YAYA FOUNDATION

YAYA FOUNDATION FOR 4H LEUKODYSROPHY

2022 Year in Review

Dear Families, Friends, and Partners,

Looking back on 2022, I am so proud of all the Yaya Foundation has accomplished. We've enabled critical breakthroughs in 4H Leukodystrophy (4HL) research through our 4HL Collaboration Network initiative, collected data that will help us prepare for future clinical trials, and continued to grow our research and clinical network.

One of the greatest parts of the year was being able to come together as a community, both virtually and in person (!). We hosted the largest convening in the history of our diagnosis focused on improving clinical care for people affected by 4HL and got together in Illinois for a session dedicated to research updates. For many of us, it was the first time meeting each other; for others, it was a time to see the people they trust most on this 4HL journey.

All of this fills us with **HOPE** for what lies ahead. I am inspired by our community and know that, working together, we can change the world and improve outcomes for people affected by 4HL.

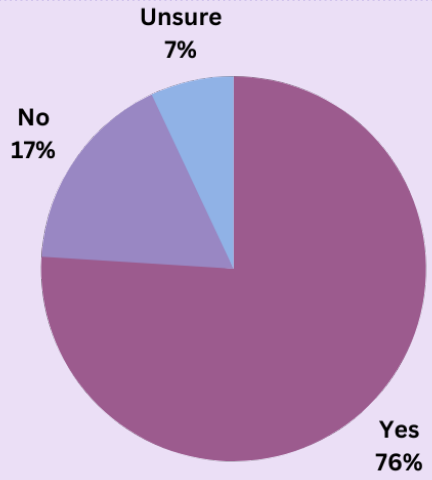
Thank you for your continued support this year and for being a part of our community.

Ron Garber

Yaya Foundation Co-Founder, Board President and Yaya's Father

YAYA FOUNDATION

MEET JOSH



Has the person had issues with their eyes and/or vision?

DATA COLLECTION PROGRAM

This past year, Yaya Foundation worked closely with RARE-X, our data collection program platform, and our community to enroll 4HL families worldwide. Today, we have more than 70 families enrolled in the data collection program, which has allowed us to learn valuable information about trends, commonalties and differences among 4HL patients.

RARE-X provided the first summary of findings, which gives the 4HL community a glimpse into where 4HL impacted individuals are located, shared symptoms, demographic information (race, ethnicity, gender at birth) and much more. We are hopeful to continue to add 4HL individuals to the data collection program to better understand 4HL and next steps in research.

4HL CONFERENCES

VIRTUAL CARE AND DISEASE MANAGEMENT DAY

In April, Yaya Foundation hosted its first virtual conference. ***40 families and 11 world-renowned clinicians participated,*** providing important care and educational resources to families and updates on research findings. This meeting represented the largest gathering of 4HL families in the history of the diagnosis, with families joining from Canada, Italy, Mexico, Romania, Russia, United Kingdom, Netherlands, and the United States.

UNITED LEUKODYSTROPHY FOUNDATION (ULF) CONFERENCE

In June, we led a 4HL-specific track within the larger ULF conference. This hybrid conference included presentations from leading 4HL researchers and clinicians from around the world and was one of the first times we were able to see families and our clinicians in person.

VIRTUAL CARE DISEASE MANAGEMENT CONFERENCE

Fighting for a cure. Bringing HOPE to our community.

MEET ALEXIS - UNITED STATES

Nothing could have prepared us for learning that our little three year old daughter had a deteriorating brain disease, not even a medical career. She had met all her milestones and was talking, walking and running, but had random falls that were unexplained. We went in wanting answers and walked out absolutely horrified and frightened for our baby girl's life, knowing there was no treatment or cure. One thing we knew was that we wanted to make sure we gave her the absolute best life possible and provide her with resources so that she could be the best version of herself, no matter what condition her body was faced with. Alexis loves to dance and move so we joined a dance studio! She loves to ride her tricycle and recently learned to ride a bicycle with training wheels.

"Her determination amazes us daily and we anticipate many more beautiful memories to come!" *Eileen, Alexis' Mom*

MEET MYLES - UNITED KINGDOM

Myles was diagnosed with 2x mutations in his POLR3B gene of December last year, this summer I took him to Amsterdam to meet the amazing Dr. Nicole Wolf. Following this appointment, I learned unfortunately Myles has a very severe form of 4HL. He is now four years old and is wheelchair bound, however his smiles and laughter are like no other. I will continue to fight for a cure. Myles loves all animals I bought him a hamster recently and he continues to be mesmerized!

"His smiles and laughter are like no other. I will continue to fight for a cure."- *Georgia, Myles' Mom*

MEET ERINN - UNITED STATES

Although I've had (seemingly unrelated yet progressive) symptoms for decades and have been searching for answers for a decade, I first learned about 4H Leukodystrophy in October 2021 and my official diagnosis came April 2022. This last year has been a challenge for me in finding the right balance between recognizing my physical limitations, wanting complete independence, and accepting support from my loved ones (although I don't always like to admit that I need it). I still LOVE traveling, hanging out with family and friends, and trying new things. At the same time, I'm still learning how to adapt to the changes and new ways to stay strong both physically and mentally.

"I want to give a special shout out and thank you to my family, the Yaya Foundation, and my 4H community of families, doctors, researchers, donors, and other advocates for all the great work you do and the continued support you provide." *- Erinn*

MEET TIA - NETHERLANDS

Our week is full of therapist and doctors appointments. But after getting knowing diagnosis for our Tia we decided to make loving memories as a family. Tia has every Wednesday hippotherapy and every Friday hypotherapy. Between those days she has physical therapy and occupational therapy and speech therapy . Our weekend is reserved for family so we travel somewhere and have fun.

"Tia likes animals, especially horses, dogs and cats. She also like to dance a lot!" - *Kristina, Tia's Mom*

We are taking on the work needed to unlock a cure with the same zeal Yaya had for life." - Ron Garber

GENE THERAPY PROGRAM

This year, we completed the first half (Phase 1) of our first ever 4HL Collaboration Network prioritized research initiative, focused on disease model and gene therapy development. The project, which teamed up one of the world's foremost 4HL experts with the President of the American Society of Gene and Cell Therapy, met its objectives and we are looking forward to publication of findings in the coming months.

2022 HIGHLIGHTS

6 Number of bi-monthly #4HLFamilies virtual calls held. These calls provided opportunities for individuals and families to share their experiences and offered educational resources. Sessions included mental health and caregiver wellness with child psychologist Dr. Chrissy Salley, and updates from 4HL researchers including Dr. Genvieve Bernard.

3 Number of Global Scientific Seminars hosted, featuring speakers from Harvard Medical School and the Broad Institute of MIT and Harvard.

8 Number of incredible families who spread awareness and raised funds for Yaya Foundation and its work in research and family support.

17 Number of new clinicians, genetic counselors and researchers we connected with this year through presentations to the Broad Institute and CZI Rare As One Network events.

Help us to continue to deliver HOPE

We are excited about all of our accomplishments, but we know there is so much more we want to do.

Your support makes our work possible and helps us to continue the fight for people just like Josh, Alexis, Myles, Erinn, Tia, and Dude. Join us and our community in fighting 4HL today by donating.

DONATE

TEXT HOPE4HL to 53-55
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