the calliope joy foundation

CURES • COMMUNITY • CUPCAKES
FOR KIDS WITH LEUKODYSTROPHY

Annual Report

2018

Table of Contents

The 2018-2019 year saw the continuation of established programs as we prepared for the gala and after the major gift to CHOP totaling $110,000. We also hosted our largest Cupcake Challenge raising $30,000 and welcoming 1100 attendees. The Challenge, now in its seventh year, is now an established event here in the Main Line. Volunteers in the Boston area even organized a successful Cupcake Run on December 1st, 2018 in Lexington, Massachusetts.

We know our support and leadership has been crucial for the growth of the Center at CHOP and increasingly supports other programs across the globe through Cal's Care Packages, TJ's Travel Grants, our series of children's books, and the soon to be published Living with Leukodystrophy guide and soon-to-be launched Cure MLD website.

TJ's Travel Grants

This program is a key program the foundation administers and it is an important part of our outreach to support families seeking care at CHOP and other partner programs. To date, we have funded 60 grants totaling over $50,000 to families around the globe including Hong Kong, Egypt, Israel,Australia, Argentina, Ireland, and New Caledonia in the South Pacific.

The University of Minnesota and the Super Gav Fund

As you learned at the gala, newborn screening (NBS) for leukodystrophies is growing, primarily driven by work on behalf of ALD (or the Lorenzo's Oil disease). The inclusion of ALD on the Recommended Uniform Screening Panel (RUSP) means that 10 states now screen all newborns for the disease. This has been a game-changer for the community as you heard with the Flynn family's story at the gala. Because of ALD NBS, children can be monitored and treated and stay healthy. The growing number of newborn screening families represents an opportunity and a challenge for our community as we move from a reactive model of medical treatment to a proactive one. To prepare for this change, we have used some of our funds (raised in partnership with the Quimby family – a longtime friend and donor to Cal's Foundation) to help support ALD NBS families treated at the University of Minnesota's Masonic Children's Hospital. Dr. Paul Orchard and his team at the University of Minnesota are among the leading centers in the world when it comes to performing bone marrow transplants to treat ALD children. He is also the principal investigator of the bluebird bio gene therapy clinical trial. Minnesota has done a truly exceptional job in monitoring and supporting the first generation of ALD NBS families.

To support the University of Minnesota's transplant program, we produced two videos for the University of Minnesota about newborn screening and genetics counseling. The videos are used on the University of Minnesota's hospital website and our own

Leukodystrophy Family Forum website. The videos offer key information for newly diagnosed families. We also partnered with the Quimby family and through peer-to-peer fundraising on Crowdrise and we raised $8000 to support the Super Gav Fund at the University of Minnesota to help support research on the genetic variations in ALD that will lead to the childhood cerebral adrenoleukodystrophy or (CALD). Cal's Care Packages are also provided to patient families at Minnesota.

We have expanded Cal's Care Package Program. The typical Care Package costs $100 per family. While Maria sends some packages out herself when families contact the foundation, Cal's Care Package program is overseen by the centers themselves with t-shirts, blankets, books and gift cards provided to families when they visit our partner Leukodystrophy centers at Emory, Lurie, Minnesota, Cornell, and CHOP. In 2018, we launched a special holiday and Mother's Day gift program with our friends at Kendra Scott and CHOP, and we heard from the medical team that these little gifts for the caregivers were truly appreciated. That is changed dramatically since 2013. Of all the projects we have supported, the children's book Loie's Disease has been among the most impactful projects for the community. Dr. Waldman has been known to read the books with families when they visit. To date, we have published 500 copies of the book and hope to complete our French and Spanish translations by 2020. We are also nearing completion on a guide for families and pediatricians titled "Living with Leukodystrophy." This will be a hands-on guide to aid families caring for a loved one impacted by leukodystrophy.

