

NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
FALL 2019

CHILDREN'S
TUMOR
FOUNDATION
ENDING NF
THROUGH RESEARCH

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Classrooms That Care

Classrooms that Care is gearing up for the Back to School season! This youth-focused fundraising program educates students, teachers, and parents about neurofibromatosis. Through educational activities and programming designed to fit into curriculum standards, Classrooms that Care allows participating schools to raise NF awareness, create empathy for those facing medical and health challenges, and empower students to celebrate diversity and embrace inclusion in their communities.

NF parent Stacey DeCillis and her husband Rob included their three children in the fight to end NF through participating in Classrooms that Care. Read about Stacey's experience on page 14, and learn how you can bring this program into your child's school.



FROM the President

Annette Bakker, PhD



This fall as we send our children back to school, the Children's Tumor Foundation (CTF) is infusing Classrooms that Care, one of our signature programs, with a renewed vigor. We are a Foundation that serves people of all ages who live with neurofibromatosis (NF), and we have a unique opportunity to educate our young NF Heroes and their classmates in their formative years. In the words of one of our star volunteers, Stacey DeCillis, who shares her story in this newsletter, this program has the power to build up the next generation of CTF volunteers and NF activists.

Indeed, education and awareness opportunities abound in the days ahead. Our NF Conference takes place at the end of September in conjunction with our NF Forum. Patients and caregivers will meet on Saturday to learn more about their diagnosis and how to take charge of their

care, while the greatest researchers and NF experts will gather to uncover solutions and potential treatments for those same patients. In keeping with our ongoing globalization efforts, Saturday evening there will be a panel discussing NF care around the world. These events fill me with enthusiasm and passion for what we do!

In this newsletter we recap the wonderful work of our network of fundraisers and families, who light up buildings and attain proclamations to spread awareness of NF. We are so grateful to the attendees, volunteers, and NF Fighters who swim, sell cookies, attend Shine A Light NF Walks, run marathons, and hold local fundraisers so that CTF can continue to fund the research needed to end NF.

The outstanding commitment of our NF community is what makes it possible to fund some of the most cutting-edge research on neurofibromatosis, and position ourselves as leaders in the research community. You, our donors, are funding that research, and empowering patients, caregivers, and researchers with hope and promise. Your gifts are making a difference, because together we all play a part in the fight to end NF.

Gratefully Yours,



2019
NF CONFERENCE
SEPT. 21 - 24

NF FORUM
SEPT. 20 - 22

NF HACKATHON
SEPT. 13 - 15

The NF Conference is a global event, attracting researchers and clinicians worldwide, sharing key advancements in research and clinical management guidelines for NF1, NF2, and schwannomatosis.

NF CONFERENCE | SEPTEMBER 21 - 24

The NF Forum brings families and patients together to connect and learn from each other, while attending seminars on relevant topics pertaining to NF.

NF FORUM | SEPTEMBER 20 - 21

At the NF Hackathon, over 120 researchers, data scientists, and engineers will come together to explore and analyze data from the NF Data Portal.

NF HACKATHON | SEPTEMBER 13 - 15

To register for these events, go to: ctf.org

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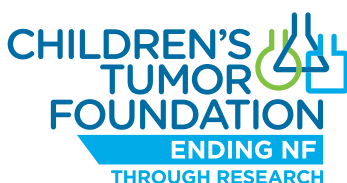
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Updated Guidelines Published about NF1 Diagnosis and Management in Children

CTF hopes that families impacted by NF1 have seen and shared the new publication titled “Health Supervision for Children with Neurofibromatosis Type 1.” The main focus of this medical article is to provide guidance to pediatricians and other medical providers to better identify a child who might have NF1, understand the variability of NF1 signs and symptoms, and recognize and treat NF1-related concerns. This highly anticipated publication is an updated document from the original pediatric guidelines published in 2008 and was created by a team of dedicated

NF clinicians including: David Miller, MD, PhD; Debra Freedenberg, MD, PhD; CTF Clinical Care Advisory Board members Elizabeth Schorry, MD, Nicole Ullrich, MD, PhD, and David Viskochil, MD, PhD; and Medical Advisory Committee Chair Bruce Korf, MD, PhD. We encourage all to increase awareness of this new article by sharing with your local providers.

For a link to this article online, go to: ctf.org/NF1update

The NF Collective Working Together

The NF Collective was formed by a number of organizations focused on addressing various needs within the NF community. Recognizing a critical need for NF patients and families to find reliable NF care, the member organizations of the collective joined forces to provide a reliable, updated resource of NF providers. For more information, go to nfcollective.org. CTF continues work with the collective to explore additional collaborative projects towards a common mission of Ending NF.

EXPANSION OF THE NF CLINIC NETWORK

The Children’s Tumor Foundation understands how difficult it can be to find healthcare professionals experienced in diagnosing and caring for individuals with NF. The NF Clinic Network (NFCN) was established in 2007 to standardize and raise the level of NF clinical care nationally and integrate research into clinical care practices. Applications into the network are reviewed or declined by the CTF Clinical Care Advisory Board based on several factors including NF expertise, patient volume, multidisciplinary approach, research support and connections with CTF. Two additional clinics have recently been accepted into the NFCN:

- House Clinic in Los Angeles, California, directed by Drs. Gregory Lekovic and William Slattery III with extensive clinical and research experience serving individuals with NF2.
- John R. Oishei Children’s Hospital in Buffalo, New York, directed by Drs. Lorna Fitzpatrick and Veetai Li with clinic coordinator, Michelle Ventola. The Comprehensive NF Program fills a geographic need in western New York state and provides NF care to both children and adults with NF.

The NF Clinic Network has now grown to 57 clinics across the country. Although we have made great progress, CTF recognizes that there are still many states without established NF providers, or some areas that do not serve adults with NF. Our efforts continue to identify additional providers and improve access to quality NF care.



Patients Help FDA Understand Life with NF

On June 13, a group of six NF patient advocates addressed the FDA to share their experiences of living with neurofibromatosis.

Five young people with NF1 and one with NF2 traveled to Bethesda, Maryland with members of the Children's Tumor Foundation staff on June 13, 2019 to offer vivid, unforgettable personal accounts of living with NF to officials from the Food and Drug Administration (FDA). The format, Voice of the Patient, is an informal discussion designed to help those involved in the drug approval process understand the obstacles and unmet needs of those who might benefit from emerging new drug treatments.

