USHER 1F
COLLABORATIVE

THE TEN TO CURE
INITIATIVE

PROBLEM

Usher Syndrome is the leading cause of inherited deaf-blindness, and type 1 is the most severe. Children with Usher 1 are born profoundly deaf and progressively lose their sight.

Usher 1F Collaborative is a 501c3 nonprofit foundation whose mission is to fund medical research to find an effective treatment to save or restore the vision of those with Usher Syndrome type 1F.

IMAGINE IF...

We could leverage the world's best brains, current science, cutting edge engineering and new technology to find a cure for blindness caused by a debilitating genetic disease.

What would this mean?

THIS

INSTEAD OF THIS

TEN TO CURE INITIATIVE

ZACHARY'S FUTURE WOULD BE TRANSFORMED

....AND HIS PARENTS COULD SLEEP AT NIGHT

Zachary Root has Usher 1F. He is a typical little boy who doesn't stop talking and has these magic ears. Those who know him best find it hard to believe he is deaf. Zachary is happy, curious, funny, smart, creative and he loves his family and friends deeply. He is oblivious to the future in store for him which includes blindness.

"Our family puts on a brave face, but we have debilitating fear that consumes our lives. Each time we get a call from school that Zachary has tripped, or when Zachary enthusiastically talks about driving -- or the many other aspects of his future, typical of a little boy, our hearts ache. My husband and I have sleepless nights, suffocating worry, hidden tears and outright panic which we don't typically discuss with others. We are scared of the day this cruel disease will rob our amazing Zachary of his vision -- a fear no parent should ever have to worry about."

- RACHEL ROOT, ZACHARY'S MOM

"There are countless mothers who rise each day determined to move mountains."

-PHILIP REILLY

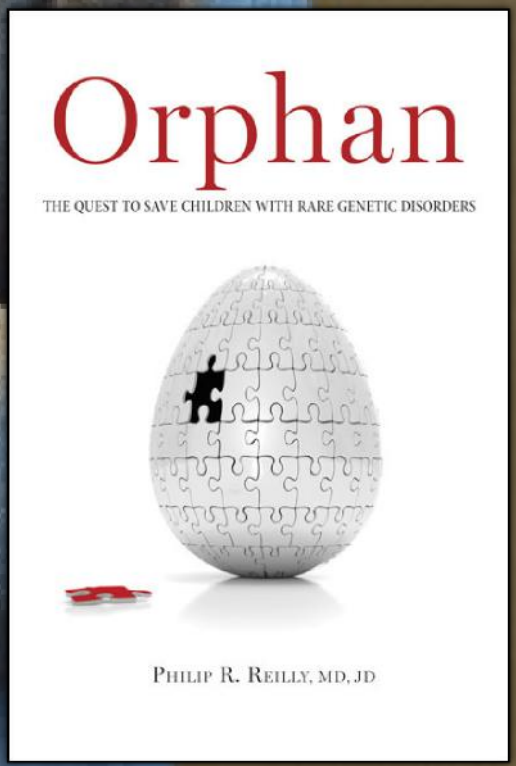


Photo of Philip Reilly taken at May 2017 Usher 1F research conference. Dr. Reilly, MD, JD is a **world renowned expert on medical genetics** and a venture partner focusing on starting companies to develop innovative therapies for orphan genetic diseases.

"We chose to fund the Usher 1F Collaborative, in part, because of the strength of the organization's leadership and relationships to the scientific research in its rare disease area."

-CHAN ZUCKERBERG INITIATIVE REPRESENTATIVE

CRACKING THE CODE

The ripple effect of cracking the code to this genetic mystery would lead to other scientific breakthroughs, uncovering cures for additional rare diseases falling through the cracks of big pharma and academic research.

New paradigms would eliminate so much needless suffering.

An extraordinary team of parents, doctors, academic researchers, biomedical engineers and world-renowned scientists are doing more than imagining....

