

NF NEWS

CHILDREN'S
TUMOR
FOUNDATION
ENDING NF
THROUGH RESEARCH

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
SPRING 2024



BORN
A FIGHTER.

Make NF Research Visible

For NF Awareness Month, CTF has launched "Make NF Research Visible," a portrait collection featuring clinicians and researchers along with patients that looks at the impact of visibility on NF research. Making NF Research Visible affects access to funding, quality care, progress, discoveries, and the pairing of patients with clinical trials. From bench to bedside, making NF research visible has a meaningful impact on everyone touched by NF.

INSIDE:

- 3** CTF Preclinical Hub
- 4** Blood Test to Predict Cancer Risk
- 8** NF Awareness Month
- 10** Extraordinary Spirit: Patti Ptasznik

Pictured: Cristina Fernandez-Valle, PhD, a neuroscientist with a special focus on NF2-related schwannomatosis, and Jake Lipe, an adult living with NF2-related schwannomatosis.



At the Children’s Tumor Foundation (CTF), we are passionate about accelerating the development of treatment options for NF, which includes neurofibromatosis type 1 (NF1) and all forms of schwannomatosis (SWN), including NF2-related schwannomatosis (NF2-SWN). This incredible organization was founded more than 45 years ago, at the initiation of NF patient Lynne Courtemache, who recently passed away. It is my joy to honor her memory by leading this Foundation toward the treatments and cures that she so boldly envisioned.

As we prepare for our May NF Awareness Month initiatives, I extend my sincere appreciation for your continued support. The Foundation’s all-new Make NF Research Visible campaign builds upon our past campaign and captures NF patients alongside the variety of NF professionals who work to improve the lives of the millions worldwide living with NF. My utmost thanks to our new CTF Board member Kim Snipes for bolstering our NF Awareness Month fundraising campaign by matching donations, dollar-for-dollar.

Our recent gatherings at the NF Summit and the NF Clinicians meetings in San Antonio, Texas, were truly inspiring. The collaborative spirit demonstrated by patients, clinicians, and other “NF-ologists” drives us forward at CTF. Looking ahead, I eagerly anticipate the Global NF Conference in Brussels, Belgium, this June. It will be the largest gathering of NF professionals in the world, hosted by CTF Europe and organized by CTF and the European NF Group. We are thrilled that Nathalie Moll, the Director General of the European Pharma Trade Association (EFPIA), will deliver the Opening Address.

I invite you to explore the stories and impact featured in this newsletter. From scientific advancements to community events, each narrative underscores the collective efforts of this dedicated community. I am particularly moved by the story of Patricia Ptasznik, whose extraordinary spirit and innovative mind are helping us steer our course with tools for data-driven decisions and improved efficiency.

The dedication and support shown by you, our CTF family, are the driving forces behind our mission. I extend my deepest gratitude to our incredible CTF Staff and Board, and to our NF patients, whose resilience and courage inspire us every day, as we work to bring an end to NF.

With shared determination,

Annette Bakker, PhD
President

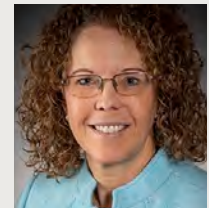
IN THE NEWS:

BioSpace Publishes Opinion Piece by CTF President Annette Bakker

BioSpace, a life sciences digital destination with a mission to improve productivity in the process of discovery, published CTF President Annette Bakker’s latest opinion piece, “Healthcare Nonprofits Drive Positive Change for Patients.”

To read the full article, search “Biospace” at ctf.org/news

Welcome New Board Members

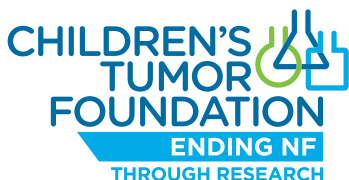


Kim Snipes



John Morris

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CTF Preclinical Hub: Case Studies

The Children's Tumor Foundation is accelerating the path to drug discovery by constructing an NF-focused Preclinical Hub to supercharge the development of NF treatments. The Preclinical Hub is built on the successes of the Preclinical Consortium (2008-2016) and Synodos for NF2 initiatives (2014-2017), both of which efficiently delivered treatments to patients. Due to limitations in scalability of both initiatives, CTF decided to expand the Preclinical Hub to become a full public-private partnership.

The Preclinical Hub will speed the approval of potential treatments by offering the following to academic, research, and pharmaceutical industry partners:

- Access to disease models, data tools, drug libraries and biological material
- Expert advice and support during preclinical study design and execution

- Prenegotiated Master Service Agreements
- Predetermined protocols and tests

The Preclinical Hub is already underway and has announced a CTF-funding opportunity to support the generation of clinically relevant models for NF. Additionally, several case studies serve as examples of the function and power of this bold new initiative.

