NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION

WINTER 2024

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

The Children's Tumor Foundation's annual patient and family conference, the NF Summit, was held April 11-13, 2024 in San Antonio, Texas. The NF Summit brings together NF patients and families, volunteers, event organizers, researchers, clinicians, patient advocates, friends, and supporters. Volunteers came together from around the country to celebrate those who have made a significant impact by raising awareness for NF.

Read more about the NF Summit on page 14.

INSIDE:

Volunteer of the Year Gwen Coverdale at the 2024 NF Summit.

FROM the CEO

Annette Bakker, PhD

As we near the close of a great 2024, I want to take a moment to reflect on the powerful strides we've made together and to express my deepest gratitude for your unwavering support. At the Children's Tumor Foundation (CTF), we are on a mission to deliver life-changing treatments for all types of neurofibromatosis and schwannomatosis (NF). This year, your involvement has driven that mission forward in ways that inspire hope and produce life-changing results.

With your support, CTF is NF's drug discovery engine. From the lab to the clinic, we unite patients, doctors, scientists, and industry leaders to ensure the accelerated development of treatments that improve lives today and pave the way for tomorrow's breakthroughs.

More life-changing NF treatments are on the horizon! We keep our fingers crossed for FDA approval of mirdametinib, a treatment poised to expand options for adults with NF, thanks to CTF's pioneering work to establish SpringWorks Therapeutics. In addition, we published in the *New England Journal of Medicine* that brigatinib showed very encouraging results in our first-ever *NF2*-SWN platform clinical trial. Our partner, Healx, is ready to start their clinical trial with the first-ever AI-discovered drug for NF.

These milestones, along with the hard work of our Clinical Care Advisory Board to continuously improve care, the excellent results of the first multi-institutional NF biomarker study to detect cancer early, and the growth of our Global NF Conference and NF Summit, underscore the transformative impact you've made possible. It's not just about research; it's about life-changing treatments for patients.

As we reflect on all that's been achieved, I invite you to continue contributing to this vital work through our year-end **GIVE** campaign. Your generosity has been the catalyst for change, and as we look ahead, there's so much more we can accomplish together.

Whether through your involvement in Shine a Light NF Walk, running in support of Cupid's Undie Run, competing in NF Endurance marathons, or directly contributing to our efforts, you play an integral role in this community's success. Thanks to you, we continue to make remarkable strides toward a future without NF.

With shared determination,

Annette Bakker, PhD
CEO, Children's Tumor Foundation

IN THE NEWS:

PUBLICATIONS FROM CTF CEO Annette Bakker

In a recent interview with *PharmaPhorum*, Annette Bakker discussed the game-changing potential of drug repositioning. In June, the *Chicago Tribune* published an op-ed from Bakker about patient tissue and how those providing their biomaterial deserve input on how it is used. Read more about these publications at ctf.org/news

Welcome New Board Member

Laura Grannemann

Board of Directors

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

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CTF is the DRUG DISCOVERY ENGINE for NF

The Children's Tumor Foundation (CTF) is the drug discovery engine for NF. By bringing together patients, doctors, scientists, and pharma, we drive treatments, advance care, and deliver faster results for millions affected by neurofibromatosis or schwannomatosis. Our patient-first collaborative approach accelerates drug development and brings life-changing therapies to patients faster – driven by our mission to end NF.

The Impact of Your Donations

First FDA-Approved Treatment for NF1: Koselugo

CTF's research investment proved that MEK inhibitors shrink NF tumors. We spearheaded the first-ever FDA session on NF, building critical support for FDA approval.

Second NF1 Treatment Awaiting Approval: Mirdametinib

CTF helped identify and reposition this drug for NF, leading to its transfer to SpringWorks, a company spun off from Pfizer.

Topical Treatment for cNF : NFX-179

CTF's early impact investment could soon lead to a gel for cutaneous neurofibroma (cNF) skin tumors.

Breakthrough for *NF2*-SWN: Brigatinib

CTF-funded team uncovered and fast-tracked this potential treatment in the first NF platform clinical trial, which is showing great promise.

First AI-Driven NF Drug: HLX-1502

CTF's investment accelerates the development of Healx's drug, which has been granted Fast Track designation by the FDA.

This progress is powered by you.
Discover more at ctf.org/impact

First NF1 Biomarker Study

CTF-funded blood test shows promising potential to detect early signs of NF1 tumors that could become malignant.

Triple the Number of Clinical Trials

CTF's leadership and research funding have expanded the NF field, boosting clinical trials from 21 a decade ago to 68 today, unlocking new possibilities for treatment and care.

20+ Companies in the NF Space

CTF catalyzed unprecedented interest in NF, transforming a once-overlooked field into a hub that now attracts over 20 pharmaceutical and biotech companies.

1,000+ Experts at the NF Conference

Organized and led by CTF, this 'must-attend' global gathering for NF research drives innovation and collaboration among scientists, clinicians, and industry leaders.

NF Clinic Network: 20,000 Patients Annually

CTF established the NF Clinic Network to ensure access to high-quality NF care, now spanning over 70 locations across North America.

Global Patient Registry with 11,000 Patients

CTF established the NF Registry, a vital resource accelerating clinical trial recruitment and driving breakthroughs in NF research.

$1.7 Million Research Study Propels Schwannomatosis Research

The **Children's Tumor Foundation** and **Dolphins Challenge Cancer** (DCC) have partnered to launch a $1.7 million research initiative at the **Sylvester Comprehensive Cancer Center**, University of Miami Miller School of Medicine, to advance understanding and treatment of schwannomatosis. This collaboration reflects a solid commitment to innovative research and patient care.

Recognizing the urgent need for effective treatments, this partnership seeks to unravel the complexities of schwannomatosis through groundbreaking research led by Antonio Iavarone, MD, a distinguished pediatric neuro-oncologist and Deputy Director of Sylvester Comprehensive Cancer Center. The study will leverage state-of-the-art technologies and methodologies to explore the interplay between tumor cells and their microenvironment, utilizing advanced single-cell analyses to better understand the role of different cell types in schwannomatosis challenges.

"We have kicked off a partnership that dreams are made of," said Gabriel Groisman, Board Chair of CTF. "The collaboration between CTF, DCC, and Sylvester Comprehensive Cancer Center to fund groundbreaking NF research is a perfect match that will benefit millions living with NF."

CTF has a proven track record in driving collaborative research initiatives, including programs like Synodos, which has led to significant advancements, such as the first FDA-approved drug for a subset of NF patients and a tripling of ongoing clinical trials in the past decade. This new study builds upon the insights gained from CTF's previous $1 million schwannomatosis-focused Synodos study, which highlighted the distinct roles of individual cells in schwannomatosis challenges.

Dr. Iavarone's expertise in cellular tumor composition, including NF low-grade gliomas, positions him to apply innovative approaches to understanding schwannomatosis and its implications for other NF variants.

This pioneering research not only aims to benefit schwannomatosis patients but also holds potential for enhancing pain management in all NF patients and providing insights into other forms of neurofibromatosis and schwannomatosis.

