TO CURE A ROSE
IMPACT REPORT

2023

We develop therapeutics for children
with rare genetic diseases

A letter from our

FOUNDER & CEO

Dear Friends, Supporters, and Champions of To Cure a Rose Foundation,

As I sit down to write a synopsis of the year, it occurs to me that I'm in awe of how far we've come. Every step of the way, we have not only moved a treatment for Rose and others, but we have built partnerships, systems, and models that will be used to significantly cut costs and time for our next disease we go after. I can't tell you how excited I am of what's to come. I am reminded as I looked through videos of Rose as a younger child, of how many things she has lost. The nuances of communication, attention, and curiosity. We must not give up.

A Journey of Resilience: Our journey with TCAR has been one of determination and strategy. Instead of giving up or going for easy goals, the science team, the board, and the staff have consistently pushed through. One instance is the incredible detail of optimizing the design, manufacturing, and chemistry of our ASOs. These small very detailed decisions could be the difference in a drug that is safe or is not. I'm so proud of our team's ability to learn, grow, and ask the right questions, and people for answers. Building an organization is hard. I've sat on boards before, helping make decisions but have never been the one executing it, especially running teams of brilliant neuro scientists and drug developers. Every event, campaign, or scientific risk we have taken have been made after diligent board members, teammates, and mentors weighing in. And of course, maybe most importantly, you showing up when we needed you. 2021 - 2023 was largely us learning how to be TCAR, building partnerships, and how to make genetic treatments effectively. My hope is that we continue to grow into a more mature organization, with an even larger impact in the year and years to come.

Hope in a Vial: The year 2023 saw us realizing the dream of providing effective treatments for rare genetic diseases. Our work with this first drug we are naming, "Rosipherson" has yielded remarkable results, kindling a spark of hope in the entire team. This treatment, as we further its development, is the whisper of a scaled approach bringing treatments to so many more children.

The Power of Voice: My learnings have showed me that largely the problem in healthcare for Rose and the millions of other children is how we have organized our healthcare system. We have catered to single large profit drugs, as opposed to many small profit drugs. We are entering an age where personalized genetic treatments require a new model. I have spoken all over the United States about this. I've been Invited to be the keynote for organizations like the Milken Institute and the National Organization for Rare Diseases. It's imperative that we continue to spread TCAR's mission and innovative approach, as the need is great, but the workers are few.

A Pledge for Tomorrow: I am excited for 2024 and beyond. I know that many things will need to change, due to the millions of dollars needed to get our first drug into a trial. But already, plans are coming together. We will continue to build that bridge to technology with sustainability in mind for these children who so desperately need it.

Our promise is simple: we will work tirelessly to bring these genetic treatments to fruition. But that will only work if you continue to support us. For that, I can't tell you how grateful I am. Together, we are mixing cutting edge science, sustainable strategies, and scalable technologies to save children's lives. What could be better than that?

With profound gratitude and boundless hope,
Casey McPherson
CEO, To Cure a Rose Foundation

THE GLOBAL PROBLEM

There are over **10,000** rare diseases but only **5%** have cures.

400 million people worldwide are living with a rare disease

Half of them **are children**

~30% of children diagnosed with a rare disease will die before their **5th birthday**

We have the technology to cure many of these diseases in as little as **12 months.**

OUR KEYS TO DEVELOPING PRIORITIES

Heart

Driving connections and creating real, human, emotional value

Mind

Leveraging creative & critical thinking for change

Science

Systematic study to save the lives of children with rare diseases

Vision

We see a world where the next child like Rose has hope. Where the next child born with a rare genetic disease has the ability to receive a customized drug within days of their diagnosis.

Mission

We believe every child deserves access to a cure. We create genetic treatments faster and less expensive than ever done before, to cure children with rare genetic diseases

Goals

Cure Rose and her disease in HNRNPH2. Then scale our platform approach to pave the new path, and save children with rare diseases at an exponential rate.

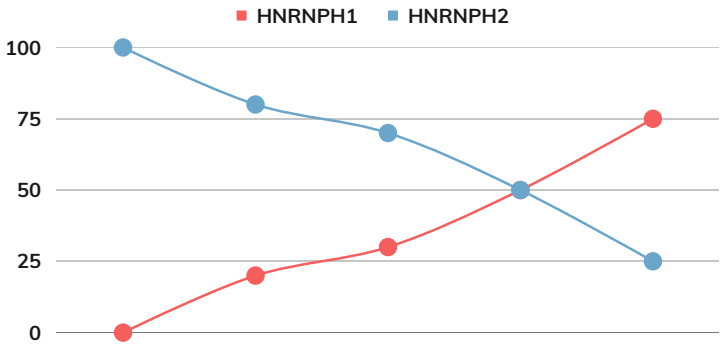
"What started with a blood draw from Rose has now become a potential treatment for thousands. It's the fundemental belief and generous support by our donors that has allowed us to accomplish this!"

