

# NF NEWS

CHILDREN'S  
TUMOR  
FOUNDATION  
ENDING NF  
THROUGH RESEARCH

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION  
FALL 2018



## NF Camp

Over 100 youth attended the 22nd Annual NF Camp, which was held in three sessions this July. NF Camp is held annually at the beautiful Camp Kostopulos in Emigration Canyon, Utah. Campers enjoyed horseback riding, rope courses, "Survivor" day, and day trips to local theme parks. At NF Camp, often for the first time, campers can talk freely about NF, share their experiences, and make lasting friendships.

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# FROM the President

Annette Bakker, PhD



This fall, we continue our yearlong celebration of the 40th anniversary of the Children's Tumor Foundation (CTF). As we do, we are deeply grateful to the many insightful individuals who established and then grew this important organization over the years. This Foundation momentously contributed to the body of knowledge about NF, and is impacting and improving the lives of the millions of people living with neurofibromatosis (NF).

As children have now returned to school, I am reminded that, 40 years ago, the parent of a child diagnosed with NF had little hope, and almost no information. This

Foundation was created by parents looking for answers, and I am grateful that we continue to provide hope and reassurance to families in need of education and support.

We are making great strides in NF research. We recently initiated a gene therapy initiative, and released significant data from the Synodos for NF2 project. That data is available through the CTF-funded data portal, which is now becoming a standard for open data sharing in NF. Our researchers are testing drugs on animal models developed through CTF funding, and our upcoming NF Conference in November is expected to be the largest ever, with over 700 experts in attendance.

Even more exciting, the MEK inhibitor selumetinib has attained orphan drug status for the treatment of NF1 from the FDA as well as from the European Medicines Agency. This drug discovery is a result of CTF's multi-million dollar investment in the NF Preclinical Consortium, and our funding of the first in-human study of selumetinib. We are now closer than ever to the first approved treatment for NF!

Thanks to decades of work by NF researchers, a dedicated Board, and

most importantly you, supporters of the Foundation, we are seeing incredible advances in NF research and tremendous growth in our Foundation. The collaboration of a continuously growing NF community has brought us to a time of great optimism for NF treatments.

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 fabulous Fighters who swim, hit homeruns, attend NF Walks, run 5Ks, and hold local fundraisers so that CTF can continue to fund the research needed to end NF. The incredible work of our NF community is what makes it possible to assemble "dream teams" of NF Experts, and position ourselves as leaders in the research community.

You, our donors, are funding more than research—you are instilling hope and promise. As individuals we can make a difference, but together we all play a part in the fight to end NF.

Gratefully Yours,

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## IMI Launches Significant Neurofibromatosis Call for Patient-Centric Drug Development

The Innovative Medicines Initiative (IMI), a jointly-funded partnership between the European Union (EU) and the European Federation of Pharmaceutical Industries and Associations (EFPIA), will soon issue a call for an integrated research platform enabling patient-centric drug development. The Children’s Tumor Foundation (CTF) has strongly advocated for the inclusion of neurofibromatosis (NF) as a rare disease in the call, and we are thrilled to announce that not only is CTF an IMI Associated Partner, but that NF is included in the upcoming IMI call. CTF and IMI will work together to establish integrated research platforms that enable more effective and accelerated drug development in areas of unmet medical need.

As the world’s largest private/public partnership in the life sciences, the IMI brings the best minds together to turn knowledge into treatments by speeding up vital research in order to overcome

serious medical challenges faced by patients. Funded by residents of the European Union (represented by the European Commission), as well as leaders in the pharmaceutical industry (represented by EFPIA), the IMI facilitates open collaboration, cross-sector coordination, and accelerated patient access to medicine. The organization currently has an overall €3.3 billion budget (\$3.8 billion), as well as the support of major global pharmaceutical companies for its initiatives.

The Children’s Tumor Foundation, the largest nongovernmental funder of NF research in the world, has developed an innovative research model aimed at translating discoveries into treatments, and then bringing those treatments to trials more quickly and at a lower cost. This unique research model, called Synodos, is driven by a collaborative open science platform consisting of multidisciplinary experts from across the globe, real-time data sharing, and patient-driven insights at every step of the

process. This collaboration with the IMI represents a significant extension of a coordinated global effort to tackle the complications of NF, and also demonstrates the impact of NF research as a prototype for other rare diseases.

“NF knows no geographic boundaries,” said Annette Bakker, PhD, President of the Children’s Tumor Foundation. “We have committed to expanding the focus of NF so that we increase knowledge about the disease at all levels, attract the involvement of the world’s best researchers and pharmaceutical companies, provide answers, and hopefully soon, effective treatments, for NF patients wherever they may be.”

The IMI conducted an online webinar, open to all researchers, that provided information about this call, entitled “Integrated Research Platforms Enabling Patient-Centric Drug Development,” on July 5, 2018. The recording of the presentation can be viewed at: [ctf.org/IMI](http://ctf.org/IMI)

“NF Camp is the highlight of my year! It is so much fun hanging out with people who have the same challenges as me and helps me feel like I am not alone in what I go through on a daily basis. I love NF Camp because I get to see my friends every year as well as make new ones.

KALA SCHVANEVELDT,  
UTAH



2018

## DRUG DISCOVERY INITIATIVE REGISTERED REPORTS (DDI-RR) AWARDEES

In 2017, CTF introduced a rigorous new way of reviewing and funding projects. Through a collaboration with top scientific journal *PLOS ONE*, in a new process known as “Registered Reports,” awardees are offered financial support by CTF and in-principle acceptance for publication by the journal. This model will allow for more rigorous, reproducible, and transparent science, guaranteeing its awardees publication, regardless of study outcome.