The Leukodystrophy Family Forum (our online resource for families and clinicians) is constantly being updated and expanded. In 2018, we created new content on newborn screening and natural histories. And, we hear from our medical partners how the Leukodystrophy Family Forum is an important resource for clinicians. https://www.leukodystrophyforum.com/ald-newborn-screening

The Cupcake Tea in April 2018 honored Spark Therapeutics Jeff Marrazzo and provided a valuable networking opportunity to acknowledge our donors and supporters and start preparations for the Cupcake Gala in 2019. The biennial luncheon will happen again in 2020 and we will host our Board Meeting in Philadelphia at that time. The Cupcake Tea is a crucial part of strategy for cultivating and aligning industry partners. I have some ideas about individuals to be recognized at the Tea.

The Cupcake Run took place on December 1, 2018 in Lexington, Massachusetts, Maria (and Tim's) hometown. Her sister, Nicole Orcutt, organized the race. The inaugural event grossed $10,000 without any major industry sponsorships. Plans are underway for a 2019 race and we hope to enlist our Boston based partners (bluebird, Takeda, Orchard and Homology) to come out and support the event. We would like to enlist the support of the gene therapy patient families who have undergone treatment at Mass General/BCH to be the ambassadors of the event.

Global Leukodystrophy Initiative

Maria served on the steering committee for the Global Leukodystrophy Initiative meetings in Philadelphia. The May 2 – 3rd meetings hosted 175 attendees from advocacy groups, industry, and research/clinicians from around the globe. This was the largest and most well-attended meeting to date. It is truly remarkable to see the investment and activity in leukodystrophy research.

National Organization of Rare Disorders, American Society for Gene and Cell Therapy, and Global Genes

Maria has had the opportunity to serve on panels for the NIH, NORD, ASCCT and Global Genes. Given the demands of work and family, it is difficult and costly for Maria to travel, but she welcomes the opportunity to speak about gene therapy. On her sabbatical this year, Maria will complete her book Investing in Miracles for Beacon Press. The proceeds from book sales, which might be a few thousand dollars per annum, will go to support The Calliope Joy Foundation.

Partnerships

Sponsorships or grants of $1000 or more.

Amicus Therapeutics
Takeda
Orchard Therapeutics
Thermo Fisher Scientific
Absorption Industries
Carlino's Specialty Foods (in-kind)
Passage Bio
Spark Therapeutics
Bluebird bio
Homology
Traffic Planning and Design
Provco Group
Au Fournil Inc.
Goodman Properties
Dreifuss Fireplaces
EGM Construction
Giant Foods
Wawa
Weathergage Capital

Orchard Therapeutics, the FDA, and MLD Newborn Screening

We expect Orchard Therapeutics to go to the FDA and EMA in the coming year. We will welcome the opportunity to discuss the promise gene therapy at the FDA. Needless to say, the demands on the foundation's time and resources has grown with the start of Takeda enzyme replacement trial and the efforts of Orchard to gain FDA approval.

Also, the research into developing an effective MLD newborn screening has made tremendous progress, and, over the course of the next several months, we expect to see consented pilot studies for MLD newborn screening in Minnesota, New York, and Pennsylvania/NJ/DE. We are excited to support this effort since gene therapy is only effective in pre-symptomatic children. Needless to say, the speed of the progress in the field given the interest from industry has made it difficult for our mom-and- pop foundation to keep up with the work that needs to happen. Maria will be partnering with researchers, clinicians and advocates on a myriad of projects that will be crucial for the effective commercialization of MLD gene therapy. Cure MLD is at the center of these efforts.

What is Cure MLD? With unrestricted grants from Takeda and Orchard, we will be launching an interactive website with partner MLD groups called Cure MLD. The grants will be used to get high search engine scores and provide high quality content with information about clinical trials, treatment options and even a monthly online support group. The proposed budget is $6000 and we hope to produce a video discussing MLD with the doctors at CHOP (budget around $8000). Such information will help us communicate with the wider patient community and establish a contact patient registry that will be controlled by CURE MLD. There will be no fundraising as part of the CURE MLD project. We just want to be sure that families go to the web and seek out information about MLD, they find CURE MLD first so we can provide them with the best resources. The website will utilize translation algorithms in German, French, Spanish, and Arabic. We would like to bring on a person to assist with developing a contact registry and we have some ideas about hiring a retired patient advocate to take on this project.

