

NF NEWS



THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
WINTER 2022

INSIDE:

- 3** NF Summit
- 4** Deep Science Ventures
- 5** 2022 DDI Awardees
- 6** Fall Fundraising Events

More than 400 people came together in person and virtually this July for the first annual NF SUMMIT in Chicago, Illinois. Those who gathered defined themselves in many ways: individuals living with NF, advocates caring for NF patients, NF clinicians and researchers, and representatives from NF organizations, academic institutions, pharma, and industry — a community committed to ending NF!

Continued on page 3



FROM the President

Annette Bakker, PhD



In the words of Paul Raci, “**Believe. Believe. Believe.**” If you aren’t familiar with his work, Paul Raci is an Academy Award Nominee for the Oscar Nominated Film, *The Sound of Metal*. The film is about a young musician who becomes deaf and struggles to accept his new life until he believes he can live his life to the fullest, even while deaf.

Paul spoke these exact words to us at the recent NF SUMMIT, the largest gathering of the NF community worldwide and in CTF’s history, which took place this past July in Chicago. Bringing together over 400 patients, family members, researchers, clinicians, industry partners, and other NF partner advocacy and research organizations, the NF SUMMIT was all about belief, education, empowerment, and action.

Paul’s words resonated with all of us.

While only one drug is now approved for NF1, because of the progress made and promising developments to come, at CTF we believe we will find more new treatments. We believe in the power of patients as partners in our research, and we believe in the unstoppable nature of the NF Community.

We have come a long way since the first days of the Children’s Tumor Foundation and have leaped forward with both promise and progress globally — but I know we have much more to do to END NF. I believe that we will get there faster than ever.

Like the lead character in *The Sound of Metal*, the struggle that each of our patients and family members endures is all too real, but YOU are the reason we **BELIEVE** in the future of research and in our researchers. YOU are the reason we **BELIEVE** in the power of educating and empowering our patients. And YOU are the reason we **BELIEVE in ENDING NF.**

I hope you will join Paul and me. **BELIEVE.**

Sincerely,

Annette Bakker, PhD
President

CTF President Annette Bakker Receives High Honor from Prime Minister of Belgium

On September 22, as world leaders and diplomats gathered in New York City for the General Assembly of the United Nations, a special ceremony took place at the residence of the Belgian Consul General, honoring those who have distinguished themselves in their respective economic, scientific, or cultural fields.

Amongst those honorees was our very own CTF President Annette Bakker, who received the title Officer of the Order of Leopold. The decoration was presented to Dr. Bakker by the Prime Minister of Belgium, Alexander De Croo on behalf of the Kingdom of Belgium, for her leadership in the field of science, particularly for groundbreaking work done in the field of NF.

Congratulations to Annette for this well-deserved honor and for a life dedicated to helping patients and advancing cures. Special thanks to the Kingdom of Belgium, the Prime Minister of Belgium, and the Belgian Consulate General in New York. To read more, including links to European press coverage, go to ctf.org/news.



Board of Directors

Officers

Tracy Galloway
Chair

Gabriel Groisman
Vice Chair

Randall Stanicky
Treasurer

RB Harrison
Secretary

Annette Bakker, PhD
President

Richard Horvitz
Chair Emeritus

Board Members

Daniel Altman
Robert Brainin
Daniel Gilbert
Sally Gottesman
Carol Harrison Kalagher
Frank Haughton
Simone Manso
Emily Parker
Laura Perfetti
Michael Peterson
Liz Rodbell
Kenneth Rudd
Richard Soll
Stuart Suna
George Thuronyl

Advisory Board Chairs

Bruce R. Korf, MD, PhD
Chair, Medical
Advisory Committee

Scott Plotkin, MD, PhD
Chair, Clinical Care
Advisory Board

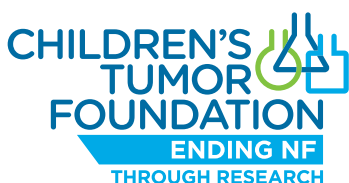
D. Wade Clapp, MD
Chair, Research
Advisory Board

Volunteer Leadership Council Chair

Anita Gribben

Honorary Directors

Suzanne Earle
John Golfinos, MD
Linda Halliday Martin
Steven L. McKenzie
Carolyn E. Setlow
Nate Walker
Ed Stern,
Pro Bono Counsel
Allan Rubenstein, MD
Director of Medical
Affairs Emeritus



NF SUMMIT Attracts NF Patients, Clinicians, Families, and Pharma

Continued from cover



This summer's inaugural NF SUMMIT was an educational and networking conference that evolved from CTF's annual NF Forum and Volunteer Leadership Conferences, which have taken place for over a decade. Those annual meetings built the necessary foundation for a new, dynamic, inclusive event intended for anyone with a connection to NF. In the spirit of new, there were 212 newcomers – more than 50% of those in attendance were first-time attendees. Tandy Wolters and Michael Peper were among this group, present as parents of NF Hero Leo and volunteers dedicated to learning and building their leadership skills.

“Michael and I had the most amazing time. It was just incredible, all parts of it, especially meeting other families. We are hopeful. We loved it.”

TANDY WOLTERS
MOM OF NF HERO, LEO

The NF SUMMIT also brought together over 50 speakers covering a wide range of content from chronic pain to using social media to raise awareness and build community. Of those speakers, 36 had never before attended or spoken to the NF community at a CTF event. One of those exciting newcomers was Dr. Charles Argoff, Vice President of Scientific Affairs at the American Academy of Pain Medicine (AAPM), who said, “On behalf of the

AAPM, I want to thank you for inviting me to participate in your annual meeting. The energy at the meeting, as well as during the panel discussion, was incredible.”

Additionally, NF organizations from around the country came to Chicago for the NF SUMMIT, including NF Midwest, NF Network, NF Northeast, NF Texas, Littlest Tumor Foundation, NF Team, NTAP, University of Alabama NF Clinical Trials Consortium, and members of the NF Collective. Another important NF organization, B the Difference, generously donated financial scholarships through their ‘B Kind’ program so several NF patients could attend in person.

