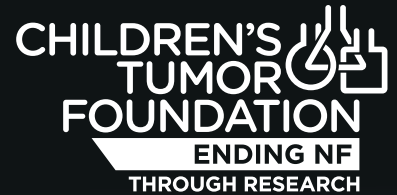


NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
WINTER 2019



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NF Innovation Week

The Children's Tumor Foundation (CTF) had a monumental presence in San Francisco this September as neurofibromatosis (NF) patients, clinicians, researchers, families, caregivers, volunteers, and data experts came together for a series of educational and inspiring events. The NF Conference and NF Forum were held in conjunction at the Hyatt Regency Hotel, San Francisco from September 20 – 24, 2019. Nearly 500 clinicians, researchers, pharma representatives, patient advocates, and other healthcare professionals attended this year's NF Conference. Adding to this number were over 150 Forum participants, including our Volunteer Leaders, which brought the number of individuals whose sole focus is to end NF to well over 650 people, and all under one roof! The week prior to these annual events, the second NF Hackathon was held to mine the data collected from years of CTF research.

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*Michael Fisher, MD,
Andrea Gross, MD, and
Brigette Widemann, MD
at the NF Conference.*

FROM the President

Annette Bakker, PhD



As we reflect on the past year here at the Children's Tumor Foundation, I am deeply grateful to all of you, who have made our success possible, thereby improving the lives of all those living with neurofibromatosis (NF). Because of you, we are closer and closer to FDA approved treatments for NF.

Nowhere was the importance of your support more palpable than at this year's NF Conference, NF Forum, and NF Hackathon in San Francisco, California. Researchers, clinicians, and data experts from around the world gathered to share the latest findings in the field of NF. In the same San Francisco hotel, patients, volunteers, and caregivers came together to learn and encourage one another. I am tremendously

proud to represent such a wonderful group of patients and families, and a group of experts so enthusiastic about serving them.

Autumn is a season of fundraising for the Foundation, and this year many delightful events brought our donors together to fund research. At the National Gala in New York City, we celebrated the launch of the Discovery Fund, a \$8 million initiative supporting the Foundation's longstanding commitment to driving the most promising NF research. At the Gala, it was an honor to express our gratitude to the Discovery Fund Founders: Shelley and Frank Haughton, Richard Horvitz and Erica Hartman-Horvitz, and Jim Bob and Laurée Moffett.

NF Hero Brianna Worden was named our incoming 2020 National Ambassador, an honor passed to her from our current Ambassador, McKinnon Galloway. Brianna spoke passionately about her journey with NF, describing a story full of exceptional strength, resilience, and courage.

At the Detroit beNeFit, which is held each year by our esteemed Board Member Dan Gilbert and his lovely wife Jennifer, another remarkable NF Hero was honored.

Aidan Fraser received the Strength and Honor Award on that fun-filled evening. I have had the pleasure of getting to know Aidan this year through his work with CTF and his advocacy at the FDA.

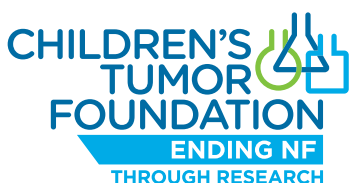
The Children's Tumor Foundation is dedicated to finding treatments for all types of neurofibromatosis: NF1, NF2, and schwannomatosis. This year marked the launch of the NF2 Accelerator Initiative, and we are incredibly excited about the progress being made toward treatments for NF2.

NF has no boundaries and CTF's global activities are moving forward to help the 2.5 million people worldwide who are living with NF. You can read more about CTF Europe in this newsletter, and I especially want to thank WuXi AppTec for their help in spreading NF awareness in China this past year.

Gratefully Yours,

Annette Bakker, PhD
President, Children's Tumor Foundation

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NF CONFERENCE RECAP

The Conference's three-and-a-half-day agenda was packed with sessions covering a vast array of important topics. With a nod to our opening keynote speaker and borrowing from the title of his talk, one could say, the theme of this year's Conference was, "From Challenge to Challenge - and a whole lot in between."

The half-day pre-conference Clinical Care Symposium was organized by the CTF Clinical Care Advisory Board (CCAB), and focused on themes of NF diagnostic criteria revision, newly published guidelines for NF1 management, and the integration of these guidelines into clinical practice. The symposium was chaired by CCAB Chair Scott Plotkin, MD, PhD, of Massachusetts General Hospital.