### ANDREA MCCLATCHEY, PhD

Massachusetts General Hospital

#### ***Exploiting macropinocytosis for the development of exosome-mediated drug delivery in NF2 mutant tumors***

Merlin, the protein encoded by the *NF2* gene, is unique among tumor suppressors because it controls cell reproduction. Merlin controls how growth receptors respond to changes in the mechanical and physical properties of the cellular environment. We have recently discovered that Merlin regulates how cells take up fluids and nutrients from the environment, through a process called macropinocytosis. In the absence of Merlin, cells take in more fluids and nutrients. This project will seek to take advantage of this feature of *NF2*-mutant cells by utilizing this pathway for the delivery of drugs for the treatment of *NF2* tumors. These studies will provide a foundation for the testing of this kind of treatment delivery for this and other therapies for *NF2* patients.



### LEI XU, MD, PhD

Massachusetts General Hospital

#### ***Reprogramming the Tumor Microenvironment to Enhance Anti-Tumor Immunity in NF2 Vestibular Schwannomas***

The hallmark symptom of *NF2* is benign bilateral vestibular schwannomas (VS). Over time, these tumors grow and may cause progressive hearing loss, which may lead to social impairment and increased clinical depression. In patients with progressive VS, a thickening of the connective tissue, called fibrosis, correlates with hearing loss. Fibrosis results in high collagen content. Recently, we found that *NF2* patients with VS demonstrate elevated collagen content and fibrogenic signaling, and are plagued by hypoxia and immunosuppression. Based on these, we propose to target the fibrogenic signaling pathway to improve hearing and enhance immunotherapy efficacy. Our research will generate important and translatable results for new combination therapy paradigms that are desperately needed for this dreadful disease.



### AARON SCHINDELER, PhD

The University of Sydney, Australia

#### ***Dietary Intervention for NF1 Muscle Weakness***

*NF1* is associated with a high tumor burden in adulthood, but for many children it most profoundly impacts their school experiences through learning disabilities, muscle weakness, poor coordination, and fatigue. Recent clinical studies indicate that muscle strength is reduced by 30-50% on average in children with *NF1*. Our breakthrough research has not only revealed that this weakness is linked to problems in fat metabolism in muscle, but also that dietary changes and supplements can overcome this weakness. We propose to complete a final series of preclinical studies with different diets and supplements to fine-tune our design for a dietary intervention trial in children.

2018

## YOUNG INVESTIGATOR AWARDEES

The Children's Tumor Foundation is pleased to announce the funding of five Young Investigator Awards (YIA) for 2018. The YIA is the Foundation's oldest research award program and serves to advance the understanding of the biology of NF1, NF2, and schwannomatosis.

The NF Research Initiative (NFRI) and David Miller, MD, PhD, of Boston Children's Hospital are funding two of this year's YIA's in full. The NFRI is a newly established initiative that will focus exclusively on malignant peripheral nerve sheath tumors (MPNSTs).

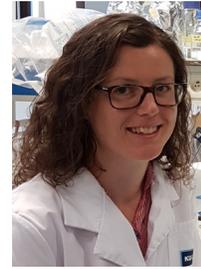


### MICHELLE WEGSCHEID

Washington University  
School of Medicine

***"Employing human induced pluripotent stem cells (hiPSCs) to define brain developmental defects in NF1"***

The overall objective of this project is to understand how germline *NF1* mutations affect the human brain. Ms. Wegscheid will employ stem cell cultures to characterize the cellular and tissue abnormalities in brain development due to different *NF1* gene mutations.



### SARAH CATHERINE BORRIE, PhD

Katholieke Universiteit  
Leuven

***"Deciphering and treating ASD-like social behavior deficits in mouse models for NF1"***

NF1 patients frequently have cognitive and behavioral problems, with many having a diagnosis for autism spectrum disorder (ASD). This project will utilize *NF1* and *Spred1* transgenic mouse models to further our understanding of mechanisms underlying autism spectrum disorder in patients with NF1, and will investigate possible treatment options.



### EVAN O'LOUGHLIN

Harvard University

***"Three-dimensional culture systems to assess the consequences of NF2/Merlin loss"***

Merlin, a protein that is encoded by the *NF2* tumor suppressor gene, controls many of the cell's signaling pathways and behaviors. Furthermore, the way *NF2* deficiency leads to the formation of tumors is not fully understood. This project will use a three dimensional cell culture system to examine the signaling pathways controlled by Merlin, and study how Merlin loss impacts the behavior of cells. This study will advance our understanding of Merlin deficiency, and will provide a framework for further examinations of the signaling pathways that may contribute to NF2 tumors.

## FUNDED BY NFRI



### JAMIE GRIT

Van Andel Research  
Institute

***"Genetic and molecular mechanisms of targeted therapy resistance in NF1-associated MPNSTs"***

Understanding how MPNSTs develop and progress in individuals with NF1 is key to understanding how to effectively treat these aggressive tumors. The goal of this proposal is to identify how RAS signaling is different between MPNSTs with mutations in *NF1*, compared to those with mutations in both *NF1* and *p53* genes. The most important result of this study will be the identification of a drug combination that blocks RAS signaling and stops MPNST growth even in *p53* mutant tumors.

