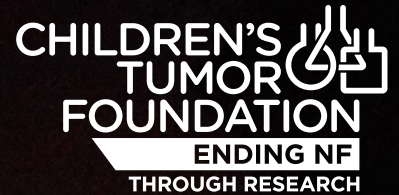


# NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION  
SPRING 2019



## MAY IS NF AWARENESS MONTH: Wear **Blue & Green** on May Seventeen

Each year, the Children's Tumor Foundation dedicates the 31 days of May to NF awareness, elevating the spirit, energy, and passion that the NF community exudes year-round. On World NF Awareness Day, we unite our voices and dedicate our sartorial choices: Wear Blue & Green on May Seventeen. We encourage you to show your support for our NF Heroes and the need for research by wearing the colors of the cause, blue and green on May 17, then posting your photos on social media with the #endnf hashtag.

*Continued on page 8*

### INSIDE:

- 3** MEK Funders Collaborate
- 4** Improved Treatments for LGG patients
- 9** Extraordinary Spirit - Sweet Tea for Sophie
- 10** The Thoms Family





# FROM the President

Annette Bakker, PhD



The month of May is NF Awareness Month, and a time for the Children's Tumor Foundation (CTF) to ignite the country through exciting outreach initiatives, campaigns, and advocacy efforts. We hope to shine through to make neurofibromatosis (NF) better known to the world. This unique time illustrates a tenacity that fuels the NF community—a determination to never give up in our fight against NF.

Throughout the month you'll see hundreds of buildings and icons across the country and around the world "Shine a Light on NF," turning their lighting blue and green to raise awareness of NF. There will be media stories about our NF heroes,

proclamations by civic leaders, and community events from Sweet Tea for Sophie stands to Shine A Light on NF Walks. We want more people to have heard of NF than when the month started. Knowing our NF family, this is a goal we will accomplish.

The Foundation's research portfolio has been focused on funding collaborative science – bringing together 'dream teams' of leading researchers from around the world, to tackle problems that you, our family of NF patients, have told us are most important. Our initiatives are aimed at accelerating the pace toward effective treatments, and ensuring that those treatments make their way to FDA approval.

Our family of patients and caregivers, who live each day with bravery and courage, perhaps uncertain where their NF story will take them next, inspire this focus. I am confident that together, our combined efforts will lead us to a cure for NF.

## ON THE HILL:

### CTF Advocates for Federal Funding of NF Research

This past February, Annette Bakker, President of the Children's Tumor Foundation, and Simon Vukelj, Chief Marketing Officer, visited the U.S. House of Representatives to advocate for the Congressionally Directed Medical Research Program-Neurofibromatosis Research Program (CDMRP-NFRP). CTF requested Congress support the NFRP by continuing a \$15 million investment in NF research in fiscal year 2020. CTF had 13 meetings with staff for Members of Congress who sit on the House Committee on Appropriations Subcommittee on Defense: Full Committee Chairwoman Nita Lowey (D-NY), Subcommittee Chairman Peter Visclosky (D-IN), Subcommittee Ranking Member Ken Calvert (R-CA), and Reps. Hal Rogers (R-KY), Dutch Ruppersberger (D-MD), Peter Aguilar (D-CA), Tom Cole (R-OK), Tim Ryan (D-OH), Robert Aderholt (R-AL), Steve Womack (R-AR), Henry Cuellar (D-TX), Charlie Crist (D-FL), and Betty McCollum (D-MN). Staffers were enthusiastic about the progress made due to the unique CTF research model, which coordinates CTF, Department of Defense (DOD), and National Institutes of Health (NIH) NF research. Notably, staff suggested that the CTF model should be viewed as the preferred template for all CDMRP research areas. Following the meetings, CTF submitted written appropriations requests to all lawmakers on the House Committee on Appropriations Subcommittee on Defense. As the FY 2020 appropriations process progresses, CTF will continue to serve as a trusted resource for Members of Congress and advocate for NFRP funding.

CTF also is a member of the Defense Health Research Consortium (DHRC), and recently represented the DHRC with four other consortium members at a meeting in March with Republican and Democrat staff from the Senate Committee on Appropriations Subcommittee on Defense. Staff voiced their strong support of the CDMRP and said the Chairman and Ranking Member are committed to supporting the various CDMRP research programs.

## Board of Directors

### Executive Members

Richard Horvitz  
*Chair*

Tracy Galloway  
*Vice Chair*

Randall Stanicky  
*Treasurer*

Gabriel Groisman  
*Secretary*

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

Linda Halliday Martin  
*Chair Emeritus*

### Board Members

Daniel Altman  
Amy Boulas

Robert Brainin  
Daniel Gilbert  
John Golfinos, MD

Simone Manso  
Steven L. McKenzie

Renie Moss  
Laura Perfetti

Michael Peterson  
Scott Plotkin, MD, PhD

Kenneth Rudd  
Richard Soll

Stuart Match Suna

Rachel B. Tiven

Peggy Wallace, PhD

### Honorary Directors

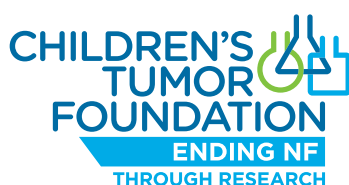
Suzanne Earle  
Michie Stovall O'Day

Alan Robbins, MD  
Carolyn E. Setlow

Nate Walker  
Ed Stern,

*Pro Bono Counsel*

Allan Rubenstein, MD  
*Director of Medical Affairs Emeritus*





## Funders Collaborate to Efficiently Develop Treatments for Rare Childhood Tumors

One of the exciting developments in neurofibromatosis (NF) research today is the success of MEK inhibitors in clinical trials for various NF1 manifestations. For example, a recent *New England Journal of Medicine* paper showed that in a phase I study of selumetinib, 70% of children with progressive or symptomatic plexiform neurofibromas had a reduction in tumor volume of 20-55%. The phase II results of this study are looking equally promising which may support this becoming the first-ever approved drug for NF.