Following an emotional session in which representatives ranging in age from 14 to 26 and their parents described their medical journeys and hopes for the future, Andrea-Furia Helms, Director of the Patient Affairs Staff, expressed gratitude on behalf of the agency, saying, "You are the experts in NF. We need to hear from you. Your stories are critical for us to understand."

Also present at the session were FDA officials from the Center for Biologics Evaluation and Research (CBER), Center for Drug Evaluation and Research (CDER), Oncology Center of Excellence (OCE), Office of Orphan Products Development (OOPD), and Office of Clinical Policy and Programs (OCPP). The session ran well past the one hour allotted as the FDA asked the youngsters for more details about the burden of NF, access to clinical trials, and ways to change the process to serve patients better, for example, with de-centralized clinical trials that would reduce the amount of time away from work, school, and family.

The patients, Aidan Fraser, Jack Burke, Jesse Sorman, Brianna Worden, Maureen Hussey (speaking for her daughter, Maggie), and McKinnon Galloway, were eloquent and impressive in both their knowledge of NF and the skill with which they communicated it.

"I am actually one of the lucky people with NF, I am fortunate to have access to great doctors and the best care I could hope for. At the same time, I have been bullied, ignored and disregarded because of the way I look. When I walk down the street people stare at me. I understand because no one has seen someone who looks like me, but it is very, very hard," Aidan Fraser shared.

Answering questions from the clearly engaged and concerned FDA audience members about how FDA

could improve the clinical trial experience, the patient reps highlighted difficulties in finding opportunities and deciding about clinical trial participation, getting different specialists to communicate with one another, and the lost potential of talented young people who spend too much of their time with doctors and in hospitals. Unspoken, but strongly implied, was the idea that efficient FDA approval of safe, effective drugs now in development could revolutionize lives and free up much of this potential.

The FDA was also interested in hearing about CTF-sponsored efforts to standardize clinical trial endpoints by supporting Response Evaluation in Neurofibromatosis and Schwannomatosis (REiNS), which REiNS and CTF Patient Representative Maureen Hussey explained in detail.

While the combination of long-distance travel and reliving physical and emotional challenges took a toll, all agreed the day was a great success. As CTF President Annette Bakker noted, the interaction between FDA and patients representatives demonstrated an amazing evolution toward empowered patients. Ms. Helms of the FDA expressed similar feelings, and noted that the day's meeting would not be the last opportunity for patient reps to engage with FDA.

CTF's participation was initiated and organized by CTF Patient Engagement Director Traceann Rose, who worked closely with the FDA staff to bring this opportunity to the NF community and prepare the young representatives for their roles.



From left: Jack Burke, Brianna Worden, Jesse Sorman, Aidan Fraser, and McKinnon Galloway

CTF Patient Advocates Participate in AstraZeneca NF1 Day



A group of patient advocates from the Children's Tumor Foundation and the broader NF1 community attended an NF1 Day event at the Gaithersburg campus of AstraZeneca on July 18, 2019. The goal of the meeting was for NF1 pediatric patients and caregivers to share their stories about living

with NF. They worked to help this important drug company to further understand NF and how selumetinib, an AstraZeneca developed drug, can benefit patients with NF1.

The group from CTF that attended included Renie Moss, Philip Moss, Aidan Fraser, Mariah Booker, and Teresa Williams. They had the opportunity to meet with AstraZeneca

leaders from the medical, marketing, and corporate affairs teams. They actively participated on an NF1 Patient Advisory Board, toured the lab, and engaged in other patient-centered activities. In addition to our ongoing collaboration with AstraZeneca, the Children's Tumor Foundation helped facilitate the NF1 Day by recruiting patients to attend the meeting.

MEK MAKES A DIFFERENCE

<p>NF HERO: Philip Moss</p>  <p>57% shrinkage</p>	<p>NF HERO: Ryker Bennett</p>  <p>42% shrinkage</p>
<p>NF HERO: Paige Doane</p>  <p>32% shrinkage</p>	<p>NF HERO: Jane Constable</p>  <p>30% shrinkage</p>

AstraZeneca and Merck & Co., Inc. were granted Orphan Drug Designation (ODD) for selumetinib, a MEK inhibitor, for the treatment of NF1 by the Food and Drug Administration (FDA). This progress is because, thanks to your support, CTF's investment in the NF Preclinical Consortium led to groundbreaking research that demonstrated that MEK inhibitors have significant impact on tumor size. That research now informs the clinical trials taking place at the NIH, and their incredible results (see images at left).

SPRINGWORKS THERAPEUTICS ANNOUNCES FDA FAST TRACK DESIGNATION FOR PD-0325901 FOR NF1 PATIENTS

One of the Children's Tumor Foundation's partners, SpringWorks Therapeutics, announced earlier this year that the Food and Drug Administration (FDA) granted Fast Track designation for the MEK inhibitor PD-0325901, for NF1 patients over the age of 2 with inoperable plexiform neurofibromas. This will help expedite the development and review of this potential treatment for NF patients. SpringWorks expects to initiate a phase 2b clinical trial for pediatric and adult patients later this year.

NF1-associated plexiform neurofibromas (NF1-PNs) are characterized by mutations in the MAPK pathway, leading to the growth of peripheral nerve sheath tumors that cause significant pain, disfigurement and morbidity. NF1-PNs are most often diagnosed in the first two decades of life and are characterized

by aggressive tumor growth, which is typically more rapid during childhood. There are currently no therapies approved for the treatment of NF1-PN.

"The Fast Track designation recognizes that plexiform neurofibromas have a substantial impact on the lives of patients, and that our MEK inhibitor has the potential to address the significant needs faced by this patient community who currently do not have an FDA-approved treatment," said Saqib Islam, Chief Executive Officer of SpringWorks Therapeutics. "We look forward to continuing to work closely with the FDA on our upcoming Phase 2b study, which will enroll pediatric and adult NF1 patients with plexiform neurofibromas."

YOUR DONATIONS AT WORK

CTF awards more than 950K in the first half of 2019

YOUNG INVESTIGATOR AWARDS 2019

The Children's Tumor Foundation is pleased to announce the funding of two Young Investigator Awards (YIA) for 2019. Both awards are focused exclusively on malignant peripheral nerve sheath tumors (MPNSTs) and are funded by the NF Research Initiative, which is made possible by an anonymous donation to Boston Children's Hospital.