We extend our heartfelt gratitude to CTF Board Chair Emeritus Richard Horvitz and Erica Hartman-Horvitz for their generous support of both the previous Synodos study and this new partnership. Their commitment to advancing NF research is invaluable and deeply appreciated.

Healx receives investment from CTF to advance NF1 treatments

The Children's Tumor Foundation's support of the NF1 programs at Healx is accelerating progress! We are happy to share that Healx's lead compound, HLX-1502, has been granted Fast Track designation by the Food and Drug Administration (FDA) for the treatment of NF1. This recognition affirms the critical patient need for new therapeutic solutions in NF1 and opens an expedited regulatory pathway, helping bring the HLX-1502 drug candidate to patients more swiftly.

The Fast Track designation is awarded by the FDA to facilitate the development and review of drugs that treat serious conditions and fill unmet medical needs. Ultimately, it helps streamline the path to patients, ensuring more regulatory support and reducing time to marketing approval.

Healx partnered with CTF in 2020 to combine its cutting-edge AI platform and drug discovery expertise in identifying novel treatments for rare diseases such as NF1 with CTF's preclinical and clinical research and patient knowledge.

Tim Guilliams, PhD, co-founder and CEO at Healx, said in 2020: "CTF is a long-standing partner of Healx, and this investment underscores their confidence in the potential of Healx's neurofibromatosis program to bring novel treatments to patients with unmet need."

Simone Manso, Head of NF Strategic Partnerships at Healx, leads the collaborative drug discovery efforts and serves as a member of the Board of Directors of CTF Europe and CTF.

Brigatinib Shows Promise for *NF2*-SWN: A Direct Result of CTF's Synodos Initiative

A landmark study published in the *New England Journal of Medicine* has revealed promising results for the use of brigatinib in treating *NF2*-SWN. This breakthrough is a direct outcome of the Synodos for NF2 research initiative spearheaded by the Children's Tumor Foundation, highlighting the power of collaborative research. A multi-institutional global research team, established and funded by the Children's Tumor Foundation, found promising evidence that brigatinib can help shrink *NF2*-SWN patients' tumors. Using a highly innovative phase II platform-basket trial design, the multi-center team found that brigatinib shrunk 10% of growing tumors and 23% of all tumors.

Read more at ctf.org/news

2024 **Drug Discovery Inititative** (DDI) **Awardees**

The Children's Tumor Foundation is pleased to announce a significant investment of more than $995,000 in Drug Discovery Initiative (DDI) Awards focused on drug discovery for the most challenging NF manifestations.

LJUBICA CALDOVIC, PhD
Children's Research Institute (CNMC)

Targeting PRMT5 in MTAP-Deleted NF1 High Grade Gliomas

NF1 is characterized by mutations in the *NF1* gene, which drive tumor growth by increasing Ras/MAPK pathway activity. While low-grade gliomas (LGGs) are common in children with NF1, these can transform into high-grade gliomas (HGGs) during adolescence, leading to poor survival outcomes. One frequent genetic mutation in NF1 HGGs is the deletion of the *MTAP* gene, found in 90% of cases. *MTAP* loss results in vulnerability to protein methyltransferase 5 (PRMT5) inhibitors. This project aims to test the efficacy of PRMT5 inhibitors, both alone and in combination with MEK inhibitors, in treating *MTAP*-deleted NF1 HGG cells. Initial studies suggest that PRMT5 inhibitors are more effective in *MTAP*-deleted cells and work synergistically with MEK inhibitors.

KIMBERLY OSTROW, PhD
Johns Hopkins University School of Medicine

GsMTtx-4 as a Novel Inhibitor to Block Non-NF2-SWN Pain

SWN frequently causes multiple painful tumors along major nerves, with limited treatment options available. Surgical removal is often impractical, and current pain management relies on trial-and-error medication approaches. This project seeks to investigate the use of GsMTx-4, a protein that has shown promise in blocking pressure-sensitive ion channels that trigger pain. Preliminary research has demonstrated that GsMTx-4 can reduce pain sensitivity in animal models, and this project aims to explore its efficacy in treating SWN-related pain. The research will evaluate whether GsMTx-4 can block the resulting pain sensitivity by using tumor-secreted products from painful schwannomas.

DAOCHUN SUN, PhD
The Medical College of Wisconsin

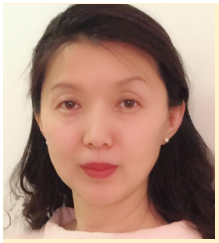
Repurposing Montelukast to Treat Plexiform Neurofibromas in Combination with Selumetinib

Selumetinib is the only FDA-approved drug for treating pediatric plexiform neurofibromas (PN), but its side effects and long-term safety raise concerns. This study proposes repurposing montelukast, an FDA-approved asthma drug, to reduce the required dose of selumetinib and enhance efficacy. Montelukast affects immune cells, specifically macrophages, which are abundant in the tumor microenvironment of NF1. Preliminary data indicate that montelukast can inhibit tumor-promoting macrophages while also reducing tumor cell growth. The study aims to test the combination of montelukast and selumetinib in cell lines and mouse models, exploring the impact on tumor growth and macrophage activity.

KEILA TORRES, MD, PhD
University of Texas M.D. Anderson Cancer Center

In Vivo Assessment of BET Blockade Combined with PARP Inhibition to Sensitize MPNST to Radiation Therapy

Malignant peripheral nerve sheath tumors (MPNSTs) are aggressive, highly lethal tumors that affect up to 15% of NF1 patients. These tumors are resistant to chemotherapy and radiation, leaving few effective treatment options. This project explores a combination approach using BET inhibitors, which reduce DNA repair gene expression, and PARP inhibitors, which block DNA repair signaling. Both drugs have shown some efficacy in slowing tumor growth in MPNST models, but neither fully stops progression. The hypothesis is that combining these inhibitors with radiation therapy will enhance tumor cell death by impairing the tumor's ability to repair DNA damage.



LEI XU, MD
Massachusetts General Hospital

Targeting HIF-2 for the Treatment of NF2-SWN Vestibular Schwannoma

Patients with *NF2*-related schwannomatosis (*NF2*-SWN) often develop vestibular schwannomas, which can lead to hearing loss and significant tumor growth. Bevacizumab, an anti-VEGF therapy, has been a primary treatment, but some patients either cannot tolerate its side effects or experience tumor progression despite its use. This project investigates the use of belzutifan, an FDA-approved HIF-2 inhibitor, as a potential treatment for *NF2*-SWN. Since vestibular schwannomas exhibit abnormal blood vessel growth and low oxygen levels, belzutifan may help inhibit these processes. The research will test belzutifan in a mouse model of schwannomas, comparing its effectiveness to bevacizumab and evaluating its ability to rescue tumors that are resistant to anti-VEGF therapy. The ultimate goal is to identify new combination therapies that could prevent tumor growth and hearing loss in *NF2*-SWN patients.