We've arrived at this important moment because federal and private funders have come together to successfully find drugs for rare childhood tumors by maximizing the efficiency of grant spending. Five years ago, the Children's Tumor Foundation brought together the major funders in the NF field to proactively and strategically coordinate to ensure

that NF funding is efficient and not duplicated, and to include the patient voice in the middle of the partnership. The funders involved in this "MEK story" are the Children's Tumor Foundation (CTF), the National Institutes of Health (NIH) – including the Pediatric Oncology Branch of the National Cancer Institute (NCI) and the National Institute of Neurological Disorders and Stroke (NINDS), the Congressionally Directed Medical Research Programs (CDMRP), and the Neurofibromatosis Therapeutic Acceleration Program (NTAP) at Johns Hopkins University.

This unique collaboration has brought selumetinib along its path from repurposed oncology drug, to testing in preclinical consortia for its applicability to NF, to its current clinical success in NF patients, with the potential to become important to a broader group of cancer patients as NF1



is a common driver mutation in many cancers such as melanoma, lung cancer, and breast cancer. Keeping in mind that this is a first step, and that more treatments are under development for the many different types of NF – including NF1, NF2, and schwannomatosis – this example of how funders can work together not only serves as a model for NF research endeavors, but has the potential to be applied to other disease areas.

In order to make this example more readily available to all researchers, the funders have just published a paper outlining the steps to this achievement, and the impact that it will have for the future of NF patients. That paper, titled "Delivering on the Vision of Bench to Bedside: A Rare Disease Funding Community Collaboration to Develop Effective Thera-

pies for Neurofibromatosis Type 1 Tumors" is available at [ctf.org/funders](http://ctf.org/funders).

Fortunately, the growing interest in NF is drawing the attention of many pharmaceutical and biotech companies who are growing the pipeline for both NF1 and NF2, with MEK and other still-developing drug candidates. Their significant efforts – and investments – are generating a revolution in the possibilities for NF research and in cancer in general.

Our common goal is to end NF in all its forms, for all NF patients. By working together, we can take complex NF research – in this instance, from the validation of the MEK target and through the necessary steps to show clinical activity – and turn that research into results that improve the lives of NF patients and their families.



### NEW CLINICAL TRIAL FROM SPRINGWORKS THERAPEUTICS

CTF's partner organization SpringWorks Therapeutics has a MEK inhibitor (PD-0325901) being evaluated for NF1 patients with plexiform neurofibromas. The company expects to start an open-label Phase 2b study in pediatrics, adolescents, and adults with plexiform neurofibromas this summer. More information about the trial will be available at [ctf.org/news](http://ctf.org/news) in the coming months when the study is open for enrollment.





## A Letter of Thanks to the NF Community

*Robert A. Avery, DO, MSCE, is a pediatric neuro-ophthalmologist in the Division of Pediatric Ophthalmology at Children's Hospital of Philadelphia. He sent this letter to CTF President Annette Bakker and to you, our family of donors.*

Dear Annette,

I wanted to provide some feedback about the CTF "Clinical Research Awards" (CRA). Back in 2010, numerous foundations rejected my grant applications to study optical coherence tomography in NF1-associated optic pathway gliomas (NF1-OPGs). CTF believed in my work and generously provided a \$94,000 CRA to continue that work. The data from my CTF CRA was critical to me being awarded a career development award from the National Institutes of Health (NIH) award for \$1.1 million. In 2014, you and your team boldly supported the international multi-center NF1-OPG Natural History study lead by Michael Fisher, MD, and myself. To further support this huge undertaking and advance our knowledge of visual field loss in NF1-OPGs, you also funded a \$150,000 CRA lead by Gena Heidary, MD, PhD.

In the spirit of "Team Science," Drs Heidary, Fisher, and I leveraged our collective expertise and research—all supported by CTF—to apply for a grant from the NIH. I am excited to report that this \$3.4 million grant was officially funded yesterday. This five-year study includes three clinical sites (Boston Children's Hospital, Toronto Sick Kids, and Children's Hospital of Philadelphia) as well as two additional sites for advanced analysis (University of Pennsylvania and New York University).

On behalf of my co-investigators, we would again like to thank you (and all those who donated to CTF) for believing in our projects and funding our CRAs. We are proud that our CTF awards have now produced over \$5 million in federal research grants.

Sincerely,

Rob Avery, Michael Fisher, and Gena Heidary

## 2018 Clinical Research Awardee

*The Clinical Research Award program supports early stage pilot clinical trials of candidate therapeutics, or interventions for treatment of physical or psychosocial manifestations of NF1, NF2, and schwannomatosis.*



**L-Carnitine Safety and Effects on Muscle**  
**Aaron Schindeler, MD**  
*Children's Hospital at Westmead, Australia*

Low muscle tone, muscle weakness, and high levels of fatigue are often concerns for individuals with NF1. Evidence suggests that an underlying change in metabolism may be a cause. Both human NF1 muscle biopsies and NF1 mouse models show a build-up of lipid (fat) droplets in muscle cells. In the mouse model, treatment with the nutritional supplement L-carnitine reduced muscle lipid and improved fatigue resistance, as reported by Aaron Schindeler, MD, of The Children's Hospital at Westmead, Australia. Based on these findings, CTF recently awarded Dr. Schindeler a \$20,000 Clinical Research Award for a pilot study of L-carnitine supplementation to determine whether this intervention is safe and to explore whether it can improve quality of life and functional outcomes in children with NF1.

## CTF RESEARCHERS SECURE FEDERAL FUNDING

Ana-Maria Vranceanu, PhD, Associate Professor of Psychology at Harvard Medical School and founder and director of the Integrated Brain Health Clinical and Research Program at Massachusetts General Hospital was recently awarded a \$1.5 million dollar Clinical Trials Award from the Department of Defense for her project entitled "Resiliency training in adolescents with NF1 and NF2; A randomized controlled trial via secure live video to improve emotional, social and physical health." This upcoming clinical trial will compare two different stress resiliency programs. Two hundred English-speaking adolescents (ages 12-17) who report challenges coping with stress and NF symptoms will be enrolled in one of the two programs. Adolescents will meet in small groups (5-6 participants) via a secure platform called Vydio that can be easily accessed on a smartphone or computer. A trained clinical

psychologist with expertise in NF will lead the sessions. Adolescents in each group will meet for 45 minutes over 8 consecutive weeks. At the end of the study we will be able to understand which of the two programs works best in improving emotional, social, and physical function in adolescents.