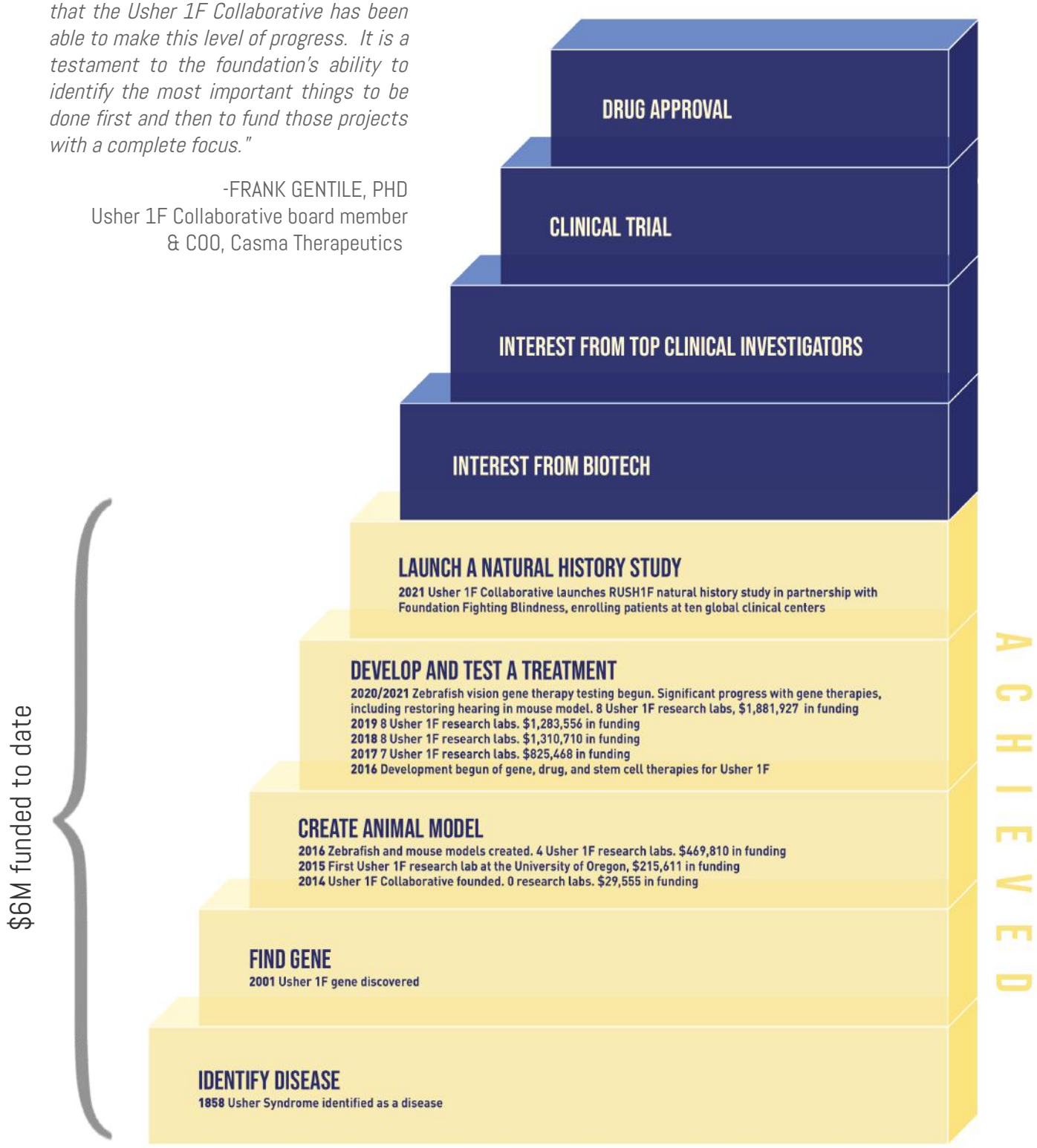
They have embarked on a laser-focused initiative under the Usher 1F Collaborative to find a cure for this devastating rare, disease, causing hearing loss and eventual blindness.

BUILDING BLOCKS TO DEVELOP A TREATMENT FOR A GENETIC DISEASE

OUR PROGRESS TOWARD A CURE

"I believe it is nothing short of remarkable that the Usher 1F Collaborative has been able to make this level of progress. It is a testament to the foundation's ability to identify the most important things to be done first and then to fund those projects with a complete focus."