YOUNG INVESTIGATOR AWARDS 2024

The Children's Tumor Foundation is excited to announce a substantial commitment of over $888,000 in research focused on NF1, *NF2*-SWN, and *LZTR1*-SWN. These funds will be distributed through CTF's Young Investigator Awards (YIA), a grant program designed to support pioneering research by early-career scientists and clinicians.

ISAM NABER, MD

University of California, Los Angeles

Proteome Analysis of Inner Ear Fluids in NF2 Mouse Models with Hearing Loss

NF2-related schwannomatosis (*NF2*-SWN) often leads to gradual, irreversible hearing loss, but the degree of hearing loss does not always correlate with vestibular schwannoma growth. Also, vestibular schwannoma patients have a buildup of precipitated protein in their inner ear, but its significance is not understood. Using two different mouse models, both of which experience hearing loss but show differences in inner ear protein buildup and schwannoma development, this study will investigate hearing loss associated with vestibular schwannoma. Examining the proteins detected in the two mouse models could identify potential biomarkers linked to and the mechanism behind hearing loss in *NF2*-SWN vestibular schwannoma patients.

PERNELLE PULH, PhD

INSERM, France

Identification and Functional Validations of Actionable Targets for Prevention or Treatment of Cutaneous Neurofibromas and Characterization of NF1 Healthy-Looking Skin

Almost all NF1 patients develop non-cancerous tumors called cutaneous neurofibromas (cNFs) in the skin. These tumors cause significant cosmetic burden, and currently, there is no treatment to prevent or reverse their development. This study will identify the proteins overexpressed in growing and mature cNFs and validate them as targets for the prevention or treatment of cNFs. The project's secondary goal is to analyze the impact of a double mutation of *NF1* that occurs during embryonic development long before the development of cNFs. This will help to understand better the cNFs-related heterogeneity seen in NF1 patients.

GEORGIA DARAKI

Leibniz Institute on Aging, Germany

Exploring the Interplay Between Lipid Metabolism and LZTR1 in Peripheral Nerve Pathologies

Patients with *LZTR1*-related schwannomatosis (*LZTR1*-SWN) experience higher levels of pain compared to schwannomatosis patients with other pathogenic variants, but the association between *LZTR1* gene and neuropathic pain is poorly understood. Preliminary studies indicate a role for *LZTR1* in lipid metabolism, with the deficiency leading to problems with fatty acid metabolism and composition, affecting the protective myelin sheath around nerves. This research will investigate the mechanism of pain development due to *LZTR1* loss, the role of *LZTR1* in lipid metabolism, and the effects of *LZTR1* deficiency in peripheral nerve disease.

ALEXIS STILLWELL

Pennington Biomedical Research Center

Developmental Analyses of Skeletal Manifestations in "Mild" NF1 Patient Mutation p.M992del in Knock-In Mouse Model

NF1 is characterized by a wide range of clinical manifestations, some of which are very severe complications while some others may be mild. One such mild manifestation is due to a single amino acid deletion at position 992 in the neurofibromin protein. Patients with this deletion (p.M992del) are clinically described as having Noonan-like phenotype due to short stature, scoliosis, heart defects, and abnormal chest wall development, along with learning disabilities and cognitive impairment. Using a novel mouse model recapitulating the "mild" p.M992del *NF1* gene variation, this study will study how bone cells and their precursors are affected due to this variation and will tease out the pathways disrupted to more thoroughly understand how skeletal defects happen in NF1 patients.

ANNA NAGEL, PhD
University of Central Florida

Deciphering Crucial Cell Death Pathways in NF2-SWN

Histone deacetylase (HDAC) inhibitors are being investigated as therapeutic agents for *NF2*-related schwannomas. This study aims to understand how a dual HDAC/PI3K inhibitor, CUDC-907, induces apoptosis of *NF2*-related schwannomas. Because HDAC inhibition affects many cell processes that can lead to adverse drug effects, understanding the mechanistic details can help identify novel targets or safer drug combinations for *NF2*-related schwannoma therapy.

ALEX DYSON, PhD
Massachusetts General Hospital

Genetic and Molecular Investigation of the Neuronal Functions of NF1

Neurofibromatosis type 1 is often associated with neurological complications like learning difficulties, ADHD, and autism. However, precisely how *NF1* gene variations affect brain development and activity to cause these issues is poorly understood. Using a Drosophila (fruit fly) model of NF1, this study aims to identify the regions of neurofibromin required for its interaction with other proteins involved in brain cell function, how disrupting these interactions results in behavioral changes, and how these changes can be improved by administering small-molecule drugs.

RAMYA RAVINDRAN
Cincinnati Children's Hospital Medical Center

Pathways that Drive Inflammation and EMT in Schwann Cells after NF1 Loss

The development of plexiform neurofibromas in NF1 is marked by the activation of inflammation-associated pathways in Schwann cells and by the increased presence of markers of a cellular differentiation process called epithelial-to-mesenchymal transition (EMT). This study will test the hypothesis that *NF1* loss in Schwann cells activates the NF-kB inflammation pathway to cause EMT, which promotes plexiform neurofibroma development. This study will also test if inhibiting the NF-kB pathway affects plexiform neurofibroma formation in vivo.

SARAH MORROW
Indiana University

Investigating the Role of ZNF423 in NF1-Related MPNST

The leading cause of death in NF1 patients is the development of malignant peripheral nerve sheath tumors (MPNST). These tumors are rare and highly aggressive and can arise from non-cancerous growths called plexiform neurofibromas (PNs). When tumors progress from PN to MPNST, levels of a protein known as ZNF423 markedly increase, indicating that ZNF423 may be vital for MPNST survival. Experimentally reducing the levels of ZNF423 in MPNST cells significantly reduced their growth. Hence, this study aims to analyze further how ZNF423 contributes to the growth and survival of MPNST to uncover novel, druggable targets or therapeutic strategies.

Fall Fundraising Events

2024 NATIONAL GALA

On Monday, November 18, 2024, the NF patient community gathered with leading philanthropists, medical experts, civic leaders, and businesses at Gotham Hall in New York City for our annual National Gala. For nearly fifty years, CTF has driven remarkable progress in NF research and clinical care. We were proud to celebrate this progress at this year's Gala.

Kimberly Snipes was recognized with a Humanitarian Award for her outstanding contributions to the Children's Tumor Foundation. Her daughter Madison was diagnosed with NF1 at the age of four, and despite the challenges, Kimberly has remained resilient in her commitment to supporting NF research and raising awareness. As a member of the CTF Board, she has played a vital role in advancing therapies for NF patients, combining her passion as a parent with her expertise as a Chief Information Officer. Her dedication has made a lasting impact on the CTF mission.

Sarah Wengel was also honored with a Humanitarian Award. Sarah has supported CTF since 2015, when she first started volunteering for Dancing With Our Stars in Little Rock, Arkansas. She served first as a Star, then as a judge, and most recently on the Arkansas Advisory Board of Directors. Her involvement and leadership in Little Rock helped establish the funds needed to open the Adult NF Clinic at UAMS Winthrop P. Rockefeller Cancer Institute, providing much-needed multidisciplinary care to adult NF patients since officially opening in 2022.