OUR HOPE

Casey, Rose, and Weston in his lab, Everlum Bio

DATA FOR OUR POTENTIAL NEW DRUG - ANTISENSE OLIGONUCLEOTIDE TARGETING HNRNPH2 GENE EXPRESSION

We see in animal models that have an H2 mutation like Rose's, that many of their abilities can be rescued by knocking down the expression of the H2 gene. Once knocked down, H1 over expresses, bringing the much needed protein back to the brain. We have developed seven versions of “Rosipherson” antisense oligonucliotides that are working in human neurons!

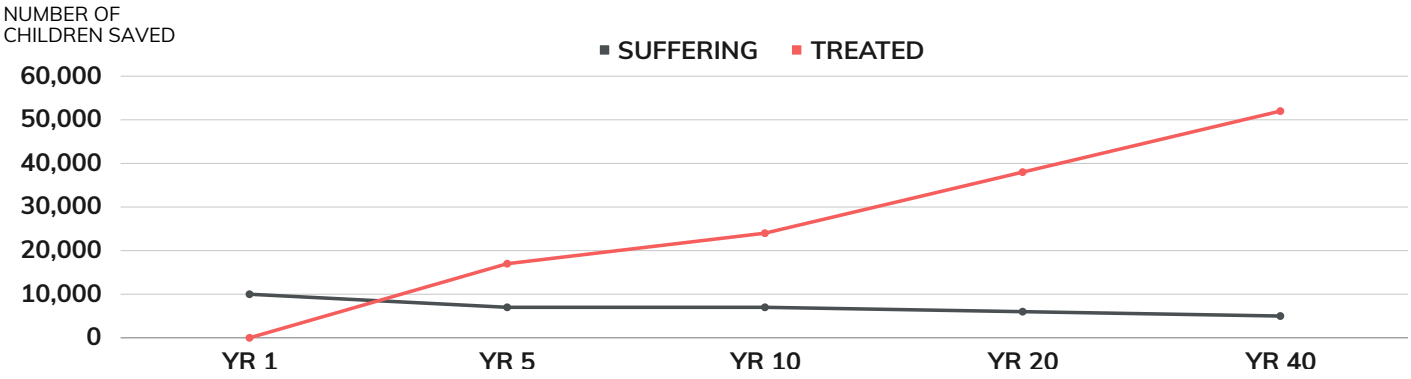


YOUR LEGACY

How starting with Rose echoes through generations.

THE ACTIONS OF ONE, RIPPLE THROUGH TIME

New data estimated that there are 10,000 people currently suffering worldwide, and 2100 born every year from mutations in the HNRNPH2 gene. A genetic treatment makes an impact forever, for the next Rose that’s born, and for generations to come.



*(*Numbers based on Broad Institute calculations)*

MAJOR WINS 2023

We Have a TEAM

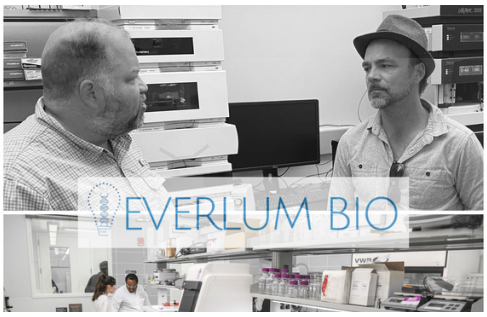
We are a part of a vast network of donors, scientists, and drug developers who are organizing the science, our partners, and creating our regulatory path for this treatment to get through the FDA into the lives of patients.

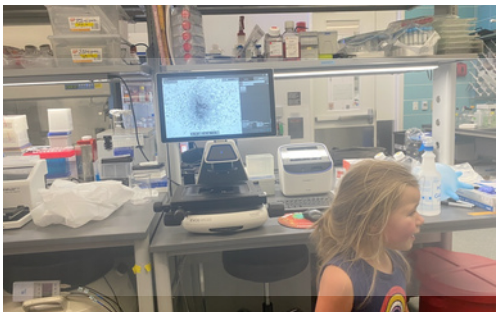
We Have a LAB

In June of 2022, Casey McPherson founded Everlum Bio, a lab to offer preclinical services to all family foundations. Affordable, and unencumbered by IP issues.