Case Studies

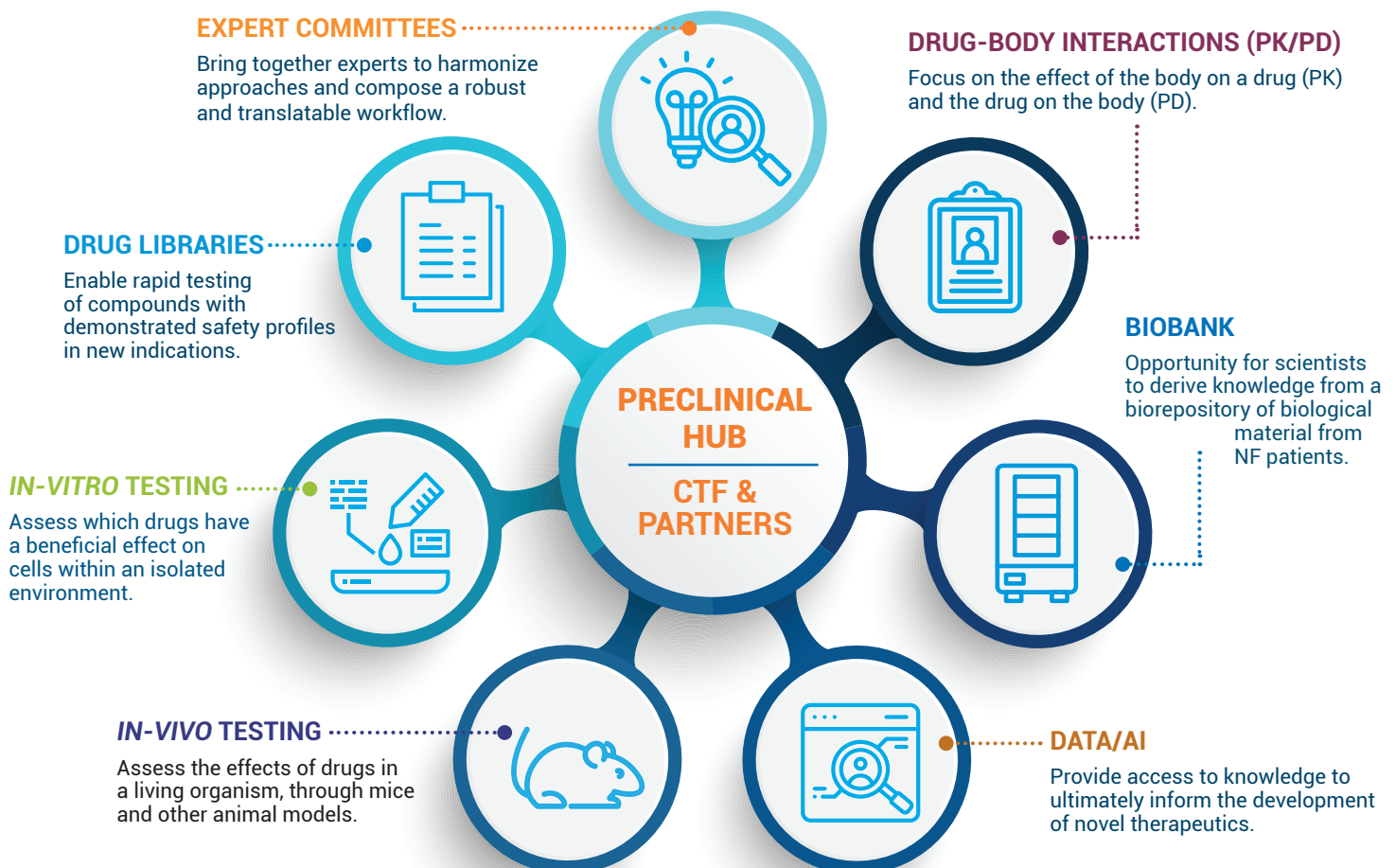
As part of the Preclinical Hub efforts, CTF was able to connect an AI-powered drug discovery company in the rare disease space with researchers holding key preclinical models for NF. Making this connection and sharing our prenegotiated workflows between the company and the expert research facility running the preclinical experiments is a great way to streamline drug development efforts.

This will significantly shorten the timeline to the clinic.

We have also helped companies initiate an NF program and support early proof of concept studies that could ultimately lead to a clinical program for NF. In such cases, working through the Preclinical Hub has provided expert advice, connections to appropriate research experts running preclinical models, and resources for gathering the initial data necessary to potentially launch an NF program.

These examples are an early glimpse into what is possible with the Preclinical Hub initiative. We are delighted to share this progress as we continue to identify, validate, and share the most robust preclinical models for clinical translation, and accelerate the identification of clinical trial-ready therapeutics for NF.

To find out more, visit ctf.org/preclinicalhub



Promising Blood Test for Predicting Cancer Risk in NF Patients

The Children's Tumor Foundation announced a groundbreaking three-year study, which it will fund for nearly \$2 million, to determine if a DNA-based blood test can offer better understanding and ultimately earlier diagnosis of cancer predisposition in neurofibromatosis type 1 (NF1) patients.

In a publication led by Taylor Sundby, MD, and others, and funded by the Children's Tumor Foundation, researchers are developing a remarkable blood test capable of predicting the risk of malignant peripheral nerve sheath tumors (MPNSTs) in individuals with NF1. This cutting-edge advancement offers hope for early detection and intervention, potentially transforming the landscape of NF management. MPNSTs are rare but aggressive tumors and pose significant challenges in NF1 patients, often eluding early detection until they reach advanced stages. However, this innovative blood test could revolutionize how healthcare providers monitor patients, enabling proactive measures to mitigate the risk of MPNST development. This research heralds a new era in

NF care by providing a simple and non-invasive method for assessing MPNST likelihood, offering patients and healthcare professionals a powerful tool in the fight against this complex condition.

Collaboration across the NF funding spectrum is making this research opportunity possible. The Principal Investigators (PIs) of this project are Angela Hirbe, MD, PhD of Washington University and John Shern, MD of the National Cancer Institute (NCI) at the National Institutes of Health (NIH), in PI-level collaboration with Aadel Chaudhuri MD, PhD at Washington University.

Read more about this study and CTF's nearly \$2 million investment at ctf.org/news.

CTF-Funded Research Published in *Nature*

In the quest to determine why Schwann cell tumors can become more aggressive and resistant to treatment, a significant study (funded by a CTF Young Investigator Award and others) dove deep into the understanding of Schwann cell tumors, which are often found in individuals with either neurofibromatosis type 1 (NF1) or NF2-related schwannomatosis (NF2-SWN). This work has now been published in the leading publication *Nature Communications* titled "Functional Interactions Between Neurofibromatosis Tumor Suppressors Underlie Schwann Cell Tumor Differentiation and Treatment Resistance."

Using cutting-edge techniques in genomics and biochemistry, the researchers examined samples from humans, cell lines, and animal models. The study uncovered a significant link between NF1 and NF2-SWN in the context of Schwann cell tumors. While NF1 and NF2-SWN are separate conditions caused by pathogenic variants (mutations) in different genes, this research reveals how changes in the NF2 gene can impact the behavior of Schwann cell tumors, particularly in individuals with NF1 pathogenic variants.

This has the potential to be a game-changer for patients. By understanding the connection between NF1 and NF2-SWN in the context of Schwann cell tumors, researchers may be able to develop targeted therapies that can benefit patients. This could lead to more effective treatments for Schwann cell tumors and improved outcomes for individuals. This work is a significant step forward in the fight against NF.

CTF-FUNDED RESEARCH SHEDS LIGHT ON EXPERIENCES OF CHRONIC PAIN

Investigators at Yale University and the National Cancer Institute recently published new research in the *American Journal of Medical Genetics* on the experiences of chronic pain in NF1. This study, which recruited participants through the NF Registry and was funded by a CTF Clinical Research Award, aimed to evaluate a new way to measure chronic pain in individuals with NF1.