Leanna Scaglione was named the Foundation's **2025 National Ambassador.** This award is bestowed upon a courageous individual living with NF and recognizes their efforts to further the Foundation's goals of research, public awareness, and patient support. Leanna lives with *NF2*-SWN and joined the CTF NF Endurance Team in 2022. She has since represented CTF in numerous on-camera media appearances and live events.

We were honored to recognize **Allison and Kip Clarke** and celebrate the legacy of their son Quinn, who passed away in 2021 following his third battle with cancer, with a **Cloud Carrier Award.** Allison, founder of Flashes of Hope, produced "Big Shots Little Stars," a popular Cleveland fundraising event that, over the years, raised millions of dollars to fund critical research into malignant peripheral nerve sheath tumors.

The evening also acknowledged the **CTF Junior Board**, a group of young professionals impacted by NF whose youthful energy and unwavering passion advance the CTF mission.

Award-winning songwriters **Kristen Anderson-Lopez and Robert Lopez** gave a delightful live performance. The songwriting duo are best known for co-writing the songs for the animated musical film *Frozen*, its sequel *Frozen II*, and the animated musical film *Coco*. Robert and Kristen's cousin, Markus, lives with *NF2*-SWN.

Our gratitude to our esteemed group of Gala Co-Chairs who made the evening a resounding success: Erica Hartman-Horvitz, Tila Falic Levi, Liz Rodbell, and Clara Wilpon. The evening's program was live-streamed and can be found on the CTF YouTube channel.

BENEFIT XII

NF Forward's signature event is the ***beNeFit***, a gala held at Huntington Place in downtown Detroit to support the innovative research of NF organizations, including the Children's Tumor Foundation. This year, ***beNeFit XII*** took place on November 23, with a theme of *A Moody Masquerade to Cure NF*.

Since 2013, this festive event has raised nearly $70 million for NF research and attracted more than 1,700 supporters annually to join the fight to end NF. Our continued thanks go to longtime CTF supporters Dan and Jennifer Gilbert, passionate advocates in the fight to end NF. Read more about this year's ***beNeFit*** at **ctf.org/news**.

Dancing With Our Stars

Mady Donoff, R. Taylor Sundby, MD, Richard Soll, Leslie Kates, and Shannon Chandley at the Boston Reception

DANCING WITH OUR STARS

After 17 remarkable years, Dancing With Our Stars Little Rock graced the stage for its grand finale, a night filled with heart, hope, and celebration. Six exceptional community leaders participated in a spirited dance competition, raising an astounding $300,000 in support of NF research. An audience of 500—including family members, friends, devoted supporters, and most importantly, our NF families—gathered to enjoy this unforgettable evening of entertainment and generosity.

We extend our heartfelt congratulations to Austin Booth, crowned Champion of the 17th Annual Dancing With Our Stars, and Monica Alexander, whose dynamic performance earned her the Best Performance Award.

While we bid a fond farewell to this cherished event in Little Rock, we are thrilled to announce one final DWOS celebration in Northwest Arkansas in March 2025. We warmly invite you to join us for this encore event as we continue to drive critical research. CTF's mission to discover effective treatments for NF is powered by the incredible support of the Arkansas NF community, and together, we will keep dancing toward a cure. For more information about this not-to-miss event, go to **ctf.org/dwosnwa**

HALLOWEEN BASH

The 19th Annual Halloween Bash lit up the night on October 19th, continuing its tradition of raising vital funds for CTF and raising over $110,000. Hosted each year by the Thoms family in honor of their daughter Camille, who bravely faces *NF2*-related schwannomatosis, this beloved event again brought the community together for an evening of fun and philanthropy. Our deepest gratitude goes to Roland and Nicole Thoms of Varsity Painting, whose dedication, alongside their family, has made the Halloween Bash a tremendous success year after year. Special thanks to the Varsity team of friends, employees, and customers, whose unwavering support has fueled critical research to develop treatments for those living with NF. Together, this community has made a profound impact.

ANNUAL CELEBRITY POKER TOURNAMENT

On October 24th, CTF hosted its Annual Charity Poker Tournament in partnership with Poker4Life, bringing together nearly 100 players for a night of competition and camaraderie. With over $100,000 raised in support of NF research, the evening was a resounding success. The coveted grand prize—a $10,000 seat at the World Series of Poker Main Event—was awarded to Zack Marshak.

The tournament attracted several celebrity participants, including Mark Feuerstein, Jonathan Sadowski, Josh Brener, Gina Hecht, Phil Gordon, Andy Buckley, and Ben Shenkman.

We extend our heartfelt appreciation to our sponsors, the Nimmons Family and Alex Elsik, whose generous support helped make this event possible.

BOSTON DONOR RECEPTION

On September 24th, the Boston community came together for the third consecutive year at Season's 52 in Chestnut Hill, raising an impressive $130,000 in support of CTF. The evening was filled with engaging conversation, cocktails, and a compelling panel discussion that brought NF research to the forefront. R. Taylor Sundby, MD, from the National Cancer Institute and Ben Guikema from Alexion AstraZeneca Rare Disease shared essential updates on NF research, including insights into the ongoing MPNST project funded by CTF. The discussion was expertly moderated by Kim Robinson, CTF Individual Giving Officer.

We extend our sincere thanks to our gracious host and the dedicated planning committee—Shannon Chandley, Mady Donoff, Leslie Kates, Stacy Kates Levy, Lisa Utzinger Shen, Judy Shwachman, CTF Board Member Richard Soll, and Carol Walsh—whose efforts made the evening a true success.

We extend our deepest gratitude to the donors, attendees, participants, and organizers of these recent events. Whether through dancing, poker, or coming together to share knowledge, every contribution helps drive our mission forward. Thank you for being an essential part of our progress.

Lab Tour: Cincinnati Children's Hospital

The Children's Tumor Foundation was proud to host a group of patients and families affected by NF for a close-up view of the groundbreaking research happening at Cincinnati Children's Hospital. Thanks to the hospitality of Nancy Ratner, PhD, and Elliott Robinson, MD, PhD, six patient representatives from CTF Engage and two local members of the NF community toured two NF research labs.

From left: Judy Hall, Matt & Stephanie Reeve, Dr. Elliott Robinson, Michael Peper, Jessica Samblanet, Micki Cole, Nancy Ratner, PhD, Betty Schorry, MD, Jay Pundavela, PhD, and Caitlin & Theo Ottesen

The day began with presentations from Dr. Ratner and Dr. Robinson to provide an overview of their work at Cincinnati Children's Hospital. The group was then given a behind-the-scenes tour of Dr. Ratner's and Dr. Robinson's labs, where we heard from Avery Volz, a student in the Robinson Lab, and Robert Hennigan, PhD, an institute investigator in the Ratner Lab. This allowed everyone to learn about and interact with the high-tech equipment researchers are using to better understand NF.