HARISH VASUDEVAN

University of California,
San Francisco

"Elucidating the clonal architecture and functional impact of genetic variants in MPNSTs using single cell genomics"

Malignant peripheral nerve sheath tumors (MPNSTs) are rare but aggressive tumors associated with NF1. There are currently no molecular biomarkers or targeted therapies available for MPNSTs due to the rarity of these tumors and incomplete understanding of their molecular composition. The overall aim of Dr. Vasudevan's proposed work is to use high throughput sequencing in single cells to characterize the molecular heterogeneity in MPNSTs at the DNA, RNA, and protein levels as well as evaluate the functional effect of these changes in primary Schwann cells. Given the heterogeneous nature of MPNSTs, single cell analyses will be critical to define clinically relevant tumor subpopulations, mutational co-occurrence, and rare genetic variants.



JORDAN KOHLMAYER

University of Iowa

"The RABL6A-RB1 pathway in MPNST pathogenesis and therapy"

The overall objective of this study is to determine the role of RABL6A, a novel oncogenic GTPase, in regulating the retinoblastoma (RB1) tumor suppressor signaling.

Preliminary work by Ms. Kohlmeyer support the hypothesis that RABL6A drives MPNST pathogenesis by RB1 inactivation, and therefore, the RABL6A-RB1 pathway can be an important, new therapeutic target. This study will determine the mechanisms and biological significance of RB1 pathway regulation by RABL6A in MPNSTs and the value of targeting this pathway to develop novel MPNST therapies.

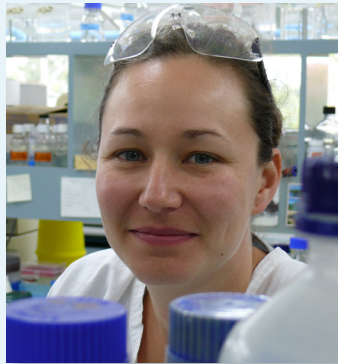


NF1 GENE THERAPY INITIATIVE

The Children's Tumor Foundation is pleased to announce the funding of two awards as part of the CTF NF1 Gene Therapy Initiative. Each award is for \$240,000 for a total duration of two years. Peggy Wallace, PhD, a longtime associate of the Foundation, is the chief consultant for this initiative.

SAMANTHA GINN, PHD

Senior Research Officer, Children's Medical Research Institute, Australia
"A mutation-independent genome editing approach for the treatment of neurofibromatosis type 1 (NF1) using novel AAV vectors"



Dr. Ginn and her team propose to use a clustered regularly interspaced short palindromic repeat/Cas9 (CRISPR/Cas9) based homology-independent targeted integration (HITI) approach to replace large sections of mutated NF1 gene. In contrast to methods targeting individual patient-specific mutations, this approach has the advantage of targeting multiple mutations with a single gene editing vector, and thus will be applicable to many NF1 patients. To ensure clinical applicability, they will optimize the recombinant adenoassociated virus (rAAV) vector by screening and directed evolution, and test the approach in primary human Schwann cells. The ultimate goal of this study is to combine optimal gene editing tools with the most functional rAAV vectors to create reagents for in vivo NF1 editing.

JAMES WALKER, PHD

Assistant Professor, Harvard Medical School
"Development of NF1 therapeutics with CRISPR-based technologies"



Dr. Walker and his team aim to investigate the feasibility of using genome editing (both CRISPR-based homology-directed repair and base editing) as a therapeutic approach to correct three pathogenic NF1 mutations in cultured human Schwann cells. They will capitalize on recently developed CRISPR/Cas9 and -Cas12a variants, which increase the targeting range, activity, and fidelity (reducing off-targets) of gene editing. With a view to developing the most promising strategies into potential therapies for NF1 tumors, they will also initiate a screen to optimize viral vehicles for Schwann cells that will be essential for in vivo delivery of CRISPR genome engineering tools.

DRUG DISCOVERY INITIATIVE REGISTERED REPORTS

Two Drug Discovery Initiative Registered Reports Awards were granted for work on therapy resistance in NF2 and DNA damage in malignant peripheral nerve sheath tumors (MPNST).

KEILA E. TORRES, MD, PHD

MD Anderson Cancer Center
"Targeting DNA damage signaling and epigenetic deregulation as a combination therapy for malignant peripheral nerve sheath tumors"



People with neurofibromatosis 1 (NF1) are at risk of developing malignant peripheral nerve sheath tumors (MPNSTs) over the course of their lifetime. MPNSTs are aggressive tumors for which the only effective treatment is surgery. Often, though, the tumors grow back in the same location; thus, surgery to remove the MPNST may not always be an effective long-term treatment. The goal of this study is to address the lack of non-surgical treatments for MPNST by developing a therapy that combines two anti-cancer drugs to target pathways in the cell that are altered in MPNSTs. This research will measure how effective these anti-cancer drugs are when used together and will provide the preliminary results to help researchers decide whether this combination works well enough to be tested in people with MPNST.

CHUNLING YI, PHD

Georgetown University
"Evaluate Novel Hippo-Yap/Taz Inhibitors in Overcoming Therapy Resistance in NF2"



Hippo-Yap/Taz signaling pathway was identified as a major mechanism showed that NF2 tumor cells might be eradicated by combining a novel class of direct Hippo-Yap/Taz inhibitors with MEK inhibitors. Moreover, several other classes of drugs (including drugs that are FDA-approved and/or in clinical trials for NF2) synergize with Yap/Taz blockade in selective killing of NF2 schwannoma cells. This project will perform high throughput combination studies of four classes of drugs predicted by preliminary studies to be synergistic with clinical Hippo-Yap/Taz inhibitors developed by industry partner Vivace Therapeutics, and select the most efficacious combinations for testing in mice.

Foundation to Invest \$2.3 Million in NF2 Accelerator Initiative

On the occasion of World NF2 Awareness Day, the Children's Tumor Foundation announced the establishment of a significant research initiative dedicated to finding effective treatments for NF2, along with a substantial investment of \$2.3 million in this bold new effort. Called the 'NF2 Accelerator Initiative,' this three-year undertaking will be dedicated to bringing active NF2 treatments to the clinic, and to patients, by expanding the clinical drug pipeline for NF2, improving drug selection through the development of innovative testing models, and the development of gene therapy options that address the underlying genetic causes of NF2.