Chronic pain is an important aspect of living with NF1, but it's usually not well understood and, therefore, hard to treat. Thanks to this research team led by Frank Bruno, PhD, CTF-funded researcher and NF2-SWN patient, and the individuals who participated in the study, results show that the tool studied, called the Neurofibromatosis Pain Module (NFPM), can characterize NF1 pain in many different ways – giving a clearer picture of what it's actually like to experience it. By understanding all the different aspects of chronic pain, clinicians can more reliably measure a patient's experiences with pain over time and make smarter decisions about treatments. It's a significant step forward in improving care for those affected by NF1.

A summary of this study, as well as a link to the complete publication, can be found at ctf.org/news

CTF AND NFLECTION PRESENT WEBINAR ON NFX-179 TOPICAL GEL RESULTS

The Children's Tumor Foundation hosted an informative webinar from NFlection Therapeutics to share results from the Phase 2b Study of NFX-179 Topical Gel in the Treatment of Cutaneous Neurofibromas in Neurofibromatosis Type 1 (NF1). NFX-179 is a topical (on the skin) treatment for cutaneous neurofibromas (cNF), which are tumors that grow in the skin or right underneath the skin and result in painful, disfiguring bumps on the skin.

Recorded live on January 18, 2024, Kavita Sarin, MD, PhD, Associate Professor of Dermatology at Stanford University School of Medicine, gave an informative summary of the clinical trial results. Following the presentation, the NFlection Chief Executive Officer William Hodder and Chief Operating Officer Gern Kochendoerfer, along with Dr. Sarin, answered attendees' questions and provided additional information.

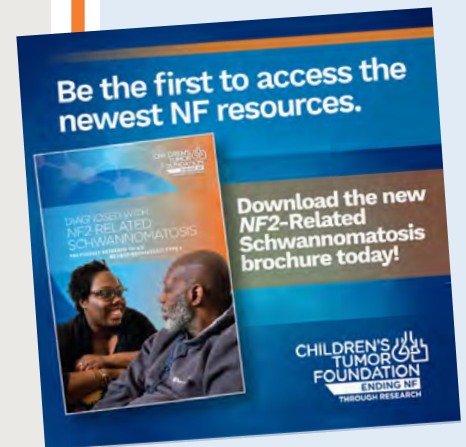
Last year, the Children's Tumor Foundation

entered a new phase in its strategic NF research model by announcing a significant impact investment in this Phase 2b clinical trial at NFlection Therapeutics, a biotechnology company focused on the discovery and development of effective, targeted therapies for rare diseases. CTF's investment strategy in NF drug development is a diversified portfolio approach to tackling all forms of NF, from early-stage investments in young investigators to open data collaboratives such as Synodos. This impact investment further establishes CTF as a co-investor with pharma/biotech in innovative NF research, expanding opportunities to increase the NF drug development pipeline.

To view this webinar, please visit the videos section of our Resource Library at ctf.org/education

AN ALL-NEW
ctf.org:

Improving
Your Source for
NF Information



The Children's Tumor Foundation's website has a whole new look! Reinforcing our dedication to providing the latest news and education about NF, the new Foundation website includes a robust search function and an enhanced user experience. Visitors to ctf.org can now enable accessibility features such as larger text size, a screen reader, and visual adjustments, according to their needs and preferences. The site also personalizes the experience for visitors interested in specific topics, and translates into nine different languages at the click of a button.

At the CTF Resource Library, you can search our newly updated resources by NF type, topic, and language, with many new translations added and more in progress. All CTF patient resources are freely available to read, download, and share.

See what's new at
ctf.org/education

Innovative Health Initiative Highlights CTF's Game-Changing PLATFORM TRIALS

The Innovative Health Initiative (IHI), the European Union public-private partnership funding health research and innovation, recently highlighted CTF's leadership in spearheading a pioneering platform trial for patients with neurofibromatosis or schwannomatosis. Collaborating with the Global Coalition for Adaptive Research (GCAR), CTF is leveraging the groundbreaking framework it developed with EU-PEARL to revolutionize rare disease clinical trials.

By championing a 'plug and play' system, CTF is ushering in a new era of cost-effective and time-efficient drug development, with patients at the forefront. This visionary approach offers access to potential life-changing medications and addresses critical challenges in patient recruitment and trial design. Each drug plugged into the trial costs an estimated \$2 million per year, compared to tens of millions

typically needed, offering a cost-effective and time-efficient approach to drug development. Within approximately 36 months, clear signals regarding the efficacy of drug candidates can be obtained, a departure from traditional clinical trial timelines. Patients enrolled in the trial have continuous access to treatments, promising improved outcomes. By addressing the key challenges in patient recruitment and trial design, CTF and its strategic partners are setting a new standard in NF and rare disease research.

As these platform trials gain momentum, pharmaceutical companies are invited to partner with CTF to accelerate the discovery of innovative treatments, making this a pivotal moment in the fight against tumors.

For links to the article from the IHI, as well as the press release announcing the CTF and GCAR strategic alliance, visit ctf.org/news



2024 NF Clinicians Meeting

The Children's Tumor Foundation held an educational and networking meeting for NF clinicians to discuss relevant and practical information about neurofibromatosis and schwannomatosis on April 12 and 13, in San Antonio, Texas, in conjunction with the NF Summit. This professional education opportunity was open to all physicians, advanced practice providers, genetic counselors, and other healthcare providers with an interest in NF.

The meeting started with a Friday evening reception and case discussion titled "NF1 throughout the Lifespan" with Drs. Laura Klesse and Kaleb Yohay, and was sponsored by Alexion, AstraZeneca Rare Disease. Saturday's educational sessions included interpretations and implications of genetic testing, vestibular dysfunction in NF2-SWN, transitioning from pediatric to adult care, and many more. Co-chairs for the meeting included Ashley Taylor, PA-C, University of Oklahoma Health Sciences Center, Ledare Finley, MS, LCGC, and Heather Radtke, MS, CGC, Children's Tumor Foundation.

2024 NF Summit

In April, more than 300 members of the NF community convened in San Antonio, TX, for the 2024 NF Summit. The NF Summit is CTF's annual patient and family gathering, bringing together NF patients and families, volunteers, event organizers, researchers, clinicians and supporters—who all have a connection to NF.