The group reconvened to hear presentations from Jay Pundavela, PhD, Associate Staff Scientist in the Ratner Lab, Dr. Robinson, and Dr. Hennigan. Presentations focused on how their early-stage basic research might one day lead to clinical applications for those living with NF.

To learn more about the CTF Engage program, reach out to **info@ctf.org**

NFCN Welcomes New Clinic

CTF's NF Clinic Network (NFCN) welcomes the **UF Health Neurofibromatosis Clinic** at the University of Florida, Jacksonville, of the Jacksonville Health Science Center Clinic to the NFCN. Clinic Director Pam Trapane, MD, also serves on the Children's Tumor Foundation Clinical Care Advisory Board.

The NFCN was established by CTF to standardize and raise the level of clinical care for NF and integrate research into clinical care practices. The network now includes 74 NF Clinics that have demonstrated their ability to provide specialty care to NF patients and their families. To learn more, go to **ctf.org/doctor**

LEGACY GIFT: Melissa D. Skyer

CTF is deeply honored and grateful to receive a legacy gift from the late Melissa Skyer, who passed away in 2022, just days after her 40th birthday due to complications from *NF2*-SWN.

Melissa lived her life with incredible passion and purpose. As an environmentalist, she was fiercely dedicated to protecting the planet. She earned both a Bachelor's in Biology and a Master's in Environmental Science from the Rochester Institute of Technology. During graduate school, she lost her hearing completely, but that didn't stop her from pursuing her dreams. She worked as an environmental scientist for five years in Chicago with Burns & McDonnell, and another five years with Southern California Gas Company, before moving back to Rochester. There, she dedicated her time

to teaching and tutoring deaf students in biology and environmental science—making a lasting impact on her students' lives.

Melissa was also a a talented artist and writer. She poured her creativity into beautiful glass mosaics, paintings, and drawings, and published two poetry collections.

She was a devoted daughter, sister, and a cherished friend to many. Despite the challenges of living with *NF2*-SWN, Melissa embraced life with resilience and strength. Her father and three of her aunts also lived with *NF2*-SWN, and she is survived by her loving mother and her brother, Michael, who also has *NF2*-SWN.

Melissa's generous legacy gift to CTF was made with the hope of accelerating a cure for *NF2*-SWN—a reflection of her unwavering spirit and her desire to make a difference for others facing this disease.

Our utmost thanks to Melissa and her family for their faith and generosity.

To learn more about leaving the Children's Tumor Foundation in your will or estate plan like Melissa did, please visit **ctf.org/legacy**, or reach out to us directly at **donorrelations@ctf.org**

Year End GIVING

give ctf.org
Children's Tumor Foundation

When Emmanuel's family first learned of his NF1 diagnosis, they were filled with questions and uncertainty. "We didn't know what to expect," his father, Henry, recalls. "As we learned more about NF, we realized that it might get worse with time, and that there was no cure."

Emmanuel had always dreamed of becoming a professional soccer player. But as a plexiform neurofibroma grew in his right hip, displacing organs and compressing his bone marrow, it became clear that his life would change.

The tumor causes him extreme pain, and he now uses a wheelchair much of the time. Walking is a daily struggle, and Emmanuel's dream of playing soccer has become a distant memory. The family traveled across several countries in search of treatment, facing robbery, discrimination, and even homelessness.

But last year, Emmanuel started taking Koselugo, the first FDA-approved treatment for inoperable plexiform neurofibromas, thanks to research driven by the Children's Tumor Foundation and supporters like you.

Since then, his tumor has softened, and Emmanuel's family **is filled with hope!**

CTF announced the FDA approval of selumetinib (now Koselugo) in 2020. Since then, it has been made available for thousands like Emmanuel who battle plexiform neurofibroma tumors.

But there is so much more to be done! That's why we need your help. Will you consider a gift today to help give children and adults like Emmanuel access to care and find new solutions to fight NF?

Emmanuel's story is one of resilience and gratitude for the CTF-initiated research that paved the way to drug approval—research made possible by you and others.

But this is just the beginning. Koselugo remains the only FDA-approved treatment for NF, and children like Emmanuel need more options. They shouldn't have to travel across countries to access care. With your help we can fight daily for faster treatments and more breakthroughs that will improve the lives of all who live with NF.

Thanks to your gifts, CTF is the drug discovery engine for NF, and, together, we are achieving significant milestones. Another treatment option has been submitted for FDA approval and a treatment for cutaneous neurofibromas is in the final phase of clinical trial.

Right now, over 60 clinical trials are underway, bringing us closer to more treatments for every type of NF. Among them is brigatinib, which is showing promise in the first-ever platform clinical trial for *NF2*-SWN patients. **None of this is possible without your commitment and support!**

Will you make a year-end gift today? Your donation will impact the lives of families like Emmanuel's and accelerate the search for more treatments, more breakthroughs, and more hope for millions around the world living with NF.

Please return the envelope, go to **ctf.org/give** or scan the QR code below with your phone.

Thank you for making a difference for millions like Emmanuel who live with NF.

Scan this QR code with your mobile phone to donate online.

Extraordinary Spirit: MELISSA BRUNNER

Melissa Brunner first encountered the Children's Tumor Foundation through a deeply personal connection: her nephew Owen, now 22 years old, was diagnosed with NF1 at age six months. Since then, Melissa has channeled her career and platform as a prominent media figure in Topeka, Kansas, into raising awareness and providing invaluable media coverage about NF for many years.

Throughout her years as a news anchor and station director at WIBW-TV in Topeka, Melissa has covered NF stories in her community, from highlighting the challenges of living with the condition to celebrating advancements in research. In 2023, she took on a national role by hosting our World NF Awareness Day Live event on May 17, highlighting the need for continued support and research.

In addition to her journalistic efforts, Melissa has taken on significant roles in key events for CTF. Most notably, she was the host of both the 2023 and 2024 Shine A Light NF Walks in Kansas City. This event has become a powerful tradition, with families gathering to not only raise funds but also celebrate the strength of the NF community. Melissa's leadership has made a lasting impact, helping to foster a sense of hope and connection among participants.

> "My hope for the next 10 years is that we don't have just one treatment, we have a handful of treatments. We have all of these tools in our arsenal to be able to attack NF."
>
> **MELISSA BRUNNER**

As someone who understands both the professional and personal impact of NF, Melissa advocates for the visibility of the NF community. As an invited panelist at CTF's 2022 and 2023 NF Summits, Melissa gave valuable insights to patients and families about the power of storytelling in spreading awareness. She was also pivotal in bringing the Shine a Light campaign to Topeka, which has lit the city's downtown plaza blue and green for NF awareness, having a powerful emotional impact on local families. "Seeing blue and green in our city meant the world to them," Melissa recalls. "One little boy now says he has to go see his lights every year."

Melissa is also driven by her nephew Owen's journey with NF. While Owen's tumors are not always visible, they have caused complications in his brain, impacting his quality of life and leading to seizures. "For Owen, it's not just about what you can see on the surface," Melissa explains. "His tumors are in his brain, causing significant issues with movement and pain." Yet, Owen's resilience and Melissa's drive to help others with NF have been a continuous source of motivation.