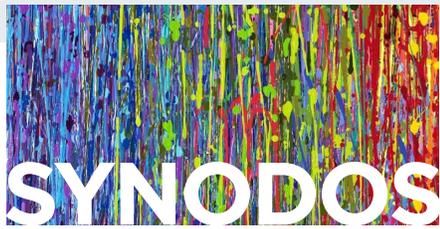


### DONG HYUK KI, PhD

Dana Farber Cancer  
Institute

***"The role of cap-dependent translation in NF1-Associated MPNSTs"***

The focus of this proposal is to test several drugs specific to the eIF4E pathway for antitumor activity and toxicity, and to also identify key proteins that depend on this pathway for expression. This study will clarify the mechanisms underlying the activity of mTOR inhibitors against NF1-associated MPNSTs, potentially leading to new and complimentary strategies to inhibit these pathways.



## SYNODOS FOR NF2 CONSORTIUM PUBLISHES KEY RESULTS

*Study shows that Drug Combination Therapies Effective in Treating Schwannomas and Meningiomas*

CTF's Synodos for NF2 consortium published its first set of results and released its second set of data in the leading scientific journal *PLOS ONE* in June of this year. Entitled "Traditional and Systems Biology Based Drug Discovery for the Rare Tumor Syndrome Neurofibromatosis Type 2," the full article is openly available to all. Allowing immediate access to data is consistent with the Foundation's principles of open-access and data sharing.

The primary aim of the Synodos for NF2 team was to find treatments that could be used for both meningioma and schwannoma by using a drug discovery approach of selecting mechanistically relevant compounds. The team integrated innovative research approaches such as Next Generation Sequencing (NGS) and Kinome profiling. These two techniques allow analysis of the alteration in expression of genes and proteins in cells or animal models.

A core finding was that while schwannomas and meningiomas are both initiated by Merlin loss, the distinctly different responses to that loss of function are likely to prescribe that a single drug (target) is unlikely to be optimally effective in both cell types. Instead, it is likely that different drugs or combinations (a cocktail of two or more drugs) aimed at distinct targets will be required to treat these two tumor types.



## NF1 Clinical Trial of Selumetinib "Most Exciting" Says National Cancer Institute Director

Speaking before the House Energy and Commerce Committee of the U.S. House of Representatives on the implementation of the 21st Century Cures Act, Norman (Ned) Sharpless, M.D., Director of the National Cancer Institute (NCI) was asked by Missouri Congressman Billy Long, "Can you tell us about the most exciting thing that is being supported in the Cancer Moonshot?"

Sharpless focused his answer on the NCI's Rare Tumor Initiative and the clinical trial presented in June at the ASCO Conference by Andrea Gross, MD of the NCI about the selumetinib (MEK inhibitor) clinical trial treating inoperable NF1 plexiform neurofibromas.

At the time, Dr. Sharpless had tweeted "tumors shrink, kids feel better, drug seems safe," which he repeated at the Congressional briefing, additionally noting that several parents of children in the clinical trial had contacted him to share "before and after" photos showing dramatic reductions in tumor size. One of those parents whose message was highlighted at this briefing was Renie Moss, a member of the Board of Directors of the Children's Tumor Foundation, and Chair of the Foundation's Volunteer Leadership Council. Her son Philip has seen a 53% reduction in the size of his tumors while participating in this trial,

and Dr. Sharpless showed the before and after photos of Philip to the congressional committee, noting that "his windpipe, his airway is not being compressed by the tumor anymore, and he has not the social stress of going to school with a big lesion like that."

This clinical success is the result of an investment by the Children's Tumor Foundation in the NF Preclinical Consortium, which showed the potential for clinical benefit in NF patients. That work was instrumental in the development of this selumetinib clinical trial, led by Dr. Brigitte Widemann, who announced at the Foundation's 2015 NF Conference that over half of the patients in the trial were seeing significant reductions in tumor size of their inoperable plexiform neurofibromas.

Since that time, the trial has entered registration phase (the last step before submission to the FDA for approval), with continued positive results. Over 70% of trial participants have seen tumor reductions of at least 20% in size, a first in NF research. This study has also been reported in the prestigious *New England Journal of Medicine*, and selumetinib has received Orphan Drug Status from both the Food and Drug Administration (FDA) in the United States and the European Medicines Agency.

## Pig Models Advance the Study and Treatment of NF1

Children's Tumor Foundation research, led by its Synodos for NF1 team, has demonstrated the ability to replicate NF1 manifestations in pigs, as reported in the *Journal of Clinical Investigation*, a leading peer-reviewed publication of the American Society for Clinical Investigation. These manifestations include café-au-lait spots, neurofibromas, axillary freckling, and learning and memory neurological deficiencies. The significance of this advancement is that it allows researchers and clinicians, for the first time, to test and study potential NF treatments in large animal models that mimic human manifestations of neurofibromatosis.

Traditional NF1 research models utilize mice, which can replicate certain aspects of NF1, but not in a comprehensive manner, and hence are not as easily replicable for human application. This new, innovative approach conducts NF studies in pigs, which allows for more relevant and accurate analysis, and aids in better understanding of the disease, thereby increasing the potential for effective treatments.

This research was initiated out of the Children's Tumor Foundation's Synodos initiative, a collaborative team science and open data model that brings together a multidisciplinary 'dream team' of leading experts to solve the complex problems of NF. This team included researchers from Sanford Research, the University of Iowa, and the University of Arizona.