As a global leader of NF research, CTF is dedicated to developing cures for all three forms of NF. The Foundation and its partners

have bolstered the NF2 research field through a team science approach, called Synodos. Launched in 2014, Synodos for NF2 brought together a multidisciplinary team of scientists from 12 world-class labs at academic and medical centers of excellence who pledged to work together. The Synodos teams shared information, datasets, and results in real-time at every step of research development. All that data is now publicly available at nfdataportal.org. That collaborative effort resulted in the identification of promising new clinical candidates for NF2, and the announcement of the NF2 Accelerator Initiative leverages that new knowledge into a new and ambitious structure, with the goal of speeding up the drug discovery process.

To read more, go to: ctf.org/NF2accelerator

CTF Provides Funding to NF2 Therapeutics, Inc. to Develop a First-in-Class Gene Therapy for NF2



The Children's Tumor Foundation has provided funding to NF2 Therapeutics, Inc. (NF2RX) to develop a first-in-class gene therapy for neurofibromatosis type 2. This funding follows an initial \$100,000 round of funding previously provided by CTF. Both rounds were structured as venture philanthropy funding. The initial funding enabled experiments in the lab of Dr. Helen Morrison, Leibniz Institute on Aging – Fritz

Lipmann Institute (FLI) in Jena, Germany, which demonstrated that biologic therapies can successfully rescue the function of NF2 in a cell culture model. Dr. Morrison is a key opinion leader who has published extensively on the function of human merlin, the protein that is mutated in NF2, and has helped elucidate its role in tumorigenesis.

CTF and NF2 Therapeutics Inc.

are optimistic that an NF2 gene therapy will provide a disease-modifying treatment for patients. NF2RX is working with key opinion leaders and leading technology partners to develop vectors tailored for delivery to Schwann cells where schwannomas occur and arachnoid cells where meningiomas occur. Michael Wootton, CEO of NF2 Therapeutics, said, "We are pleased to receive a second round of funding from the Children's Tumor Foundation, and look forward to developing a gene therapy that we hope will be a disease modifying treatment for NF2."

Annette Bakker, PhD, President of the Children's Tumor Foundation said, "We are excited to work with NF2RX and provide funding to develop a gene therapy that could greatly benefit the quality of life of those affected by NF2."

Research Published from Synodos Group

Research from one of the Synodos for NF1 research teams was recently published in the prestigious science journal *Communications Biology*. The University of Minnesota Synodos team is focused on developing and perfecting swine models that will most accurately replicate human reaction to drugs in preclinical testing.

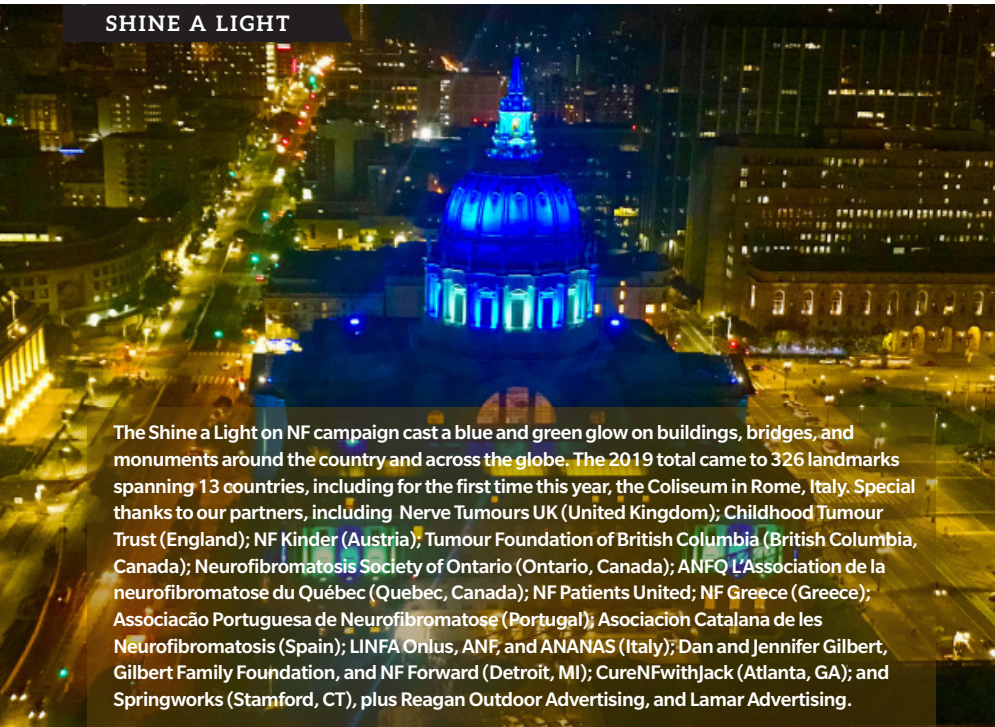
A link to the full article can be accessed at:

ctf.org/synodospub

Awareness Month HIGHLIGHTS

The annual NF Awareness Month campaign raises awareness about neurofibromatosis through numerous campaigns and gatherings during the month of May.

SHINE A LIGHT



The Shine a Light on NF campaign cast a blue and green glow on buildings, bridges, and monuments around the country and across the globe. The 2019 total came to 326 landmarks spanning 13 countries, including for the first time this year, the Coliseum in Rome, Italy. Special thanks to our partners, including Nerve Tumours UK (United Kingdom); Childhood Tumour Trust (England); NF Kinder (Austria); Tumour Foundation of British Columbia (British Columbia, Canada); Neurofibromatosis Society of Ontario (Ontario, Canada); ANFQ L'Association de la neurofibromatose du Québec (Quebec, Canada); NF Patients United; NF Greece (Greece); Associação Portuguesa de Neurofibromatose (Portugal); Asociación Catalana de les Neurofibromatosis (Spain); LINFA Onlus, ANF, and ANANAS (Italy); Dan and Jennifer Gilbert, Gilbert Family Foundation, and NF Forward (Detroit, MI); CureNFwithJack (Atlanta, GA); and Springworks (Stamford, CT), plus Reagan Outdoor Advertising, and Lamar Advertising.

The NF Awareness Month "Picture a World Without NF" photo contest asked the NF community to share how they would feel if we cured NF tomorrow.

"In my photo I am taking the NF from out of 'I Know A Fighter.' If we found a cure we would still be fighters but without NF. I would be excited to have the health, energy, and quality of life to fight for others who may not have a cure."

