

NF NEWS

CHILDREN'S
TUMOR
FOUNDATION
ENDING NF
THROUGH RESEARCH

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
WINTER 2017

Believe
WE CAN END NF

Galas to End NF

This fall, exciting events took place across the country to benefit the Children's Tumor Foundation (CTF) and drive neurofibromatosis (NF) research forward.

At the New York Gala on November 9, two extraordinary young siblings, Frankie and Olyvia Moriguchi, were named the 2018 Children's Tumor Foundation NF Ambassadors. These two courageous young people have a long history with CTF that is heartening, as is the bond they have as part of a family fighting NF.

This honor was passed down to the Moriguchi siblings from last year's ambassador, Sarah Rodbell, whose photography exhibit "Through the Lens of an NF Ambassador" was on display at the event.

Held at The Lighthouse on Pier 61 in NYC, the evening's theme was *Shine a Light on NF*. It offered a unique opportunity to celebrate and honor "The NF Patient," a group that includes over 2.5 million individuals around the world living with NF. With a wealth of NF patients in attendance, a new light was shed on the struggles and triumphs of the NF patient. It was the Foundation's privilege to use its signature gala to honor the individuals who are at the heart of everything we do. *Continued on page 9*

INSIDE:

- 3 New Pharma Partnership
- 4 Synodos Progress
- 9 Believe We Can End NF Campaign
- 10 Gala Highlights

Siblings Frankie and
Olyvia Moriguchi,
2018 NF Ambassadors



FROM the President

Annette Bakker, PhD



Now more than ever, I believe that treatments are in sight for the millions of people around the world living with NF. I believe that through the interminable, relentless work that CTF researchers are doing today, those living with NF will have a better life tomorrow. I believe this, and in this final newsletter of the year, as you read of groundbreaking progress and vital new partnerships, my hope is that you will believe along with me—we can end NF.

This year we have seen the impact of your generosity through the results that NF patients are experiencing. We are seeing tumor shrinkage of 20-50% in 70% of the patients enrolled in the MEK clinical trial at the National Cancer Institute. The images of NF Heroes Jane Constable and Philip

Moss speak for themselves; their lives have improved, both visibly and physically. The MEK trial has been revolutionary, and as we move into a new year we keep in mind our bold goal of 100% tumor reduction in 100% of patients.

Yet there is much work to do, which is why the researchers from all five CTF Synodos consortia met together for the first time this October. The data that these teams are producing is moving NF research forward at championship speed, and even more exciting, that data is being made openly available to the entire world. The energy in the room at this full network meeting was tangible—these are clinicians and researchers who deeply care about the NF patient.

We celebrated and honored the NF patient at the New York Gala this fall. Our courageous 2018 Children's Tumor Foundation Ambassadors, Frankie and Olyvia Moriguchi, are a wonderful example of the tenacity and fervor that characterizes the NF patient, and the exceptional families who fight NF alongside them. This extraordinary brother and sister have a long history with CTF, and there is a palpable bond between them as they fight NF together. I could not have been more proud to stand next to these two brave ambassadors at the Gala this year.

I am also extremely inspired by the two Unconventional Vision Awardees that were recognized that night, Freda Lewis-Hall,

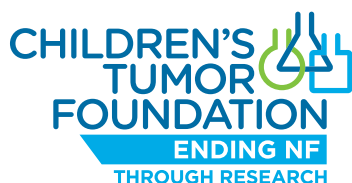
Pfizer's Chief Medical Officer, and Lara Sullivan, the President of SpringWorks Therapeutics, a new pharma company that will partner with CTF to create new treatments for NF.

This year marks the fifth annual Detroit "beNeFit," and I am profoundly grateful to Dan and Jennifer Gilbert for their energy, commitment, and dedication to CTF and NF in general. The creativity and imagination surrounding this beautiful event is delightful; each year it proclaims to the world the Gilbert's philanthropic passion for an end to NF. I also want to congratulate Dan and Jennifer on the creation of NF Forward—a great new partner foundation battling NF1.

NF patients, I thank you. Your faith in us, and in the work we are doing, propels us forward and fills us with purpose. Please know that you are cherished; indeed, everything we do is for you. Thank you to all those who support NF patients in so many ways—through fundraising, volunteering, planning, donating, or advocating. Together we are changing the lives of these patients, as they are also changing ours.

Gratefully,

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CTF Collaborates with New Pharma Company

The Children's Tumor Foundation is pleased to announce an exciting new collaboration with SpringWorks Therapeutics, a Pfizer-conceived new company focused on developing much-needed therapies for underserved patient communities. SpringWorks Therapeutics launched in September with news of promising investigational therapies, including one for neurofibromatosis. The Children's Tumor Foundation is collaborating with this new company to connect the NF community with these much-needed therapies.

SpringWorks represents an innovative approach to drug development, with a business model designed to deliver both social and financial returns via partnerships with a variety of stakeholders, including scientists, biopharmaceutical partners, patient groups, funders, and philanthropists. It is receiving funding from Pfizer, Bain Capital Life Sciences, Bain Capital Double Impact, OrbiMed, and LifeArc.

SpringWorks Therapeutics also has rights to four clinical-stage experimental therapies from Pfizer. One of those therapies is of benefit to patients with plexiform neurofibromatosis. SpringWorks Therapeutics is planning to initiate a Phase 3 program to establish safety and efficacy of its MEK 1/2 inhibitor (PD-0325901) in the NF1 population, in collaboration with the Children's Tumor Foundation in order to meet the needs of the patient community.