This work is based on a pilot study funded by the Children's Tumor Foundation, which showed that adolescents enjoyed participating in the resiliency programs and improved their quality of life after participation. When the study is complete CTF will also help to make the intervention available for all adolescents in need.

**“By teaching adolescent stress management and resiliency skills at this critical time, there is tremendous opportunity to improve the trajectory of their quality of life.”** —ANA MARIA VRANCEANU, PhD



## Selumetinib granted US Breakthrough Therapy Designation in NF1

AstraZeneca and Merck & Co., Inc. announced that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) for the MEK 1/2 inhibitor and potential new medicine selumetinib. This designation is for the treatment of pediatric patients aged three years and older with NF1. Selumetinib was granted Orphan Drug Designation for the treatment of NF1 by the US FDA in February 2018 and the European Medicines Agency in August 2018.

“This new designation validates our ongoing development of selumetinib. As a result of this, selumetinib has the potential to receive expedited regulatory review and we hope to bring this medicine to patients as soon as possible.”

—ROY BAYNES, MSD RESEARCH LABORATORIES

Breakthrough Therapy Designation is designed to expedite the development and regulatory review of medicines that are intended to treat a serious condition and that have shown encouraging early clinical results, which may demonstrate substantial improvement on a clinically-significant endpoint over available medicines.

## Open Science and Data Initiative Announced for Neurofibromatosis

The Children’s Tumor Foundation, together with the Neurofibromatosis Therapeutic Acceleration Program (NTAP) and Sage Bionetworks (Sage), has announced the first-ever open data portal for scientific research results in the field of neurofibromatosis. The NF Data Portal marks the first major milestone in all three organizations’ commitment to the development of the larger NF Open Science Initiative (NF-OSI), which draws experts from across research disciplines and disease areas in order to develop treatments for neurofibromatosis.

The unique challenge for researchers studying NF, and particularly for patients living with the disease, is that NF is extremely variable. This makes diagnosis and treatment particularly difficult, and as a result, many patients are told to ‘watch and wait’ as their tumors grow. Researchers working on the disease have likewise faced the challenge

of working in independent groups and relying primarily on data made public through published works.

The Children’s Tumor Foundation looked to turn this ‘traditional research model’ on its head, and in 2014 launched the NF Open Science Initiative, in collaboration with Sage Bionetworks, in order to enable CTF-sponsored project teams to share their data in real-time through Sage Bionetworks’ Synapse platform. This was started in connection with the Foundation’s Synodos research model, which brings together multidisciplinary experts from leading institutions and across basic, translational, and clinical disciplines, in order to solve complex NF issues.

This spirit of collaboration has attracted other significant partners, and in 2015, CTF committed to supporting data collection and integration from a large National Cancer Institute (NCI) project, called DHART SPORE (Develop-



mental and HyperActive Ras Tumor Specialized Programs of Research Excellence). That same year, NTAP initiated a relationship with Sage requiring that data generated through NTAP-funded initiatives be uploaded, harmonized and indexed within Synapse. Across all of these projects – from CTF to NTAP to the NCI - Sage Bionetworks interacts directly with the project scientists to coordinate data upload and organization, and applies expertise in computational biology, oncology, data harmonization, and community-building to expand and enhance the value of the data generated across all of these initiatives.

All content in the NF Data Portal is compatible with global scientific

initiatives to establish common vocabularies and protocols for sharing data across all diseases. Furthermore, the NF Data Portal hosts analysis tools to integrate different modalities of data, including drug efficacy across model systems, drug-target analysis, clinical trial data, and molecular adaptive responses in patients. The datasets coming from funded research are subject to a short embargo period (generally up to 18 months), during which the researchers will use the Data Portal for their own research before releasing the dataset to be shared with the entire research community.

The NF Data Portal can be accessed at: [www.nfdataportal.org](http://www.nfdataportal.org)

## NF Clinic Network Ensures Quality Care

The Children's Tumor Foundation has distributed over \$2.3 million in stipends to members of the NF Clinic Network (NFCN) to support quality NF care and educational enrichment for both patients and professionals. There are now 55 NF clinics in the U.S. that meet CTF standards for providing quality specialized care and participate in the NFCN.

In 2018, the NFCN served a total of 14,187 individual patients with some form of NF. The 2018 clinic education stipends supported 27 educational or

symposium events throughout the country.

Each year, as they apply for CTF stipends, member clinics send reports to CTF detailing patient volume, as well as clinic staffing and organization, research participation, and involvement with the CTF community. This information is made available at [ctf.org](http://ctf.org) to assist patients in finding the best clinic for their needs.

Some of the highlights of this year's report are the following:

- NFCN clinics average 2.9 physicians serving as a main

NF care provider

- Among all clinics, 38 nurse practitioners and 56 genetic counselors routinely see NF patients
- More than half report that they enroll patients for on-site NF clinical trials or clinical research

To encourage young practitioners to get involved in NF care, in 2018 CTF launched a new stipend category, awarding ten 'First-Time Conference Attendee Stipends' to be used for clinic members or trainees who have not previously attended the NF Conference.

## Furthering Patient Engagement Initiatives

The Children's Tumor Foundation was invited to join an expert panel from around the world, with a goal of developing a tool to identify practices about patient engagement with a high potential for reaching the desirable outcome in the design of Clinical Trials. Traceann Rose, CTF Director of Patient Engagement, attended the face-to-face meeting in Brussels sponsored by Paradigm, a public-private partnership, co-led by the European Patients' Forum and European Federation of Pharmaceutical Industry and Associations. At this meeting, a group of 18 organizations decided upon criteria and categories that will compose the evaluation tool for patient engagement practices.



### A Huge Thank You to **Dave Viskochil**

CTF extends a huge thank you to Dave Viskochil, MD, PhD from the University of Utah for his many years of dedication as the Chair of the Clinical Care Advisory Board (CCAB). Dr. Viskochil has made numerous contributions to the field of NF. As the Foundation's CCAB Chair, he has led the expansion of the NF Clinic Network to the current number of 55 NF Network Clinics (five years ago, it was only 40). He has served as an invaluable advisor to CTF on decisions about clinical research awards, approval of registry and biobank projects, and review of patient-

oriented materials. He continues to be an active member of the CCAB and many other CTF-related efforts (at NF summer camp, he is known as "Dr. V").