Representatives from Lurie Children’s Hospital, Stanford Medical Center, and Massachusetts General Hospital were present and recruiting for clinical trials relevant to the NF community. Connecting patients to researchers is something CTF is passionate about, and we are excited to grow these opportunities at future events.

The NF SUMMIT was generously supported by Alexion AstraZeneca Rare Disease, as well as SpringWorks Therapeutics, NFlection Therapeutics, Recursion, and Healx.

NF SUMMIT 2023 will take place June 21-24, 2023 in conjunction with the NF Conference in Scottsdale, Arizona. To learn more, go to nfsummit.org



Make NF Visible Recognition Awards 2022

Make NF Visible Community Recognition Awards acknowledge individuals or groups, at every level of involvement, who Make NF Visible in the local community, nationally, or globally. These awards recognize a diverse group of committed people who work tirelessly throughout the year to help CTF fulfill its mission. Award presentations took place at a special reception on July 22, 2022, at the NF Summit in Chicago, Illinois.



Volunteers of the Year, Diane Owens and Lara Mukabonov

2022 Make NF Visible Recognition Awardees

Volunteer(s) of the Year:

Diane Owens and Lara Mukabonov

Community Advocate Award:

Naomi Cuka

Clinician Award:

Tena Rosser, MD

Young Leader Award:

Maddie Sassa

Patient Advocate Award:

Dale Berg

Researcher Award:

Kevin Bruemmer, PhD
Young Investigator Awardee

Corporate Champion Award:

Bourbon Charity

Global Reach Award:

Magda Chlebus

Fight NF Your Way Award:

Mark Ehrli

Honorable Mention Award:

McKinnon Galloway

Deep Science Ventures and Cancer Research Horizons Amp Up Efforts to Create Oncology Ventures

CTF partnership provides resources, expertise, and connections with the NF community

Deep Science Ventures (DSV), a London-based venture creator, announced it has expanded its alliance with Cancer Research Horizons, Cancer Research UK's innovation organization, to further power the creation of cutting-edge ventures in oncology. The new ventures, which the two organizations will be able to invest in through pre-seed capital, will be incubated within Cancer Research Horizon's Therapeutic Innovation laboratory network. The ventures will build on the success of a previous partnership which led to the formation of three new cancer therapeutics companies.

The Children's Tumor Foundation has joined the alliance as a strategic partner, which will aid efforts in identifying possible venture-creation opportunities in neurofibromatosis.

While there have been considerable improvements in therapies available to many cancer patients over the last 20 years — particularly with the advent of immunotherapy approaches — significant subsets of patients remain unresponsive or ineligible for existing treatments. Through the alliance, DSV and Cancer Research Horizons will aim to identify and tackle some of those critical remaining barriers across up to 10 fundamental areas in oncology.

The first venture will focus on novel technologies for deep reprofiling of the immune system within specific immunotherapy-resistant contexts. The following business will seek new approaches in all forms of neurofibromatosis. This latter effort will be supported by the Children's Tumor Foundation, which provides resources, deep expertise, and close network connections with the NF community.

Each venture will be led by a DSV Founding Analyst and overseen by a joint steering committee, who will work together to design a customized solution that leverages promising technologies in development throughout the scientific ecosystem. It will spin out to develop the technology and potential medicines if successful.

DSV and Cancer Research Horizons are exploring additional commercial and non-profit strategic partners whose interests align with other challenge areas. The alliance will leverage DSV's proven ideation-based innovation and venture creation approach. In its first alliance with Cancer Research UK, DSV established the creation of Enedra Therapeutics, Stratosvir, and Neobe Therapeutics, which are among 35 companies DSV has established.

Cancer Research Horizons brings deep oncology expertise and insights: the new alliance will see it add wet lab incubation space and scientists to support the proof-of-concept work for the new ventures.

To learn more, please go to ctf.org/news

“ We look forward to the creation of additional high-impact ventures, which will benefit from hands-on resources from the laboratory team at Cancer Research Horizons Therapeutic Innovation, as well as invaluable support from the Children's Tumor Foundation. ”

DR. LAURA FLETCHER
HEAD OF BUSINESS DEVELOPMENT AND STRATEGIC PARTNERSHIPS AT DEEP SCIENCE VENTURES



SAVE THE DATE

Join us in Los Angeles
NF2 & Schwannomatosis State of the Art 2023

UCLA Luskin Conference Center
JANUARY 12-15, 2023

For more information as it becomes available, visit ctf.org/news, or reach out to mgiovannini@mednet.ucla.edu or pnghiemphu@mednet.ucla.edu

2022 Drug Discovery Initiative (DDI) Awardees

Four investigators were awarded a Children's Tumor Foundation Drug Discovery Initiative (DDI) Award for the 2022 grant cycle, a significant investment toward potential NF drug treatments.

Sylwia Ammoun,
University of
Plymouth

***In vivo* Testing
of AXL inhibitor
BGB324 and
MERTK inhibitors
UNC2025 and
MRX2843 in
the Postn-Cre;**

***NF2*flox/flox Mouse Model of Schwannoma**

The protein receptors AXL and MERTK have been newly identified as potential therapeutic targets in NF2-related tumors. Inhibitors of these proteins successfully reduced the growth and survival of patient-derived schwannoma and meningioma tumor cells *in vitro*. The goal of the present study is to examine the efficacy of these inhibitors in mouse models of NF2 schwannomas. The data generated would enable the testing of these inhibitors in human clinical trials.