SpringWorks Founder and President Lara Sullivan, MD said, "SpringWorks Therapeutics will pursue the development of medicines across therapeutic areas, focused on diseases where there is an urgent need and the potential for the greatest impact for patients." Pfizer's Chief Medical Officer Freda Lewis-Hall, MD added, "Pfizer sees SpringWorks Therapeutics as a groundbreaking new model for collaboration to deliver on the promise of medical research and development, so that more people have the potential to overcome disease."

SAVE THE DATE

2018 Joint Global Neurofibromatosis Conference

Maison de la Chimie
Paris, France
November 2-6, 2018

Join the World NF Community of Researchers, Clinicians, and Patients in Paris in 2018

ctf.org/nfconference



Neurofibromatosis



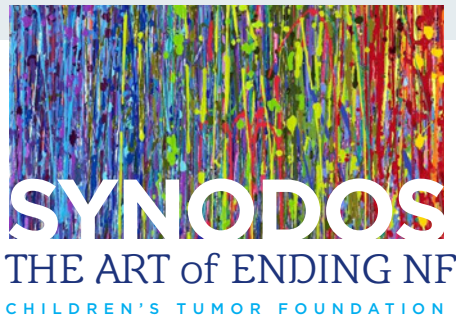
CTF Board of Directors member Robert Brainin presented Annette Bakker with a RARE Champion of Hope Award

2017 RARE Champion of Hope Award

Children's Tumor Foundation President and Chief Scientific Officer Annette Bakker, PhD, was the recipient of a 2017 RARE Champion of Hope award. Hundreds of individuals and organizations worldwide were nominated by their peers to receive the RARE Champion of Hope award for notable efforts in rare disease advocacy, science, collaborative sciences, and medical care and treatment. From the extraordinary list of nominees, esteemed panelists selected the recipients to be honored. Dr. Bakker was recognized for her notable efforts in science.

These awards are presented by Global Genes, one of the leading rare disease patient advocacy organizations in the world. Global Genes builds awareness, educates the global community, and provides critical connections and resources that equip advocates to become activists for their disease.

Global Genes presented the award to Annette at the 6th Annual RARE Patient Advocacy Summit, a gathering of rare disease advocates from across the globe. These advocates come together for three days in September in Irvine, California to share best practices, create important introductions, and help catalyze powerful collaboration.



CTF HOSTS FIRST SYNODOS NETWORK MEETING

Over 70 researchers, clinicians, patients, staff, and other experts gathered in Palm Beach, Florida this October for the first Synodos Network Meeting, hosted by the Children's Tumor Foundation, and supported by the Jin Hua Foundation. The theme of the meeting was Synodos: The Art of Ending NF, and included the vibrant artwork of Synodos patient advocate and former NF Ambassador Jeffrey Owen Hanson. This was the first joint gathering of key leaders from all Synodos initiatives, and it provided a unique opportunity to share research updates and exchange views on specific Synodos-related matters.

Please go to ctf.org/news to read Synodos research status updates.

"The Synodos Network Meeting was an amazing and stimulating experience of brain storming."

— PIERRE WOLKENSTEIN, MD, PhD
Henri-Mondor Hospital, University
Paris Est Créteil

"Congratulations on the progress you have made with the Synodos initiatives. . . the science, the move toward 'community,' and not reinventing wheels are impressive."

— ANNA D. BARKER, PhD
Co-Director, Complex Adaptive Systems and
Director, National Biomarker Development
Alliance (NBDA); Professor, School of Life
Sciences, Arizona State University



Pig Models in NF1

A Conversation with Jill Weimer, PhD

Co-Principal Investigator Jill Weimer, PhD, of the Sanford School of Medicine discussed the Synodos for NF1 swine model program with members of the CTF staff. Below is an excerpt from that talk.

JILL WEIMER: One of the reasons it's important to develop a genetically modified pig is that we need a more accurate animal model, one that is closer to a human than a mouse. Mouse models for NF1 lack a lot of the common characteristics that we see associated with humans with NF1. So we began working in partnership with Exemplar Genetics, the company that was the first to design a genetically modified pig, to develop a more accurate animal model.

CTF has paired us very closely with the other NF1 Synodos team, headed by David Largaespada at the University of Minnesota, as they are making a pig model as well. It has been nice because we are able to trade tips on how to characterize the pig model.

All the pigs have been born with café au lait spots, which is already a step up from the mouse models. Within a few months, they often start to develop the axillary

freckling that you see in NF1 patients. Some of our founder pigs are about a year and a half old, and a number of them have started to develop cutaneous fibromas. We have a veterinary pathologist that works closely with us, who has looked at some of these cutaneous fibromas and determined that they do resemble the cutaneous fibromas that you would see in NF1 patients. He has also done a lot of pathology on the café au lait spots to ensure they are actually café au lait spots, because sometimes pigs can have spots themselves.

We developed a learning and memory test that the pigs have to go through: they have to choose a certain path to follow through a maze to get a food reward, and then they are tested again and scored based on how quickly they can learn where a new food reward is in the maze. We have definitely seen a change in our neurofibromatosis pigs that correlates with the deficits in learning and memory in humans with NF1.

So that's where we are right now. I think the pig model will be very, very useful to the community for drug testing, for advanced imaging technology, and for early diagnosis of NF1.

To read the full transcript of Dr. Weimer's discussion, please go to ctf.org/news.

NF

Q&A

Navigating the NF landscape can be complex; keeping track of appointments and tests, reading up on developments in research, and staying on top of general self-care can be overwhelming. Have you ever had a question about NF that you forgot to ask at your last doctor's appointment? We're here to help!

Q: Is there a connection between NF1 and breast cancer?