A Potential TREATMENT

We have data to show that we can treat Rose's disease in brain cells. This is a requirement of the FDA, and a potential cure for all children with HNRNPH2.





CELEBRATIONS OF 2023

Created the First Proof of Concept Treatment for HNRNPH2 Called "Rosipherson"

Through your generous donations, we have reached our first major milestone of a treatment for Bain Syndrome, a disease from mutations in the HNRNPH2 gene. Rose and potentially 10,000 others affected by this disease could benefit from this new genetic treatment we have named "Rosipherson."

Filed Patent on New Drug

Because TCAR owns the patent, we are able to ethically license this curative treatment to be further developed. Creating a potentially sustainable path for more cures for more children!

Applied for 3 Major Grants

TCAR has applied for grants with the Discovery Foundation, NIH, NCATS, and RTW Foundation, and awarded a $53,000 grant for 2024 from Discovery Foundation.

MAJOR CURE MILESTONES

Our journey has been defined by transformative moments. From the inception of groundbreaking ideas to their tangible realization, each milestone represents a leap forward in our pursuit of innovating for these suffering children with no options. These markers signify incredibly difficult cutting edge scientific accomplishments. From conceptual breakthroughs to real-world impact, these milestones narrate a story of vision, perseverance, and the profound impact of your contributions.

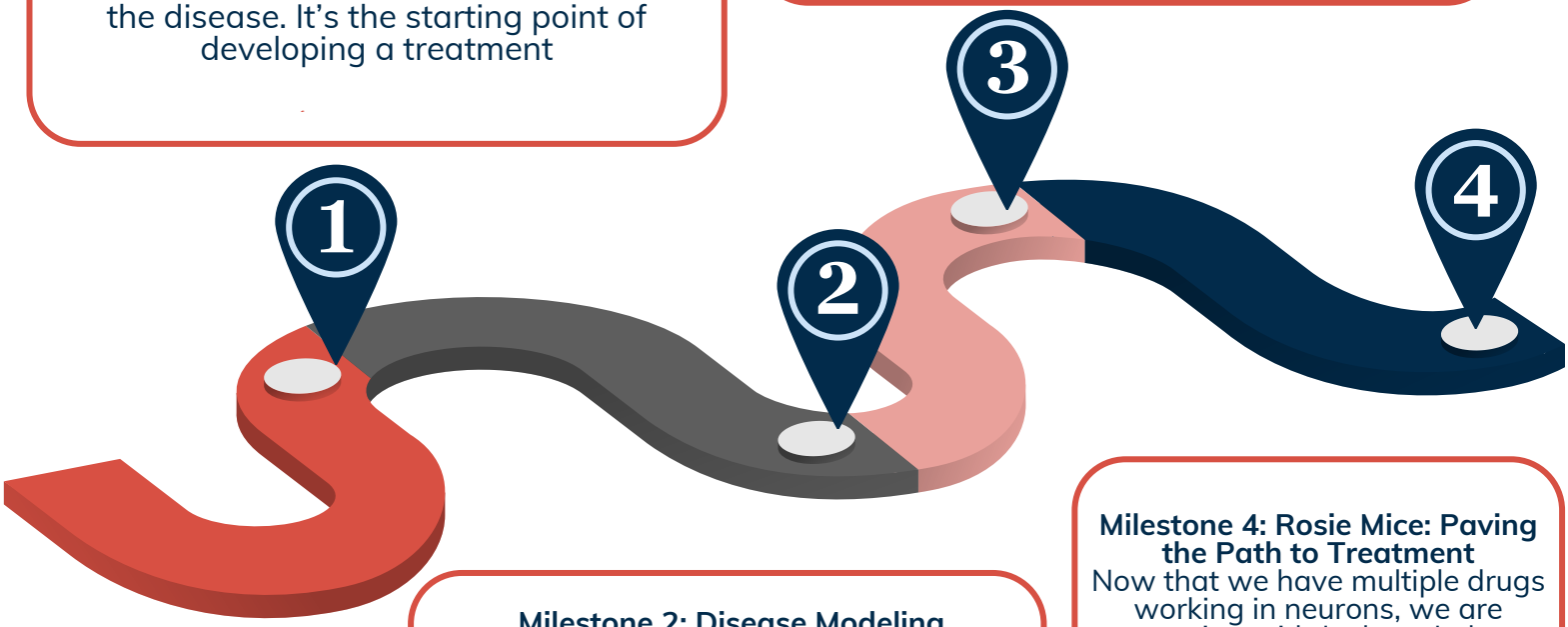
The dollar amount listed at each milestone reflects an estimate of how much each stage cost.* ***None of this would be possible without YOU.*