Thomas DeRaedt,
The Children's
Hospital of
Philadelphia

***Combined
Inhibition of MEK
and BMI-1 for the
Treatment of NF1-
associated High-
Grade Glioma***

NF1-associated high-grade gliomas (HGG) are rare but aggressive brain tumors with no effective therapy. A combination of MEK and BMI1 inhibitors were recently identified to potently kill NF1-associated high-grade glioma cells. This study will evaluate if the combination of these drugs also extends the survival of NF1 mice with high-grade glioma. The study will test the MEK inhibitor Mirdametinin and the BMI1 inhibitor PTC596 in combination.



Lawrence Sherman,
Oregon Health
and Science
University

***A Screen for Novel
Schwannomatosis
Pain Therapies***

Schwannomatosis patients suffer from chronic, untreatable pain and the degree or type of pain differs depending on mutation type (*SMARCB1* or *LZTR1*). Schwannomatosis tumor cells release proteins that influence the number of nerve cells that respond to pain signals. This study will use *SMARCB1*- and *LZTR1*-variant Schwann cells to test the ability of drugs targeting secretory proteins or pain-signaling proteins to relieve pain and any difference in their effects depending on the variant type.



Efthimios Skoulakis,
Alexander
Fleming
Biomedical
Sciences
Research Center,
Greece

***Allele-specific
Behavioral
Pharmacogenetics
of Novel NF1 Variants***

NF1 patients present a variety of behavioral symptoms, including compromised learning, attention deficits, and activity and sleep disturbances, and the variability of these symptoms reflects the nature of the pathogenic variant. *Drosophila* modeling these human variants also present similar behavioral symptoms. This study will use variant *Drosophila* strains to test potential drugs against these deficits to develop personalized therapies for NF1 patients.



Newly Named Jeffrey Hanson Optic Pathway Glioma Study Renewed for Five Years

In 2015 CTF funded a visionary study on the natural history of optic pathway gliomas (OPG) led by Michael Fisher, MD, and Robert Avery, DO, from Children's Hospital of Philadelphia. During the 2015-2021 period, the study opened 25 sites, and over 60% of the 250 patients were already enrolled. CTF recently signed a renewal for \$1.3 million for an additional five years. The new study will complete enrollment and allow for a more comprehensive collection of OPGs, additional MRI scans, and additional biology samples.

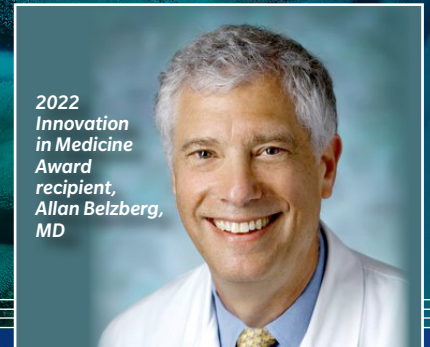
So far, 180 children with OPG have been followed for about five years, and significant findings are emerging from the study. The proposal estimates that the planned accrual (250 patients) will be complete in about two years, thanks to the addition of several large European centers with NF1-OPG expertise that have expressed interest in joining the study.

CTF and the investigators consulted with the Hanson family and decided to rename the study after Jeffrey Hanson, a former CTF ambassador. Jeffrey was an NF1 patient affected by an optic glioma who passed away in 2020. We hope the study will help patients like Jeffrey receive better care and, ultimately, a cure for NF.



Fall Fundraising Events

2022 GALA CHILDREN'S TUMOR FOUNDATION



2022 NATIONAL GALA

The Children's Tumor Foundation 2022 National Gala took place on Monday, November 14, to honor the NF Community and our collective efforts to fund vital NF research. This year's beautiful event transpired at the exquisite locale, Gotham Hall, in New York City. The evening's program was also livestreamed globally for everyone to enjoy during the event, and may be viewed on the Foundation's YouTube channel. Co-hosts for the evening were TV host/NBC correspondent Raina Seitel and actor/producer Jonathan Sadowski. Guests enjoyed an elegant evening of cocktails, fine dining, inspiration, and laughter. An impassioned speech was given by Annette Bakker, who this year is celebrating her tenth year as CTF President.

The 2022 Humanitarian Award was presented to Rachel Tiven, an attorney and activist with a long and dedicated history with the Children's Tumor Foundation. Rachel spent the past 15 years building and leading LGBT and immigrant rights organizations, including Lambda Legal, Immigration Equality, and Immigrant Justice Corps. A longtime donor to the Foundation and to the Neurofibromatosis Therapeutic Acceleration Program (NTAP) at Johns Hopkins, Rachel has significantly funded critical NF research and been instrumental in the successful leadership of the Foundation.

The 2022 Innovation in Medicine Award was given to Allan Belzberg, MD, George Heuer Professor of Neurosurgery at Johns Hopkins. A longtime CTF researcher and collaborator, Dr. Belzberg is also a neurosurgeon who treats tumors of the peripheral nervous system, including schwannoma, neurofibroma, and nerve sheath tumors.

Attorney Aubrey Rothrock and the law firm Squire Patton Boggs, of which he is a senior partner, were presented with the 2022 CTF Champion Award. Aubrey and his team successfully advocate for NF research through the Congressionally Directed Medical Research Program in the U.S., and have expanded the NF fight globally with the initiatives of CTF Europe.

Florida native Michele Holbrook was named the 2023 National Ambassador. Michele was diagnosed with schwannomatosis at age 25; she has endured eight surgeries, plus radiation, and lives with excruciating pain every day. Michele has worked with the Children's Tumor Foundation for many years as a volunteer and fundraiser, gaining numerous media appearances for her work with the NF Endurance team. She currently serves as a patient representative with the CTF Clinical Care Advisory Board.



Our utmost thanks to an esteemed team of Gala Co-Chairs who made the evening inspirational: Erica Hartman-Horvitz, Tila Falic Levi, Liz Rodbell, and Clara Wilpon. This newsletter was sent to the press before the evening transpired. For photos, videos, additional details, and fundraising totals go to ctf.org/news.