The opening keynote address of the Conference was given by Stephen Groft, PharmD, senior advisor at the National Center for Advancing Translational Science (NCATS) and one of the leading experts in the rare disease space. His keynote focused on the challenges facing the rare disease community, and called for researchers to recognize the value of patient registries, natural history studies, research consortia, and inter-

national networks.

Natural History of Neurofibromatosis

Brigitte Widemann, MD, of the National Cancer Institute (NCI) provided an overview of the Natural History Studies for Drug Development in Rare Disease at the National Institutes of Health (NIH). She explained that elucidating the natural history and biology of plexiform neurofibromas will help identify strategies for cancer prevention.

Invited speaker Belinda Barton, PhD, from the Children's Hospital at Westmead, Australia presented on the natural history of the NF1 cognitive phenotype in early childhood. The aim of this study was to identify early predictors of later cognitive and reading difficulties.

Pam Wolters, PhD, of the NCI gave a platform talk on physical functioning outcomes in children with NF1 and plexiform neurofibromas on the SPRINT trial, which is a Phase II trial of the MEK inhibitor selumetinib. One objective of the study was to evaluate the effects of selumetinib on physical functioning.

Ina Ly, MD, of Massachusetts General Hospital, presented a platform talk on long-term



Keynote Speaker Stephen Groft, PharmD, of the National Center for Advancing Translational Science

follow-up of adult NF1 patients using whole-body MRI, which demonstrates dynamic changes in internal neurofibroma size.

Jonathan Payne, PsyD, of the Murdoch Children's Research Institute, Australia, described preliminary findings of autism spectrum disorder in children with NF1 from the multi-site PANDA study, which established a framework to better understand and treat autism.

Kristin Lee, MD, of the University of Wisconsin-Milwaukee gave a platform talk describing her team's work on examining attention problems and anxiety in children with NF1, and suggested that psychosocial functioning for children with NF1 needs to be regularly monitored over time.

In his platform talk, Cody Peer, MS, PhD, of the National Cancer Institute, presented data from a 10-year NIH natural history study to characterize plexiform neurofibroma growth, with a goal of using this model to predict the growth of these tumors.

NF CONFERENCE

PSYCHOSOCIAL ISSUES OF THE NF PATIENT

Ana-Maria Vranceanu, PhD, and Ethan Lester, PhD, of Massachusetts General Hospital/Harvard Medical School, co-chaired this session. Dr. Vranceanu noted the importance of increasing funding for psychosocial clinical trials, as well as ensuring that we retain talented psychologists in the field of NF. Dr. Lester presented his and Dr. Vranceanu's research on resiliency across adults, adolescents, and adults with NF2 who are deaf. Dr. Lester presented data from three randomized controlled Phase II clinical trials, which showed substantial improvement in resiliency factors and quality of life after group-based psychosocial interventions were delivered via secure live video.

Following their presentation, Staci Martin, PhD, of the National Cancer Institute, spoke about exploring treatment engagement as a key feature of treating chronic pain symptoms and demonstrated that, when given an Acceptance and Commitment Therapy (ACT) intervention, 60% of patients participate in at-home practices on a weekly basis. Allison Del Castillo of the Children's National Health System presented data highlighting the relationship between internalizing and externalizing psychiatric symptoms and social functioning within children with NF1.

These three talks comprehensively showed that psychosocial factors are common across all neurofibromatoses and all ages, and that psychosocial interventions show high promise in improving quality of life, resiliency, and pain in patients with NF1, NF2, or schwannomatosis.

NF CONFERENCE

Cancer

Sunday began with a session focused on cancer and was led by a keynote given by Lloyd B. Minor, MD, Dean of the Stanford School of Medicine. His talk focused on the future of precision medicine, and this new incredible opportunity to not just treat a disease, but to predict it, prevent it, and cure it.

The cancer session featured presentations discussing new therapeutic approaches and insights for multiple NF1-related tumors, including plexiform neurofibromas, malignant peripheral nerve sheath tumors (MPNST), optic pathway glioma, and NF1-associated leukemia. Several groups also discussed novel drug targets for NF1-related cancers.