- Ricki Jensen, who lives with NF2



PHOTO CONTEST WINNERS

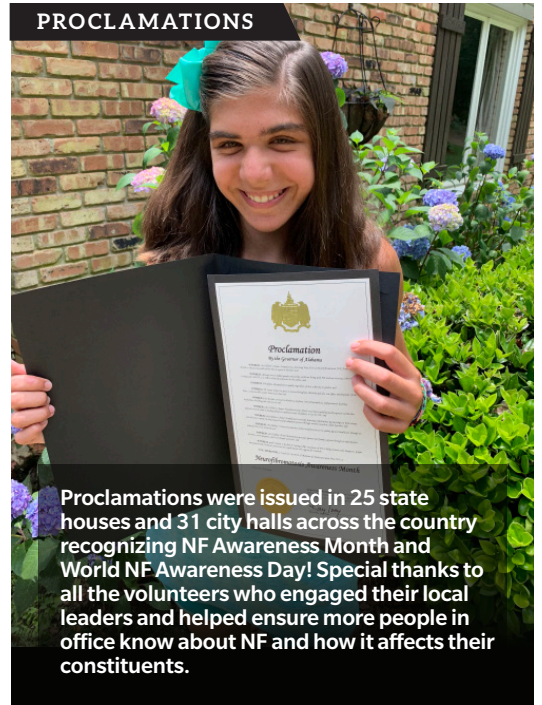
"We adopted our son with NF 20 months ago and he hadn't had a chance to have treatment for NF until coming to the US to be a part of our family. Finding a cure for this disease would mean the world to a kid like him. He has such a huge heart. Every time you ask him what his dream is, it always involves helping others who are less fortunate. He wants to be a chef. So usually he wants to cook for others in need."

- Lindsay Gibson, mom to Maddox (pictured) who lives with NF1



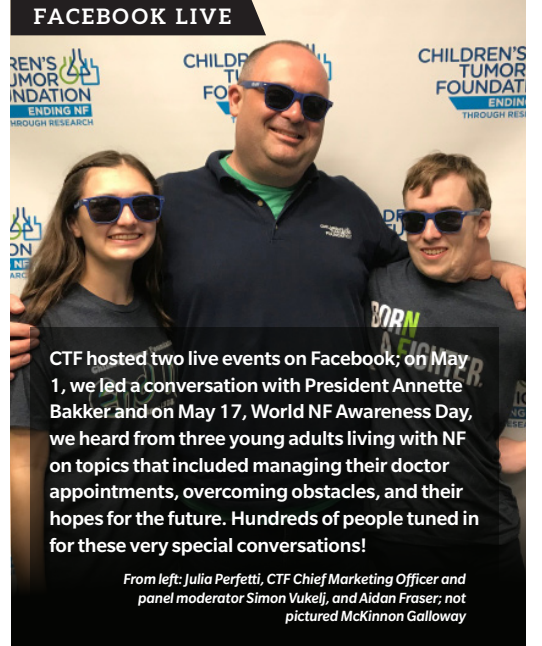
Watch our NF Awareness Month Recap Video at: ctf.org/recap2019

PROCLAMATIONS



Proclamations were issued in 25 state houses and 31 city halls across the country recognizing NF Awareness Month and World NF Awareness Day! Special thanks to all the volunteers who engaged their local leaders and helped ensure more people in office know about NF and how it affects their constituents.

FACEBOOK LIVE



CTF hosted two live events on Facebook; on May 1, we led a conversation with President Annette Bakker and on May 17, World NF Awareness Day, we heard from three young adults living with NF on topics that included managing their doctor appointments, overcoming obstacles, and their hopes for the future. Hundreds of people tuned in for these very special conversations!

From left: Julia Perfetti, CTF Chief Marketing Officer and panel moderator Simon Vukelj, and Aidan Fraser; not pictured McKinnon Galloway

New for 2019, the NF community showed our passion through fashion on May 17 (to recognize World NF Awareness Day) and May 22 (to recognize NF2 Awareness Day) by wearing blue and green.

WEAR BLUE & GREEN



Shine A Light Walks 2019

shinealightwalk.org

ALABAMA
11/2/19

ATLANTA
10/6/19

BAY AREA
(Previously San Jose)
9/28/19

CENTRAL FLORIDA
(Previously Orlando)
9/7/19

CHICAGOLAND
9/7/19

DENVER
9/28/19

HAGERSTOWN, MD
(New walk!)
9/14/19

HOUSTON
11/9/19

JACKSONVILLE
9/7/19

KANSAS CITY
10/5/19

LOS ANGELES
10/19/19

LONG ISLAND
9/14/19

MICHIGAN
(Previously Addison Oaks)
9/14/19

MOOREHEAD
9/8/19

NEW JERSEY
10/5/19

PHOENIX
11/10/19

ROCHESTER
10/12/19

SAN ANTONIO
10/5/19

SAN DIEGO
11/16/19

SEATTLE
9/15/19

SOUTH FLORIDA
DECEMBER
MARCH 2020

VIRGINIA
(Previously Richmond)
9/7/19

WASHINGTON, DC
10/26/19

WESTCHESTER, NY
(Previously NYC)
9/14/19



Shine A Light on NF Walk program got off to a great start with the first three walks of the year happening during NF Awareness Month. Thanks to participants like you, this program has raised over \$10 million since 2009 to fund research initiatives that will one day put an end to NF.



Cincinnati Shine a Light Walk

The Cincinnati Walk continues to grow every year with the help of local volunteer Walk Organizer, Jessica Samblanet. The Walk has so far raised more than \$90,000, exceeding the event's overall fundraising goal. With over 580 Walkers, the day was full of fun, and hope as the local NF community took steps together to help CTF get closer to finding effective NF treatments through funding research.

Utah Shine a Light Walk

This May was the largest NF Walk in Utah history under the leadership of Brandie Evans. The first ever evening Shine a Light NF Walk raised almost \$40,000 and was attended by over 475 walkers. Thank you to Presenting Sponsor Recursion Pharmaceuticals for their \$10,000 gift, which was a huge factor in the Walk's success.



Idaho Shine a Light Walk

Over 200 walkers in Meridian, Idaho came out on May 18th to Shine a Light on NF. Live entertainment and fun activities filled the beautiful Scentsy campus. More than \$15,500 was raised to fund NF research thanks to Walk Organizers Shannon McNall and Freedom Macky, the Shine a Light NF Walk Committee, many donors, and the participants who joined us that day. First time walker Evlyn said, "Being at the Shine A Light Walk in Idaho was so inspiring. It was the first time we'd seen so many other families affected by NF, all gathered together, lending support, understanding, and just having fun together! It was such an incredible reminder that our little family is not alone, that we are all in this quest together to find a cure for NF."