A: To date, many studies show that women with NF1 have an increased risk for breast cancer at a relatively young age (between 30 and 50). In the general population, breast cancer is typically a risk for women over age 50. The knowledge of this increased risk for women with NF1 helps to address potential issues these patients may face. What is needed is an

awareness of this fact by women with NF1 and by their doctors. For a doctor, this means not to ignore a new lump as "just another neurofibroma." For women with NF1, this means paying close attention to changes in the breasts.

If detected, the treatment of breast cancer in NF1 should follow the standard protocols.

Thank you to Juha Peltonen, MD, PhD, University of Turku, Finland for the answer to this NF question.

CTF Receives New Grant from Patient-Centered Outcomes Research Institute (PCORI)

We are proud to announce that the Children's Tumor Foundation is the recipient of a \$50,000 grant from the Patient-Centered Outcomes Research Institute (PCORI) for the support of the 2018 NF Forum, a Patient-Centered Outcomes Research conference for patients, families, and other stakeholders. PCORI was established to fund research that can help patients and those who care for them make better-informed decisions about the healthcare choices they face every day, guided by those who will use that information.

The PCORI grant will provide critical funding to support the NF Forum being held in Atlanta, Georgia on May 4-6, 2018. The NF Forum is an educational opportunity that conveys to patients the importance of including their voice in research, and helps stakeholders incorporate that voice into their mission. We extend our thanks to PCORI for this recognition, and for contributing to the Foundation's efforts to serve the NF community across the nation.

Access to Quality Care Improves with Four New NFCN Clinics

The NF Clinic Network continues to expand with new clinics added in California and Wisconsin, as well as two new states, Arizona and Tennessee.

Mayo Clinic Arizona Phoenix, Arizona

Clinic directors:

Radhika Dhamija, MD and Maciej Mrugala, MD, PhD

Clinic coordinator:

Mary Madden, RN
The clinic serves adult patients and is led by Dr. Dhamija with a dual specialty of genetics and neurology, as well as Dr. Mrugala from neuro-oncology.

University of California, Los Angeles (UCLA) Los Angeles, Ca.

Clinic directors:

Phioanh Leia
Nghiemphu, MD, Melissa Reider-Demer, DNP, Marco Giovannini, MD, PhD

Clinic coordinator:

Roberta Leyvas, MS
The main focus of this multidisciplinary clinic is NF2, but individuals with NF1 and schwannomatosis are also seen.

University of Wisconsin/American Family Children's Hospital Madison, Wisconsin

Clinic director: Neha Patel, MD

Clinic coordinator:

Kristin Jakubowski, BSN, RN
The Comprehensive Neurocutaneous Disorders Clinic primarily serves pediatric patients.

Vanderbilt University Medical Center Nashville, Tennessee

Clinic directors: Paul Moots, MD and Debra Friedman, MD

Based in the Vanderbilt-Ingram Cancer Center, this weekly clinic provides care to both pediatric and adult patients.

For more information on our network clinics, go to ctf.org/understanding-nf/find-doctor.

2018 NF Forum



CHILDREN'S TUMOR FOUNDATION

**NF Patient-Centered Outcomes Research (NF-PCOR) Forum
MAY 4-6, 2018, ATLANTA, GEORGIA, Hilton Garden Inn, Downtown**

The NF Forum is a fun family weekend in which patients, caregivers, and children come together to:

- get support and network
- meet experts in the field of NF

-find out how to get involved in patient-centered research

Registration opens January 15, 2018 - go to ctf.org/nfforum

TO LEARN MORE CONTACT [Traceann Rose, trose@ctf.org](mailto:Traceann.Rose@ctf.org)

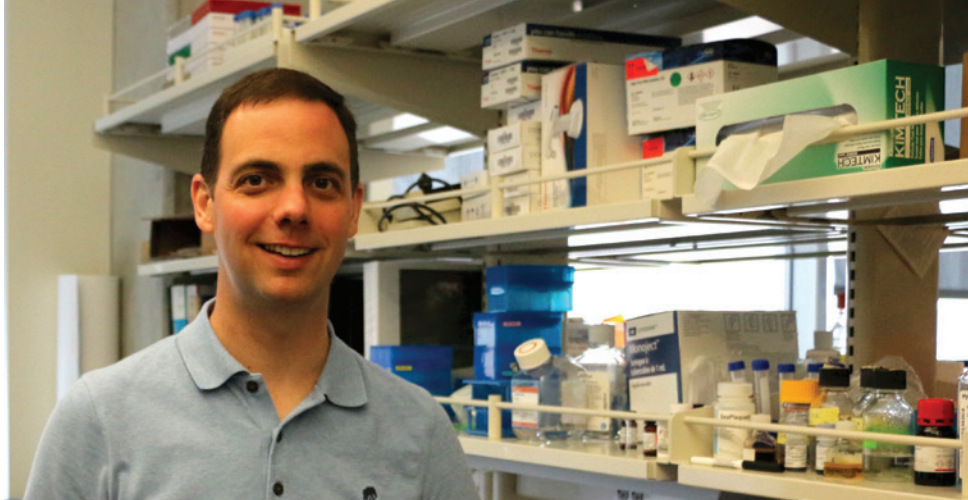
2017 Young Investigator Awardees

The NF Research Initiative (NFRI) and David Miller, MD, PhD, of Boston Children's Hospital are funding the 2017 Young Investigator Awards (YIAs) in full. The NFRI is a newly established initiative that will focus exclusively on malignant peripheral nerve sheath tumors (MPNSTs). Dr. Miller looked to CTF and its established YIA program as a resource that can readily identify young basic researchers through our outstanding peer-review process, and administrate the grants through our grant management systems.