Thanks Dr. V, for being such a great colleague and leader within our organization, sharing a common goal of improving the lives of individuals living with NF.

As of November 2018, following Dr. Viskochil's tenure, Scott Plotkin, MD, PhD became the new chair of the CCAB.

“Becoming a Patient Advocate has been very important to me, because in the past, I didn't feel like I had a voice. But now as a Patient Advocate, I feel connected to the doctors and the medical community.”

**SHANNON MCNALL**

GRADUATE OF CTF'S PATIENT ENGAGEMENT TRAINING COURSE



# MAY is NF Awareness Month!

Visit [ctf.org](http://ctf.org) to learn more about all the ways **YOU** can take action to **End NF!**



## WEAR

our exclusive gear to show off your CTF pride



## SHINE

a Light in your community and add to the growing list of landmarks that will glow in blue and green



## CHANGE

your profile picture to one that celebrates NF Awareness



## TAKE

a photo and submit it in the 'Picture a World Without NF' contest



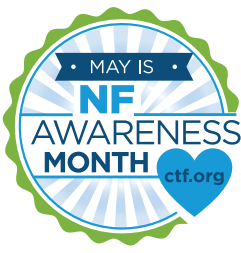
## TAG

your photo(s) on social media with #EndNF



## LIKE

Children's Tumor Foundation on social media



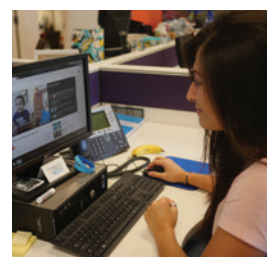
## SHARE

exclusive NF Awareness Month graphics



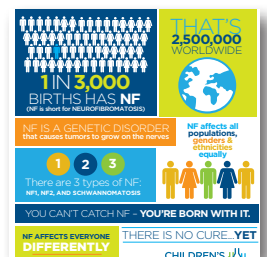
## PARTICIPATE

in an NF event in your community



## WATCH

our NF Awareness Month videos on our YouTube channel



## EDUCATE

using informative fact sheets and brochures



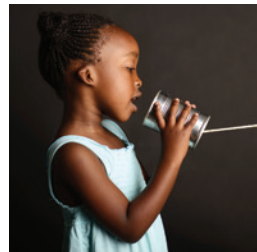
## JOIN

the NF Registry if you or your child has NF



## DONATE

to fund the cutting-edge research that will end NF



## COMMUNICATE

Spread the word and increase awareness about NF!



If we found a cure for NF tomorrow, how would that make you feel?

Enter the 2019 Children's Tumor Foundation NF Awareness Month "Picture a World Without NF" photo contest! Visit [ctf.org/pictureaworld](http://ctf.org/pictureaworld) and submit your entry. The photo must include CTF-branded gear. Contest ends May 31, 2019.

There is currently no cure for this genetic disorder that affects 2.5 million people worldwide, but through our shared efforts to raise awareness and raise money to drive research, together we will be successful in our mission to end NF.

Download your NF Awareness Month resources at: [ctf.org/nfawareness](http://ctf.org/nfawareness)





“Her big brothers wanted to find a way to raise funds and awareness for vital research being done by the Children’s Tumor Foundation. They found that selling sweet tea for their baby sister was a perfect way to do just that.”

—SHANNA NELSON, SOPHIE’S MOM



## Extraordinary Spirit / SWEET TEA FOR SOPHIE

**S**ophie Nelson is full of personality and sass! She’s strong and independent, always insisting that she do things “all by myself.” She loves to dance and play with her big brothers. And when she grows up she wants to be a deep sea diver!

When Sophie was two years old, she was diagnosed with NF1. Sophie’s brothers, Caleb and Liam Nelson, were just 5 and 3 years old then, but they wanted to do something to help their sister. They knew that Sophie had something wrong with her that meant she had to see a lot of doctors, so they asked their parents if they could start a lemonade stand to raise money to help find a cure for her condition.

“We scrambled to throw it together,” says their dad, David Nelson. “But we didn’t have any lemonade. We did have sweet tea though, and that’s Sophie’s favorite, so we did a sweet tea stand instead.” The family set up their first sweet

tea stand on their front lawn in Mint Hill, North Carolina in 2016, and they made \$125.

Sophie is now 6 years old, and her parents consider themselves extremely lucky. Sophie is growing beautifully and, while there are things that make her life a little difficult, nothing is, at present, terribly frightening.

The Nelson family is focused on awareness and they’ve found that hosting Sweet Tea for Sophie is a fun way to get all three of their kids involved, as well as other families across the country.

That first year, friends and family hosted 40 Sweet Tea for Sophie stands in 7 states raising \$7,000, and now it has grown to 145 stands across 17 States. With your help, it will continue to spread.

This family has a goal to have a sweet tea stand in every zip code! As they fill in the map, it means that awareness about NF is spreading across the country.

### How You Can Participate With SWEET TEA FOR SOPHIE

#### SWEETEST DAY OF THE YEAR

May 4th, 2019

You can host a Sweet Tea for Sophie stand on any day of the year, however once a year, we pick a special day to try to have as many stands around the country as we can! We call it the Sweetest Day of the Year, and in 2019, it will kick off NF Awareness Month on May 4th.

To see all of the sweet things we are doing throughout the year, go to:

[sweetteaforsophie.org](http://sweetteaforsophie.org)

Thank you to **McAlister’s Deli** and the **DMAC Franchise** for generously supporting Sweet Tea for Sophie in their locations throughout North Carolina and in Chicago. We raise our tea glasses to you for your sweet support of our effort to End NF!



## The Thoms Family

**V**arsity Painting owner Roland Thoms spoke with the Children's Tumor Foundation about his family's inspiring journey since his daughter Camille was diagnosed with NF2. The Varsity team of friends, employees, and customers worked together over the years to raise more than \$650,000 toward research into treatments for NF. We are extremely grateful to the Thoms Family, including Roland's father James Thoms, who is making a significant contribution to the Foundation this year.