-FRANK GENTILE, PHD
Usher 1F Collaborative board member
& COO, Casma Therapeutics

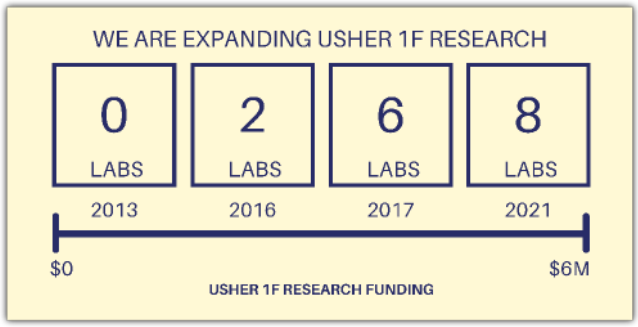


THE TEN TO CURE INITIATIVE

CUMULATIVE GOALS & TIME FRAME

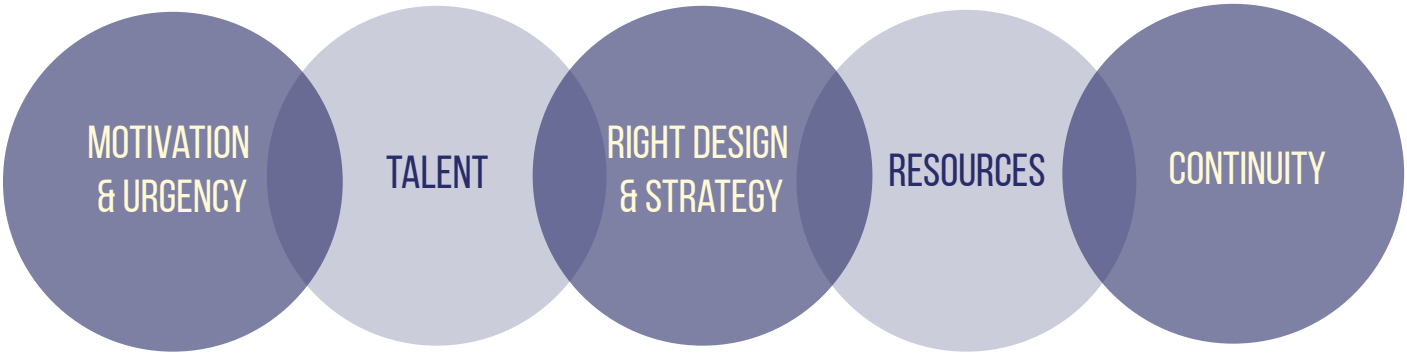
$500,000 2020 → $1.5M 2021 → $3M 2022 → $5M → $10M

CUMULATIVE AMOUNT	HOW TO EXPEND FUNDS	IMPACT ON COLLECTED WHOLE
$500,000 2021	$500,000 university research labs focused on finding a treatment that works in the lab in our animal models	Advance research in the lab toward proof of concept (treatment that works in the lab).
$1.5M 2021	Drug screen $0.5M $500,000 university research labs	Identify existing drug(s) for efficacy for Usher 1F. Continue funding university labs research to reach proof of concept.
$3M 2022	Drug screen $1.25M $750,000 university research labs	Identify existing drug(s) for efficacy for Usher 1F. Continue funding university labs research to reach proof of concept.
$5M	Drug efficacy, safety studies $2M	Pre-clinical trial work.
$10M	Phase 1/2 clinical trial	Begin testing in humans.



THE TEAM WILL SUCCEED BECAUSE

WE HAVE THE ESSENTIAL INGREDIENTS



THE RIGHT DESIGN & STRATEGY — WITH AN INCENTIVE SYSTEM TO ACHIEVE THE REQUIRED PATIENT OUTCOME IS KEY TO OUR SUCCESS. COMBINED WITH MOTIVATION & URGENCY, ALONG WITH TALENT, RESOURCES AND CONTINUITY, OUR TEAM HAS THE ESSENTIAL INGREDIENTS.

"Learning that a child has a dual sensory impairment with Usher 1F Syndrome is a life-changing moment. The Usher 1F Collaborative is converting despair into hope through action-. They are directly funding the generation of the animal models essential for understanding the molecular basis of this disease, and facilitating the exploration of a broad range of potential treatments. My service to this cause — and the people behind it — gives greater depth and meaning to my research every time I step into the laboratory."

-JENNIFER PHILLIPS, PHD

VISIONARY TEAM
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VICE PRESIDENT & DIRECTOR
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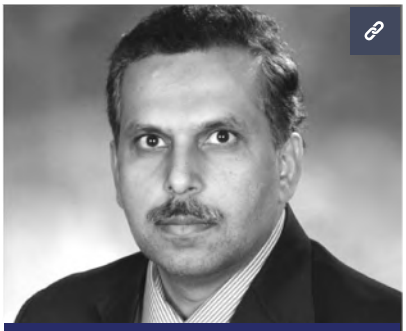
USHER 1F
COLLABORATIVE

VISIONARY TEAM
LEAD SCIENTISTS, ENGINEERS & RESEARCHERS

A multidisciplinary team pursuing numerous paths across continents comprised of internationally respected researchers and scientists.