Believe
WE CAN END NF

Because of **your gifts**, young scientists are pursuing NF research.
ctf.org/believe



Kyle Brandon Williams, PhD, University of Minnesota

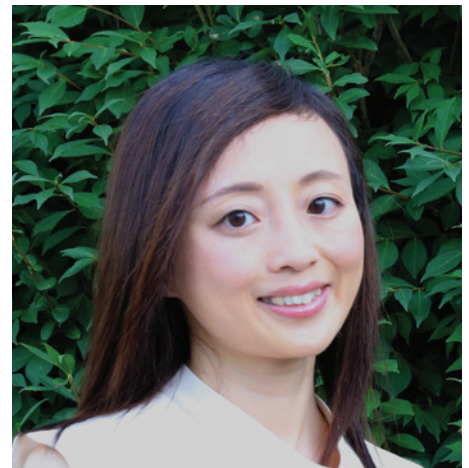
Exploiting Synthetic Lethality to Reveal Novel Vulnerabilities in NF1 Tumorigenesis
Award amount: \$108,000

New technologies such as next-generation sequencing, gene knockout libraries, and proteomics profiling now allow researchers to apply great power to the study of human cells in culture and disease modeling.

Dr. Williams has a human cell line that is deficient for the NF1 gene, which is an outstanding research tool. He proposes to use these cells to look for drugs that could combine with the promising MEK inhibitors to preferentially kill the NF1-deficient cells. This could identify new, targeted combination therapies for potential clinical use. In addition to mutations in the NF1 gene, it has been established that other mutations are required for malignant tumors (such as MPNSTs) to develop in NF1 patients. Through this YIA, Dr. Williams will take their existing NF1-mutated cell models and engineer them to be more "MPNST-like" by incorporating some of those other mutations. Testing drugs in these cells will give additional confidence that these drugs will be effective against more aggressive and malignant tumors. This work will develop their NF1 drug discovery pipeline further and should allow identified drugs to more rapidly progress into clinical trials for people suffering from complications of NF1.

Lai Man (Natalie) Wu, PhD, Cincinnati Children's Hospital Medical Center ***Molecular and Signaling Mechanisms of Malignant Transformation in Peripheral Nerve Sheath Tumors*** **Award amount: \$108,000**

Dr. Wu's initial study shows that a pathway, called Hippo, goes awry in MPNST tumors. Moreover, recent studies have reported nonsense mutations of LATS1, a Hippo-signaling molecule and a known tumor suppressor, in patients with nerve sheath tumors. This suggests that abnormal Hippo signaling activity may contribute to nerve sheath tumor growth. The goal of this study is to learn how Hippo pathway malfunction in Schwann cells causes MPNSTs, and to discover drug targets to treat these aggressive tumors.



Understanding how abnormal Hippo signaling drives Schwann cells to become cancer cells may aid the discovery of promising drug targets against MPNSTs. Dr. Wu will use a state-of-the-art genome-wide target screen to identify molecules and pathways directly regulated by the Hippo pathway during tumor formation. She will further devise treatment strategies to modulate the activity of Hippo. In principle, this research emerges as a new paradigm in MPNST research and will have practical benefits that aid us in finding key therapeutic targets to effectively cure MPNST.

Drug Discovery Initiative Awardees

CTF is proud to have recently funded four Drug Discovery Initiative (DDI) awards. We are enthusiastic about these exciting projects!

**Jeffrey Field, PhD,
University of
Pennsylvania, Perelman
School of Medicine**

*MPNST Profiling and
Screening: Extension for
Exome Sequencing of the
Cell Lines Screened*

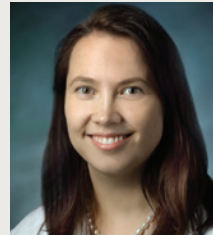


Award amount: \$25,000

In a prior DDI award, Dr. Field developed a course for students to do drug screening for 130 relevant NF drugs against 9 NF1 cancer cell lines and two NF2 cell lines. They also screened thousands of drugs against representative NF1 and NF2 cell lines. Further funding will allow Dr. Field and these students to find the mutations for each cell line to more closely correlate the sensitivity to drugs tested, and to identify new drugs to test. Additionally, the data from this project will be made public.

**Verena Staedtke, MD, PhD,
Johns Hopkins University,
School of Medicine**

*Evaluation of Mebendazole
as Chemoprevention in
a Neurofibromatosis 1
Transgenic Mouse Model*



Award amount: \$85,000

This project will explore using chemoprevention, the use of drugs to reduce the risk of cancer development, by repurposing a particular drug for the prevention of MPNST development. The drug, Mebendazole (MBZ), has shown benefits in colorectal cancer syndromes previously, and will be tested in a MPNST mouse model. If successful, the results will have an immediate impact on patient care; the highest death rate among NF1 patients is due to MPNST, and this project hopes to reduce this cancer frequency among NF1 patients.

**Andrea Rasola, PhD,
University of Padova,
Department of
Biomedical Sciences**

*Targeting the Mitochondrial
Chaperone TRAP1 to
Inhibit Plexiform
Neurofibroma Growth*



Award amount: \$40,000

Changes in cell metabolism constitute a driving force for the growth of many tumor types. Dr. Rasola and his group have also found that TRAP1, a protein that has a crucial function in the control of the energy metabolism of tumor cells, is mandatory for neurofibroma growth. The aim of this project is the identification of molecules that inhibit TRAP1, which might block neurofibroma progression. It is hoped that these new compounds will be the first step in the development of selective and effective anti-neoplastic drugs for NF1 patients.