Colfax Marathon, Half, 5K and Marathon Relay

NF Endurance was excited and happy with our first official team event at the 2019 Colfax Marathon Relay, Half and 5K! Taking place over May 18-19 in Denver, Colorado, it was a wonderful opportunity for many in our Colorado NF Community to celebrate NF Awareness Month at one of Denver's largest and most popular races. Anchored by an amazing corporate team, CFC Construction (CFCC), the Colfax team was comprised of 25 runners and walkers participating in the 5K, the Half Marathon, and the Marathon Relay. NF Dad Pat Smith suggested to his CFCC colleagues Dennis Polmateer and Nicole Skalecke that CTF be the benefiting charity for CFC's fundraising efforts. An organized team dinner on Saturday evening set a congratulatory and celebratory tone for the weekend, and a race-day team tent allowed runners to gather and connect, as well as promote CTF's mission and NF awareness to the general public. Special thank you to CFC Construction; to Jody Bartling for organizing many of the weekend team logistics; and to Green Leaf Massage & Sport Recovery for sponsoring our team tent and providing post-race massages to our team members. And finally, thank you to our committed and dedicated Colorado NF Community for their interest, support, and participation, and for raising more than \$9,000 in 2019. Next year the Colfax Marathon celebrates its 15th Anniversary on World NF Awareness Day (May 17, 2020), so this event promises to be even bigger and better!



Run with the NF Endurance Team to accelerate NF research at one of the events below! Not a runner? We'd love your help as a race weekend volunteer or cheer station supporter.

Register at nfendurance.org or reach out to adumadag@ctf.org.

UPCOMING NF ENDURANCE EVENTS:

Chicago Marathon – Oct. 13

New York City Marathon – Nov. 3

Indianapolis Monumental Marathon, Half, 5K – Nov. 9

California International Marathon, 5K – Dec. 8

NYC Half Marathon – Mar. 2020

Colfax Marathon, Half, 10 Miler, 5K, Relay – May 16-17, 2020

NEW: Berlin Marathon – Sept. 27, 2020

Choose Your Own Challenge – any event, any time, anywhere



Timothy Schuster

In honor of his close friend's son, Ari Taub, Timothy Schuster ran the London Marathon as a member of the NF Endurance team in April 2019 and raised over \$8,400 for the mission of the Children's Tumor Foundation. Here, Timothy writes about the experience, and his inspiration for joining the NF Endurance team:

One of my dearest friends from residency and a colleague in urology in my earliest years in Toledo has a son who has neurofibromatosis ... Diagnosed as a baby, Ari is now 12 (the same age as my son) and loves football, sports, science, and anything related to University of Michigan (also like my son). Although he is doing well now, he continues to be monitored annually for signs of progression.

When the opportunity arose to run a marathon to raise money for the Children's Tumor Foundation (CTF), I jumped at the chance. This seemed like the perfect next challenge for me. Not only could I continue my running, could also help spread awareness about NF, share Ari's story, and raise funds to support NF research and CTF patient support programs. . . . It's this feeling and this passion that makes me want to run.

Around the COUNTRY

The Foundation has a presence across the United States and facilitates local medical symposia and fundraising events. Learn more about the Children's Tumor Foundation in your area by visiting ctf.org.



The Night of Hope Committee:
Emily Tseffos, Hannah Brooks,
Pennie Brooks, CTF Staff
Barbara Gallagher, CTF Staff
Allyson Douglass, and Nancy
Carver

A NIGHT OF HOPE BENEFIT CONCERT

It was truly "A Night of Hope" on Monday, May 6, 2019 at the Franklin Theatre in Franklin, Tennessee, when the popular singing group Thompson Square partnered with the Children's Tumor Foundation for a wonderful event raising \$14,330 for NF research. Band members met with fans and local NF families for a Meet and Greet before the concert, and all attendees received a copy of the band's children's book, *Time to Get Dressed*. Our thanks to the planning committee, the band, and everyone involved at this musical evening.

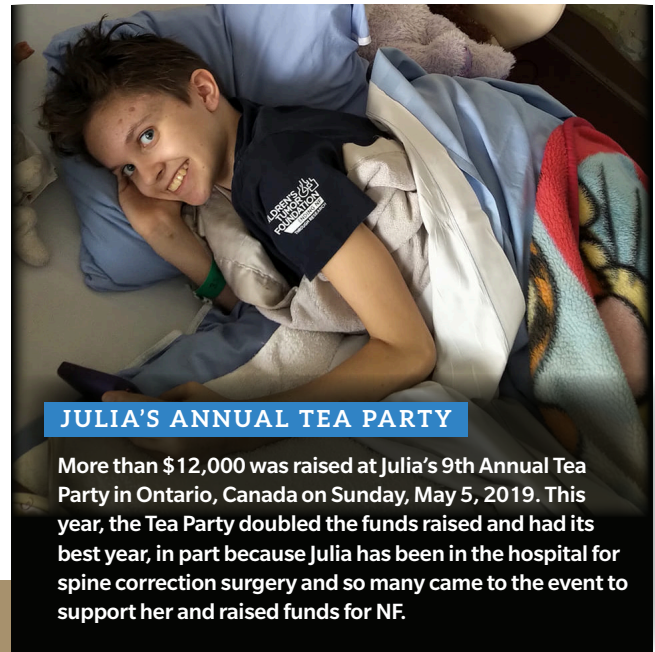


Actor and emcee Jonathan Sadowski pictured with the Kid's Fashion for NF runway models.

Kid's Fashion for NF

On Saturday May 18, 2019, 11 local NF Heroes strutted the runway in the Annual Kid's Fashion for NF event at the Federal Bar in North Hollywood, California. More than \$13,000 was raised for NF research through ticket sales, a silent auction, and a live auction of the models' artwork. Thank you to actor Jonathan Sadowski who was the emcee of the event again this year, and a big shout out to retailer Rosie G, who donated all the girl's outfits for the runway. This year's honoree Joe Jaffa told his NF story and brought jokes and laughter to the event.