DAVID COREY, PHD
HARVARD MEDICAL SCHOOL

MONTE WESTERFIELD, PHD
UNIVERSITY OF OREGON
INSTITUTE OF NEUROSCIENCE



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UNIVERSITY OF PITTSBURGH

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UNIVERSITY OF IOWA
INSTITUTE FOR VISION RESEARCH

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UNIVERSITY
OF WESTERN AUSTRALIA

VINCENT TROPEPE, PHD
UNIVERSITY OF TORONTO

ALEX HEWITT, MBBS, PHD
CENTRE FOR EYE RESEARCH AUSTRALIA

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SCIENTIFIC ADVISORY COUNCIL

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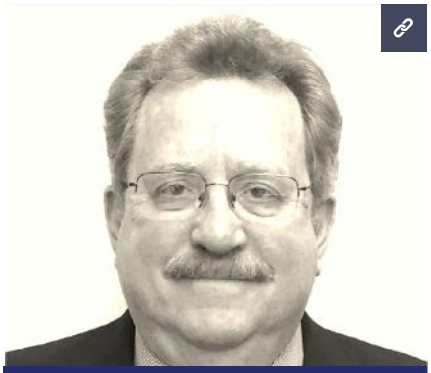
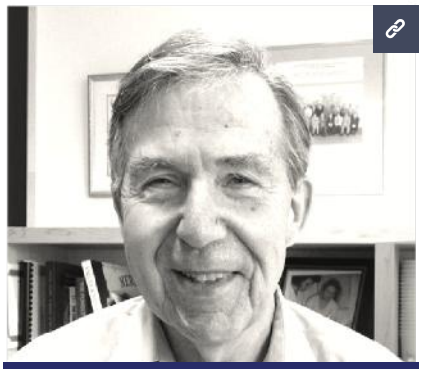
NYU GROSSMAN SCHOOL OF MEDICINE



RICHARD CUMMINGS, PHD
BETH ISRAEL DEACONESS MEDICAL CENTER AND HARVARD MEDICAL SCHOOL

DAVID LIU, PHD
HARVARD UNIVERSITY DEPARTMENT OF CHEMISTRY AND CHEMICAL BIOLOGY



EDWARD SCOLNICK, MD
BROAD INSTITUTE OF MIT AMD HARVARD

In 2013, Usher 1F Collaborative was founded with the explicit goal of finding an effective treatment to save or restore the vision of those with Usher Syndrome type 1F. Since then, there has been prolific scientific research thanks to the almost $5 million contributed by generous donors.

Step by step, progress has been steadily advancing in our laboratories.

None of this would have been possible without the researchers who devote their work to our mission. Recognizing the integral role that scientists play in the hunt for a treatment, Usher 1F Collaborative formed a Scientific Advisory Council in 2020. This body of four esteemed scientists provides strategic guidance and advises on timing and implementation of proposals.

TOP PHILANTHROPIC PARTNERS & DONORS

S. DANIEL AND EWA ABRAHAM

LEO H. BENDIT CHARITABLE FOUNDATION

BLAVATNIK FAMILY FOUNDATION

CHAN ZUCKERBERG INITIATIVE

SHEILA AND DR. JEREMY CHESS

GANZ FAMILY FOUNDATION

DAVID AND BARBARA B. HIRSCHHORN FOUNDATION

WILLIAM S. AND INA LEVINE FOUNDATION

$6.0 million raised to date
Full list of donors available upon request

ELLIOT CHAIKOF, MD, PHD

"The development of animal models of Usher 1F has been a major milestone that is allowing our investigators to dissect the mechanisms of action of PCDH15, and most importantly, approaches to therapeutic intervention through the use of gene therapy, small molecule drug therapy or other approaches. We promote team based science to ensure that all research groups are sharing resources and collaborating in our search for a cure."