$150K

Milestone 1: Unlocking Hope: Rose's Stem Cells
We took a sample of Rose's blood and derived multiple stem cell lines - these stem cells gave us the ability to turn them into neurons, and learn more about the disease. It's the starting point of developing a treatment

$250K

Milestone 3: Creating a Treatment
We decided to culture healthy human neurons, to begin our drug testing, due to the complications with how Rose's neurons performed. Our team created thousands of antisense oligos and identified 50 that emerged as promising candidates for rigorous screening. We have patented seven versions of the drug we deem as "Rosipherson" which are working in human neurons.



Milestone 2: Disease Modeling
With those stem cells, we performed different experiments to determine the best technology to use to fix the mutation. We did proteomics and additional genetic sequencing as well as turned the cells into neurons to characterize them. We learned that using an RNA therapeutic called an "antisense oligonucleotide" would help us achieve our goal.

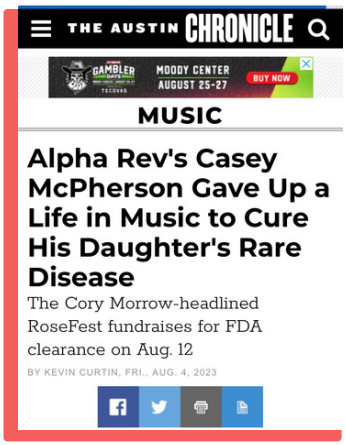
$75K

Milestone 4: Rosie Mice: Paving the Path to Treatment
Now that we have multiple drugs working in neurons, we are partnering with Jackson Labs to test the drugs in mice to determine things like safety, efficacy (does it work), and other properties before we take the candidate to the FDA. We're also doing safety testing with other experiments, as well as re-screening the candidates at a different lab to verify our data.

$250K

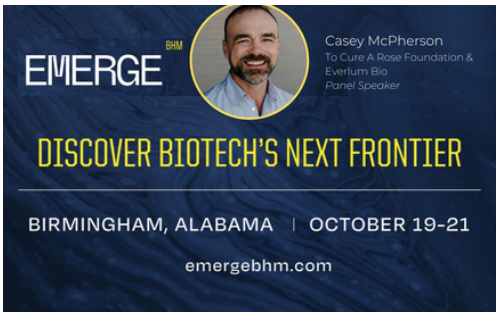
AWARENESS

(Click on each logo/image to read/watch/listen to the interview)



ADVOCACY

Casey is speaking at a number of medical research & biotech conferences, and even testifying at Senate committees.



Mind

Science

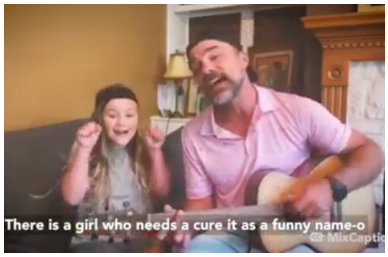
Music is intricately woven into our work, from benefit concerts to writing original songs that reflect Rose's experiences. One of rare disease's biggest issues is the human connection required to be felt in order for those in power to change the systems needed to open up this area of medicine and unmet need. Casey has used his music as a way to tell the story of these children and families that are fighting for hope. It gives a way for the scientists, regulators, and healthcare companies to connect with the core reason why they do what they do. To help people. Rare disease continues to be on the bottom of the list of people that are helped.

Heart

SONGS

I Won't Wait

Talk To Me



O-L-I-G-O

Is It Enough

What's a Rosie Say?