- **Luis Parada, PhD**, of Memorial Sloan Kettering Cancer Center reviewed his team's work using combination therapy approaches to slow MPNST growth in mice.
- **Kyle Williams, PhD**, from the University of Minnesota described results from a chemical screen in MPNST models identifying potential new treatment compounds. This research was funded by CTF's Synodos for NF1 and a Young Investigator Award.
- **Nicole Brossier, MD, PhD**, of Washington University, MO, explained new insights into the cell-of-origin for optic pathway gliomas.
- **Kevin Shannon, MD**, Stanford University, discussed his decades of work on JMML, a pediatric leukemia that can occur in NF1 patients (funded through

- CTF's Neurofibromatosis Therapeutic Consortium).
- **Rebecca Dodd, PhD**, of the University of Iowa, presented on the work in her laboratory using CRISPR/Cas9 technology to identify important immune cells that contribute to MPNST growth.
- **Eduard Serra, PhD**, Program on Hereditary Cancer, Barcelona, described his team's development of an inducible pluripotent stem cell (iPSC) model to study plexiform neurofibroma development.
- **Geraldine O'Sullivan Coyne, MD, PhD**, National Cancer Institute, presented preliminary findings from the phase II clinical trial of selumetinib in adult patients with plexiform neurofibromas.

NF CONFERENCE

Pain and Itch in NF1, NF2, and Schwannomatosis

Pain and itch are two major clinical problems for patients, but these symptoms remain poorly treated.

Allan Basbaum, PhD, from the University of California San Francisco, presented a keynote in which he discussed cutting-edge approaches to study pain and pain control in the laboratory. This includes generating drugs that specifically block proteins that cause pain-sensing neurons to fire, and using genetic engineering to introduce a "turn-off" switch into pain-sensing neurons. He also discussed the importance of understanding the role of the brain in shaping our complex experience of pain.

- **Xinzhong Dong, PhD**, from Johns Hopkins University, described his laboratory's efforts to identify the receptor proteins that cause itch in patients with obstructive liver disease and people with contact dermatitis.
- **Rajesh Khanna, PhD**, of the University of Arizona, shared data characterizing pain in a minipig model of NF1. These minipigs have increased electrical signals in sensory neurons which may cause pain.
- **Fatima Banine, PhD**, of the Oregon Health Sciences Center, presented data showing that the pain receptor TRPV1 is upregulated in a mouse model of schwannomatosis. Her data also suggest that the increased pain receptor is caused by different molecular pathways depending on whether the schwannomatosis is caused by mutations in the *SMARCB1* or *LZTR1* genes.
- **Stephanie Taylor, PhD**, of the Veterans' Administration Los Angeles, shared information on complementary and integrative health (CIH) approaches to pain treatment, which include things like meditation, yoga, acupuncture, and therapeutic massage. Early research evidence compiled by Dr. Taylor's team suggests that CIH approaches can improve pain in a wide variety of diseases.



NF CONFERENCE

NF2 AND SCHWANNOMATOSIS

There were three sessions specific to NF2 and schwannomatosis, which covered basic biology, genetics, and mosaicism. Basic science researchers talked about their work focusing on the Hippo, YAP, and Taz tumor suppressor pathways as potential therapeutic targets for NF2. The clinical session featured clinical and molecular presentations on mosaicism in NF2 and the results of a drug trial of an mTOR inhibitor drug in a tumor biopsy phase 0 study. Finally, Dorothy Halliday, BSc, MBBS, PhD, FRCP, from the Oxford NF2 service, presented on the clinical implications of mosaicism. This included the lower risk of mortality and risk of passing NF2 on to any children.

CUTANEOUS NEUROFIBROMAS - Bench to Bedside

The vast majority of adults with NF1 cope with cutaneous neurofibromas (cNF) without approved drug therapies. Jaishri Blakeley, MD, of Johns Hopkins Medicine, Matthew Steensma, MD, of Spectrum Health, and Eduard Serra, PhD, Program on Hereditary Cancer, Barcelona, moderated talks on the current status of clinical management and translational studies for cNF.