The Thoms Family: Ash, Camille, Roland, Nicole, and Carter

Camille and her grandfather, James Thoms

**CTF: Can you tell us about your journey with NF and what led to Camille's diagnosis?**

Camille was born with a cataract in her left eye, and she had a small surgical procedure to remove a neurofibroma when she was almost two years old, but she wasn't diagnosed until she turned seven. The circumstances of her diagnosis were unique – she was presenting with tongue spasms (her tongue would spasm uncontrollably).

We took her to the hospital, and they did a CAT scan and then an MRI, and they came in and said, "Mr. and Mrs. Thoms, your daughter has a brain tumor." That absolutely floored us. They scheduled Camille for immediate surgery to remove a tumor that was about the size of a tangerine from her 7-year-old brain.

We tried to stay strong in front of her, but privately we were on our hands and knees in tears. But thankfully she had a very successful surgery; they removed the tumor and she came out with flying colors. But of course, the other news was that she had a diagnosis of neurofibromatosis type 2, NF2. So that began the journey.

**CTF: How is Camille doing now?**

That was when she was 7, and today she's 21 years old, so we've had 14 years of MRIs multiple times a year, and we've been monitoring the fibromas and tumors that are growing. We consider it a good year when we don't do a surgery, but each year often brings a surgery. She's had surgery twice on her left ear to remove the two neurofibromas that have rendered her

deaf in that ear. She's legally blind in her left eye because of the cataract. She has schwannomas up and down her spine, a large bladder tumor, a few surface lesions, a growth on her neck, and some smaller neurofibromas on her back and arm. So we keep an eye on all of those.

I'm proud to say that now she's a thriving junior at the University of Utah, and loves life! She's studying child therapy and wants to work with children who are going through medical challenges as they come to the hospital, and help guide them through that. She's transitioned in the last three or four years into an adult who can accept that this is who she is, and that NF does not need to define her. She faces her challenges bravely.

**CTF: How did you first learn of the Children's Tumor Foundation, and how did you get involved?**

The year after Camille had her surgery, so in October of 2005, we held a Halloween party at our house with the theme "Celebrate Life," which has become our family's motto. And it was a big success, so for the next few years we kept doing it, but we added a fundraising component and my father Jim Thoms would match whatever we raised. We raised around \$400,000 in those years.

In the past four years, I've become more involved with the Children's Tumor Foundation. I've had a long relationship with Tracy Galloway, who is a great NF warrior. I've also had the opportunity to meet and speak with the CTF President Annette Bakker on several occasions, and I'm very impressed

with her resolve. So we've raised about \$300,000 for CTF in the last few years.

**CTF: Can you tell us a little bit more about the Halloween Bash?**

My wife and I organize it every year – my wife is really the star here. Every year the Halloween Bash has a theme. We had a toga theme, an 80s theme, a 70s theme, a Rocky Horror theme, and so on. This past year it was a pajama theme, and we had a huge pillow fight!

**CTF: Your father, Jim Thoms, is very devoted to Camille and this organization. Can you tell us more about him?**

My father approached me in early December, and said, "I'd like to make a sizable donation." We did a bit of research to figure out how he could do it, because my father lives in Canada. With the help of the Bedouin Foundation, he was able to make a donation of \$750,000 (Canadian dollars).

What I really want people to know is that, even though my wife and I hold the Halloween Bash, the real heroes in this story are my daughter, Camille Thoms, and my father, Jim Thoms. My father has donated over a million dollars toward NF research over the years, and he would give his life to help his granddaughter. And at the same time, Camille is a true warrior. She faces each day and challenge as it comes. Her balance is an issue, her eyesight and hearing are compromised, and yet she is so brave and strong. I would do anything I could to change things for her, but despite her challenges, she keeps on moving forward. She is just incredible.



# stories

OF NF



## Victor Santiago

**M**y son was born with a café au lait spot on his back, so immediately his pediatrician referred us to a pediatric neurologist. She ordered the NF1 test when he was a one-year-old and the results were positive. At the age of five he was tested again to know his mutation, and the results were that he has a *de novo* mutation.

Even though he has NF1, the most challenging aspect of his condition has been his hearing loss. Victor's hearing loss has impacted his

communication skills, and he also has learning disabilities.

At the beginning, it was not easy for me or for my family. We had never heard about the condition and the prognosis was really scary. We didn't know what to expect. Now he is 12 years old and he has a lot of energy, he's always happy, and he is a smart kid. I am always paying attention to any changes on his body or in his behavior. I have done everything in my power to stay positive and help him along this process, taking him to all his appointments and learning as much as I can about NF.

—Victor's mom, Jossie

*"My name is Victor, I am 12 years old and I am in sixth grade. I love to play video games and board games, my favorite is chess. I love the beach and spending time with my family. I have a Chihuahua, her name is Paw. I love to play violin and I am really silly. People are happy when they are around me and they always say good things to my mom about me."*

— Victor



## Brianna Worden

**B**rianna was diagnosed with NF1 when she was 6 months old. She has had several surgeries to debulk large tumors in her left arm, and has had spinal fusion surgery. She has also had three

malignancies in peripheral nerve sheath tumors.

Brianna is a constant role model and inspires everyone she meets. She raises awareness for neurofibromatosis everywhere she goes. She highlights what it is like to live (and succeed) with NF on her YouTube channel, and she mentors other NF patients online through her NF Facebook page. She was named Miss Teen New York in 2013 and used that platform to talk about NF and self acceptance. She is truly an inspiration!

A senior at Boston University, Brianna is studying psychology and human development. She plans to work with children with special needs and medical challenges.

Brianna loves spending time with her family; she loves the fall season and all of the pumpkin and apple spiced goodness that comes with it!

— Brianna's mom, Janna

## ask Kate A YOUTUBE RESOURCE

**Kate Kelts, RN, BSN  
Patient Support Coordinator**

**I** joined the Children's Tumor Foundation in 2015 as the Patient Support Coordinator, and since that time I have explored many different ways to support and connect with the NF

community. I get the opportunity to email or speak with patients and families all over the world looking for answers, and fighting for better care for themselves or a loved one. There are many questions that are asked frequently because there are challenges that are common to a lot of our NF fighters. This work was the inspiration for our most recent project, "Ask Kate!"