### Why Pigs? And Next Steps

There are many similarities between pigs and humans in terms of metabolism, biochemistry, physiology, weight, lifespan, and genetics. As demonstrated in other disease areas, including cystic fibrosis, pig models offer a unique ability to

monitor disease progression and have shown better predictability in human drug response and behavior. The pigs in this study are born with NF1, and exhibit human-like NF characteristics, such as café-au-lait spots. By 10 months of age, the pigs start showing neurological impairments similar to that in humans with NF. These new models are now an invaluable tool in furthering and accelerating NF1 research and therapies, and will open the door for the very first time to the testing of drugs that improve the neurological impairments of NF1.

Lead funding for the Synodos for NF1 project was provided by James and Laurée Moffett.



### NF1 Gene Therapy Initiative: Request for Applications

The Children's Tumor Foundation is pleased to announce the initiation of a research program in gene-based therapeutic approaches for the treatment of NF1. The first goal of this initiative is to fund two proof-of-principle studies in NF1 gene editing. Funding for each study will be \$240,000 total for a duration of two years. The

ultimate goal of this initiative is to explore the development of genome editing as a potential therapeutic tool for NF1.

Applicants must submit an LOI by November 9, 2018. For more information, please contact: [grants@ctf.org](mailto:grants@ctf.org)

## CTF GRANT DEADLINES & IMPORTANT DATES

**November 2 - 6, 2018**

NF Conference

**November 9, 2018**

LOI Gene Therapy Initiative

**December 28, 2018**

Deadline for NF Conference travel grants

**January 04, 2019**

YIA LOIs due

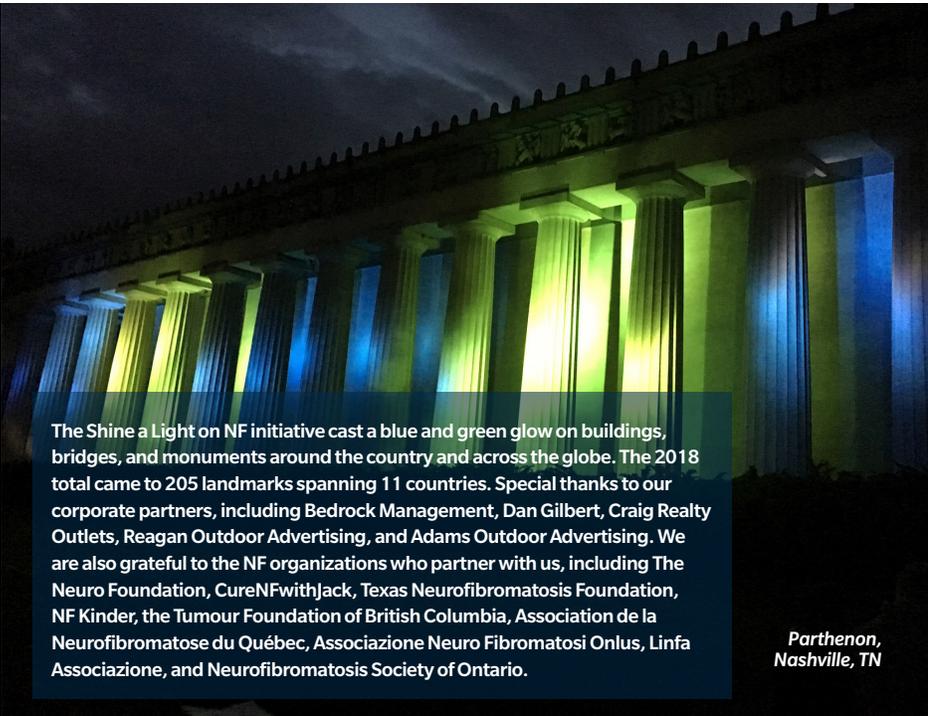
### Care of Adults with NF1, a new resource

Care of Adults with NF1, a new resource, has been published by the *American College of Medical Genetics and Genomics (ACMG)*. It offers voluntary guidelines for medical management based on published reports and the expertise of the authors, who are leaders in NF1 medical care.

We are sharing this news with you as a resource to manage your own care. You can get this article free of charge at Springer Nature: <https://rdcu.be/MFHJ>

# Awareness Month HIGHLIGHTS

The annual NF Awareness Month campaign raises awareness about neurofibromatosis through numerous events held during the month of May.



The Shine a Light on NF initiative cast a blue and green glow on buildings, bridges, and monuments around the country and across the globe. The 2018 total came to 205 landmarks spanning 11 countries. Special thanks to our corporate partners, including Bedrock Management, Dan Gilbert, Craig Realty Outlets, Reagan Outdoor Advertising, and Adams Outdoor Advertising. We are also grateful to the NF organizations who partner with us, including The Neuro Foundation, CureNFwithJack, Texas Neurofibromatosis Foundation, NF Kinder, the Tumour Foundation of British Columbia, Association de la Neurofibromatose du Québec, Associazione Neuro Fibromatosi Onlus, Linfa Associazione, and Neurofibromatosis Society of Ontario.

Parthenon, Nashville, TN



## PHOTO CONTEST WINNER

The NF Awareness Month "I Know a Fighter" photo contest showcased the fighting spirit of many NF Heroes. "I Know a Fighter" gives voice to those living with neurofibromatosis and is a reflection of the bravery that NF patients exhibit in their daily lives.

“Both boys are your typical energetic, fun-loving boys. They delight in getting on each other’s nerves and pestering their sisters. The photo was taken at our local park. They are partially dressed in their karate giis, a sport they both recently started in January.”