Who Would You Be

WE'RE BUILDING POWERFUL PARTNERSHIPS

We have developed partnerships with Jackson Labs, La Jolla Labs, Facet Life Sciences, NCATS, and Weave Bio, all to create a more cost-effective, streamlined platform to develop more cures for more children.

weave

La Jolla Labs

NIH National Center for Advancing Translational Sciences

The Jackson Laboratory

Leading the search for tomorrow's cures

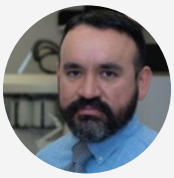
FACET LIFE SCIENCES

SCIENTIFIC LEADERSHIP TEAM

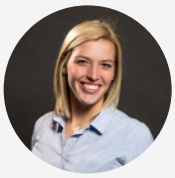
Our Leadership Team

Casey McPherson
CEO / Founder

Dr Richard Finnell
SAB Chair



Robert Cabrera Phd
Chief Science Officer



Courtney Banks PhD
Director of Programs

Huiping Zhu
Chief Medical Officer

Mentors, Partners, and Advisors

Dr Cat Lutz
Jackson Labs

Dr Rodney Bowling
Everlum Bio

Yael Weiss
Mahzi Therapeutics

Ethan Peralstein
Perlara

Julia Vitarello
Board Member

“I WANT TO HEAR ROSE SAY DADDY AGAIN, PLAY IN THE PARK, AND MAKE FRIENDS. BUT WITHOUT A GENETIC TREATMENT, SHE NEVER WILL.”

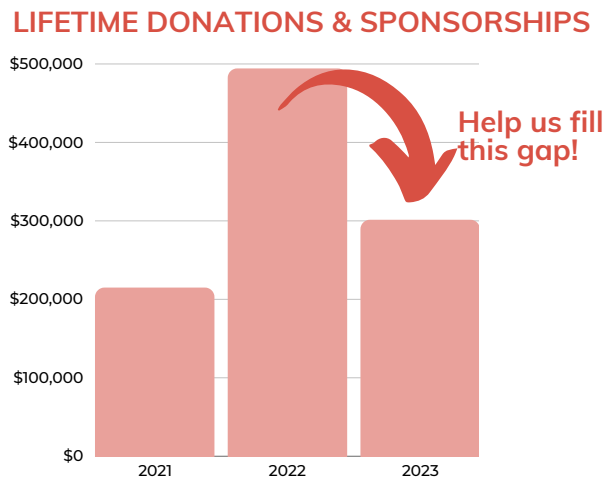
WE HAVE THE TECHNOLOGY TO TREAT MANY OF THESE DISEASES. TCAR IS DETERMINED TO CONNECT THIS TECHNOLOGY TO THESE CHILDREN LIKE ROSE WHO SO DESPERATELY NEED IT.

RAISED TO DATE

2023 SCIENTIFIC VOLUNTEER HOURS: 624

2023 EVENT VOLUNTEER HOURS: 176

2023 ALL IN-KIND DONATIONS: $627,460



Since the organization started in 2021, we have raised $964,071, and $587,000 as in-kind scientific donations to come as far as we have, with 80% going to our mission and 20% going to administrative costs to keep the organization running. (*See appendix A for breakdown.)

TCAR must raise $500,000 by December 31st to continue our work. We have raised 60% of the funds needed but have an urgent need to raise an additional $200,000 by the end of the calendar year. There is still time to step into this gap with a meaningful gift. Please make a donation TODAY!