- **Lu Le, MD, PhD**, of University of Texas, Southwestern, gave a talk on the cellular and animal model studies that characterized and defined the likely cell of origin for cNF.
- **Pierre Wolkenstein, MD, PhD**, of Hôpital Henri-Mondor, France spoke on the management and treatment landscape for cNF.
- **Yanan Yu**, a PhD student from the University of Florida, presented data in support of the idea that there are genes that

influence the variability of neurofibroma presentation beyond the *NF1* gene. One of the genes they identified that could be responsible for increased neurofibroma activity is *ATM*, a cancer-related gene well known for influencing cell division and DNA repair.

- **Jianqiang Wu, MD**, of the Cincinnati Children's Hospital, presented data about the role the *MiR-155* gene plays in regulating neurofibroma formation, and suggested that this could be a promising drug target.
- **Katherine White and Sara Osum, MD**, of the University of Minnesota, funded by CTF's NF1 Synodos, revealed new data about the development of skin and deep neurofibromas in minipig models of NF1. The minipig models had clinical features that echo the human experience with cNF and provide a valuable system in which to study the complexities of cNF development over time.

DIAGNOSTICS & IMAGING

Keynote speaker Heike Daldrop-Link, MD, PhD, at Stanford University School of Medicine, discussed how integrated imaging techniques can be both convenient and time-saving, and that this approach for whole body evaluations for NF patients can provide a great deal of information, including pinpointing presence of lesions that are suspicious for malignancy.

The talks in this session underscored the importance of imaging as a diagnostic tool and as a measurement of response to therapy.

- **Wenli Cai, PhD**, from Massachusetts General Hospital, discussed exciting new developments using computers to assess internal characteristics of plexiform tumors, called tumor texture analysis, increasing the reliability of evaluating response to treatment.
- **Caitlin Tydings, MD**, of the Children's National Medical Centre, described a new technique called magnetic resonance-guided high intensity ultrasound as an alternative to surgery in removing nodular neurofibromas.
- **Shivani Ahlawat, MD**, from Johns Hopkins University School of Medicine, showed that contrast enhanced MRI sequences were not necessary to characterize neurofibromas, and that non-contrast whole body MRI with diffusion weighted imaging is sufficient.
- **Michel Kalaramides, MD, PhD**, of the Hôpital Pitié-Salpêtrière Paris, talked about the emerging technique of cranial nerve tractography to determine the position of cranial nerves before tumor surgery and to reduce damage to these



nerves.

- **Rosalie Ferner, MD, FRCP**, of Guy's Hospital London and Dr. Ahlawat presented difficult clinical problems in neurofibromatosis, where imaging was pivotal in making a diagnosis.
- **Eva Dombi, MD**, from the National Cancer Institute, presented an observational study of 42 individuals with NF1 and non-ossifying neurofibromas that are benign developmental bone lesions found mainly in the legs.

NF CONFERENCE

Gene Editing and Gene Therapy for NF1 and NF2

On Monday afternoon two concurrent sessions focused on a variety of basic science advancements in understanding new aspects of NF1 and NF2 biology, as well as understudied clinical features of both conditions.

The session on Targeted Gene Editing and Gene Therapy for Neurofibromatosis drew a lot of interest, as gene editing is a rapidly growing technology that is finding increasing traction outside of neurofibromatosis, and often driven by the discovery of CRISPR gene editing.

- **Aaron Schindeler, BSc, PhD**, from the University of Sydney, introduced the historical background and recent advances in CRISPR/Cas9 and noted its potential utility in both medical diagnostics and treating genetic diseases.
- **Marco Giovannini, MD, PhD**, from University of California, Los Angeles discussed his recent work advancing gene

therapy, which may have a major impact on the treatments.

- **Bruce Korf, MD, PhD**, from the University of Alabama at Birmingham, noted the wide range of NF1 mutations that can be causative for disease; thus a variety of personalized approaches not only involving CRISPR but also exon skipping have therapeutic potential.
- **Rajesh Khanna, PhD** of the University of Arizona, presented work using gene editing to reveal novel molecular mechanisms for pain that have enabled him to devise new strategies for pain treatment.
- **Scott Plotkin, MD, PhD**, of Massachusetts General Hospital, discussed the ethical and governance issues associated with bringing gene therapy treatments to NF. These conditions are amongst the most challenging to apply gene therapy solutions to compared to other diseases, and the growing consensus is that safety is a paramount consideration in any trials moving forward.