This year, attendees heard from experts on various topics, including genetic variants, family planning, vestibular rehabilitation, and treatments for cutaneous neurofibromas. The event's medical co-chairs, Devorah Segal, MD, PhD, pediatric neuro-oncologist at NYU Langone Medical Center, and Manikum Moodley, MD, a pediatric neurologist at Dell Children's NF Center, presented on NF1 through the lifespan, answering family's questions on when to worry about what symptoms. Scott Plotkin, MD, PhD, shared insights about researchers currently studying pain in neurofibromatosis and schwannomatosis.

The 2024 agenda also included a renewed focus on the role of patients and care advocates in advancing NF research, not only as research

subjects but as representatives of the NF community. Attendees got the chance to hear from Josh Denny, MD, MS, of the National Institutes of Health's "All of Us" project, on how the study can impact the rare disease and NF communities, as well as updates on CTF Engage, CTF's patient representative program. Closing the Summit, social media influencer and NF1 patient Amit Ghose inspired attendees with this motivational keynote address.

The CTF Volunteer Leadership Council Members hosted an in-person Round Table meeting for all members to get updates on foundation-wide initiatives and network with other CTF volunteers. The entire community enjoyed a time of fellowship with a walk to San Antonio's historic landmark, The Alamo.

Closing the meeting, the Make NF Visible Community Recognition Awards were presented to recognize individuals or groups who Make NF Visible locally, nationally, or globally.

All educational sessions from the 2024 Summit are available to view at nfs Summit.org.

NF Patients Speak at FDA Listening Session

On May 21, 2024, patients living with NF1 will offer their unique and unforgettable personal accounts of living with cutaneous neurofibromas (cNF) to officials from the Food and Drug Administration (FDA).

Whereas cNF have been historically considered a "cosmetic" issue, we know that they can greatly impact the quality of life for some NF1 patients in unexpected ways and remain a top priority for the community. The listening session intends to help professionals involved in the drug approval process understand the obstacles and unmet needs of patients living with this specific manifestation of NF, and how those

patients might benefit from emerging new drug treatments. As with all listening sessions, this meeting will be closed to the public.

This is the second FDA Listening Session initiated by the Children's Tumor Foundation's Patient Engagement initiative, CTF Engage. In 2019, five individuals offered vivid, unforgettable personal accounts of living with NF to officials from the FDA.

The Patient Listening Sessions are a joint initiative of the FDA Patient Affairs Staff

(PAS) and the National Organization for Rare Disorders (NORD). These meetings facilitate the sharing of patient perspectives on disease burden, treatment burden, impact on daily activities, quality of life, and priorities to consider in medical product development programs. The objectives of this session are to inform regulatory decision-making, educate review staff about NF, help patients understand the FDA's mission and work, and provide a starting point to inform research & development.

Watch for a recap of the May 21 session on the CTF newsfeed at ctf.org/news

stories

OF NF

Gianna Cochran

NF1

Gianna's father has neurofibromatosis type 1 (NF1), so we knew when she was one year old and had more than 30 café au lait spots [that she also had NF]. Her doctor referred her to the Genetics Department, where she was diagnosed. Neurology performed a brain MRI, and we were set up through the Ophthalmology Department as well.

Living with NF has been difficult. While Gianna seems like a healthy child, she has developed a brain tumor called a tectal plate glioma that caused hydrocephalus (a condition in which fluid accumulates on the brain) and needed surgery to correct it. Gianna has also been diagnosed with MoyaMoya disease related to NF1. The many surgeries and doctor's appointments can take their toll on her fight.

Gianna is very creative and artsy. She also loves tumbling, cheer, make-up, and being silly!

—Submitted by Amber, Gianna's mom



Shaborah Ball

NF1

I was diagnosed with NF1 at birth, and I was 3 years old when I developed an optic glioma in my right eye. I had many surgeries growing up to debulk the tumor in my eye. Growing up with NF1 has been difficult; there have been adult bullies and child bullies who have made me feel bad about myself for having this condition. It was difficult coping with bullying from teachers and students while trying to learn in school with a learning disability and my facial differences.

As an adult, I've suffered through adult bullies both in the workplace and random strangers who like to yell out hurtful things, people thinking I'm dangerous or someone to be afraid of, avoided, and often stared at. Sometimes, this condition makes me tired more quickly than others, or I need to rest more because physically I am in so much pain. Two more tumors were discovered in my brain recently, and they are being monitored. Thankfully, none of my tumors were cancerous. Sometimes, I have trouble with my speech impediment, learning disabilities, chronic pain, depression, and anxiety. However, most of my anxiety comes from being in social situations because people can be curious, and some people can be cruel. NF has impacted my confidence and impacts my social life. I try to push through the negativity and remind myself that I am fearfully and wonderfully made.

I am thankful for my family and friends, who have given me the strength to keep going and push through all the negativity that comes with people who are different in this world. I thank God for giving me my life, family, and friends. I am thankful for continuing my education and getting my Bachelor's in psychology with a minor in criminal justice, and getting a job where I can give back to my community.

The gift NF1 has given me is to not give up on yourself and your dreams. NF1 has given me the drive to fight and the compassion to see and accept others for who they are. We are all different, and some of us just stand out even more differently among the different. My wish and hope is to have people be accepting of others' differences. I don't like to say "deformity" because I feel like that term has a negative connotation when people think of it. I like to say my face is beautiful just as it is facially different.

My love for supporting my community is not only in my work but also in my everyday life.

"The gift NF1 has given me is to not give up on yourself and your dreams. NF1 has given me the drive to fight and the compassion to see and accept others for who they are."

—SHABORAH BALL, WHO LIVES WITH NF1

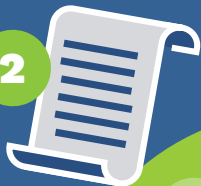


MAY IS NF AWARENESS MONTH Map to May!

This Map to May from the Children's Tumor Foundation includes the meaningful actions you can take before and during NF Awareness Month. Visit [makenfvisible.org](https://www.makenfvisible.org) to learn more ways you can spread the word about NF, and Make NF Research Visible!