BENEFIT X

On Saturday, November 19, over 1,800 supporters gathered at Huntington Place in Downtown Detroit for beNeFit X, NF Forward's annual fundraiser hosted by Dan and Jennifer Gilbert. For the first time since 2019, the event welcomed its supporters back to a fully in-person gathering. Attendees dressed in their disco best in a nod to the gala's 70's theme and a decade devoted to curing NF.



Dancing With Our Stars



benefit X

A Decade Devoted to Ending NF

Over the past ten years, the beNeFit has raised more than \$50 million to fund audacious research in the areas of gene therapy, vision restoration, and more, giving hope for a future without NF. In honor of the 10th anniversary of this landmark event, past Strength and Honor Awardees were welcomed back to Detroit and gave updates since sharing their NF story as an honoree.



Kylie Earle

This year's Strength and Honor Award was presented to Kylie Earle, who educated millions on TikTok about her prosthetic eye, what NF is, and how it has impacted her vision and her life. She cast aside negative comments and encouraged others with NF to be themselves. Kylie has shown bravery as an extraordinary advocate for the NF community. We

congratulate Kylie and thank the Gilberts for their support in propelling NF Forward and CTF into another decade of research and hope.

DANCING WITH OUR STARS

The annual Dancing With Our Stars events raised more than \$500,000 for the Children's Tumor Foundation this fall. Hosted in two locations, the events took place in Little Rock, Arkansas, and in Northwest Arkansas. Twelve dancers took the stage in pairs that included one local celebrity and a dance instructor in Little Rock and Northwest Arkansas to compete for the Best Performance Trophy and the Championship Trophy, awarded to the top fundraiser.

Special thanks to Lesley Oslica and her team in Little Rock, as well as Mimi Stewart, Malorie Marrs, and Courtney Norton in Northwest Arkansas. These incredible, yearly events are bringing us closer to our goal of ending NF.



Jonathan Sadowski and Roland Thoms at the 2022 Halloween Bash

HALLOWEEN BASH

The 17th annual Halloween Bash was held on Saturday, October 29, in Walnut Creek, California, and was emceed by actor/producer Jonathan Sadowski. This year's fun-filled event raised more than \$150,000.

Thank you to Roland and Nicole Thoms of Varsity Painting, their family, and the attendees and supporters of the annual Halloween Bash. The Varsity team of friends, employees, and customers have worked together over the years to raise more than \$700,000 for research into treatments for those living with NF.

CELEBRITY POKER TOURNAMENT

On October 6, CTF hosted an online Celebrity Charity Poker Tournament in which 100 players raised more than \$80,000 for NF research. The event featured celebrities including Jason Alexander, Elizabeth Berkley, David Costabile, and more. The organizing committee was led by Board Member Dan Altman and actor and NF Uncle Michael B. Silver.

The Children's Tumor Foundation extends our utmost gratitude to the organizers, attendees, sponsors, and donors who participated in each of these festive events. Your support is vital to the success of our mission to find treatments for all those living with NF, and we thank you.

Nick, Angela, and Bill are Making NF Visible

We BELIEVE in ending NF.

We believe for the 2.5 million people around the world living with NF, life-altering genetic disorders that cause tumors to grow on nerves anywhere in the body.

Every day, 120 babies are born with some type of NF, which includes neurofibromatosis type 1 (NF1), NF2-related schwannomatosis (NF2), or one of the rare types of schwannomatosis.

We **BELIEVE** in a hopeful future for every NF patient. We have seen lives changed thanks to discoveries and advancements that YOU, supporters and friends of the Children's Tumor Foundation, have so generously funded.

Your donation today at www.ctf.org/believe will help speed NF research toward better treatments, more options, and improved lives for every person living with NF.

People like 14-year-old Nick (pictured here), who lives with NF2.



NF2 Hero Nick just after surgery

When Nick was three, his parents, Peter and Angie, noticed their toddler walking a little funny. Finally, after six months of appointments and tests, a scan of Nick's lower back and hip revealed a tumor growing on his left sciatic nerve. That was the first time Nick's parents had ever heard of NF.

In the following years, Nick had major surgery on his foot, a harrowing experience that left him incapacitated for an entire summer. Later he had another surgery to remove a tumor on his upper spine. Shortly after, yet another surgery to remove a brain tumor, which was cancerous and required six weeks of radiation therapy.



Nick Lindeback, who lives with NF2, and his father Peter.

Around the age of twelve, Nick's hearing started to deteriorate due to a vestibular schwannoma tumor on his right hearing nerve. In January, Nick had a grueling 10-and-a-half-hour procedure to take the tumor out. The family was shocked when he woke up after surgery and couldn't hear or understand his parents because the vestibular schwannoma on his left side had grown substantially.

"I'm practically fully deaf now," said Nick.

Children and adults around the world who live with NF deal with manifestations like deafness, disfigurement, blindness, bone abnormalities, learning disabilities, disabling pain, or cancer. **It is a lifelong disorder**, and the Children's Tumor Foundation is committed to people living with NF throughout their lives. We believe there will one day be more treatment options for people like **35-year-old Angela (pictured on the following page), who lives with NF1.**

Angela was diagnosed with NF1 at birth. She had her first surgery at just eight days old and lost vision in her right eye at around the age of two. As a young child, Angela underwent reconstructive surgeries on the right side of her skull to correct a bone abnormality in her face. At age ten, Angela had surgery to remove her right eye.

Now, at the age of 35, Angela has had 23 surgeries during her life.

“I have a facial disfigurement due to neurofibromatosis. My right eye, which is a prosthetic, protrudes out, and the right side of my face is slightly lower than that of my left,” writes Angela in her blog, *Life with Neurofibromatosis*.