NF CONFERENCE

Poster Award Winners



Oral presentations were given by the top three poster awardees in the clinical and basic science categories. Two of this year's winners in the basic science poster contest were CTF Young Investigator Awardees.

BASIC SCIENCE POSTER WINNERS

FIRST PLACE:

Juan Mo, UT Southwestern Medical Center
 "Humanized Neurofibroma model to delineate tumor pathogenesis and preclinical therapeutic testing"

SECOND PLACE:

Evan O'Laughlin, Massachusetts General Hospital/ Harvard Medical School
 (Young Investigator Awardee) "Modeling NF2-deficiency in the liver"

THIRD PLACE:

Jordan Kohlmeyer, University of Iowa, Holden Comprehensive Cancer Center
 (Young Investigator Awardee)
 "Targeting RABL6A-RB1 Signaling Suppresses MPNST Pathogenesis"

CLINICAL SCIENCE POSTER WINNERS

FIRST PLACE:

Yang Hou, National Cancer Institute
 "Academic Achievement and Cognitive Functions of Children and Young Adults with NF1 and Plexiform Neurofibromas: Stability and Change Across Six Years"

SECOND PLACE:

Danielle Griffin, Children's National Medical Center
 "Morphology of the Corpus Callosum and Neuropsychological Development in Children with Neurofibromatosis Type 1"

THIRD PLACE:

Line Kenborg, PhD, Danish Cancer Research Center, Copenhagen, Denmark
 "Pregnancy Outcomes in Women with NF1: A Danish Population-Based Cohort Study"

Final Keynote

The final keynote was presented by Vincent Riccardi, MD, past von Recklinghausen Awardee. His talk pointedly challenged attendees to think outside the box, and explore the many areas of research that could potentially yield fruitful insights into NF1 research. His talk “Perspectives of Long-term Recklingologist: Focusing on the Future” was an apt way to not only begin the final day of the conference, but as a thought-provoking challenge to future “recklingologists” who followed his keynote at the podium to present their poster prize-winning research.



Von Recklinghausen Award Winner
Scott Plotkin, MD, PhD, with CTF
President Annette Bakker, PhD

2019 Friedrich Von Recklinghausen Awardee Scott Plotkin, MD, PhD, a Champion for NF2

This year's Friedrich von Recklinghausen Award was presented during the Saturday evening combined Forum/Conference Gala Dinner to Scott Plotkin, MD, PhD, Director of the Family Neurofibromatosis Center at Massachusetts General Hospital, and Professor of Neurology at Harvard Medical School.

For many years, Dr. Plotkin has sought tirelessly to improve the lives of patients with NF2: as an investigator on clinical trials of bevacizumab and other

candidate therapeutics; as a co-PI of the Synodos for NF2 drug discovery program; and as co-founder of NF2 Therapeutics, Inc. Dr. Plotkin has also made major contributions to NF clinical trials as founder and co-Chair of the Response Evaluation in Neurofibromatosis and Schwannomatosis (REiNS) International Collaboration. Launched at the 2011 CTF NF Conference, REiNS now consists of 150+ clinicians, researchers, and patient representatives working across 8 topical groups to standardize and improve clinical outcome measures for use in NF clinical trials.

Dr. Plotkin has demonstrated consistent leadership in the field through his service in numerous committees and conferences; he currently serves as chair of the CTF Clinical Care Advisory Board and co-chair of the International Collaboration for Revision of Diagnostic Criteria for Neurofibromatosis. Finally, this award recognizes the outstanding contributions Dr. Plotkin has made in mentoring new NF clinicians and researchers, many of whom have gone on to receive funding for new lines of NF research from CTF, the Department of Defense, and other agencies.

The Children's Tumor Foundation's Friedrich von Recklinghausen Award is given to individuals in the neurofibromatosis community who have made significant contributions to NF research or clinical care.

NF Forum

Another year and another successful NF Forum! While the event was ideally located in downtown San Francisco and attendees were treated to delicious meals and Ghirardelli chocolate throughout the weekend, we believe what made this Forum so special was the range of important topics covered in the programming.