PROCLAIM

2



Approach your local government and ask for an NF Awareness Month proclamation.
[ctf.org/proclaim](https://www.ctf.org/proclaim)

Share NF facts and infographics
[ctf.org/education](https://www.ctf.org/education)



LEARN



Create an event to spread the word!
[ctf.org/fightnfyourway](https://www.ctf.org/fightnfyourway)

PLAN

3



Wear our exclusive CTF blue and green gear all month, and especially on May 17 & 22.
[ctf.org/store](https://www.ctf.org/store)

SHOP

4



SHINE

1



Approach local landmarks and ask them to Shine a Light on NF by lighting up in blue and green, the official colors of the End NF movement.
[ctf.org/shinealight](https://www.ctf.org/shinealight)

Record a video to tell the world what NF Research means to you!
[ctf.org/myvideo](https://www.ctf.org/myvideo)

RECORD

5



7 SPREAD THE WORD

Follow @childrenstumor on social media and share our posts, and tell your stories using the hashtag #EndNF

[makenfvisible.org](https://www.makenfvisible.org)

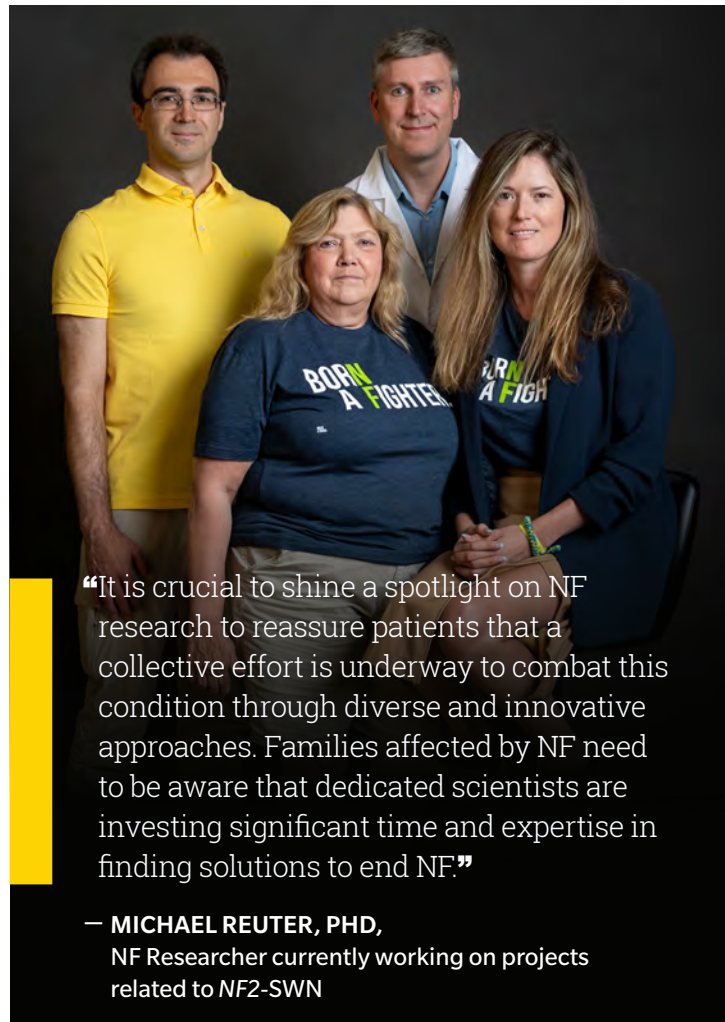
MAKE NF RESEARCH VISIBLE

Throughout the year and especially during NF Awareness Month, the Children's Tumor Foundation is passionate about working with you, our dedicated donors and friends, to Make NF Visible. We want to make sure the world sees NF, and sees each person living with it.

This year, we've expanded our Awareness Month campaign to Make **NF Research Visible**. We believe that the NF community stands on the brink of transformation, and NF Research has the power to transform lives.

We want adults and children living with all types of neurofibromatosis and schwannomatosis to be free of the pain and fear that come with these conditions. Make your donation now at ctf.org/give or scan the QR code below to help fund more treatment options for all types of NF.

Your donation today will be **MATCHED dollar-for-dollar**, thanks to a generous gift from our new Board member, Kim Snipes. Thank you for joining us—there is so much more work to be done, and we need your help today.



“It is crucial to shine a spotlight on NF research to reassure patients that a collective effort is underway to combat this condition through diverse and innovative approaches. Families affected by NF need to be aware that dedicated scientists are investing significant time and expertise in finding solutions to end NF.”

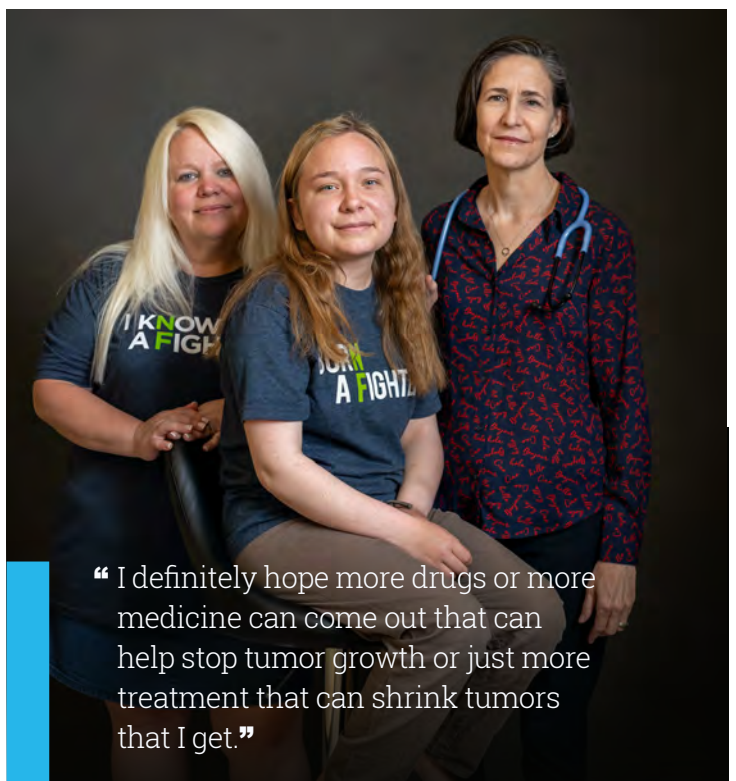
— **MICHAEL REUTER, PHD,**
NF Researcher currently working on projects related to *NF2-SWN*

Our thanks to NF Dad and photographer Craig Warga for the beautiful photography and videography featured throughout this campaign.



“Making NF research visible is important to give all individuals with neurofibromatosis or schwannomatosis the opportunity to participate in cutting-edge clinical trials. If patients participate, it may help them today, but more importantly, it will help generations of patients in the future to solve the difficult questions of NF care. We cannot answer tough questions about NF without NF patients.”