CTF is incredibly grateful for Melissa's unwavering dedication. Whether through her journalism, her leadership in key events, or her powerful personal story, Melissa continues to be an extraordinary advocate for NF awareness.

Awareness Month HIGHLIGHTS

NF Awareness Month

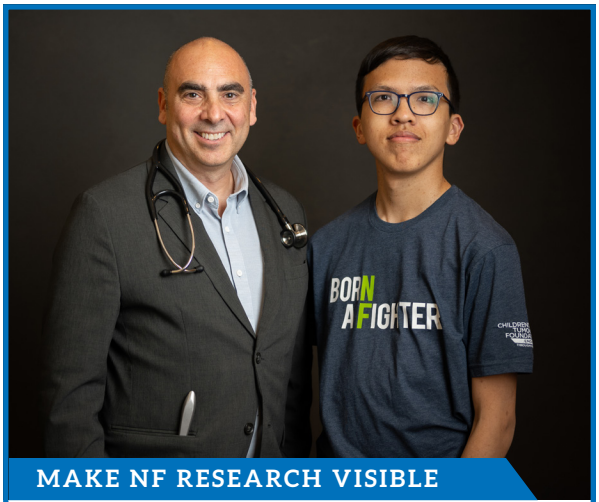
Each May, the Children's Tumor Foundation proudly leads NF Awareness Month, a dynamic global campaign where we join forces with you—our passionate donors and supporters—to Make NF Visible. Together, we're spotlighting NF and amplifying the voices of those living with it. On May 17, World NF Awareness Day, we celebrate the inspiring stories of individuals with NF while underscoring the urgent need for research to improve their lives.

Members of the CTF community were invited to ring the opening bell at the NY Stock Exchange.


Stone Bridge, Pennsylvania

SHINE A LIGHT ON NF 2024

More than 400 world-famous buildings, bridges, and architectural icons participated in this year's Shine a Light on NF campaign, showing their support in the global fight against NF by lighting up in blue and green, the official colors of the NF cause. Launched by CTF in 2014 to increase public awareness of NF, the Shine a Light on NF campaign has grown substantially. CTF partners with NF organizations, medical and research institutions, and corporate and media partners worldwide to expand awareness globally.



MAKE NF RESEARCH VISIBLE

During NF Awareness Month, CTF released the "Make NF Research Visible" portrait series, showcasing clinicians and researchers alongside patients to highlight how visibility drives progress in NF research. Raising awareness enhances access to funding, improves quality care, accelerates discoveries, and connects patients with clinical trials. From the lab to the clinic, making NF research visible transforms the lives of everyone affected by NF.

PROCLAMATIONS & EVENTS

Ten states and 23 cities officially recognized NF Awareness Month or NF Awareness Day on May 17 through formal proclamations. Throughout the month, the NF community came together, both online and in person, for family and community events, proudly wearing blue and green, sharing daily NF Facts, and posting videos and photos highlighting their NF journeys. Raising awareness is the crucial first step toward securing the funding needed for life-changing research. Thanks to our expanding partnerships with Lamar Advertising and other digital billboard networks, messages of NF awareness lit up 139 digital billboards across 34 cities nationwide.

For more information about NF Awareness Month and Make NF Visible, visit **makenfvisible.org**.

NF SUMMIT

The 2025 NF Summit will bring NF patients, families, and friends to Washington, D.C., from June 19-21 at the Omni Shoreham Hotel. While this will be the fourth annual NF Summit, long-time friends of CTF will remember when this annual event was called the NF Forum. Some may also remember that the very first NF Forum in 2009 was in none other than Washington, D.C. While many things have changed since then, we have remained steadfastly dedicated to creating an event that empowers all of you, the NF community, to advocate for yourselves and your loved ones throughout this NF journey and at every level of research and clinical practice.

What has changed since 2009?

- The first FDA-approved treatment for NF
- Improved and updated diagnostic criteria for all forms of NF
- The launch and rapid growth of the NF Registry, currently with over 11,000 members
- The establishment and expansion of the NF Clinic Network, now over 70 clinics strong

Our 2025 NF Summit Co-Chairs are Miriam Bornhorst, MD, and Carlos Romo, MD. Dr. Bornhorst recently joined the Comprehensive NF Clinic at Lurie Children's Hospital after many years with Children's National and brings decades of experience to our planning efforts. Dr. Romo is part of one of the largest comprehensive NF clinics in the country at Johns Hopkins University and has spoken at countless NF patient gatherings. We are excited to bring both of these dedicated clinicians to Washington, D.C.

2025 is promising to be another exciting year of advancing treatments and improving the lives of all those affected by NF. The NF Summit is the place to celebrate, connect, learn, grow, and get inspired as we continue to fight this fight to end NF together.

Learn more at nfsummit.org

Miriam Bornhorst, MD, receiving the Make NF Visible Clinician Award

2024 Make NF Visible Award Winners

The 2024 NF Summit celebrated the third annual Make NF Visible Community Recognition Awards. These awards celebrate individuals or groups who have made a significant impact by raising awareness for NF in their local community, nationally, or globally. CTF hosted a special ceremony at the NF Summit to honor and thank volunteers, clinicians, researchers, community partners, and patient advocates worldwide who are dedicated to making NF visible.

2024 Volunteer of the Year
Gwen Coverdale

Make NF Visible Researcher Awards
R. Taylor Sundby, MD
Nancy Ratner, PhD

Make NF Visible Global Reach Award
Jaishri Blakeley, MD

Make NF Visible Clinician Award
Miriam Bornhorst, MD

Young Leader Award
Brianna Worden

Community Advocate Award
Leanna Scaglione

Patient Advocate Award
Michael Peper

Corporate Champion Award
EOG Resources, Inc.

Fight NF Your Way Award
Christine Gallager

Community Connector
Sheila Drevyanko

Share the Love Award
Stoney Jesseph

Community Champion Award
George Thuronyi

NF REGISTRY

The NF Registry is a patient-driven resource for accelerating research and finding treatments for all forms of NF. It is the most efficient way to raise awareness, expand the NF community, and participate in research to end NF. Joining the NF Registry is easy, and will make an important difference.

Join today at nfregistry.org

"The NF Summit was an opportunity for my husband and me to learn about NF and connect with other families who just 'get it'... Hearing from professionals about research, medical knowledge, and NF warriors themselves gave us so much hope for our son."

stories OF NF

Abbi Evans *NF2*-SWN

I was diagnosed with *NF2*-SWN entirely incidentally at age 16. I went to my optometrist for an annual eye exam. She said my optic nerve looked slightly inflamed, so she referred me to a specialist. The specialist ordered MRIs of my brain and it turned out my eyes were fine, but I had three brain tumors! My family and I were shocked. I was then referred to a pediatric neurologist for follow-up care. The neurologist diagnosed me with [what was then called] NF2 as my scans showed that I had bilateral vestibular schwannomas and a meningioma. I had genetic testing to confirm the results. The neurologist ordered an MRI and the scans showed that I had a giant ependymoma inside of my thoracic spinal cord that was compressing it. Two days later I had surgery, and I was left with weakness, pain, fatigue, and loss of sensation in the lower half of my body.