**Dr. Marco Giovannini,
MD, PhD, University of
California, Los Angeles**

*Preclinical Safety and
Efficacy Evaluation of
Long-Term Anti-VEGFA
Treatment Administration in
a GEM Model of NF2-Related
Schwannoma*



Award amount: \$84,999

Case reports and clinical trials have reported that bevacizumab (Avastin), can induce both tumor regression and hearing improvement in patients with NF2-associated vestibular schwannomas. Dr. Giovannini will test Avastin in an NF2 mouse schwannoma model to analyze its efficacy in terms of tumor shrinkage and hearing performance. Setting the Avastin response baseline in mice will allow prioritization of new drugs by comparing their efficacy, and will therefore aid the choice of new drug candidates for clinical trials in NF2 patients.

The Children's Tumor Foundation and the scientific journal PLOS ONE are collaborating on a new funding program in the area of neurofibromatosis research. The new initiative, called the Drug Discovery Initiative Registered Report (DDIRR) Awards, is a funder-publisher partnership to integrate the Registered Reports model in the grant application process.

Registered Reports pre-determine the research question, methodology, and design of a study to be carried out, and are designed to enhance the rigor, reproducibility, and transparency of the science produced. Upon thorough review of the study design at the time of grant application, awardees are guaranteed an in-principle acceptance to publication in the journal PLOS ONE. Provided the study is conducted according to the plan, acceptance in principle is honored regardless of study outcome—as such the Registered Report model contributes to eliminate publication bias. This new award will serve as a pilot and will evolve from the Foundation's classic Drug Discovery Initiative Award program.

The Request for Applications for the 2017 DDIRR Awards is now open. Learn more at ctf.org/research.

stories

OF NF

Katelyn Watkins

Katelyn recently graduated college from Anderson University in Anderson, South Carolina. She is currently working as a teacher at a local middle school in a Special Education classroom. This has always been her dream job, and to have it as a first year teacher is very amazing. She also purchased her first home and is enjoying living in it and being out on her own. If you try hard you can succeed and accomplish anything that you want to in life.

Having NF never stopped Katelyn from doing what she needed to do to reach her goals and dreams. As a young child she played basketball and participated in cheerleading. In high school she was an officer in Future Business Leaders of America and was a member of the color guard. In college she was frequently on the Dean's list, was the President of the Council for Exceptional Children, and still found time to be the manager of the Women's Basketball team. While NF has never stopped her, she has faced many challenges, including countless surgeries and vision problems. The vision problems made driving very difficult for her, but she succeeded in obtaining her license. The challenges she has faced have greatly impacted



where she is today. She works with children who also face challenges in their life. Katelyn's goal is to help them face those challenges head on and overcome them.

Katelyn has always been a fighter with a heart of gold. She enjoys helping people and has made a great impact on her family. Katelyn does not procrastinate, and is always on top of everything. She is a fine Christian young lady and excels in everything that she does. I am so proud of her and love the fact that I am her grandmother.

—Katelyn's grandmother, Priscilla

“Katelyn has always been a fighter with a heart of gold. She enjoys helping people and has made a great impact on her family.”

—PRISCILLA, KATELYN'S GRANDMOTHER

Jamalyvett Jimenez

Jamalyvett, or Jamaly for short, is a very positive young girl who has been fighting every day to do her best to achieve her goals. She was only 8 months old when she was pre-diagnosed, and 3 years old when we received the results of a genetic test confirming she has NF1.

Jamaly is currently receiving speech, language, and occupational therapy. She is well known in her elementary school due to her positive attitude. It is hard for Jamaly to communicate with other kids and sometimes she feels left out, but she doesn't let that discourage her. On the contrary, it drives her to keep working hard to improve her speech. Jamaly has had three surgeries so far. In 2013, a neurofibroma was removed from the abdominal muscle. In 2014, she developed hydrocephalus and we almost lost our beautiful princess. But thanks to the great physicians Dr. Miller and Dr. Niazi at Miami's Children Hospital, an endoscopic third ventriculostomy was conducted on time and she was saved. The last surgery took place in 2015 to correct strabismus; she was losing the vision in her left eye. It has been a long battle and full of surprises. But there's no other beautiful being as positive as Jamaly. She always has a big smile, even when she is in pain.

—Jamaly's mom, Sonia



This holiday, join the
NF Registry
at nfreistry.org

Believe

WE CAN END NF

Jane Constable was only a couple of months old when her pediatrician pointed out to her parents, Kristina and Todd, that she had a number of café au lait spots on her body. They were referred to another specialist, and around that time started to notice a swelling in her cheek. Her first MRI at only 18 months old revealed the large tumor that she has now. It wasn't long before her doctors diagnosed Jane with NF1.



Only 2% of the charities we rate have received at least 8 consecutive 4-star evaluations, indicating that the Children's Tumor Foundation outperforms most other charities in America. This "exceptional" designation from Charity Navigator differentiates Children's Tumor Foundation from its peers and demonstrates to the public it is worthy of their trust.

Michael Thatcher
Charity Navigator President and CEO

DONATE TODAY!
ctf.org/believe



In Spring 2011, Philip and Renie Moss noticed that their son "Little" **Philip**'s neck was unusually swollen. The pediatrician thought it could be a swollen lymph gland and it was treated as such. However three weeks later, their doctor informed them that the biopsy revealed that Little Philip had a neurofibroma tumor and that he had NF1.

Seeing Is Believing

The images above speak for themselves. Jane's tumor has shrunk 30%, and Little Philip's tumor has shrunk an incredible 50% percent since their participation in a clinical trial at the National Institutes of Health (NIH). While not yet a cure, their lives have improved, both visibly and physically, with enhanced mobility and better health. Their smiles speak a thousand words!