— **HEATHER L. THOMPSON, PHD, CCC-SLP,**
Speech-Language Pathologist and NF Researcher



“ I definitely hope more drugs or more medicine can come out that can help stop tumor growth or just more treatment that can shrink tumors that I get.”

— **HOLLY BEEMAN,** who lives with NF

Extraordinary Spirit: **PATRICIA PTASZNIK**



The Children's Tumor Foundation proudly celebrates Patricia Ptasznik, also known as Patti, a loving NF mother and a leader in data management at American Airlines. Through her roles as both a passionate NF advocate and an accomplished IT professional, Patti exemplifies our mission to end NF with her unwavering dedication and resolve.

Patti's journey is a testament to the impact one individual can have on a cause close to

their heart while showcasing how team efforts can drive meaningful change. At American Airlines, she catalyzed a unique collaboration with the Children's Tumor Foundation, emphasizing teamwork and problem-solving for a cause aligned with their motto to "care for people on life's journey."

In 2021, on American Airlines' Charity Day, a day designated by the IT and select business teams to give back to their communities, she assisted the Foundation with donor data management and analysis, leveraging the collective expertise of over 135 data technologists. Meanwhile, another group of experts worked on NF scientific data to advance NF research. Patti orchestrated the collective effort, uniting the company's expertise and diverse talents to support the Foundation's mission.

In 2024, the collaboration continues as the American Airlines team again embarks on Charity Day in support of CTF, this time focused on advancing a data warehouse and robust analytical tools to further support the

Foundation's objectives. Patti's leadership extends beyond these initiatives as a guiding force of CTF's Data Advisory Board and other American Airlines data leaders she has recruited.

Beyond her instrumental role in American Airlines Charity Day, Patti's involvement spans a myriad of initiatives, from leading Team Amanda at the Dallas Shine a Light NF Walk to championing NF awareness efforts.

Patti's leadership in harnessing collective brainpower brings innovative solutions and strategies to grow the Foundation's reach and effectiveness. Her passion inspires a chain reaction of generosity and compassion that continues to resonate.

In Patti, we find not just a volunteer but a driving force—a beacon of hope whose extraordinary spirit uplifts and inspires us all. Amanda, her beloved daughter, and eternal inspiration infuses Patti's journey with love and determination, enriching the lives of all those around her.

Amanda as Patti's Inspiration

From Patti's remarks upon accepting CTF's 2023 Make NF Visible Corporate Champion Award

My daughter, Amanda, was born with NF1. She had an optic pathway glioma and faced learning challenges. When she was 17, neurofibroma tumors started growing inside of her, and within five years, she had extensive painful tumors throughout her entire body. She passed away in 2017 at the age of 22.

Losing a child is one of life's most difficult trials a parent can go through. I prayed that God would take all the pain and suffering Amanda and our family experienced and asked God to help me bring good out of it. The Children's Tumor Foundation was there for our family when we needed them most. I shared my daughter's NF experiences and my background in IT, and we started slowly collaborating on possible ideas over the next several years.

I've been a developer and manager in the American Airlines (AA) IT department for 34 years. And over the past 16 years, I've led the enterprise data analytics and data warehouse team. We integrate extensive amounts of operational and customer data from across the company to analyze opportunities to improve our operations and customer service and increase revenue. Data is at the heart of every decision made at American Airlines. You can't

manage what you don't measure. Organizations that are data-driven will outperform those that are not.

I worked with my AA upper management to allow team members to be able to have a Charity Day dedicated to CTF, and we were able to leverage our technical skills to analyze CTF data, identify information gaps, create data visualizations, and help CTF improve donor outreach and engagement through enriched information strategies.

CTF staff received insights and expertise to serve the Foundation in its future planning and growth. CTF is focused on medical research based on data to help find a cure for NF. My goal is to help CTF organize its data in a way that drives additional donations to fund medical research to end NF. I can't change the past and bring my daughter back, but I can do everything in my power to leverage my knowledge, skills, and time to help bring a better outcome for the families still living with NF.



*Patti's daughter,
Amanda*

Our thanks to Patti and the entire American Airlines team for their **extraordinary spirit**.

Special EVENTS

Let Good Take Flight: American Airlines Charity Day

More than 100 data technologists at American Airlines (AA) will come together on May 30, 2024, for the second AA Charity Day, committed to supporting the Children's Tumor Foundation. These dedicated experts have invested significant time 'hacking' strategies and devising solutions to advance the CTF mission of ending NF. This year's Charity Day is showcasing their work, including the advancement of the CTF data warehouse and the creation of tools to analyze donor data, identify information gaps, and create data visualizations. This will help CTF improve its constituent outreach and engagement through enriched information strategies.

The first AA Charity Day dedicated to the CTF occurred in November 2021. Under the American Airlines banner of "Let Good Take Flight," the American Airlines team found inspiration from colleague and fellow team member Patricia Ptasznik, whose daughter Amanda lived with NF. What began as an endeavor to harness individual strengths for the betterment of CTF quickly gained traction across AA's diverse data teams, evolving into a dynamic event focused on enhancing comprehension and analysis of available data. Leveraging the skills that propel AA's customer-centric success, team members ventured into the nonprofit sphere on behalf of CTF,



spreading their wings to apply their unique expertise.

CTF will benefit from the insights and expertise provided by the AA team, empowering the Foundation in its future planning and growth while at the same time utilizing its collective brainpower and teamwork to address challenges and advance philanthropic efforts. Charity Day stands as a testament to the powerful collaboration between corporations and nonprofits in addressing significant challenges together.

To honor this enduring impact, the data warehouse developed through this initiative will be named "Amanda," paying tribute to her resilience and courage, as well as to that of all NF heroes. The name symbolizes our unwavering commitment to supporting NF patients and advancing the Foundation's mission.

Dancing With Our Stars: Northwest Arkansas

On Saturday, March 9, 2024, the 3rd annual Northwest Arkansas Dancing with Our Stars Gala took place at Crystal Bridges Museum of American Art in Bentonville, Arkansas. A spin-off of the popular annual dance competition that has taken place in Little Rock since 2006, Dancing with Our Stars brings together local celebrities, professional dancers, and NF Heroes for a one-night ballroom competition to raise money and awareness for NF research.