SUBMITTED BY LATASHA BINFORD  
TIMMOTHY AND CLAYTON’S MOM

Our ongoing photo series "This is NF" tells a side of the NF story that isn't often told—that of the passion that those living with NF bring to their daily lives. Nine adults joined the photo series this May and demonstrated that living with NF doesn't mean letting NF define your life. Read all the profiles at: [ctf.org/thisisnf](http://ctf.org/thisisnf)

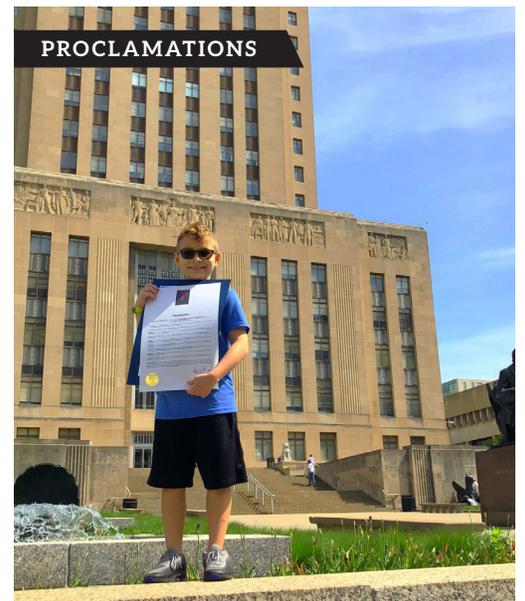


“A neurofibromatosis diagnosis isn't always what people see when they ask Dr. Google. There is a lot of support available and having NF isn't necessarily the end of the world.”

—STACEY FRIED  
lives with neurofibromatosis type 1. She is a Human Resources Executive and lives in New York, NY.

[ctf.org/thisisnf](http://ctf.org/thisisnf)

## PROCLAMATIONS



Proclamations were issued in 31 state houses and 40 city halls across the country recognizing NF awareness month! 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.

Please visit [ctf.org/photos](http://ctf.org/photos) to view all of the NF Awareness Month photos.

# May ATLANTA RECAP

In May 2018, the NF community gathered in Atlanta, Georgia for a long weekend of patient engagement training, leadership training, research updates, and patient and family support.

## NF FORUM

The Children's Tumor Foundation's NF Forum took place in Atlanta from May 4-6, 2018. This national patient education and family gathering allows those living with NF, and their families, to connect, support, and learn from each other. Families and patients learn together while attending seminars on relevant topics pertaining to neurofibromatosis. The Foundation was awarded a prestigious PCORI grant (Patient-Centered Outcomes Research Institute) for the 2018 NF Forum.

"What I was hoping to get out of the Forum was a chance to meet other people in the NF community and gain more knowledge about the latest medical advancements for NF. What I was not expecting was to immediately feel so welcomed by everyone. I also looked forward to the expert guest speakers, but did not expect to get the chance to speak with them personally, which was really great."

**HANNAH DUBY**  
NF2 MOM

## Volunteer Leadership Council

In celebration of the Foundation's 40th Anniversary, 62 registered members of the Volunteer Leadership Council (VLC), our most active and dedicated volunteers, gathered for their annual Leadership Training Conference in Atlanta, Georgia. The conference allows VLC members the opportunity to have face-to-face interactions with staff and one another. They gain new insights about the latest scientific research, CTF program enhancements and marketing initiatives, as well as to have time to share with and learn from each other. Volunteer Recognition Awards (VRAs) were presented to volunteers to celebrate the contributions of Volunteer Leaders in several categories from our newest up-and-comers to our most tried and true champions.



## Patient Rep Training Program

CTF's Patient Representative Training Program graduated its first class of "Patient Advocates" this May. This Patient Engagement initiative is designed to prepare individuals with NF and their families to participate as advocates in NF research. Through online and in-person learning, the program's goal is to show patients how to add their perspective during all phases of the research process. Patient Advocates are trained to work with stakeholders such as researchers, research institutions, the pharmaceutical industry, the Food and Drug Administration, and patient advocacy organizations. Congratulations, graduates!



## Ida Sulaiman

My acoustic neuromas had caused me to lose hearing in both my ears. It started with a ringing sound in my left ear. Gradually the left hearing diminished and by the time I was 15, I had one brain surgery done to remove the tumor on my left auditory nerve and by 16, I had to rely solely on my right hearing. In 2004, when I was 15, I was diagnosed with NF2.



Up until I was 22, my right hearing got affected. Today, I am 80% deaf and my residual hearing is very minimal. I am hearing-impaired but because I wasn't born deaf and am not fluent in sign language, people doubt my deafness when I speak. It's really difficult to integrate and interact.

In 2015, I gave birth to my first child and I understand that pregnancy can contribute to tumor growth because of hormones. Last year, I went for my second brain surgery, as one of the tumors had grown in size and was pressing on my nerve, causing me to have constant headaches. After this brain surgery, my balance got really bad and I no longer can walk properly or straight. It eased my headache but I have to deal with new challenges; besides my balance, my left eye became drier (dry eye) and I developed facial paralysis. Half my face is not working (affected facial nerve) and I had people mistakenly thought I have cerebral palsy, which I don't. My speech can also be a bit slurred due to this.

NF2 is slowly complicating my life with all the different problems it is causing. Currently, due to my spinal tumors, I have to put on hold, my plan to try for a second baby. I need support just to walk too because a recent brain

surgery have messed up my balance.