“The NF Fashion Show is one of the only times that my daughter gets to celebrate overcoming the difficulties of living with NF. It connects us back to her strength, her heart, and her will to overcome. The NF Fashion Show is a time where we feel the support of the NF community in full force and it fuels each of us for the year to come.” —LAURA WALLOF, NF MOM



JULIA'S ANNUAL TEA PARTY

More than \$12,000 was raised at Julia's 9th Annual Tea Party in Ontario, Canada on Sunday, May 5, 2019. This year, the Tea Party doubled the funds raised and had its best year, in part because Julia has been in the hospital for spine correction surgery and so many came to the event to support her and raised funds for NF.

SPRING LUNCHEON FOR NF

The 2019 Spring Luncheon for NF was held on March 2, 2019, at the Cedarburg Cultural Center in Cedarburg, Wisconsin and raised more than \$4,000. Luncheon organizers Elaine Pankow, Bonnie Wellnitz, and Wayne Stemo hosted a great event with over 80 people in attendance. The Luncheon organizers have volunteered for CTF in numerous ways for more than 20 years, and were surprised with plaques from CTF thanking them for their years of volunteering, as this is their last year to host the luncheon. We thank the organizers and attendees for a wonderful event.



stories

OF NF



of 80 degrees in the lower thoracic and upper lumbar spine.

Together, the world class Neuro and Spinal Team worked hard to come up with a plan that would allow Kloe, now five years old, to have major spinal surgery in a timely fashion and that would also allow her to lead a lifestyle that did not see her in surgery every six months. Although Kloe has undergone major traumatic surgery for the insertion of Magec spinal rods, they are very delicate and easily prone to damage.

Post-operative, Kloe has been fitted with a spinal jacket for her safety. Her follow-up from the hospital requires three monthly hospital visits to lengthen the spinal growing rods with subsequent surgery following at two and five years independently, with a final spinal surgery for a definite fusion when Kloe's growth is completed.

Although Kloe has a magnitude of obstacles to overcome she is a vibrant and determined little girl who doesn't have time to be sick. She continues to live her life with the same tenacious spirit she did prior to her diagnosis.

Kloe does not let her NF define her and we take solace from her strength which inspires us to live for each moment. Life is not promised to any of us, so we make a conscious effort to make it count.

—Candice, Kloe's mum

Kloe van der Merwe, NF1

Kloe is a happy, fun, go-getter with an infectious zest for life! She is a real people pleaser and loves spending time at school with her friends. Born a fighter, she doesn't let NF define her but instead inspires us to all be better versions of ourselves on a daily basis.

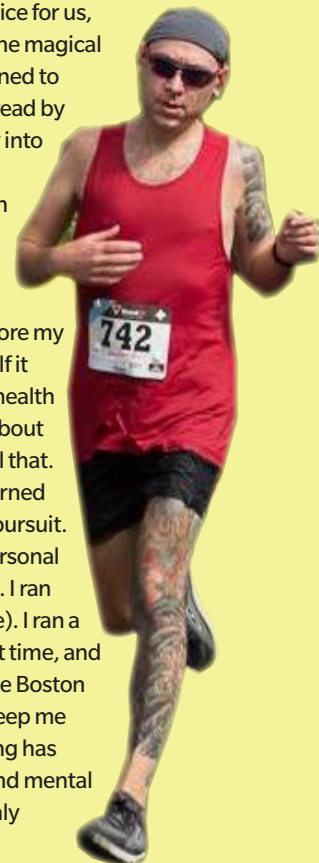
Kloe had been under investigation for NF when her café au lait spots started to appear before she was one year old. Kloe was later diagnosed at age four when she developed severe scoliosis. In March 2018, Kloe's parents took her to the doctor as they had witnessed a deterioration in her spine. Kloe was seen by the complex team at Manchester Children's hospital who confirmed their worst fears, Kloe had been affected by a secondary condition, a progressive and severe scoliosis

Chris Cook, NF2

I was diagnosed with NF2 in my late 30s. It was around Summer 2015, and I had a ringing in my right ear that wouldn't go away. At the time, I was worried that I'd done permanent damage to my hearing due to too many metal rock concerts. If only that had been my issue. My family doctor sent me to an ENT, who sent me for an MRI "just on the off chance." His secretary called and said the doctor needed to see me first thing the next morning. I knew that wasn't good. And that's when I learned all about NF2.

When I first was diagnosed, I felt like it was a death sentence. But then something funny happened. I mean, I'm sure not going to say that anything about it was "good," but it gave me a new outlook and sense of urgency about life. Other than the prognosis and physical systems, it has actually impacted me more positively than negatively, if that makes any kind of sense at all. It has pushed me to accomplish things that I either didn't think I could, or felt I could get around to later. Also, over time and after coming to terms with it, it helped me be an inspiration to others around me. That's become a responsibility I've taken very seriously. It also reaffirmed an earlier decision that adoption might be the right choice for us, and hopefully we'll have someone magical in our family's life soon! I've learned to deal with the uncertainty and dread by channeling that nervous energy into other pursuits. Not every day is good, but I have more good than bad.

Running may have literally saved my life. I was a runner before my diagnosis, but always told myself it was just for fitness and general health and I didn't need to be serious about it. My NF2 diagnosis changed all that. It made me double down and turned running into a truly passionate pursuit. Since then, I've broken every personal record I had. I've placed in races. I ran Ragnar DC (a 200 mile relay race). I ran a full marathon in under my target time, and have aspirations to qualify for the Boston Marathon someday. NF didn't keep me down. It fired me up. And running has brought me both the physical and mental well-being I've needed to not only survive, but thrive.



CLASSROOMS THAT CARE

Classrooms that Care is a youth-focused program that teaches students about neurofibromatosis, and then empowers them to make a difference through a school-wide change drive. NF parent Stacey DeCillis brought Classrooms that Care into her children's schools. We spoke with Stacey about her experience and the impact it had on her family and community.

What motivated you and your family to participate with Classrooms that Care?

We were motivated by a number of things, but first by the opportunity to raise additional funds for NF research in a way that got the kids involved. Our NF Hero Jackson is now 8 years old, and wants to actively participate in the fundraising efforts of the family. This program allowed him and his siblings, Madison (age 10) and Alexa (age 5), to also participate. In addition, some of Jackson's classmates had been asking him about his café au lait spots, asking why his back was "dirty." I realized that the best way for me to help him with his peers (in addition to our continuing work on high self esteem) is to teach his classmates about NF and show them what Jackson and other NF Heroes deal with on a daily basis.

