly into the storm – facing the adversity head-on and limiting the amount of time that they endure the weather. The symbolism is powerful for the Usher 1F Collaborative community. Armed with the knowledge that there is a treatment on the other side, our network of families, donors and scientists face the storm head-on. While the challenges can be overwhelming now, a brighter future is in sight for those who live with Usher 1F. ◆