The Cupcake Gala

We are pleased to report that our Cupcake Gala exceeded our goal of grossing $200,000 and actually raised $214,000. We had 325 guests and we saw increases in sponsorship and more effective and successful fundraising. We will be pleased to solicit LOIs from CHOP and our partner hospitals. It is clear that the event is evolving and becoming quite successful. Co-chair Kristin Roosevelt (from Thermo Fisher) believe we can work towards $500,000 in 2021.

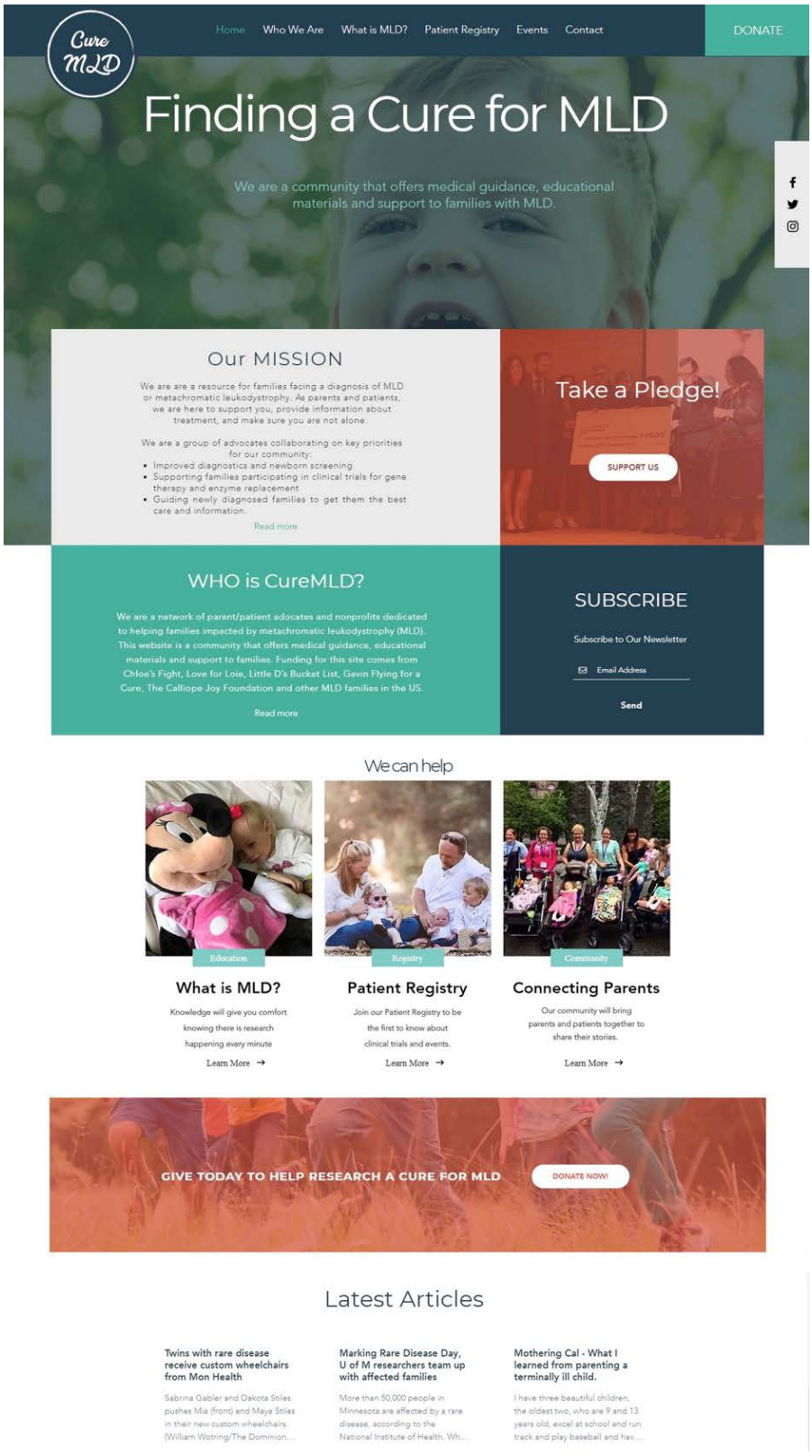
The board can review assorted options for the distribution of these funds to CHOP and other partner programs. Our planning committee, we are happy to report, seems willing to return for 2021 gala with a new goal of $500,000.