The Northwest Arkansas stars vying for the chance to take home the coveted mirror ball trophy were Ryan Fox, Lee Hollingsworth, Melissa Parrish, Misty Van Hooser, Tony Waller, and Allisha Watkins.

"I'm excited to bring light to a condition that not many people know about through a fun-filled night of dancing and entertaining," said Ryan Fox.

Melissa Parrish added, "Dancing may be a personal challenge for me, but the excitement of supporting the Children's Tumor Foundation fueled every step."

The 2024 event raised over \$120,000 and the winners were:

- **Best Performance:** Ryan Fox, Co-Owner Foxy Tees
- **Community Advocate:** Allisha Watkins, CEO of Paradox
- **Top Fundraiser:** Tony Waller, Vice President, Constituent Relations and Racial Equity at Walmart

Mark your calendars for the Little Rock Dancing with our Stars, which will be held on Thursday, September 12, 2024, in the Wally Allen Ballroom at the Statehouse Convention Center.



SHINE A LIGHT NF WALK

The 2024 Shine a Light NF Walks are presented by **ALEXION**
AstraZeneca Rare Disease

The Shine a Light NF Walk had over 5,000 participants walk and raised close to **\$1.5 million dollars in 2023**. Thank you to our local NF Heroes and their supporters, Walk Organizers, volunteers, participants, fundraisers, donors, and our National Walk Sponsor, Alexion. Join us in 2024 as we look forward to welcoming more Walkers and raising even more money to fund critical NF research and improve diagnoses and treatments for NF patients!



IOWA

The Iowa Shine a Light NF Walk has had incredible growth! With 297 attendees and 11 teams, the Iowa Walk continues to strengthen the NF community – and raised over \$41,000 this year! Iowa welcomed several new teams and had plenty of support from the community with sponsorships from seven local businesses. The Walk had face painting, balloon animals, safety services officers, dance teams, mascots, a DJ, food, and more! There was plenty of family fun for everyone.

Join an upcoming Shine A Light NF Walk!

8/17	Iowa
9/7	Philadelphia
9/14	Chicagoland
9/14	Minnesota
9/14	South Dakota
9/14	Washington State
9/15	Michigan
9/21	Kansas City
9/21	Utah
9/28	Cincinnati
9/28	Denver
10/5	Atlanta
10/5	Carolinas
10/5	New Jersey
10/5	New York
10/12	Dallas
10/27	Southern California
11/2	Houston
11/3	Arizona
11/10	Florida
Virtual	New England
Virtual	Virginia

Registration is open for the 2024 Shine a Light NF Walks at shinealightwalk.org

CINCINNATI

Cincinnati had its biggest year ever in 2022 and carried that momentum into their 2023 campaign. With 495 attendees, the day was filled with family, friends, and fun, culminating in over \$104,000 already raised to help end NF. Cincinnati consistently produces a day of engagement and fun for everyone with characters, mascots, games, lunch, live music, and more. Congratulations to walk organizers Jessica Samblanet and Shannon Savage on another successful year!



CUPID'S UNDIE RUN

Once again, nearly 6,000 undie runners hit the streets, sidewalks, and beaches across the country in over 30 cities throughout the month of February for the Cupid's Undie Run! Having raised more than \$1.7 million this season (and still climbing), this unique event continues to evolve and grow year over year.

Many of our cities were able not only to reach but exceed their fundraising and participant goals this season, including Baltimore, Charleston, Chicago, Cincinnati, Cleveland, Denver, Kansas City, Los Angeles, Phoenix, Reno, St. Louis, Virginia Beach, and Wilmington.

We owe much of our success to those who supported us nationwide. Thank you to our selfless and hard-working Event Directors, committees and volunteers, local partners, and our National Partner, **Love, Tito's**, the philanthropic arm of Tito's Handmade Vodka, and our National Underwear Partners, **Pair of Thieves** and **Woxer**.

Registration is open for Cupid's Undie Run 2025 at cupids.org

A Party with Purpose

Cupid's Undie Run, long-time participant and Event Lead in Cleveland, Carrie Keagler, states, "It's really anything goes, ya know? We're so inclusive. You can run in your undies or in a costume or have a theme with your team."

"When I was younger, I had so many migraines and head pains. NF is on the nerves, and you never know if there's damage. . . . It's a little uncomfortable to strip down to underwear. It gives people a little bit of awareness of how it feels to be different and be out of their comfort zone. I also love the awareness and that people will stop us and ask why we're running in undies," said Carrie.

Carrie was diagnosed with NF1 at age two and has made it her mission to educate and fundraise so that future generations won't suffer from NF as she does. Our gratitude goes out to Carrie and her team, **WOOT2ACURE**, which has participated in the Cleveland Cupid's Undie Run for 13 years and raised over \$200,000.



NF Endurance Team Profile: Los Primos

Tell us about the athletes on the Los Primos team.

Los Primos is a diverse tapestry of cousins, spanning various age ranges and stages in life, united with a common purpose to conquer the Chicago Marathon. We come from different walks of life and are engaged in various professional fields, but we share a collective commitment to both personal achievement and charitable endeavors.

Tell us a little bit about Kathlyn, your NF Hero.

Kathlyn was diagnosed with NF as a newborn. Kat had a tumor under her spine from birth and in 2013 doctors removed it. They had to cut a piece of her spine out, remove the tumor, and fuse her spine back together. She was up and walking within 48 hours.

When Kat was graduating high school, educators at her school told her that she would never hold a job due to struggling with school and her learning disabilities due to NF. But Kat never hesitated. She has since graduated from college with a degree in Early Childhood Education and is currently a kindergarten teacher working on her master's degree. She has always wanted to teach early childhood and is thriving in the school she is currently working at.

Kat currently has a tumor on her brain that doctors have been monitoring for a while and although she struggles with pain on a daily basis, she continues to show up to work day in and day out without wanting any pity on her. We are inspired daily by her tenacity and perseverance in every aspect of her life.

Why did you choose to fundraise with the NF Endurance team?

Running the Chicago Marathon offers a unique blend of personal challenge and community impact. Beyond a personal achievement, we chose this marathon as a platform to raise awareness and funds for NF. The Chicago Marathon's rich history and diverse atmosphere make it an enticing challenge for those seeking both individual accomplishment and a chance to contribute to a greater cause. It is also a fun way to be competitive and bring our family together for a good reason!

What's one (other) thing that you want people to know about Kathlyn?