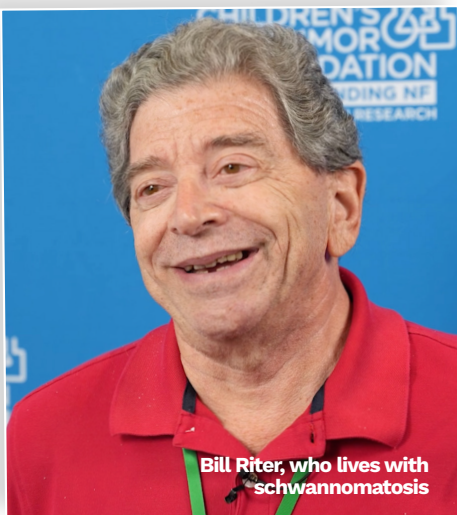
Angela is a fighter despite a life of seizures, surgeries, dyslexia, and disfigurement. She has a thriving career as an advocate for individuals with disabilities of all kinds, and is an accomplished writer and blogger.

In January of 2022, the same month Nick woke up from surgery having lost his hearing, Angela had the most difficult surgery of her life. She is recovering, but still has quite a road ahead.

We BELIEVE in a future where patients like Angela and Nick have more options than yet another surgery. We believe in treatments, preventive therapy, and clinical care.

With your help, CTF is driving the research and discovery that will give NF patients more options:

- The first FDA-approved treatment for NF1
- The INTUITT-NF2 platform clinical trial
- The first schwannomatosis clinical trial



And more. So much more! But even with so many recent discoveries, **patients are often told no treatment is available.** They can only “watch and wait” to see if their tumors will grow, and only then can they determine how those tumors might impact their lives.

Schwannomatosis patients like Bill Riter

(pictured here) often live in terrible pain and wait for decades before receiving a diagnosis.

“At 23, I had my first tumor removed from my hip. It was not done at an NF center, and they simply said it was a benign tumor, which we thought was good news. I continued to have pain for the next 25 years,” said Bill.



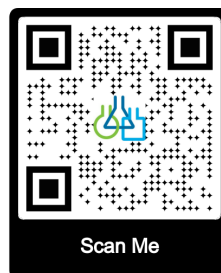
Finally at the age of 48, an NF specialist confirmed Bill’s diagnosis of schwannomatosis. Later that year, Bill was forced to retire due to schwannomatosis pain.

“I’m now 67 and have lived with this pain for most of my life. We want the younger people with this condition to have a better quality of life, whether through gene therapy or one of the other potential treatment options CTF is funding,” said Bill.

We agree with Bill. Important things are happening in NF research. From CTF-funded gene therapy to our vital optic glioma study to worldwide partnerships to repurpose lost drugs for NF patients, **we believe that there are treatment options to be found for all patients with NF.**

And we need your help.

Please return the enclosed envelope, scan the QR code below, or donate at www.ctf.org/believe to help develop further treatments that will change the lives of the millions of people worldwide living with NF.



Extraordinary Spirit: **LILLY SIMON**

Her TikTok video about living with NF garnered over 2 million views after a stranger posted a video of her riding the train.

33-year-old Lilly Simon does not have monkeypox. She does have neurofibromatosis type 1, a genetic condition that causes tumors to grow on nerves. During her commute to work last July, a stranger surreptitiously videoed Lilly while riding the subway wearing summer shorts and a T-shirt. Lilly had no idea she was being recorded.

That stranger then posted the video on TikTok, adding a monkey emoji and a question mark. TikTok users understood the implication - that Lilly might have a case of monkeypox, the virus that was declared a global health emergency by the World Health Organization.

After discovering the video, Lilly was initially devastated. However, with an NF Fighter's spirit, Lilly decided to create and post a video response. She used the moment not only to stand up for herself but all those living with NF.

"The tumors are benign, but they are still all over my skin and give me a lot of health complications, both physical and mental," Lilly said in her video. She went on to say, "I will not let any of y'all reverse any years of therapy and healing that I had to endure to deal with the condition and, of course, to exist around people like you."

Lilly's video went viral with over two million views, and soon Lilly's response was highlighted in the *New York Times*, on *Last Week Tonight With John Oliver*, and many other media outlets. Prior to this incident, NF wasn't something she spoke openly about. But after, she became an inadvertent spokesperson for a condition that many were learning about for the first time.

Lilly has partnered with the Children's Tumor Foundation to make sure that the NF community's message about acceptance and understanding is heard loud and clear. While she didn't intend to Make NF Visible, she's now at the forefront of that conversation.

Lilly Simon is courageous and resilient; one of the NF community's true Extraordinary Spirits.

The following is a press statement from the Children's Tumor Foundation, issued at the time of this incident.

NF Is not Monkeypox

As the monkeypox outbreak has garnered increased attention both in the news and social media, individuals with NF who have cutaneous neurofibromas (also known as dermals, or 'bumps' on the skin) are reporting a range of reactions both online and in public - from outright harassment to quizzical looks and more.

At CTF, we are not monkeypox experts (visit the NIH website for more info on that disease), but we are NF experts, and the two conditions could not be more different. NF, also known as neurofibromatosis, is a genetic condition that causes tumors to grow on nerves throughout the body. Sometimes these tumors present on the nerve endings on the skin, and can appear as bumps called cutaneous neurofibromas, or dermals. They are considered tumors (growths), but are also benign (non-malignant). The person living with these NF 'bumps' is not contagious - in fact, quite the opposite. Because NF is genetic, the patient is born with it (which is why we are called the Children's Tumor Foundation, and we also serve the NF patient population of all ages). While these dermals can become more prevalent as one gets older (just like the rest of the body changes as we get older), their effects are unique to each individual.

We reiterate: one cannot catch NF - it is not contagious. People are born with it. NF affects 1 in 3,000 births of all populations - all races, ethnicities, genders equally - which means that over 2.5 million people worldwide have NF. And chances are, with those statistics, you've probably met someone with NF and never realized it. Each NF case is as unique and



“The tumors are benign, but they are still all over my skin and give me a lot of health complications, both physical and mental,” Lilly said in her video. She went on to say, “I will not let any of y'all reverse any years of therapy and healing that I had to endure to deal with the condition.”