Just in the first week of September this year, almost a year after the 2nd brain surgery, I had seizures (side effect of the surgery). I cannot remember what happened. They detected a brain infection/swelling after an MRI scan. I become more susceptible to infections and have to take greater care of my immune system. Due to my poor balance and weakness affecting the strength in my legs and left side of the body, I was advised against pregnancy in the near future. I also cannot carry my child too much because of this and have been caring for him in other ways that I can.

I managed to get a job but resigned before I went for my surgery last year. I am now a stay-at-home-mom. I cannot make phone calls and hearing aids do not help me now therefore I rely mostly on texts, sms, emails or any form of writing.

I lose some and gain some but the gains have pushed me to make the best out of everything and be the best I can be. And I am blessed to have a small but tight circle of support.

## Jacob Deon

Jacob and his father were diagnosed on the same day with NF1. Jacob was two months old. The initial diagnoses were a bit of a surprise, but both Jacob and his dad are determined to overcome obstacles and spread NF awareness.

In addition to NF1, Jacob was born with bilateral sensorineural hearing loss. He has been wearing hearing aids since the he was 3 months old. He also has growth hormone deficiency and battles immunodeficiency. With medicine, Jacob is growing

taller and stronger by the day.

Jacob's NF diagnosis makes us grateful for all of the blessings he has received. It has caused our family to be more appreciative of his health and happiness. NF reminds us not to take the little things for granted.

Although Jacob has some speech and learning difficulties, he pushes himself to be the best he can be. He recently was recognized at school for his outstanding effort, earning two medals for academic achievements.



# Extraordinary Spirit / MCKINNON GALLOWAY

Meet the incoming 2019 NF National Ambassador, McKinnon Galloway. Look for more about McKinnon and the presentation of this honor in the next newsletter.

McKinnon Galloway is a young adult who lives in Charlotte, North Carolina. She was diagnosed with NF2 at age 16 after she got a concussion during a high school volleyball match. A scan revealed that she had tumors unrelated to the concussion. McKinnon has lived the last 10 years of her NF journey showing only her brave face, while hiding the brutal reality of her condition from most. She hopes that her decision to change how she lives with NF and to tell about her experience will be a source of encouragement and comfort to others, in knowing that they are not alone. She also wishes to use this next year as the NF Ambassador to share her story in order to raise funds and awareness and to educate the greater community and help find a cure.

The concussion that McKinnon sustained during the volleyball match is what prompted a routine MRI, which ended up changing the course of her life dramatically. The doctors explained that she had two tumors in her brain and predicted that she would go deaf by the time she graduated high school. She and her family were in complete shock. One day, she was a normal healthy teenage girl with the bright future and the next, an NF2 patient on an unforeseen path of uncertainty and fear.

McKinnon's parents scrambled to learn what they could about a diagnosis they had never before known. What they learned terrified them. They absorbed the very real possibilities of deafness, blindness, muteness, balance issues, paralyzation, seizures, and much more. They discovered that there is no cure, which devastated them at first, but eventually became their motivation to get involved. They established care for McKinnon in the Children's Tumor Foundation's NF Clinic Network at John's Hopkins Hospital in Bethesda, Maryland. Dr. Jaishri Blakely is the doctor who guides them with McKinnon's care and who stood beside them then as they learned about the severity of her condition. When they discovered that the tumors were growing aggressively, Dr. Blakely recommended chemotherapy and ultimately, surgery. McKinnon's first brain surgery was scheduled on her 18th birthday. The 8-hour surgery was initially

successful in preserving her hearing. Over the last 10 years as she endured multiple medical challenges, McKinnon struggled privately with feelings of self-consciousness, depression, shame, and paralyzing fear. She buried her feelings and withheld the seriousness of her deteriorating condition from her family, her friends and everyone she met. She hid behind a dazzling, but artificial, smile and forged ahead as the epitome of strength and courage when all she really wanted was to be "normal" or to disappear completely.

Eventually, McKinnon did lose hearing on one side, despite being enrolled in a phase I clinical trial for a chemotherapy drug and undergoing a second brain surgery. The surgery was successful in saving her smile from full facial paralysis, which was somewhat ironic because her desire to smile diminished as the depression continued to deepen and her resolve to not burden her loved ones grew stronger.

Today, McKinnon is completing the Bachelor's Degree that she began six years ago. She will earn a BA in Liberal Studies in December 2018 with a concentration in business, communications and psychology. Ten years after starting on Avastin, McKinnon is grateful for the research that has helped her to maintain the hearing in her left ear, but she is hopeful for continued research because she is beginning to damage other body systems as a result of the long-term use. She wants to work with researchers and to educate donors about this need on behalf of all NF2 patients.



# Regional NEWS

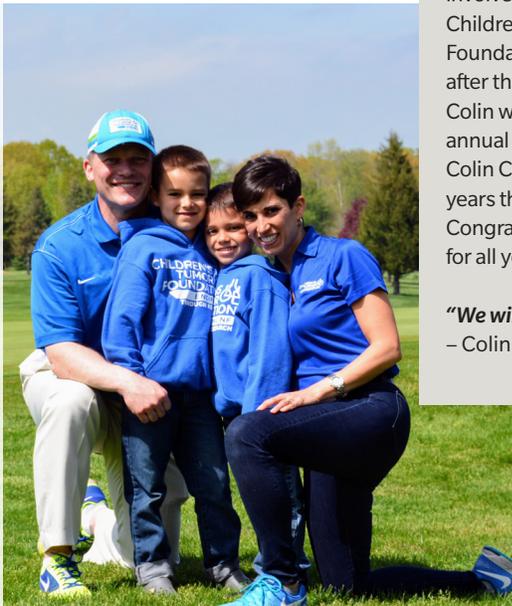
The Foundation has a presence across the United States and facilitates local patient support groups, medical symposia, and fundraising events. Learn more about the Children's Tumor Foundation in your area by visiting [www.ctf.org](http://www.ctf.org).