Conclusion and summary

We are pleased to present this report to the board, and hope that we can also discuss a more expanded role for the board as we enter a new era for the treatment and care of children with leukodystrophies, particularly MLD. We remain overwhelmed by the progress in the field over the last 5 years, and we are eager to get your guidance and leadership on how to remain relevant and keep up with the rapidly changing needs of the leukodystrophy space.

We estimate there is now hundreds of millions of dollars of industry money in the leukodystrophy research space for drug development space. This is an exciting, but challenging, time and we want to act in ways that optimize the work of the foundation with our limited resources.

-Pat Carr and Maria Kefalas


Cure MLD
Home
Who We Are
What is MLD?
Patient Registry
Events
Contact
DONATE
Finding a Cure for MLD
We are a community that offers medical guidance, educational materials and support to families with MLD.
Our MISSION
We are are a resource for families facing a diagnosis of MLD or metachromatic leukodystrophy. As parents and patients, we are here to support you, provide information about treatment, and make sure you are not alone.
We are a group of advocates collaborating on key priorities for our community:
Improved diagnostics and newborn screening
Supporting families participating in clinical trials for gene therapy and enzyme replacement
Guiding newly diagnosed families to get them the best care and information.
Read more
Take a Pledge!
SUPPORT US
WHO is CureMLD?
We are a network of parent/patient adocates and nonprofits dedicated to helping families impacted by metachromatic leukodystrophy (MLD). This website is a community that offers medical guidance, educational materials and support to families. Funding for this site comes from Chloe's Fight, Love for Loie, Little D's Bucket List, Gavin Flying for a Cure, The Calliope Joy Foundation and other MLD families in the US.
Read more
SUBSCRIBE
Subscribe to Our Newsletter
Email Address
Send
We can help
Education
Registry
Community
What is MLD?
Knowledge will give you comfort knowing there is research happening every minute
Learn More →
Patient Registry
Join our Patient Registry to be the first to know about clinical trials and events.
Learn More →
Connecting Parents
Our community will bring parents and patients together to share their stories.
Learn More →
GIVE TODAY TO HELP RESEARCH A CURE FOR MLD
DONATE NOW!
Latest Articles
Twins with rare disease receive custom wheelchairs from Mon Health
Sabrina Gabler and Dakota Stiles pushes Mia (front) and Maya Stiles in their new custom wheelchairs. (William Wotring/The Dominion...
Marking Rare Disease Day, U of M researchers team up with affected families
More than 50,000 people in Minnesota are affected by a rare disease, according to the National Institute of Health. Wh...
Mothering Cal - What I learned from parenting a terminally ill child.
I have three beautiful children, the oldest two, who are 9 and 13 years old, excel at school and run track and play baseball and hav...


NE TOO RARE
CHOP helps exceptional kids
fight exceptionally rare diseases.
for tomorrow's
BREAKTHROUGHS
The Campaign for
Children's Hospital
of Philadelphia

LIVING WITH
LEUKODYSTROPHY

A Guide for Caring for Leukodystrophy Patients