"Ask Kate" is a YouTube series started in late 2018 in which I record videos, answering

questions related to NF. In the beginning I covered a lot of NF basics, and as time has gone on and participation has increased, I've been able to respond directly to the great questions I get in response to these videos. "Ask Kate" is simple, interactive, effective, and easy to watch. I would love for the NF community to subscribe to CTF's YouTube channel and watch "Ask Kate", and respond with your own stories and questions!

# 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 [www.ctf.org](http://www.ctf.org).



Event committee members **Laura Perfetti, Cristina Spoto, Lisa Arena, Debra Acker, and Victoria Romano**

## COCKTAILS FOR A CURE

Cocktails for a Cure took place on February 2, 2019, and was an amazing event, raising nearly \$90,000. With moving speeches and a live NF Hero art auction, the energy was most certainly in the room that night. Special thanks to our dedicated committee members **Laura Perfetti, Cristina Spoto, Lisa Arena, Debra Acker, and Victoria Romano**.



## Duty Free Americas

The Children's Tumor Foundation is exceptionally thankful for the generous support of Duty Free Americas and the Falic Family Foundation through their successful golf and tennis charity tournament held in Miami on January 31, 2019. Under sunny skies and with the participation of an enthusiastic and big-hearted crowd, over \$325,000 was raised to support our mission to fund critical NF research. We are particularly grateful for the leadership in this effort shown by **Simon Falic, Jerome Falic, Leon Falic** and the entire Falic family.

On hand to accept the check on behalf of the Foundation was our 2019 NF Ambassador **McKinnon Galloway**, who spoke touchingly about being diagnosed with NF2 at the age of 16, how it continues to impact her life, and the critical importance of research: "This pain motivated me to share my story. I can lose my hearing, sight, balance, and I know my life is on the line. Your donations allow the Foundation to develop treatments that allow me to stand before you."

We wholeheartedly thank Duty Free Americas, the Falic Family Foundation, and all those who participated at this great event for their kind generosity and their belief in the work of the Children's Tumor Foundation. Together, we are making a difference in the lives of all those who live with NF.



## CLASSROOMS THAT CARE

### Two Counties, One Cause

Tappan Zee High School hosted the third annual "Two Counties, One Cause" boys basketball tournament fundraiser on January 12th, 2019. The boys high school basketball teams of Westchester County and Rockland County came together once again for a great cause! Last year, thanks to the communities of six participating tournament teams, the Tappan Zee High School Boys Basketball team raised \$14,000 to fund critical research for neurofibromatosis. This year's event hosted ten teams and raised over \$25,000 for the Children's Tumor Foundation.



## CUPID'S UNDIE RUN

### 10 Years of Baring it All for NF Research

This February, Cupid's Undie Run celebrated 10 years of bringing awareness to neurofibromatosis and raising money to benefit the Children's Tumor Foundation. To date, nearly 90,000 runners have turned heads for this cause!

Cupid's Undie Run is a great reminder of what a generous, passionate, and sometimes crazy group of people can do to advance NF research whether they are personally affected or not.



# SHINE A LIGHT WALKS 2019

MAY 11  
Salt Lake City, UT

MAY 18  
Idaho

MAY 18  
Cincinnati, OH

JUNE 1  
North Carolina

JUNE 8  
New England

JUNE 22  
Philadelphia, PA

JULY 21  
Portland, OR

AUGUST 10  
Iowa

SEPTEMBER 7  
Central Florida

SEPTEMBER 7  
Chicagoland

SEPTEMBER 7  
Virginia

SEPTEMBER 8  
Moorhead, MN

SEPTEMBER 14  
Jacksonville, FL

SEPTEMBER 14  
Westchester, NY

SEPTEMBER 15  
Addison Oaks, MI

SEPTEMBER 28  
Denver, CO

SEPTEMBER 28  
Bay Area, CA

OCTOBER 5  
Kansas City, MO

OCTOBER 5  
New Jersey

OCTOBER 12  
San Antonio, TX

OCTOBER 19  
Los Angeles, CA

NOVEMBER 16  
San Diego, CA

TBD  
Alabama  
Atlanta, GA  
Houston, TX  
Long Island, NY  
Phoenix, AZ  
Rochester, NY  
Seattle, WA  
South Florida  
Washington, DC

Sign up today at  
[shinealightwalk.org](http://shinealightwalk.org)



Shine a Light NF Walk is the signature fundraising event of the Children's Tumor Foundation (CTF), bringing neurofibromatosis (NF) out of the shadows and inspiring the community to come together to raise critical funds for NF research.

For over a decade the Children's Tumor Foundation NF Walk has made great impact on NF research and awareness, helping fund critical research that has resulted in promising discoveries that are bringing more knowledge to the NF field.

Our work is far from done, so we've given the NF Walk a jolt of new energy as the newly named Shine a Light NF Walk. Leveraging the tremendous success of our Shine a Light on NF initiative, which lights up hundreds of buildings, monuments, and bridges each May in blue and green, the new NF Walk shines with renewed energy, offering activities that inspire and help bring attention to NF, and of course, even more opportunities to raise funds needed to solve NF.

Join CTF to Shine a Light on NF! Visit [shinealightwalk.org](http://shinealightwalk.org) to learn more!



## South Florida NF Walk

On December 8, 2018 members of the South Florida community came together on a picture perfect Florida day to honor local NF Heroes and raise money at the NF Walk South Florida. The walk, organized by Amy Midlarsky, Rasheena Taub, Amy Bermudez, and Sherri DaPaolo, took place at Royal Palm Beach Commons Park. More than 200 members of the community came out to support our heroes. Following the program, an obstacle course challenge was completed, symbolic of the obstacles faced by those living with NF every day. After the walk, participants were treated to lunch courtesy of Chik-Fil-A, Panera Bread, Wawa, and Indian Spring Country Club. In addition to raising \$63,000 for the Children's Tumor Foundation, a raffle was held to sponsor a child to participate in NF camp this summer. The event was a huge success, and the local community came together to support our NF Heroes!