## CONNECTICUT

### Orange Hills Golf Tournament

On Monday May 14, 2018, the 4th Annual Children's Tumor Foundation Charity Golf Tournament was held in Orange, Connecticut. With an 8am shotgun start, 118 golfers teed off to enjoy 18 holes of golf on the beautiful greens at Orange Hills Country Club. This year marked the most successful outing in the history of the event, bringing together the largest number of foursomes and raising more than \$26,600.

Jeffrey Owens has organized the event every year since 2015 in honor of his son Alex, who at 4 months old, was diagnosed with NF1 and who continues to battle inoperable tumors on his brain and back. Jeff says, "Alex is an NF fighter who strengthens our resolve to beat NF every day." Over the past 3 years, Jeff has raised nearly \$60,000 for NF research through this event.



## NEW JERSEY

The 4th annual Caddies for Colin golf outing took place in Haworth, New Jersey on Monday, October 1st, and raised an impressive \$70,000 for the Children's Tumor Foundation. Heidi and Michael Cashell became involved with the Children's Tumor Foundation shortly after their son



Colin was diagnosed with NF1. In addition to their annual golf outing, last year they established the Colin Courageous Foundation. Over the past four years they have donated more than \$300,000 to CTF. Congratulations to the Cashell family, and thank you for all you do in the fight to end NF!

*"We will not stop until there is a cure!"*  
– Colin Courageous Foundation

## NEW YORK

### Vito's Ristorante Fundraiser

For the 4th year, Vito's Ristorante in Glen Cove, New York hosted an anniversary fundraiser in which they donate 50% of each pie they sell to the Children's Tumor Foundation. This June their annual Charity Pizza Night contribution grew to \$1,200.



NF Hero Julia Perfetti with NF Sibling Jessica Schafer and Vito, of Vito's Ristorante



From left to right: Zach Ferioli, Nora Delaney, Nola Mullins, CC Leiphart, Reese Testa, and John Kanter

## MASSACHUSETTS

### Change for NF

Massachusetts fifth grader John Kanter completed his school's Independent Learning Project on neurofibromatosis. John's uncle and cousin have NF, and he has seen his family support NF awareness and fundraising activities, so he wanted to do his part. John decided to raise money for NF research, and when he discussed it with a few of his classmates they volunteered to help. They wrote to their school principal to get permission to do a coin drive at The East School in Hingham, Massachusetts. They later did a "Change for NF" presentation to kick off the coin drive at their all-school meeting, which was attended by more than 600 kids plus faculty, staff, and parents. The drive raised \$500 for the Children's Tumor Foundation.

Hubert Barksdale, Pete Tanguay, Lesley Oslica, and Dr. Anne Trussell supporting the I Know A Fighter 5K in Little Rock, Arkansas on May 26, 2018.



### I KNOW A FIGHTER 5K

As part of CTF's year-long 40th anniversary celebration, over 1,200 runners participated in nine different cities across the country from April to June for the I Know A Fighter 5K tour! This event series attracted runners engaging with CTF for the first time, as well as runners already connected to our community. Participants as well as generous corporate sponsorships raised more than \$150,000. The family-friendly event featured a 5-kilometer/3.1-mile race and a wonderful finish line festival. The last event took place on October 20, in conjunction with our NF Walk Los Angeles.

Thank you to our National Sponsors, Dave & Buster's and Everlast, and top fundraisers around the country:

- Keira D'Arcy (Boston)
- Christopher Phillip (Atlanta)
- Linda Keller (Chicago)
- Wendi Singer (NYC)
- Anne Trussell (Little Rock)
- Clara Wilpon (NYC)
- Romy Pavolotsky (San Francisco)
- Claudia Timko (Atlanta)
- Sean Collins (San Francisco)
- Eric Payumo (San Francisco)
- Christina Cuneo (NYC)
- Richard Walsh (Boston)
- Shannon McNall (Atlanta)
- Kristina Diaz (Boston)
- Patricia Ptsaznik (Fort Worth)

# NFWALK

### NF WALK CINCINNATI

A little rain didn't stop the 2018 NF Walk Cincinnati! There were so many great things happening that the sun eventually peaked. With food, games, raffle baskets, face painters, super heroes, a magician, and a DJ, the rain didn't keep this year's participants from having a great time! The event raised more than \$84,000. Special thanks to Jessica Samblanet, Shannon Savage and the rest of the planning committee for a wonderful event.



# NF ENDURANCE

### Swim Around Key West

On June 16, Katy Freye took part in the "Swim Around Key West" Event, swimming 12.5 miles as her husband Joe kayaked alongside her. Katy swam in honor of her son Austin who has NF and raised more than \$6,000.



Katy Freye and her husband Joe

“ Our son, Austin, continues to battle the effects of neurofibromatosis. He is currently working to overcome delays in academics. Sixth Grade has proven to be Austin's most difficult school year (understandably so, it's Middle School!). He is becoming more and more aware of his own learning delays. Additionally, the tumors on his sternum multiply. I do not know how NF will affect Austin in the future, but I do know it is progressive. We worry, but we continue to advocate! ”

## ASK KATE!



Ask Kate! is an exciting new YouTube series to answer your questions about neurofibromatosis.