This is because back in 2008 - **with the support of donors like you** - the Children's Tumor Foundation made a major investment in the NF Preclinical Consortium. The groundbreaking research of that consortium of scientists would demonstrate that MEK inhibitors have significant impact on tumor size in animal models, and that research now informs the clinical trials taking place today at the NIH, and the incredible results these patients are seeing.

Jane and Philip are not alone. Over 70% of the participants in this particular trial have seen tumors reduce in size by 20-50%. The NF research field has not seen anything like this before. But as amazing as that is, we want 100%. Our goal is 100% tumor reduction in 100% of patients. A bold goal, but with your support, we can make this a reality.

What You Can Do

Between now and January we need you to give to the "**Believe We Can End NF**" Campaign at ctf.org/believe. Whatever you do, don't wait. Your support of further critical research in NF is needed today.

There is still much work to do. Please accept this invitation to further impact the lives of the millions worldwide who live with NF. It's now more important than ever that everyone join together - including you - to support this work. Your support today of NF research will ensure that the opportunity for a better life tomorrow is available to all who live with NF.

Galas to End NF

[continued]



Lara Sullivan, MD, receives an Unconventional Vision Award



Allison Clarke, Flashes of Hope founder, with one of Cleveland's "Little Stars"



Raina Seitel, NBC host and correspondent, at the New York Gala

Two individuals were also recognized at the New York Gala with the **Unconventional Vision Award**. This new honor acknowledges individuals who have rejected the status quo and are having a positive impact on the lives of children and adults living with neurofibromatosis. This year's awardees were **Freda Lewis-Hall, MD**, Executive Vice President and Chief Medical Officer at Pfizer Inc., and **Lara S. Sullivan, MD**, President of SpringWorks Therapeutics. Dr. Lewis-Hall and Dr. Sullivan are upending traditional drug development with the launch of SpringWorks Therapeutics, a Pfizer-conceived company dedicated to developing potential new treatments for underserved patient communities.

Correspondent and NBC host **Raina Seitel** served as Master of Ceremonies at the New York Gala. Through her warmth and humor, she further illuminated an evening already filled with light. The gala's research auction caused The Lighthouse to brim with excitement. At the time of this printing, the event's proceeds had not yet been finalized.

The fifth annual Detroit **beNeFit** was "A Fantastical Affaire to End NF," held at the Cobo Center in downtown Detroit, Michigan, on November 18. Esteemed CTF patrons **Jennifer and Dan Gilbert** were once again the hosts of this huge and fantastic affair, which was attended by leaders and celebrities in the Detroit community.

Jada Smoot, a middle school student, and her mother Teaquia Jacobs, both of whom have NF, received the Strength and Honor Award to recognize their uncommon courage in dealing with the disorder. Young Jada has a large tumor on her cheek, but despite her complications, she is a straight-A student and, as Dan Gilbert remarked, the "nicest, sweetest person."

The hugely successful Detroit beNeFit is a highlight of the year. At the time of this writing, the event's proceeds had not yet been finalized.

On October 18, more than 40 pediatric cancer survivors and fighters were the true stars of the night when the Cleveland Cavaliers hosted

the annual **Big Shots and Little Stars** fundraiser, benefiting Flashes of Hope and the Children's Tumor Foundation. Flashes of Hope is a volunteer-driven organization focused on children's cancer, which was founded by **Kip and Allison Clarke**. In addition to their teammates, Cavs players were joined by The Mad Hatter and Queen of Hearts, among other Alice in Wonderland characters, for this unique and entertaining evening. Thanks to Big Shots and Little Stars, a donation will be made to CTF which will go toward researching malignant peripheral nerve-sheath tumors.

In Arkansas, **Dancing With Our Stars** was held on September 7. At this 10th Anniversary Spectacular, Arkansas celebrities showed off their dance moves as they competed for the coveted mirror-ball trophy. Months of rehearsals with local dance instructors culminated in a one-time performance with the intention of raising money to fund critical NF research, supporting local NF clinics, and sending Arkansas teens to NF Camp.

The first Dancing With Our Stars was held in 2007 at the Annunciation Greek Orthodox Church, and it raised \$25,000. As the event grew, it moved to Chenal Country Club and several other venues. This year, it was held in a ballroom in downtown Little Rock, where it raised \$185,000, bringing the ten-year total to over \$1 million for NF research. Our gratitude goes out to **Lesley Oslica** for her continuing work on this wonderful event.

The New England Gala took place on Saturday, September 16, at the Dane Estate of Pine Manor College in Chestnut Hill, Massachusetts. The night's honorees included **Nicole Ullrich, MD, PhD** of Boston Children's Hospital, for her notable contributions to NF research. Other individuals honored for their commitment to the Foundation and its cause were longtime supporters Bernard & Brenda Gitlin, Phil & Judy Shwachman, and Lou & Roberta Williams.

The evening was inspired by the Roaring Twenties, and it culminated in live and silent auctions. The event brought in more than \$90,000, thanks to a wonderful planning committee, some of whom have been involved with this event for over 30 years.



Little Rock's Dancing Stars of 2017



From left: Myrna Merowitz, Matthew Shwachman, Bob Merowitz, and Honoree Judy Shwachman at the New England Gala



NF Heroes Charlotte Spoto and Julia Perfetti at Cocktails for a Cure

Cocktails for a Cure was held on October 22 at the Brookville Country Club in Long Island, New York. It was a beautiful evening filled with delicious hors d'oeuvres and flowing champagne. The event was hosted by **Laura Perfetti, Cristina Spoto, Lisa Arena, Jill Hannity, Erika Millet, and Victoria Romano**. The event raised more than \$108,000 to support the mission of the Foundation, thanks to a silent auction, a raffle, and a live auction in honor of NF Heroes, Julia Perfetti and Charlotte Spoto.