## NFWALK



## San Antonio NF Walk

On October 13, 2018, San Antonio held its 3rd Annual NF Walk at Eisenhower Park and raised more than \$30,000 for the CTF mission. Despite intermittent rain, over 200 people joined the morning's activities to honor and recognize all the NF Heroes. The Kids Dash was a wonderful way to kick off the scenic 2 mile walk! Upon returning from the walk the kids (and kids at heart) enjoyed activities including face-painting, balloon twisting, rock painting and meeting The Batman of San Antonio, Sparky the Fire Dog, and the San Antonio Fire Department.



## Atlanta NF Walk

Walk Organizers Melissa Lee and Karen Trommer, along with their amazing committee, hosted the 6th annual NF Walk Atlanta on Sunday, November 11, 2018 at Piedmont Park. Many families and friends joined together on a chilly Sunday morning in Atlanta to enjoy great music by DJ Ressie Cup, face painting by Fabulous Faces, delicious food by Taco & Tequila and many fun games. Members of the Georgia 501st Star Wars group and the Cosplay Volunteers of Atlanta helped to entertain all who attended. Each year we gather together to celebrate all of our NF Heroes and raise awareness of neurofibromatosis. This year's walk raised over \$32,000 to help end NF!



## UPCOMING EVENTS

**Colfax Marathon, Half-Marathon, 10-Miler, 5K, & Relay**

May 18, 2019 | Denver, CO

**America's Finest City Half Marathon & 5K**

August 18, 2019 | San Diego, CA

**Tour of Napa Valley Cycling Ride**

August 18, 2019 | Calistoga, CA

**Faxon Law New Haven Road Race**

September 2, 2019 | New Haven, CT

**Bank of America Chicago Marathon**

October 13, 2019 | Chicago, IL

**TCS New York City Marathon**

November 3, 2019 | New York, NY

**Indianapolis Monumental Marathon, Half-Marathon, 5K & Kids Run**

November 9, 2019 | Indianapolis IN

**California International Marathon**

December 8, 2019 | Sacramento, CA

**Rock 'n' Roll Race Series**

Nationwide throughout 2019, choose from 5K to Marathon

**Choose Your Own Challenge**

Anytime! Anywhere!



The checkered flag is waving on Team Garrett! This team's seventh and final showing will be at the 2019 America's Finest City (AFC) Half-Marathon & 5K. This dedicated group has raised over \$120,000 at AFC running in honor of NF Hero Garrett Baumann, and this year they've set their highest-ever fundraising goal in hopes of going out with a bang. To learn more about this amazing team, go to:

[nfendurance.org/teamgarrett2019](http://nfendurance.org/teamgarrett2019)

“ At only six months old, we don't know how Lydia will be affected by NF. But raising funds for CTF is an investment in her future. I truly believe that CTF's mission of advancing care for the NF community will directly benefit my daughter in the years to come. I feel empowered!

—BETH MCKENNA,  
NFE ATHLETE

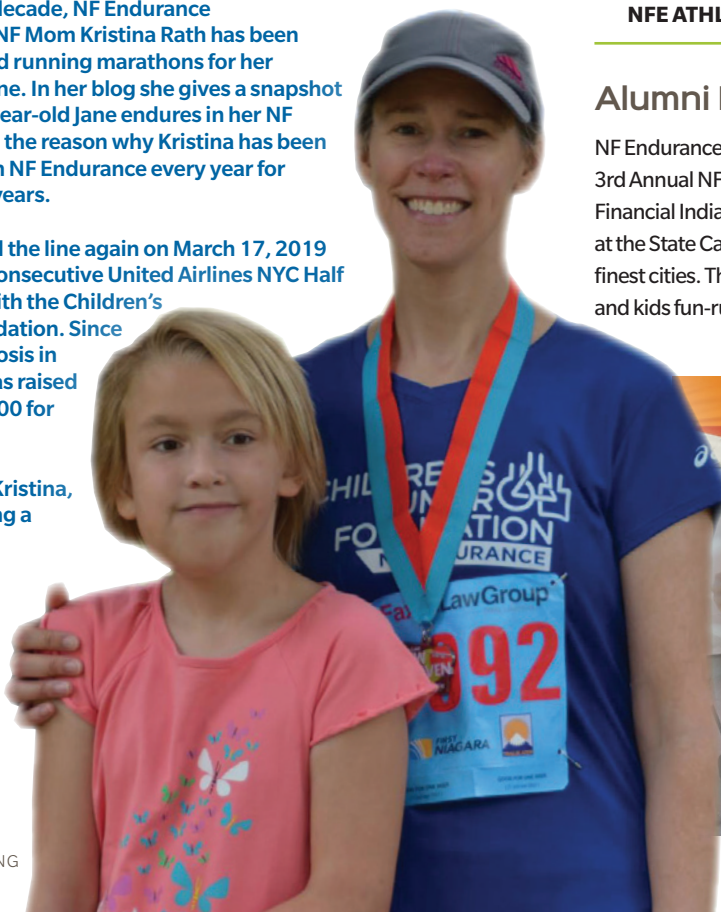


For the last decade, NF Endurance athlete and NF Mom Kristina Rath has been blogging and running marathons for her daughter, Jane. In her blog she gives a snapshot of what 11-year-old Jane endures in her NF journey, and the reason why Kristina has been running with NF Endurance every year for the past 10 years.

Kristina toed the line again on March 17, 2019 for her 4th consecutive United Airlines NYC Half Marathon with the Children's Tumor Foundation. Since Jane's diagnosis in 2009, she has raised over \$180,000 for NF research.

Thank you, Kristina, for dedicating a decade to ending NF!