Kate Kelts, RN, BSN is the Patient Support Coordinator for the Children's Tumor Foundation, and each week she will answer questions submitted by viewers.

If you have a question you'd like to see Kate answer, you can include it in the YouTube comments, or email Kate at [KKelts@ctf.org](mailto:KKelts@ctf.org).

## NYC Poker Night

The 2018 New York City Poker Tournament was held on June 6th and was the largest NY Poker event yet with more than 180 guests! The annual event brought in more than \$130,000 to support NF research. We extend a huge thank you to the night's winner, Scott Gottlieb, who graciously donated his prize back to the Foundation.

Our hearty thanks to the planning committee for all of their hard work and dedication: Dan Altman, Dan Wilpon, Corey Altman, Jon Tolpin, Frank Romano, Brandon Maymudes, Laura Raheb and Ken Goodkind.



## Remembering Joan Engel

It is with great sadness that we share the news that Joan Engel, former president of the National Neurofibromatosis Foundation (NNFF), has passed away. Joan served as president of the NNFF from 1981-1984, after Foundation founder Lynne Courtemanche, who started our organization in 1978 with Allan Rubenstein, MD and Joel Hirschtritt.

Joan's involvement with the Foundation came because of her son Ken, who lives with NF, and her incomparable leadership in those critical early days of the organization ensured that the vital work of funding NF research and developing the NF community would continue and thrive. Her accomplishments still resonate today, as the Foundation commemorates its 40th year.

Joan touched many lives with her kind spirit and distinctive grace, her vibrant energy and thoughtful compassion. She is also remembered for her wonderful sense of humor and her steadfast optimism. Earlier this year, in recognition of the Foundation's anniversary, Joan shared with NF News a remembrance about the Foundation's founding, discussed the role the organization plays in the lives of NF patients and families, and reflected on the pride she feels for her son Ken, who currently serves on the Foundation's national Board of Directors.

We will greatly miss Joan and send our deepest condolences to her family and friends.



## \$15M SECURED FOR NF RESEARCH

After receiving approval from both chambers of Congress, President Trump signed the FY 2019 Defense and Labor, Health, and Human Services Appropriations conference report into law on Friday, September 28. The conference report includes \$15 million in funding to support neurofibromatosis research and is vital to our fight to end NF. This funding will support strategic research through the Department of Defense's Neurofibromatosis Research Program (NFRP), resulting in new discoveries and better outcomes for NF patients and their families. This would not be a reality without the tireless work and advocacy done by the Children's Tumor Foundation, the NF Community, patients, and their families. This sustained federal funding will allow us to further develop scientific data, break through barriers, and forge a pathway to end NF.



*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).

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## LEAVE A LEGACY...BE A LEGACY

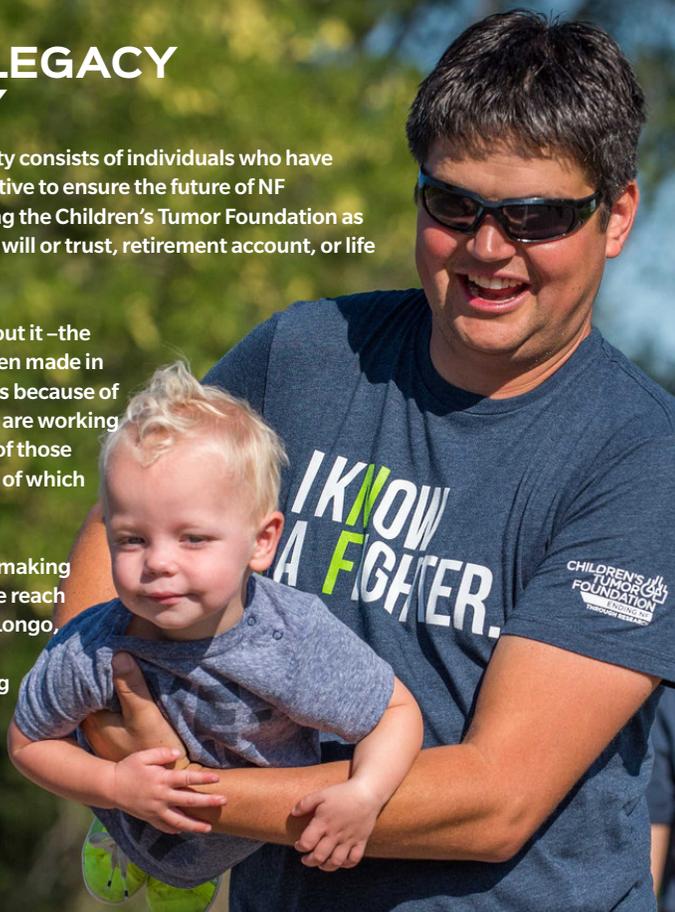
Introducing

## 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.





## Board of Directors Visits NF Campers

Members of the Children's Tumor Foundation Board of Directors spent an afternoon in Utah with teens and young adults at NF Camp. Whether young, or young at heart, the group came together for a day of fun and festivities as they learned more about each other, and their shared NF experiences.



STAY INFORMED

[ctf.org/news](http://ctf.org/news)

The next NF News print newsletter won't arrive in your mailbox until December, but in the meantime you can find something new almost every day at [ctf.org/news](http://ctf.org/news). Visit our newsfeed frequently for research updates, industry announcements, events across the country, and inspiring stories from the NF community.

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

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