NF HERO LILLY SIMON

individual as the person living with it.

By contrast, monkeypox is a viral condition that sometimes causes a rash and reddish bumps on the skin. It does not look like NF, it is not related to NF, and it is not NF. Our hearts go out to those dealing with the monkeypox outbreak, and we encourage those susceptible to that risk to take precautions, talk to their doctor, and learn more on the NIH website.

Recently, we have been stunned to see photos and videos of NF patients posted on social media - without their permission - with disparaging remarks. We want to be clear on this point: we fiercely protect the NF community and all NF patients and their families. No one should ever be harassed, accused, shamed, or bothered in any way by anyone. Not anyone with NF, or the unrelated monkeypox, or anyone ever.

To learn more about NF, please visit our website at ctf.org.



Sondra Kincaid NF2

My mom was diagnosed with NF2 when she was pregnant with me. I was tested at age five and cleared (MRI showed no tumors). At age 10, I was frequently sick with sinus infections, tonsils infections, and problems with allergies. The ENT decided it would be best to remove my tonsils and adenoids, and was also concerned and intrigued about NF2. Per my parent's request, he sent me to have another MRI. I was diagnosed then with bilateral vestibular schwannomas. My parents promptly scheduled surgery to remove my small tumors to save my hearing.

Living with NF2 has impacted my whole life. I lost hearing in my left ear at age 10. I learned to adapt and worked hard in school to earn my advanced studies high school diploma with recognition from the National Honor Society. I graduated college twice, with two associate degrees, and became an occupational therapy assistant. I chose the two-year degree because I wanted to work in my field for as long as possible before losing my hearing completely. I learned that I had to compensate for my balance and hearing issues and work harder to achieve what I want.

I am many things, despite my diagnosis. I am a daughter, wife, stepmom, and mom of four fur kids. I love to create things. I love my family, nature, and animals. I love to photograph every experience so I can constantly relive the moments. NF2 has made me the person I am. It has grounded me, and given me an understanding of what happens in the world.

stories

OF NF

Gabriel Hilburn NF1

Gabriel was diagnosed with NF1 at the age of three. He was playing with his older brothers and sisters when he started to complain that his back was hurting. Due to his dad's scoliosis, our immediate action was to check his back, and we noticed a curve in his spine and a large lump. That day Gabriel had CT scans and X-rays, which revealed a large tumor attached to his cervical/thoracic spine. After numerous doctor visits, genetic testing, and MRIs, it was confirmed he has neurofibromatosis type 1, with two inoperable tumors, one on his spine and one in his pelvic region. We recently discovered multiple small neurofibromas as well.



His spinal tumor increased in size, causing his scoliosis to worsen even with the rods. In January, he underwent a partial spinal fusion, yet he recovered quickly. His pelvic tumor also increased, pushing on his bladder and rectum which causes "accidents" every so often. His doctor appointments for oncology and MRIs occur every three months, and his spinal surgeries occur every six months. Unfortunately, despite all efforts, including Koselugo, Gabriel's tumors are still growing and another spinal fusion surgery is imminent, as well as the debulking of his tumors.

Gabriel enjoys building and taking things apart and playing video games. He doesn't know what he wants to be yet, but he would be a great architect or engineer. We don't hinder him from trying new things or doing what he already loves to do.

We started this journey with the mindset that we would make every moment count. Despite his NF, scoliosis, and ADHD, we firmly believe that Gabriel can do anything. He knows it too.

—Gabriel's mom, Tatjana

SHINE A LIGHT **NF** WALK

The Shine a Light Walk program had its most significant year ever, with over 4,300 people participating in 24 walks throughout the country. The walks have raised more than \$1.2 million to fund NF research and patient programs. We continued engaging families, friends, NF Clinics, and our corporate sponsors from all over the country by offering the chance to walk near and far and share experiences on our Facebook Event pages. Those that could join us in person were able to connect with other NF families and enjoy a fun day celebrating our NF Heroes.

Fundraising continues through the end of December, and we are confident that we will exceed our national goal of raising \$1.6 million in 2022.

Thank you to our local volunteers, fundraisers, donors, and, most importantly, the families who support their NF heroes each day. Special thanks to Alexion, who was our National Walk Sponsor.

Read on for highlights from Shine A Light NF Walks earlier this fall.

The Carolinas October 8th

The Carolinas continue to break all Shine a Light NF Walk program records. This year they hit a new milestone and became the highest-grossing walk in our history, raising more than \$150,000 in their third year.

Twenty-five teams celebrated their NF Heroes by enjoying a sunshine-filled morning of dancing, walking, and fun photo ops with Homer, the Charlotte Knights mascot. Thank you to organizer Nicole Domazos and her committee: Maureen Hussey, Alexis Stadler, Norie Gildersleeve, and Tracy Galloway, for setting the bar high.



Chicagoland - September 17th

This year, Chicago reached a new milestone by joining the walks that raised more than \$100,000. In just five years, this walk has grown from 4 teams to 17! Over 400 participants gathered together for a day full of fun, food, music, and a special appearance by Elmo! New teams joined with those that have participated for many years, forming the largest walk Chicago has ever had. Many thanks to Kristin Meek, Stacy Raymond, Amanda Gentile, Jessie Bodell, Becki Deuel, and Erin Mohideen for helping make this event so special.



Iowa - September 25th

More than 150 walkers from all over Iowa gathered for their walk day. Even though it was raining, that didn't stop families from shining a light on NF and raising more than \$47,000! Thanks to our extraordinary organizer, Alicia Tegtmeyer, and her committee for making this a fantastic event. Alicia won the dedication award this year for not only putting on this event while nine months pregnant but also leading the teams in fundraising this year.





AN INTERVIEW WITH Will Hoffman

We spoke with NF Endurance athlete Will Hoffman about his son Liam's journey with NF and what drew him to the Children's Tumor Foundation.