KEY FACTS

Usher 1F scientists and researchers span the US and multiple continents

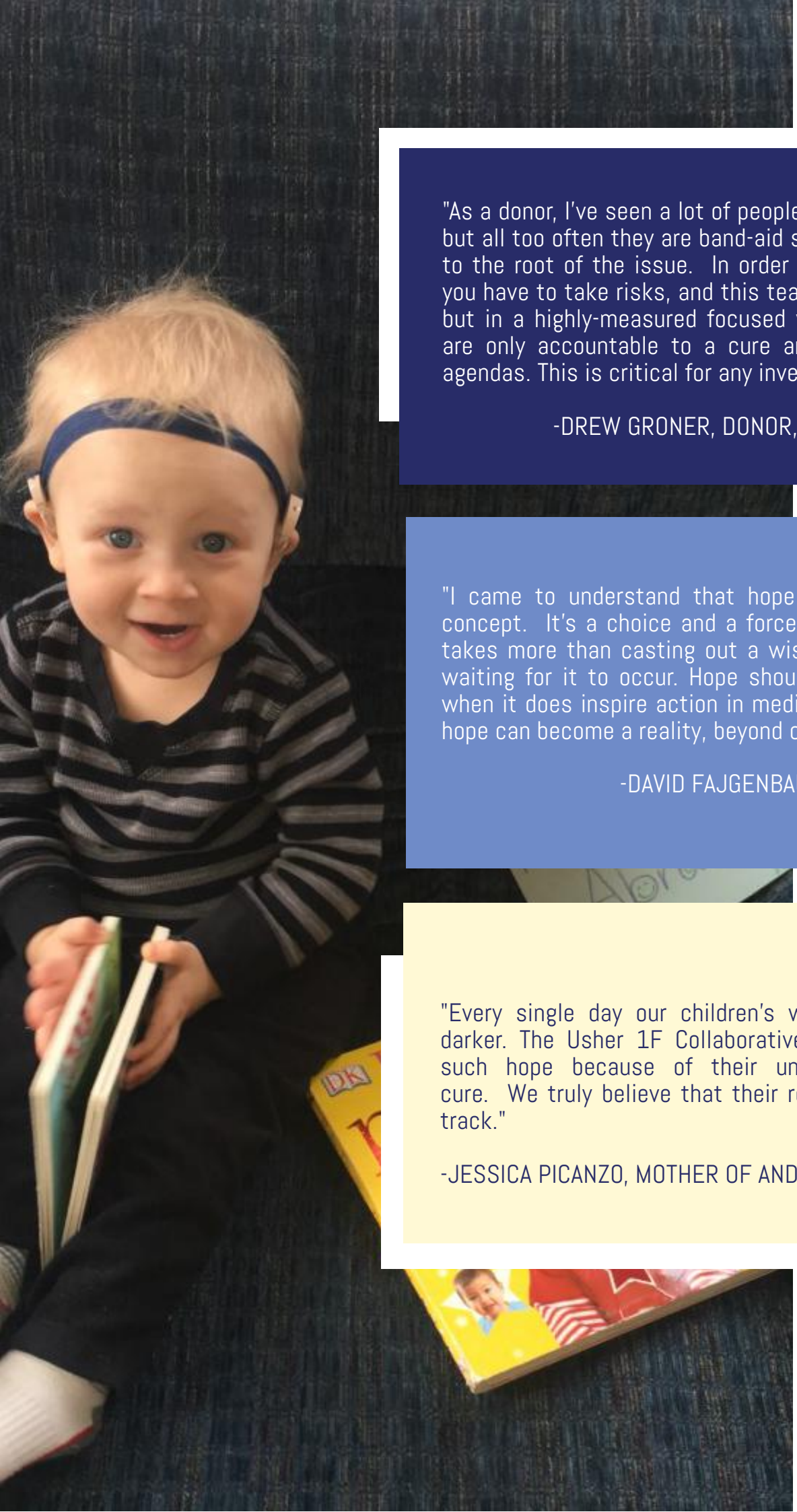
Usher 1F team is leveraging networks and knowledge built over decades

The team is collaborating vs. working in independent silos

COURAGE TO CHANGE OUTCOMES FOR OUR COLLECTIVE VISION

"As the days go by my sister and I, and everyone living with Usher 1F, continue to lose the ability to see the world and live independently. We want a cure as soon as possible to restore the abilities that we are losing."

-Rachel Chaikof



"As a donor, I've seen a lot of people give to similar things, but all too often they are band-aid solutions that don't get to the root of the issue. In order to get to root causes, you have to take risks, and this team is willing to do that, but in a highly-measured focused way. In addition, they are only accountable to a cure and not any one else's agendas. This is critical for any investor."

-DREW GRONER, DONOR, CORPORATE SPONSOR

"I came to understand that hope cannot be a passive concept. It's a choice and a force: hoping for something takes more than casting out a wish to the universe and waiting for it to occur. Hope should inspire action. And when it does inspire action in medicine and science, that hope can become a reality, beyond our wildest dreams."