To read Kristina's blog go to: [nfemom.blogspot.com](http://nfemom.blogspot.com)



## Alumni Reunion Race Weekend

NF Endurance athletes past and present will come together to end NF at CTF's 3rd Annual NF Endurance Alumni Reunion Race, held as part of the 2019 CNO Financial Indianapolis Monumental Marathon weekend. Starting and ending at the State Capitol, it's a Boston Qualifier that showcases one of the Midwest's finest cities. This incredible weekend includes a marathon, half marathon, 5K, and kids fun-run. To find out more go to: [nfendurance.org/indy](http://nfendurance.org/indy)





LEAVE A LEGACY...BE A LEGACY

## THE NF LEGACY SOCIETY

The NF Legacy Society consists of individuals who have taken the extra initiative to ensure the future of NF research, by including the Children's Tumor Foundation as a beneficiary in their will or trust, retirement account, or life insurance policy.

Make no mistake about it – the progress that has been made in the fight against NF is because of people like you, who are working to improve the lives of those with NF. It is a legacy of which you can be proud.

To learn more about making a planned gift, please reach out to **Melissa Sosa-Longo**, VP of Major Gifts, at [msosa-longo@ctf.org](mailto:msosa-longo@ctf.org) or 646-738-8549.

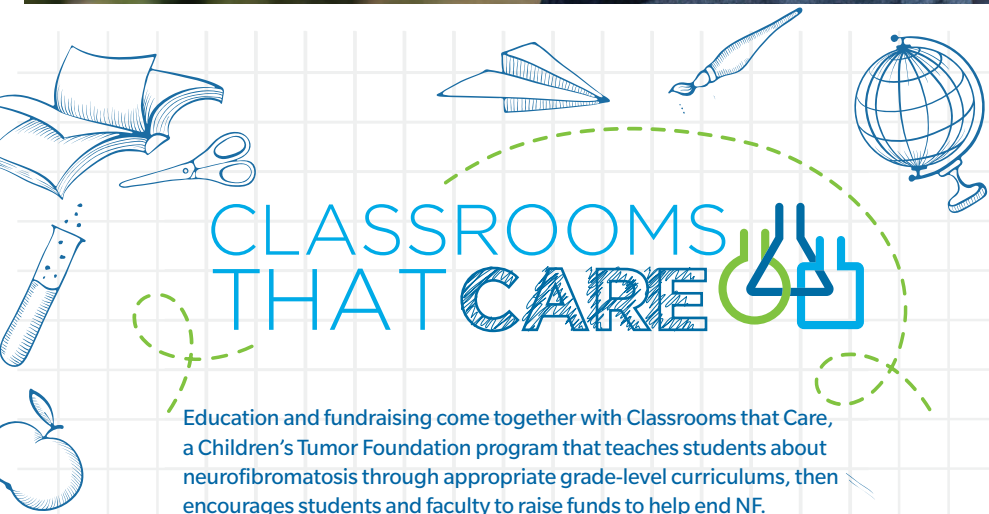


## CLASSROOMS THAT CARE

Education and fundraising come together with Classrooms that Care, a Children's Tumor Foundation program that teaches students about neurofibromatosis through appropriate grade-level curriculums, then encourages students and faculty to raise funds to help end NF.

Our curriculum for older students includes a study guide with a word-search, match-up, and crossword puzzle. Teachers of younger students will receive coloring pages and a story book appropriate for young children. The learning series can be incorporated into everyday programs to educate students about this rare disease and spark a desire to help others. After students have learned all about NF, the school will kick off a fundraising campaign. Upon the completion of this program, schools will be included on the Classrooms that Care leaderboard!

For more information go to: [ctf.org/classrooms](http://ctf.org/classrooms)



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

### FOUNDATION STAFF

**Annette Bakker, PhD**, President

#### Research and Medical Programs

**Salvatore La Rosa, PhD**, Chief Scientific Officer

**Melanie Barry**, Senior Executive Assistant

**Vidya Browder, PhD**, Basic Science Senior Manager

**Kate Kelts**, Patient Support Coordinator

**Pamela Knight**, Director, Clinical Program

**Patrice Pancza**, Vice President, External Relations

**Heather Radtke**, Clinic and Symposium Senior Manager

**Traceann Rose**, Director, Patient and Volunteer Engagement

**Dani Williams**, External Relations Coordinator

#### Development

**Michele Przepyszny**, Chief Advancement Officer

**Katie Bloom**, Director, Major Gifts

**Allison Cote**, Lifestyle Manager

**Emily Crabtree**, Director, Development Operations

**Allyson Douglass**, Development Manager, Special Events

**Angela Dumadag**, Senior Development Manager, Lifestyle

**Barbara Gallagher**, Vice President, Field Development

**Lolita Jerido**, Development Manager, Walk

**Julie Pantoliano**, Senior Manager, Youth Programs

**Kristine Poirier**, Senior Director, Development, Walk, Strategy

**Kim Robinson**, Development Manager, Special Events

**Connie Sorman**, Senior Manager, Stewardship and Volunteer Development

**Melissa Sosa-Longo**, Vice President, Major Gifts

**Jennifer White**, Development Manager, Walk

#### Finance and Administration

**Sarah Bourne**, Vice President, Finance and Operations

**Taylor Bertran**, Gift Processing Coordinator

**Monique Boucher**, Gift Processing Senior Manager

**Carey Milligan**, Senior Accounting Manager

**Brandon Weaver-Bey**, Gift Processing Coordinator

#### Marketing and Communications

**Simon Vukelj**, Chief Marketing Officer

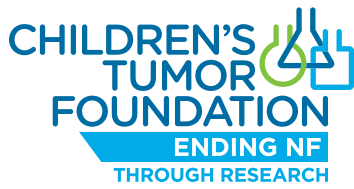
**Albert Diaz**, Director, IT and Salesforce Administration

**Rebecca Harris**, Public Relations Senior Manager

**Alissa Marks**, Marketing Senior Manager

**Susanne Preinfalk**, Design Director

**Vanessa Younger**, Communications Director



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

120 Wall Street, 16th Floor  
New York, NY 10005  
800-323-7938 | [www.ctf.org](http://www.ctf.org)

**May 17 is World NF Awareness Day**

**Wear Blue & Green  
on May Seventeen**

**Get all your gear for  
NF Awareness Month at:  
[ctf.org/store](http://ctf.org/store)**



Join the NF Registry [nfregistry.org](http://nfregistry.org)

Donate [ctf.org/donate](http://ctf.org/donate)

Connect