Can you tell us a bit about Liam's diagnosis?

We noticed the café au lait marks on Liam and, with our pediatrician, sought care from our Pittsburgh NF clinic, which is when we received his diagnosis. He is doing great! His school and teachers have been wonderful in getting him the support that helps him succeed. He is a red belt in Karate and is working towards his

Blackbelt. He is also involved with Boy Scouts. He has been excited to fundraise for our local NF clinic and CTF.

When did you start running?

I started running in high school to stay in shape for basketball, and then I got back into running to prepare for the Pittsburgh Marathon in 2014. During that process, I fell in love with training for a race and participating in big races like Pittsburgh, New York City, and Boston.

Why did you join the NF Endurance team this year?

After seeing the impact we had on the Pittsburgh NF community with our fundraising efforts for the Pittsburgh Marathon this year, I couldn't wait to perpetuate that momentum for the Children's Tumor Foundation. In addition to helping CTF raise money, I think people watching us are inspired. And if someone can see me, at 44, hoofing around Chicago and raising money for CTF, and it inspires them to start running and making a difference for a charity, then it will be worthwhile.

Why did you select the Chicago Marathon?

I love big-city marathons! I read somewhere that if you want to see the best of a city, run that city's marathon. I know that is the case with the Pittsburgh Marathon. It is one of the best ways to see our city. So, when the opportunity arose to do Chicago, I jumped at the chance.

What has been your most successful means of fundraising, and what advice would you give others who want to fundraise for CTF?

Using social media and our existing support system for the Pittsburgh race has been very successful. Having Liam involved helps ensure folks understand what the money raised goes towards and how it helps change the trajectory of our fight against NF.

Is there anything else you'd like to add?

I want to thank CTF and the NF Endurance team for the opportunity to run in Chicago. I have seen how running in these premier races can inspire others. And to do that, all while fundraising for a charity that can impact people's lives is an honor.

Our thanks to Will and his family for making a difference!

NEW NFE EVENT: EL TOUR DE TUCSON

November 18, 2023

Cyclists, take note! NF Endurance has put El Tour de Tucson on the map in blue and green! Please mark your calendars for this fabulous fall event in its 40th year. El Tour offers rides of 102, 63, or 32 miles and a family-friendly El Tour Fun Ride, with 10, 5, or 1-mile course options. Ride with us out West and you'll join over 7,000 riders in a beautiful destination event celebrating community, charity, health, and wellness.

Learn more at nfendurance.org



2023 NF ENDURANCE EVENTS CALENDAR

Los Angeles Marathon
March 18-19, 2023

United Airlines NYC Half
March 20, 2023

Flying Pig Marathon
May 7, 2023

TD Five Boro Bike Tour
May 7, 2023

Colfax Denver Marathon
May 21, 2023

Rock 'n' Roll San Diego
June 3-4, 2023

BMW Berlin-Marathon
September 24, 2023

Bank of America Chicago Marathon
October 8, 2023

TCS New York City Marathon
November 5, 2023

El Tour de Tucson
November 18, 2023

Learn more and register now at
nfendurance.org

CUPID'S CHARITY

What a Top Organizer Has to Say about Cupid's Undie Run:

"I've always wanted to participate in a Cupid's Undie Run, and now I can cross it off my bucket list. Thank you to my beautifully amazing friends Christina Ann and Felicia Benjamin who drove me to Delaware, walked alongside me, and literally held me up. I could never have done this without them. Last but not least, thank you to Caroline Mouldsdales and Jennifer Hamilton Rubert for the display of lavish love and kindness toward me and for the colossal effort in putting together a wildly successful NF fundraiser. Almost 60K raised for neurofibromatosis! The atmosphere was electrifying. You ladies are extraordinary and could run the world. So much love between strangers. We may not be related by blood but we are related by heart. I am beyond grateful for this experience and even though it's been difficult to process some things in my life recently, I realize my times are in God's hands, and I'm going to do as much as I can while I can. Carpe Diem! See you guys next year!"

—Nissa Marie, who lives with NF2 and was a top organizer and fundraiser in the 2022 Delaware Cupids Undie Run.



From left: Christina Davies, Felicia Benjamin, and Nissa Marie at the Delaware Cupid's Undie Run.



REGISTER TODAY

CUPIDS.ORG

WE BARE IT ALL TO RAISE MONEY AND AWARENESS FOR NEUROFIBROMATOSIS (NF)!

ATLANTA, GA
AUSTIN, TX
BALTIMORE, MD
BOISE, ID
BOSTON, MA
BUFFALO, NY
CHARLESTON, SC
CHICAGO, IL

CINCINNATI, OH
CLEVELAND, OH
DENVER, CO
DETROIT, MI
JACKSONVILLE, FL
KANSAS CITY, MO
LOS ANGELES, CA
MINNEAPOLIS, MN

NASHVILLE, TN
NEW YORK, NY
ORLANDO, FL
PHILADELPHIA, PA
PHOENIX, AZ
PITTSBURGH, PA
RALEIGH – DURHAM, NC
RENO, NV

SAN FRANCISCO BAY AREA, CA
ST. LOUIS, MO
ST. PETERSBURG, FL
VIRGINIA BEACH, VA
WASHINGTON, D.C.
WILMINGTON, DE

CTF and EU-PEARL: Shaping the Future of Drug Development Through Platform Trials

CTF is providing an in-kind contribution to a €26 million EU-funded project under an Innovative Medicine Initiative (IMI) joint undertaking, entitled EU Patient-centric clinical trial Platforms, or EU-PEARL. This pursuit aims to build platform trials in which multiple drugs are tested in parallel under the same clinical protocol. This approach allows more efficient identification of potential treatments. Thanks to CTF's advocacy efforts in Europe, NF was chosen as a prototype for rare diseases by the IMI, one of the world's largest public-private partnerships in healthcare. The NF component of EU-PEARL is co-lead by CTF and the Erasmus Medical Center in Rotterdam, Netherlands.