The Children's Tumor Foundation humbly extends our gratitude to the attendees, donors, honorees, and organizers of all of these galas, particularly those who support our Foundation with such remarkable fervor.

Extraordinary Spirit /

EMMA BECKER



Emma Becker has been a patient at Connecticut Children's Medical Center (CCMC) for four years. Emma has NF and is undergoing chemotherapy because of tumors in her brain.

However Emma is not taking her diagnosis sitting down—she spends her time fighting NF. For her birthday last month, she launched a campaign on her Facebook page to collect silly socks for every kid who has to come through the hospital doors. Her goal of 1,200 pairs of socks was quickly surpassed, and she collected more than 2,500 pairs of socks for pediatric oncology patients.

The 12-year-old wanted to spread love and smiles to children in the hospital because she knows what it's like to be in their shoes. "I know how other kids feel," she said, "cause hospitals are cold and I just wanted something so they could feel like they were home."

What is incredible about Emma is that this isn't the first time she's done a good deed for her friends. The young Connecticut girl has collected over 10,000 containers of Playdough, painted colorful rocks, hosted lemonade stands, and bought iTunes gift cards. This Halloween, she collected costume donations for her friends at the hospital.

Emma is fighting for her life, but she lives by one rule: "No matter how big or small you are, you just need an idea to make a lot of other kids happy."

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.

NORTH CAROLINA

CTF Night with the Charlotte Knights

Local NF Heroes, their families, and supporters gathered at BB&T Ballpark in Charlotte, North Carolina on August 17th for an evening to raise NF awareness. CTF was the featured Community Corner organization as the minor-league

baseball team Charlotte Knights took on the Toledo Mud Hens. NF Hero Colin Cashell threw the first pitch and his Dad represented the NF community, with a pregame interview broadcast throughout the stadium.



FLORIDA

Bay Hill Golf Scramble

October 9th was the 25th annual Florida Bay Hill Golf Scramble benefitting the Children's Tumor Foundation. A beautiful day of golf was enjoyed by 140 golfers at the world famous Arnold Palmer's Bay Hill Club & Lodge in Orlando, home to the PGA's Arnold Palmer Bay Hill Invitational every March. The Ehrli family has hosted this event in honor of NF Hero Joe Ehrli for the past eighteen years, the last fifteen of which have been held at the Bay Hill Club. This year's golf scramble raised more than \$62,000, bringing the event's 18-year total to over \$600,000 to fund NF research!

“I come every year because Joe Ehrli is family to me. I have watched him grow up into a fine young man who has succeeded in spite of the challenges that he has faced.”

—**DARRELL GREEN,**
PRO FOOTBALL HALL OF
FAMER AND FORMER
WASHINGTON REDSKINS
PLAYER



CALIFORNIA

Kids Fashion for NF

The third annual Kids Fashion for NF event took place on Sunday, September 24th in Los Angeles, California. Local NF Heroes strutted down the runway wearing Hudson Jeans, and dresses or T-shirts that they designed themselves. The Heroes were asked, “If we found a cure for NF tomorrow, how would that make you feel?” They were then asked to put those feelings into their fashion designs. Jonathan Sadowski, star of Freeform's *Young and Hungry* hosted the event for the third year in a row. Many new NF families joined the event, while veteran families came together once again, and collectively raised more than \$14,000 to end NF.

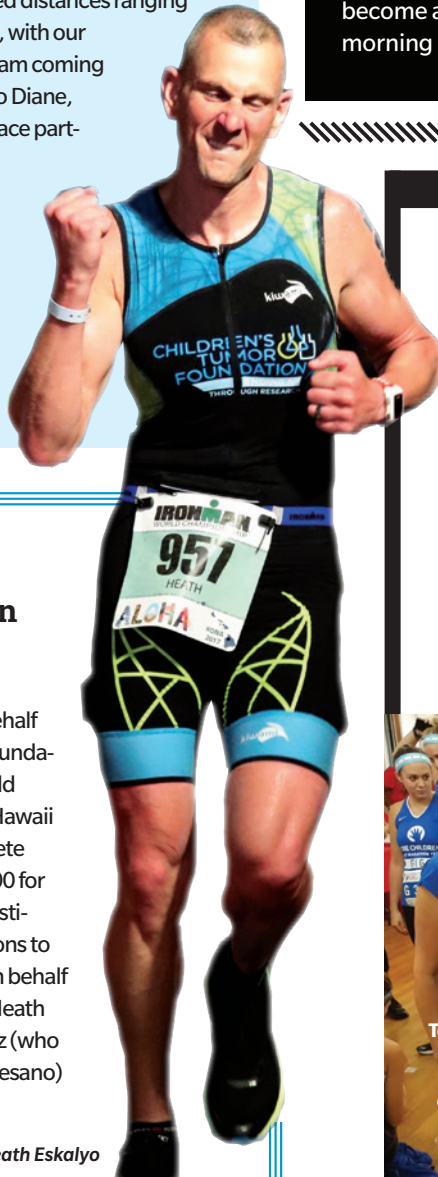


New Haven Road Race



For the second year in a row, CTF was a signature charity partner of the 40th Annual Faxon Law New Haven Road Race, which took place on September 4th. Led by CTF volunteer leader, Diane Owens, our NFE runners raised more than \$7,000 and raced distances ranging from 5K to a half marathon, with our NFE half marathon relay team coming in Third Place! Thank you to Diane, who brokered the charity race partnership in 2016 and built up the NFE team racing in New Haven, Connecticut these past two years.