Can you describe the program from your perspective?

The program includes both awareness and fundraising, so it educates kids about NF while also giving kids the opportunity to make a "change" through a change drive. Students get to experience some of the direct manifestations that Jackson faces, such as low muscle tone, during an "NF Experience" assembly, which brings empathy and understanding. Students then become active participants by reaching out to their parents and community, to explain NF and raise funds to help CTF fund research into effective treatments.

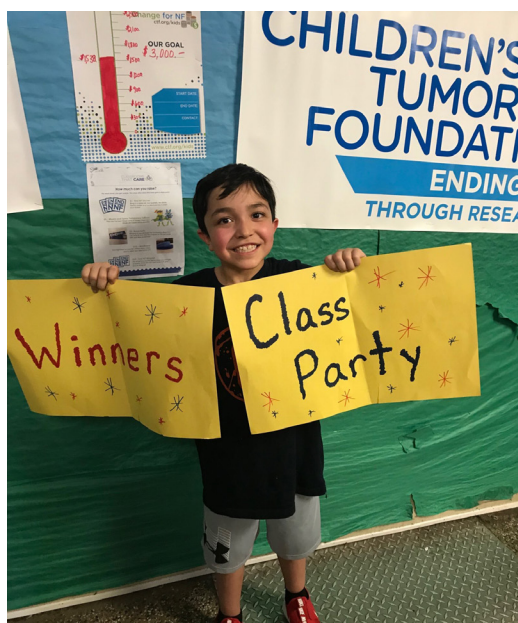
What were the steps you took to implement the program in the schools?

This program was extremely easy to imple-

ment. CTF has prepared materials and even an interactive presentation to explain NF and some of the symptoms. Depending on the district, you may need to reach out directly to administration, or at the secondary level, the person overseeing the student government. Once the contact is made, CTF's Youth Development contact Julie Pantoliano will help you move forward with everything you need.

How do you feel Classrooms that Care impacted the school children?

The program was delivered to the students in assemblies in which they learned about NF through an "NF Experience" presenta-



tion. Students then went home to raise funds and brought back their change boxes. The third and fourth grades collected more than \$1,700, and grades K-2 raised more than \$1,500. Most important, the students in both schools learned empathy not only for kids with NF, but for all students who may be facing challenges on a daily basis, or who may look or be different.

How impactful was the experience for Madison, as an NF sibling?

Madison has always been involved in Jack-



son's diagnosis. Madison has participated in NF Walks, and has even done a TED-Ed talk on "Why we need to find a cure for NF" in her school's club. This time, however, she was able to share with her classmates some of the struggles her brother faces and really take a leadership role in our family's fundraising. This program will hopefully help develop our next generation of volunteers for CTF!

What were the reactions of the teachers and school administration?

After doing Classrooms that Care, I was approached by many of the school educators who said that now they better understand what we have been trying so hard to explain. Now the alerts on his IEP mean more than just a diagnosis, and the professionals who work with him understand why his handwriting may be messy, why he rushes through his work, why sometimes he has a hard time waiting to be called, and why he has to stand at his desk because his newly diagnosed severe scoliosis (due to NF) causes him pain.

How was it meaningful for your family?

This experience was invaluable for us. Madison became a leader for her school's fundraising efforts and a spokesperson for her brother. Jackson was able to have his classmates and peers understand his disorder. One of the most heartwarming things I witnessed was from one of Jackson's friends, a second-grader who raised enough money to earn a CTF bracelet. One day at soccer practice, he ran up to his dad and exclaimed with such pride "Dad! I got my bracelet today for raising enough money for NF and for Jackson!" It was amazing to see a friend support Jackson with such pride! That is the true meaning of community service.

If you are interested in bringing Classrooms that Care into your child's school, go to: ctf.org/classrooms



Extraordinary Spirit / COOKIES FOR CARSON

On his way home from school one day, NF Hero Carson was picked on by another student. Carson's sister Millie and some other kids, including their neighbor Aiden, banded together to stop the bullying. After the incident, Millie explained NF to Aiden and the two decided to sell flowers (from the McNall's tree) to help the NF cause. Within a couple hours they decided to sell cookies instead, and got more kids to help. Aiden's mom coined the phrase "Cookies for Carson" and a fundraiser was born.

Carson is now 12 years old and was diagnosed with NF1 at 6 months old. Since then his life has been a series of doctor appointments. One year Carson went to over 170 therapy, speech, and doctor appointments with the goal to try to catch him up to the rest of his peers.

After seeing that number his mom stopped counting. But Carson is resilient! He constantly amazes his teachers with his perseverance and unique personality. He loves people, his family, animals, and video games. He is an avid Boy Scout who lives for the outdoors, and he is kind beyond words.

However Carson's biggest struggle is with fitting in socially. Earlier this year, as the rest of the family wrote down their new year's goals, Carson's goal really stood out. His only goal was to "make friends." Not to "make new friends" but to simply "make friends." NF has caused cognitive complications that make it more difficult for Carson to fit in to social norms, even though he desperately wants to. But even with setbacks, Carson keeps his focus on ways to be kind and to make friends.

"Carson is such a kind kid. When I asked him about the incident he simply said, 'Bullies are just being bullied themselves. So we need to help them,'" said Carson's mom Shannon McNall. "Kindness spreads and we are so grateful that the community wants to make this event happen to raise awareness for NF."



The Founders of Cookies for Carson: sister Millie McNall and neighbor Aiden Meyer.

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.

The Children's Tumor Foundation is a 501(c)(3) not-for-profit organization dedicated to funding and driving innovative research that will result in effective treatments for the millions of people worldwide living with neurofibromatosis (NF), a term for three distinct disorders: NF1, NF2, and schwannomatosis. NF causes tumors to grow on nerves throughout the body and may lead to blindness, deafness, bone abnormalities, disfigurement, learning disabilities, disabling pain, and cancer. NF affects 1 in every 3,000 births across all populations equally. There is no cure yet – but 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 www.ctf.org.

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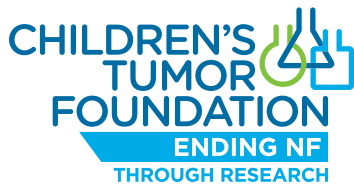
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SAVE THE DATE

2019 *National*
Gala

WEDNESDAY, NOVEMBER 20TH
Cipriani, 25 Broadway, New York, NY

ctf.org/gala

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