Kat is a Swiftie and loves concerts. She's also a fan of the Chicago Cubs and the Pittsburgh Steelers. She has four dogs that she loves to play with!

The Los Primos Team: Ethan, Tomas, Christian, Paul (back row), Eva and Emi (front row)



ROCK WITH US!

Rock 'n' Roll's reputation for staging lively, party-starting races is unparalleled. Join the NF Endurance team at one of the following events and tour your destination in a whole new way.

Rock 'n' Roll San Diego - June 1-2

- Distances: Marathon, Half Marathon, 5K

Rock 'n' Roll San Jose - October 5-6

- Distances: Half Marathon, 10K, 5K

Rock 'n' Roll San Antonio - December 7-8

- Distances: Marathon, Half Marathon, 10K, 5K

2024 NF ENDURANCE EVENTS

Rock 'n' Roll Nashville
Apr 27 & 28

Flying Pig Marathon
May 3-5

TD Five Boro Bike Tour
May 5

Denver Colfax Marathon
May 18-19

Rock 'n' Roll San Diego
Jun 1-2

BMW Berlin-Marathon
Sept 29 - SOLD OUT

Rock 'n' Roll San Jose
Oct 5-6

Bank of America Chicago Marathon
Oct 13 - SOLD OUT

TCS New York City Marathon
Nov 3 - SOLD OUT

Rock 'n' Roll San Antonio
Dec 7-8

For more information on any NF Endurance events, please visit nfendurance.org or reach out to Lydia Vanderloo at nfendurance@ctf.org

Make NF Research Visible: NF Registry

The NF Registry allows people living with all forms of NF to take an active role in research for better treatments. Joining the Registry is as simple as filling out a survey once each year about your symptoms and experiences.

The Registry now has over 11,000 participants. This is terrific—but 11,000 represents a very small percentage of the world's population of individuals living with NF. The more people who participate, the greater the impact will be, and the more we learn about the many ways NF impacts patients from around the world.

“I'm very interested in learning more about the patient perspective – how they cope with their diagnosis and how they understand their NF. The NF Registry has been really helpful for me in doing survey-based research to reach many patients across the country. Without the NF Registry, I wouldn't be able to do the studies that I've done so far.”

— KARA ANSTETT, MS, CGC
Clinical Genetic Counselor
at NYU Langone Health
Comprehensive
Neurofibromatosis
Center

*pictured here with
Jennifer Berube,
NF patient*



Join the NF Registry or update your existing profile this May at nfregistry.org

IN TRIBUTE

Celebrating the Legacy of Lynne Ann Courtemanche Shapiro, CO-FOUNDER OF THE CHILDREN'S TUMOR FOUNDATION



It is with heavy hearts that we share the news of Lynne Ann Courtemanche Shapiro's passing. Lynne was one of the original co-founders of the Children's Tumor Foundation in 1978 (known then as the National Neurofibromatosis Foundation). Our heartfelt condolences go out to her family, loved ones, colleagues, and friends as we remember

Lynne and the impact she made.

Lynne embarked on her extraordinary mission at a time when pervasive uncertainty and misunderstanding clouded the landscape for those affected by NF. In the face of her own diagnosis, she refused to succumb to despair. Instead, she turned adversity into advocacy, sparking a beacon of hope where none seemed to exist.

The genesis of the National Neurofibromatosis Foundation—now known as the Children's Tumor Foundation—is a testament to her perseverance. It emerged from a simple yet profound question: “Is there a group dedicated to people like me?” Undeterred by the absence of such a lifeline, she resolved to create it herself. Alongside her trusted physician Allan Rubenstein, MD, and the dedicated attorney Joel Hirschtritt, she forged the Foundation's humble beginnings, laying the cornerstone for what would become a bastion of knowledge, empowerment, and community.

We honor Lynne by continuing to support one another, advocating for research and awareness, and striving to make a positive difference in the lives of all those affected by NF.

To read more about Lynne Ann Courtemanche Shapiro, search “Lynne” at ctf.org/news

NF2-SWN Accelerator Meetups



CTF's NF2-SWN Meetups bring the community together to connect and hear from leading experts in the NF2-SWN space.

For more information about how to join these interactive and informative sessions, email us at nf2@ctf.org

Upcoming NF2-SWN Meetups

- May 22 - NF2-SWN Awareness Day
- August 22
- November 14

Find all the NF2-SWN Accelerator Meetup recordings on our NF2-SWN Education playlist, which can be found in the videos section of our Resource Library at ctf.org/education.

2024 Global NF Conference Brussels, Belgium June 20-25, 2024

Hosted by CTF Europe, the 2024 NF Conference is organized by the Children's Tumor Foundation and the European NF Group and will take place June 20-25, 2024 in Brussels, Belgium. The 2024 Global NF Conference is the most important annual gathering of the NF research and clinical communities, focused on drug development and improved outcomes for patients living with all types of neurofibromatosis and schwannomatosis. This worldwide event is attracting over 1,000 participants across various scientific disciplines and a critical forum for advancing basic, translational, and clinical research in NF.

The Opening Address at the Global NF Conference will be delivered by Nathalie Moll, Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA). Ms. Moll has spent 20 years working for the biotech industry at EU and national level associations and corporations. In 2013, she was awarded the Technovisionaries Women Innovation Award organized by Women & Technologies™ and was also named one of 15 leading women in biotech in Europe in 2017. Moreover, CTF Europe Board member Magna Chlebus, Executive Director for Regulatory and Science of EFPIA, will have a fireside chat with Dr. Niklas Blomberg, the newly appointed Executive Director of the Innovative Health Initiative (IHI).

Learn more at
ctf.org/nfconference

ORGANIZED BY:



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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
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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 conditions that cause tumors to grow on nerves. NF refers to all types of neurofibromatosis and schwannomatosis, which collectively affect 1 in every 2,000 individuals. These conditions 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.

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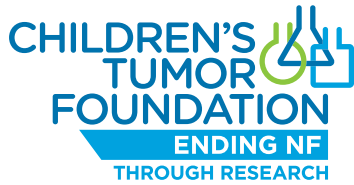
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Philadelphia, PA 19170 - 0001

Support the Children's Tumor Foundation's campaign to Make NF Research Visible with a new T-shirt that celebrates NF experts!

NF-ologists include every member of our vibrant NF community: researchers, healthcare professionals, educators, caregivers, families, and every individual living with NF, demonstrating strength and resilience in the daily battle against NF.

ctf.org/store