Above, from left: Jill Wiebke, Danielle Suhar, Diane Owens, Mauneen Kania, Sandra Lounsbury, and Maggie Converse



IRONMAN Heath Eskalyo

IRONMAN World Championships in Kona, Hawaii

Three athletes raced on behalf of the Children's Tumor Foundation at the IRONMAN World Championships in Kona, Hawaii on October 3rd. Each athlete committed to raise \$40,000 for CTF to compete in this prestigious event. Congratulations to John Joseph (who raced on behalf of NF Hero Alex Owens), Heath Eskalyo, and Liz Fernandez (who raced on behalf of Bella Paesano) for crossing the finish line!



Team Garrett - America's Finest City Half Marathon and 5K

Team Garrett participated in the America's Finest City Half Marathon and 5K in San Diego, California this August. The 85-member team raised more than \$18,000 for the Children's Tumor Foundation and drew lots of attention for NF. Each team member wore an individualized, creative penguin hat, and nine of Garrett's closest friends wore full penguin costumes, and waddled over the finish line. This is Team Garrett's fifth year racing to find a cure to end NF and they have become popular participants, due to their enthusiasm and fun costumes. It has become a yearly highlight for friends and family to come together, enjoy an early morning race through the city, and spend time with NF Hero Garrett.

CHICAGO MARATHON

Thirty-eight NF Endurance team runners from four countries each tackled 26.2 miles at the 40th Annual Chicago Marathon on October 8th. Collectively these runners raised more than \$78,000 for NF research, with top fundraising team Chafing the Dream bringing in over \$10,500 in honor of NF Hero Connor Brennan. Connor's mother Trish celebrated her birthday running her first marathon and in doing so was "fighting back against a disease that has taken too much."

Join runners like Trish next year at the 2018 Chicago Marathon, which takes place on October 7, 2018.

Sign up now at nfendurance.org/chicagomarathon.



Team Chafing the Dream, who ran for NF Hero Connor Brennan



Jacksonville NF Walk

The October Jacksonville NF Walk marked the second year of double regional participation by Team Nicholas, whose hard work brought in more than \$17,400 and earned them the title of Top Fundraising Team. Fourteen days prior to this Florida event, Team Nicholas participated across the country in the Seattle NF Walk where they were the Third Highest Fundraising Team. Walking in honor of NF Hero Nicholas Lindeback, this team is passionate about creating community awareness and raising necessary funds to end NF. Special thanks to Walk organizer Lori Linkous, and to the Lindeback family, who flew in from Seattle so Nicholas could walk with his grandparents.

“Nicholas is a fighter. We walk and raise funds for research because we want him to see that the community is fighting with him. Together we will end NF!”

—TERRI TURNER, NICHOLAS’ GRANDMOTHER



Seattle NF Walk

On Saturday, September 23rd, over 200 people gathered at Owen Beach in Tacoma, Washington for the fourth annual Seattle NF Walk and raised more than \$89,000. NF Hero Natalie Denham was in the spotlight at this Walk, as were many other NF Heroes who enjoyed a day full of fun, food, and laughter. Special thanks go to top fundraising team Team Isla, who raised more than \$50,000 for NF research. Panera Bread graciously donated delicious food for the event, and the Washington Ladyhawks and Saint Martin’s cheer squad contributed their time and talents to the NF community.

ADDISON OAKS NF WALK AND LITTLE HEROES RUN

The sixth annual Addison Oaks NF Walk and Little Heroes Run took place on September 17th at Addison Oaks Park in Michigan and was a smashing success! Lead by co-chairs Christy Wheaton and Kristen Glazer, this event raised more than \$55,000 for NF research. The event honored more than 20 local NF Heroes of all ages and celebrated their bravery with over 400 of their friends and family. The sunshine, smiles, and comradery made it a day to remember.



Chicagoland NF Walk

A new location, new date, and unseasonably high temperatures did nothing to cool off the Chicagoland NF Walk! Building on the success of previous years, the Walk was held on September 23rd and brought in nearly \$25,000—thanks to some of the biggest fundraising teams yet! Team Might Meeks once again had a great turn out, and newcomers Aburi’s Avengers also made a splash. These two teams were responsible for over 90% of the event’s fundraising! Special thanks go out to Kristen Meek and Staci Raymond for making this year’s Walk so successful.

New “Ask the Expert” Webinar Series

We know that, for some patients, it can be challenging to find a local NF expert. With that in mind we are excited to launch “Ask the Expert,” an online, interactive webinar series. Videos of NF experts speaking about a variety of topics related to NF will be posted online. After each new video is posted, we will invite you to submit any questions about what you’ve learned for two weeks. These questions and answers will then be posted online along with the video for everyone’s benefit!

It is our goal to empower the NF community by providing the latest in NF research and knowledge. Please join us! Be sure to watch the first of many webinars online beginning this January at ctf.org/patienteducation.

NF News is the official publication of the Children’s Tumor Foundation. All issues are available on our website at www.ctf.org. Please direct any questions or feedback to info@ctf.org.

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

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TO END NF

Shop the Children’s Tumor Foundation official store for your holiday gift giving, and pick up our new 2018 Calendar, which celebrates CTF’s 40th anniversary and the This is NF portrait series.

ctf.org/store



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NF News is available online! Read the newsletter of the Children’s Tumor Foundation at ctf.org

If you’d like to save the Foundation the cost of printing and mailing this newsletter to you, visit ctf.org/newsletter-unsubscribe and let us know. We’ll email you the newsletter going forward.



★ *Happy Holidays!* ★

FROM OUR FAMILY TO YOURS



The Staff of the
Children's Tumor
Foundation