In less than two months, I went from being a typical junior in high school to having a life-changing diagnosis and a spinal cord injury. I stayed in acute care for about a week, and then I was transferred to TIRR Memorial Hermann for in-patient rehabilitation. I stayed there for about five weeks doing intensive rehab to learn to walk again and do daily tasks.

As a result of my surgery, I lost sensation on my whole right side and proprioception on my left side from T8 down. I deal with pain and fatigue daily. I live scan to scan uncertain of what the future holds for me. However, this experience has taught me the value of living in the moment.

Before I was diagnosed, I planned to become a doctor and pursue a medical career. After my surgery and spinal cord injury, my fatigue and mobility issues made me realize that this was not the path for me. Instead, I chose to get involved in an undergraduate research lab where I discovered my passion for histology! Additionally, I had never really thought about having children before my diagnosis. Now that I have a 50% chance of passing this disease on, this is something I have to consider more in the future.

Each time I have been asked to share my story has been a defining moment in my NF journey. Sharing my experience and knowing I am encouraging others makes me realize that all of my pain was not for nothing!

Levi Spano NF1

Levi was diagnosed with NF1 at seven months old; we found out pretty soon after that he had several plexiform tumors surrounding his cervical spine, with one pressing into the spinal cord. He started on a MEK inhibitor when he was 11 months old and has been on one since (he's 4.5 now). Those early days right after the diagnosis consisted of a lot of doctor appointments, therapy appointments, blood draws, and MRIs. Levi spent more time around adults than he did kids his age.

Levi's life is impacted by having more doctor appointments than your average kid. He switched over to Koselugo a year ago, and now that his tumors have remained stable, we're able to stretch out the doctor visits and MRIs. His NF causes him to have low muscle tone, so jumping, going up and down stairs, and getting from sitting to standing take a little more work. He gets PT, OT, and speech therapy several times a week. Now four and a half years old, NF hasn't impacted any plans he has for the future: he still wants to hunt for dinosaurs when he's older.

I can't speak for Levi, but as his mother, I can remember back when we took a little break in between switching MEK drugs. The plan was to take a four-week break, but once he came off the MEK, his tumors began to hurt, and he was up most nights crying in pain. We ended up starting the new MEK earlier, and within days, the pain went away. Levi was a little over three at the time, and it was then that he understood he needed the pills to be ok, to not be in pain. He still doesn't completely understand his diagnosis, and sometime soon, we plan to explain it to him in an age-appropriate way. Still, for now, he will tell people he takes medicine for his tumors and that tumors are "boo-boos" in his body, and the therapy helps them.

Levi loves playing with his three older siblings. He also loves monster trucks, construction vehicles, and dinosaurs! He is our little comedian and always makes us laugh.

—submitted by Leah, Levi's mom

CUPID'S UNDIE RUN

2025 CUPID'S UNDIE RUN REGISTRATION IS OPEN!

Freezin' for a reason. After a successful 2024 season, we are gearing up for 2025 and even bringing our undie-clad friends back in person to three new cities!

We believe in putting the hilarity in charity and that fundraising can be thrilling and fun. Cupid's Undie Run has raised more than $25 million since its inception in 2010, and our passionate community won't stop until we find a cure and #EndNF.

"The best party in town with a brief run to raise money for NF and to bring awareness of the condition. The sense of community and inclusiveness at this event keeps me coming back."

"Cupid's Undie Run is cold but unforgettable...and all for a good cause."

JOIN US IN 2025 IN ONE OF THESE LOCATIONS:

Sign up today at **cupids.org**

ATLANTA
AUSTIN
BALTIMORE
BOISE
BOSTON
BUFFALO
CHARLESTON
CHICAGO
CINCINNATI
CLEVELAND
DALLAS
DENVER
DETROIT
JACKSONVILLE
KANSAS CITY
LOS ANGELES
MADISON
MINNEAPOLIS
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RALEIGH-DURHAM
RENO
SEATTLE
SF BAY AREA
ST. LOUIS
ST. PETERSBURG
VIRGINIA BEACH
WASHINGTON, D.C.
WILMINGTON

New CTF Resources for Children and Families Affected by NF1

Discussing an NF1 diagnosis with a child can be challenging. To support these conversations, CTF recently introduced two new resources.

Talking to Your Child About NF1 is a caregiver's guide offering practical tips on sharing an NF1 diagnosis with children.

Super Emerson is a children's book that explains NF1 through the story of a young child living with the condition.

CTF also hosted a **webinar** with members of the team that created these resources, including experts in genetic counseling and bibliotherapy: Ashley Cannon, PhD, MS, CGC; Madeleine Franchi, MS, LCGC; and resource author Ryan Brown-Ezell, MS, CGC.

The webinar and both resources in English, Spanish, and French are available at **ctf.org/superemerson**.

Additionally, CTF's **NF Parent Guidebook** is now available in English, Spanish, French, German, and Italian and is coming soon in Mandarin. This 160-page guide supports families navigating NF-related learning, behavioral, and social challenges. Access it at **ctf.org/nfparentguidebook**.

SHINE A LIGHT **NF** WALK

The premier fundraising event of the Children's Tumor Foundation, Shine a Light NF Walk brings thousands of participants together to celebrate our NF Heroes. Throughout 2024, there were 23 events nationwide, and supporters joined us rain or shine, or even virtually! Participants raved about the Walks, describing them as "Heartwarming and memorable," "Emotional and amazing," and "A fun family day raising awareness and helping those affected with NF." Thank you to our local NF Heroes and their supporters, Walk Directors, volunteers, participants, fundraisers, donors, and our National Walk Sponsor, Alexion AstraZeneca Rare Disease. Fundraising will continue until December, and we are well on our way to hitting our $1.5 million goal.

SHINE A LIGHT NF WALK

ALEXION®
AstraZeneca Rare Disease

IOWA

August 17, 2024

Iowa has done it again and even beat its 2023 record, raising more than $72,000! Two hundred and fifty attendees and 14 teams were ready to walk and roll and enjoy the day with many activities for the kids, including face painting, a fire truck, dance teams, mascots, a DJ, and more. The Iowa Walk continues to strengthen our NF community with support from the community and local businesses. Thanks to our Walk Director, Alicia Tegtmeyer, and her committee for providing plenty of family fun.

Kansas City

September 21, 2024

We're serious when we say, "See you rain or shine," and the rain couldn't keep our Walkers in Kansas City away! With over 200 attendees, the day was filled with family, friends, cheerleaders, mascots, and fun, culminating in more than $55,000 raised to help end NF. Congratulations to Walk Director Hannah Duby and her committee for their hard work in making the 2024 Kansas City Walk event a HUGE success!