THIS HAS ALL BEEN MADE POSSIBLE

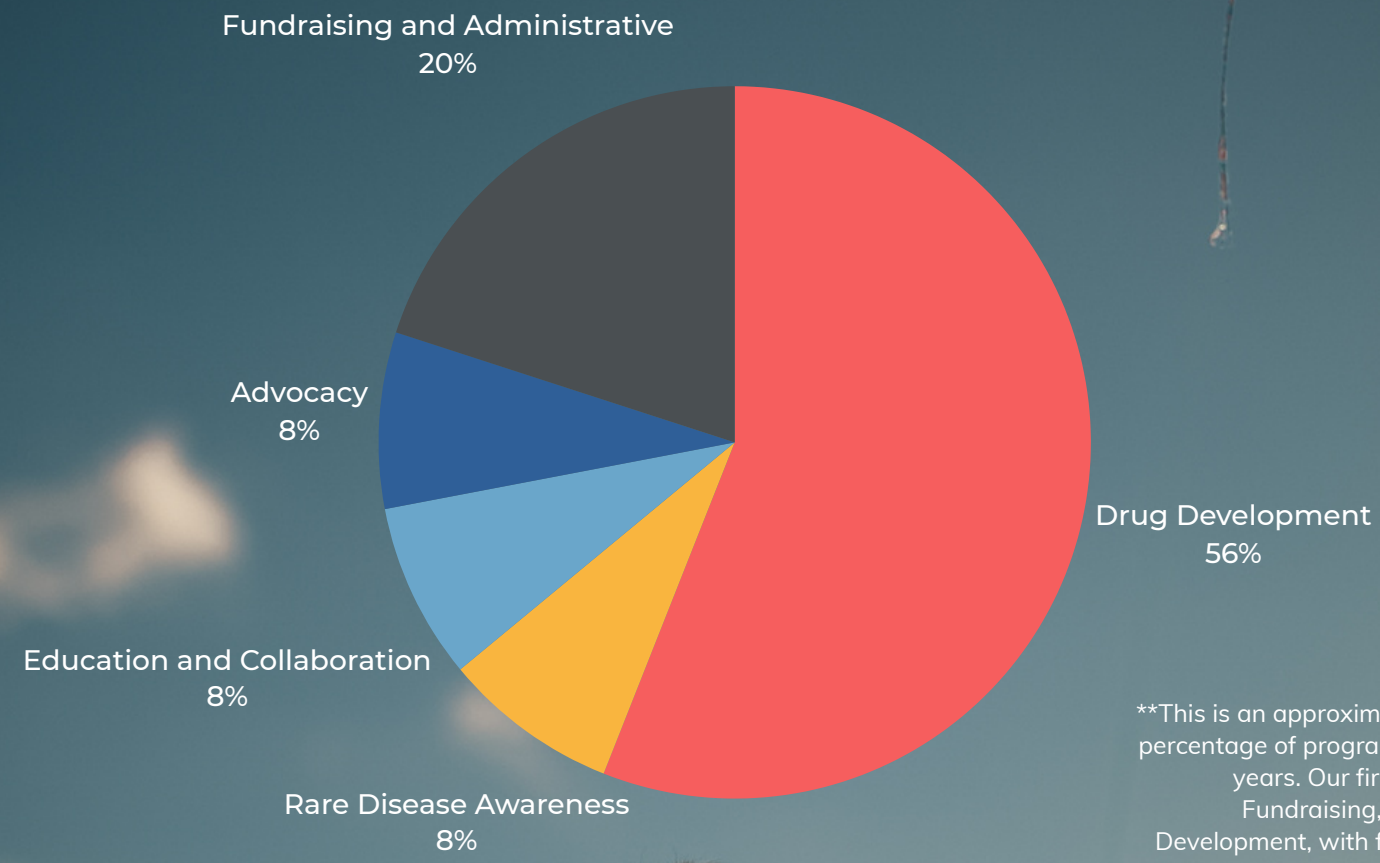
BY DONORS LIKE

YOU

If we can change behaviors in mice with the drug designs that you have funded, then we can take that evidence to the FDA and start the process for treating Rose and other children like her. If you're inspired to continue the next steps in this journey, please make your tax-deductible contribution here.

*APPENDIX A

We treat every dollar in the organization as an investment. Here is a breakdown in how we have spent your investment not just for a better future for Rose, but advancing a pathway for millions of other children.



**This is an approximate breakdown of percentage of programs over last three years. Our first two 990s show Fundraising, Admin, and Drug Development, with first two years not showing scientific in-kind donations. All awareness, education, and advocacy fell under Fundraising and Admin during 2021, and 2022.

We have raised **$964,071** and approximately **$587,000** in scientific services and consulting donated to us since our inception. Our events, conferences, and speaking engagements fall under awareness and advocacy, with some percentage going to fundraising and admin. We want to thank **Facet Life Sciences, Jackson Labs, Everlum Bio, La Jolla Labs, and JDI Marketing** for donating services, as well as all the **hundreds of scientists** that have collaborated, advised, and continue to help us build our network of hope.

HOW YOU CAN HELP

Our work is supported by people like you that want to create a brighter future for children that suffer from rare genetic diseases. Below are ways you can give these children a brighter future with cutting edge genetic technology.

TO DONATE

- CLICK ON LINK
- MAIL A CHECK
- DONATE STOCK
- SPONSOR AN EVENT
- VOLUNTEER

or go to www.tocurearose.org/donate

CLICK TO DONATE

To Cure A Rose Foundation is a 501(c)3 organization, with a mailing address of
1401 Lavaca St #890 Austin, Texas 78701

CASEYMCPHERSON@TOCUREAROSE.ORG | 512.940.4164 | TOCUREAROSE.ORG