-DAVID FAJGENBAUM, *CHASING MY CURE*

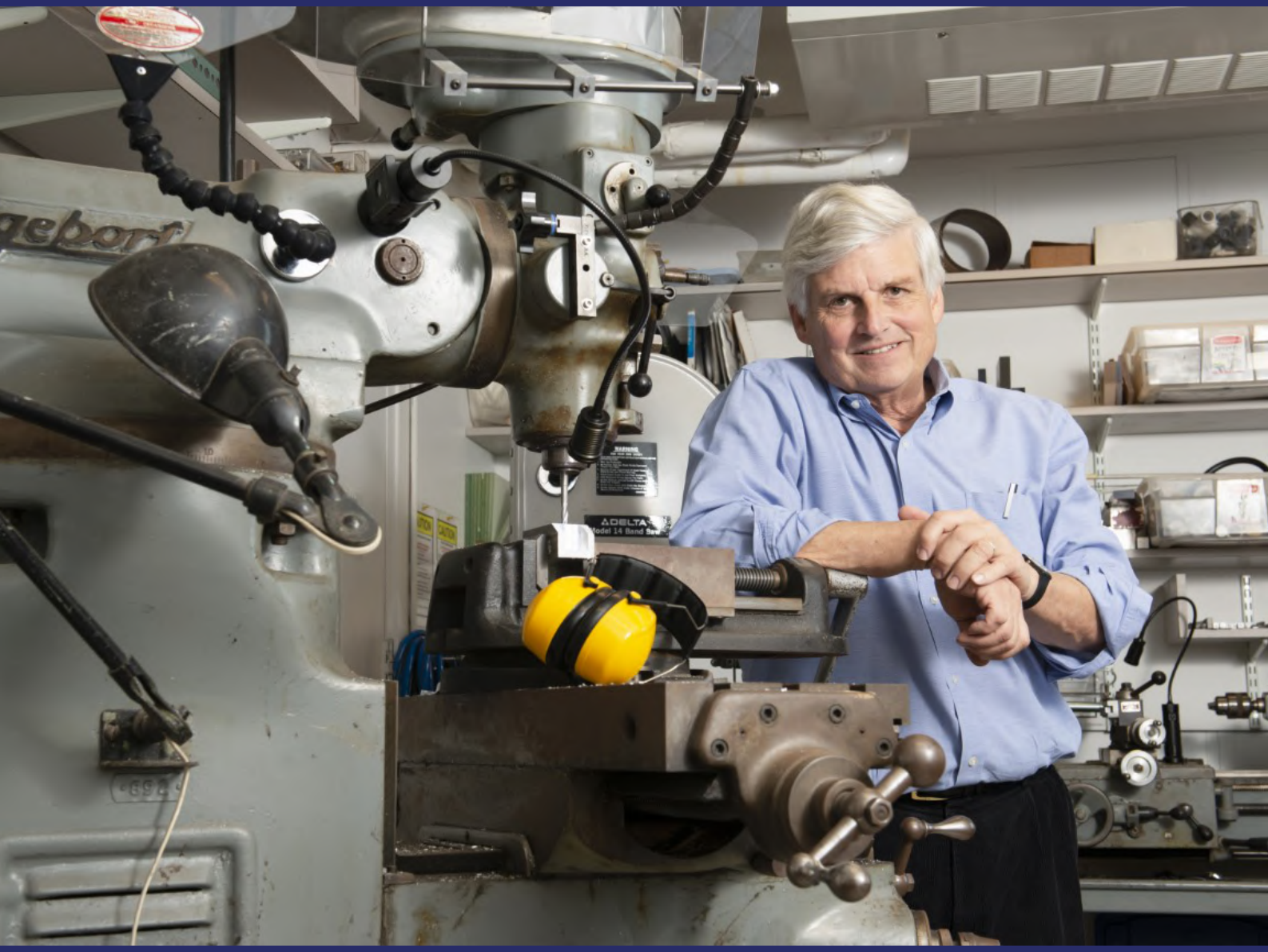
"Every single day our children's world gets increasingly darker. The Usher 1F Collaborative has given our family such hope because of their unabated pursuit for a cure. We truly believe that their research is on the right track."

-JESSICA PICANZO, MOTHER OF ANDI AGE 4 & AARON AGE 2

USHER 1F
COLLABORATIVE

OUR BOLD VISION REQUIRES

Donors who seek transformative outcomes are sought to join this team whose sole agenda is a race for a cure for Usher 1F which can instigate other innovations in genetic and Medical Research.



PLEASE JOIN US

USHER 1F
COLLABORATIVE

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