Carolinas

October 5th, 2024

The Carolinas had yet another great year in 2024. After a monumental year in 2023 with more than $227,000 raised and 278 participants, the volunteers in Charlotte used that momentum to put together an even bigger and better event for 2024. Between the fantastic fundraising being done in the Carolinas area by our walkers and the Caddies for Collin golf tournament hosted by the Cashell family, the Carolinas community surpassed their goal of $180,000 for 2024. With an appearance from local mascot Homer, volunteers from the area high school cheering on our walkers, and amazing NF families and friends, it was a beautiful and impactful October day!

Learn more about the Shine a Light NF Walk program at shinealightwalk.org

NF ENDURANCE

NF ENDURANCE ADDS FOURTH WORLD MARATHON MAJOR:

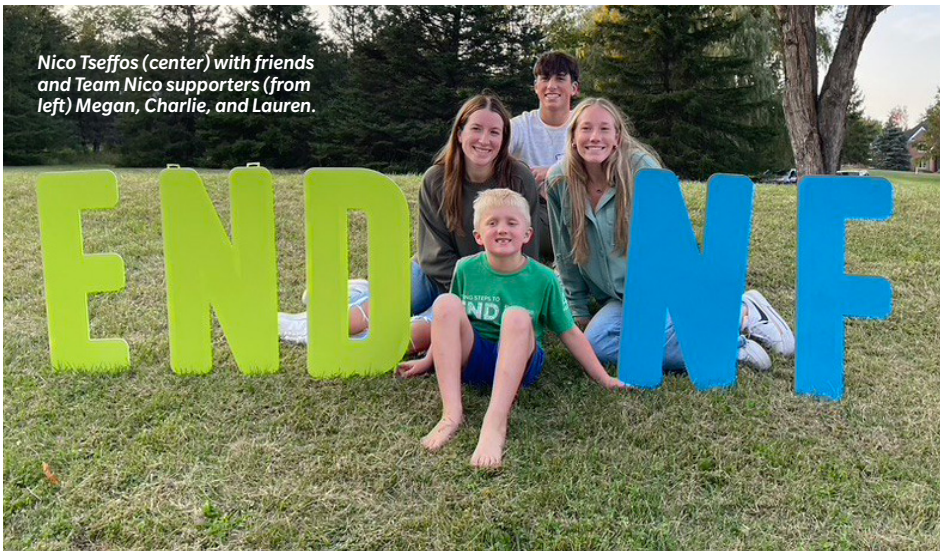
THE TCS LONDON MARATHON!

Beginning in 2025, CTF joins the list of esteemed partner charities of the TCS London Marathon. Scheduled for April 27, 2025, the event joins the BMW Berlin-Marathon, the Bank of America Chicago Marathon, and the TCS New York City Marathon (see dates below) as Abbott World Marathon Majors that allot coveted entries to CTF. Each race offers a unique course through a major international city with its own buzz and energy. For registration details, contact Lydia Vanderloo at lvanderloo@ctf.org.

TEAM NICO TOPS $200,000

Since 2018, Team Nico has worked tirelessly to raise funds for CTF in honor of Nico Tseffos, one of Emily and Nick Tseffos's three beloved children who lives with NF1.

When Nico Tseffos was diagnosed with NF1, his parents set a goal to raise $100,000 to support CTF by Nico's 10th birthday. They formed Team Nico along with Nico's grandparents and a community of family friends, and they doubled their goal before Nico turned 8. Together, they have run marathons, run in their undies with Cupid's Undie Run, walked to Shine a Light on NF, and, in recent years, staged a series of amazingly successful wine fundraisers in their backyard in Appleton, Wisconsin. "Our goal is not only to raise money but to connect with our community and educate them on the need to find a cure," said Karen and Nick Sr., Nico's grandparents. "We are filled with gratitude for our family, friends, and community. How can it get any better? Great wine, food, music, and a community of friends all coming together to END NF!!!"



Nico Tseffos (center) with friends and Team Nico supporters (from left) Megan, Charlie, and Lauren.

2025 NF ENDURANCE TEAM CALENDAR

United Airlines NYC Half
March 16

TCS London Marathon
April 27

Flying Pig Marathon Weekend
May 2-4

TD Five Boro Bike Tour
May 4

Denver Colfax Marathon Weekend
May 17-18

BMW Berlin-Marathon
September 21

Bank of America Chicago Marathon
October 12

TCS New York City Marathon
November 2

Limited Entries Available for These Destination Events:

Life Time Miami Marathon
February 2

Napa Valley Marathon
March 2

Schneider Electric Paris Marathon
April 13

Prague International Marathon
May 4

Irish Life Dublin Marathon
October 26

For event information, visit nfendurance.org or contact Lydia Vanderloo at lvanderloo@ctf.org

CTF GLOBAL

Recap of the 2024 Global NF Conference Brussels, Belgium June 2024

Hosted by CTF Europe and organized by the Children's Tumor Foundation and the European NF Group, the 2024 Global NF Conference was the premier event for the NF research and clinical communities. This significant gathering aimed to advance outcomes for individuals living with all types of NF.

The conference took place in the historic city of Brussels, attracting nearly 1,000 attendees from 43 countries. Each of the five days focused on specific aspects of NF, including Gene Therapy, Comprehensive Care, Schwannomatosis, Novel Therapeutics, and advancements in AI and Biomarkers.

Nathalie Moll, Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA), delivered an inspiring opening address to catalyze the event. A highlight of the conference was the fireside chat between Niklas Blomberg, PhD, Executive Director of the Innovative Health Initiative, and Magda Chlebus, Executive Director of Scientific & Regulatory Affairs at EFPIA and Board member of CTF Europe. They discussed Europe's unique public-private partnerships and the opportunities they present for rare disease research.

The conference also featured the inaugural Young Investigator Day and the second Patient Day, which occurred before the main event. Our partners at Sage Bionetworks conducted two essential data workshops, while dedicated satellite meetings, organized by long-term friends and partners, focused on specific topics of interest.

The von Recklinghausen Award was presented to Professor Rosalie Ferner, MD, PhD, in recognition of her life achievements in the field of NF. Throughout, the community honored the memory of our longtime friend and colleague, Ludwine Messiaen, PhD, dedicating the meeting to her legacy. Each year, this conference serves as a cornerstone of CTF's efforts, reinforcing our belief that our united community will help us achieve the mission to end NF.

Our utmost thanks to the conference sponsors: Presenting Sponsors, Alexion AstraZeneca Rare Disease and SpringWorks Therapeutics; Bronze Sponsors, the Neurofibromatosis Therapeutic Acceleration Program (NTAP), Recursion, and Healx; and Partner Sponsors, NFlection Therapeutics and Boehringer Ingelheim.

Learn more at nfconference.org

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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
NF News Design Director, Susanne Preinfalk

The Children's Tumor Foundation (CTF) is the drug discovery engine for NF, a group of genetic conditions that cause tumors to grow on nerves. By bringing together patients, doctors, scientists, and pharma, we drive treatments, advance care, and deliver faster results for millions affected by NF. NF refers to all types of neurofibromatosis and schwannomatosis, which collectively affect 1 in every 2,000 indiviiduals. 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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