Following consultations with clinicians and patients through a Delphi procedure, the protocols for two platform trials are being defined and finalized by the

Spring of 2023. An NF1 platform trial will initially focus on treatments for plexiform neurofibromas, cutaneous neurofibromas, optic pathway gliomas (OPG), and non-OPG low-grade gliomas. A schwannomatosis platform trial will enroll schwannomatosis patients, including NF2-related schwannomatosis patients, and will test the ability of drugs to shrink tumors. Criteria to be used in a platform trial to treat pain in schwannomatosis are also being discussed. CTF will coordinate efforts among schwannomatosis and pain experts to reach this goal in consultation with patients.

While a network of European clinics will be the first of many to execute these trials, the established protocols will enable trials such as this to run globally. Learn more about these efforts at eu-pearl.eu

CTF EUROPE'S GRANT WRITING MENTORING

Grant Writing Workshop for European NF Researchers

Each year, funding for NF research is released globally, yet too few European researchers have benefited from that funding. CTF Europe sponsors a mentoring program in writing for American-style grant applications for junior faculty or post-doctoral students. If interested, contact mnievo@ctf.org

CTF EUROPE TRAVEL GRANTS: Professional Development Opportunities

PARTICIPATING CENTERS

Erasmus Medical Center, Rotterdam, Netherlands

Hôpital Pitié-Salpêtrière, Paris, France

Universitätsklinikum Hamburg-Eppendorf, Hamburg, Germany

Guy's and St Thomas' Hospital, London, United Kingdom

Hôpital Henri-Mondor, Créteil, greater Paris area, France

UZLeuven, Leuven, Belgium

Medical University, Vienna, Austria

For further details, go to ctfeurope.org/research

NF News is the official publication of the Children's Tumor Foundation. All issues are available on our website at www.ctf.org. Please direct any questions or feedback to info@ctf.org.

NF News Editor, Vanessa Younger

NF News Design Director, Susanne Preinfalk

The Children's Tumor Foundation is the world's leading nonprofit dedicated to funding and driving innovative research that will result in effective treatments for the millions of people worldwide living with NF, a group of genetic disorders that causes tumors to grow on nerves throughout the body. One in every 3,000 people is born with some type of neurofibromatosis or schwannomatosis, which may lead to blindness, deafness, bone abnormalities, disfigurement, learning disabilities, disabling pain, or cancer. NF affects all populations equally, and while there is no cure yet, the Children's Tumor Foundation mission of driving research, expanding knowledge, and advancing care for the NF community fosters our vision of one day ending NF. For more information, please visit: ctf.org.

FOUNDATION STAFF

Annette Bakker, PhD, President

Research and Medical Programs

Salvatore La Rosa, PhD, Chief Scientific Officer
Vidya Browder, PhD, Senior Manager, Basic Science
Angela Dumadag, Senior Manager, External Relations
Kate Kelts, Coordinator, Patient Support
Pamela Knight, Senior Director, Clinical Program
Elana Loftspring, External Relations Coordinator
Jessica McElmeel, Coordinator, Clinical Science Team
Marco Nievo, PhD, Chief Scientific Officer, CTF Europe
Elizabeth Oliver, Senior Executive Assistant
Patrice Pancza, Vice President, External Relations
Heather Radtke, Senior Manager, Clinic & Symposium

Development

Michele Przepyszny, Chief Advancement Officer
Jamie Balhon, Director, Development, Cupids
Amy Boulas, VP Development, P2P & Field Based Events
Cassidy Brewer, Manager, Development, Cupids
Holly Cheatham, Manager, Development, Cupids
Emily Crabtree, Senior Director, Development Operations
Aidan Fraser, Development Assistant
Barbara Gallagher, Vice President, Development, Corporate
Lauren Johnston, Manager, Shine a Light NF Walk
Krysta Mochi, Manager, Shine a Light NF Walk
Julie Nassisi, Senior Manager, Special Events
Kim Robinson, Senior Manager, Advancement
Connie Sorman, Senior Manager, Stewardship and Volunteer Development
Rebecca Taylor, Director, Shine A Light NF Walk
Lydia Vanderloo, Senior Manager, NF Endurance

Finance and Administration

Sarah Bourne, Vice President, Finance and Operations
Rachel Anderson, Director, Donor Database Operations
Jennifer Ching, Director Human Resources
Brianna Daquino, Accountant
William Johnson, Salesforce Administrator
Dan McAvoy, Operations Assistant
Carey Milligan, Senior Manager, Accounting
Brandon Weaver-Bey, Senior Coordinator, Gift Processing

Marketing and Communications

Simon Vukelj, Chief Marketing Officer
Rebecca Harris, Senior Manager, Public Relations
Alissa Marks, Director, Marketing
Susanne Preinfalk, Director, Design
Ryan Sells, Coordinator, Communications
Vanessa Younger, Director, Communications
Maribel Zambrana, Manager, Digital Marketing
Marina Zouaghi, Data Analyst



NON-PROFIT ORG
U.S. POSTAGE PAID
TWIN CITIES, MN
PERMIT NO. 29626

CHILDREN'S TUMOR FOUNDATION

National Office:

697 Third Avenue, Suite 418
New York, NY 10017

Send Donations to:

Mail Code: 6895
P.O. Box 7247
Philadelphia, PA 19170 - 0001

Join the NF Registry!

**A secure, worldwide database
for people living with neurofibromatosis
and schwannomatosis.**

- Share information about living with NF to help researchers learn more
- Receive emails about research and chances to get involved
- Strength in numbers! Help grow awareness among clinicians, researchers, and industry professionals

**Increase knowledge, advance
research, and stay informed by
joining the NF Registry!**

nfreistry.org



Go to nfreistry.org or
scan the QR code for
more information.



SCAN ME

Join the NF Registry nfreistry.org

Donate ctf.org/believe

Connect