Over the past several months Dave Viskochil, MD, PhD, Andrea Gross, MD, and Patient Advocates Jessica Samblanet and Maureen Hussey, along with CTF staff members Traceann Rose and Kate Kelts, distilled feedback from the NF Community and organized a dynamic program for the weekend. Inspired by the nearby Golden Gate Bridge, the theme for the weekend was 'Bridging the Gap' between NF patients, families, and researchers.

This year an extended program started on Friday afternoon, featuring engaging sessions about the **NF2 Landscape** and **Patient Advocacy in Healthcare and Research**. During the Opening Reception, attendees were treated to entertainment from a roving magician while they browsed the patient poster session, and Breakout Sessions were held on many specific topics.

The Session for Pediatric NF1 focused on the latest updates, such as **Guidelines for the Care of the Child with NF1**, published in 2019, which was reviewed during this session. Also reviewed was **NF1 Genetics: Beyond the Basics**, which was followed by a discussion of whether to consider genetic testing, the limitations of testing, and the exciting research being done on genotypes and phenotypes in NF1. A session called **NF1 & School: Bridging the Gap for NF Fighters** covered various challenges a child with NF1 might encounter in school, such as learning differences, social-emotional issues, and the role of neuropsychiatric testing.

We were thrilled to have larger than usual **Adults With NF** attendance at the Forum, with a special networking meeting held for the group. A session on adults included a review of the **Guidelines for the Care of the Adult with NF1**, published in 2018. Also reviewed was **NF1 Genetics and the Family**, which discussed how the genetics of NF1 can impact family planning decisions, including options for fertility care and the value of genetic counseling for adults with NF1. In **NF1 & Work: Bridging the Gap as an NF Fighter**, the discussion centered around how medical needs can change related to NF over the course of a lifetime.

The NF2 Breakout Sessions had a large contingent of experts who spent time answering patient questions both in the room and over the webinar, as well as presenting on hot topics for NF2. The **Diagnostic Criteria for NF2 & Schwannomatosis** session provided an in-depth review of these two types of NF. A discussion of **Family**



Planning Options for Individuals Living with NF2 or Schwannomatosis covered the value of genetic counseling and the options for genetic testing of children and family members. **NF2 & Hearing Loss: Bridging the Gap** included an overview of the mechanics of hearing loss in NF2, including alternative forms of communication and hearing restoration.

There were general sessions that benefitted all attendees, including round table discussions held over lunch. Patients and families joined the NF Clinic Coordinators and Directors for discussion about life with NF, and we heard from two courageous NF Fighters about their journey.

Two very popular, interactive, and engaging sessions were **NF and Pain** and **The Pursuit of Therapy Development** panel discussions. Pain can play a significant role in the life of an individual with any type of NF, and this talk covered how pain impacts quality of life and the latest in pain interventions. During **The Pursuit of Therapy Development** panel, attendees heard from a variety of scientists, researchers, government regulators, and medical professionals about clinical trial designs, how patients are working with researchers in endpoint design, how drugs get FDA approval, and how patients can access drugs after FDA approval.

The NF Forum was held in collaboration with the NF Conference this year, and the combined gathering gave patients the opportunity to meet experts, discuss current research with young investigative researchers, and enjoy continued conversation during a combined dinner of clinicians, scientists, and patients. The integrated meeting brought the CTF mission full circle: driving research, expanding knowledge, and advancing care for the NF community.



Onno Faber, NF2 patient and entrepreneur, Marco Giovannini, MD, PhD, NF researcher, and Salvatore La Rosa, CTF Chief Scientific Officer



NF Hackathon

The weekend prior to the NF Conference, September 13-15, more than 75 data specialists, engineers, and artificial intelligence experts gathered at the Google Developers Launchpad Space in San Francisco to explore vast amounts of data from the NF Data Portal, a collaboration of the Children's Tumor Foundation, Sage Bionetworks (Sage), and the Neurofibromatosis Therapeutic Acceleration program (NTAP). The high-energy hackathon participants broke up into 10 teams to explore NF-related genomics, computer science and vision, statistics, and bioinformatics, and four teams were selected to present their findings present their findings at the NF Conference.

The Hackathon was managed by SVAI (Silicon Valley Artificial Intelligence), and the participants focused on three core areas: Imaging, Preclinical Drug Modeling, and Genomics.

The four winning teams that presented at the conference were (in no particular order):

TEAM NULL FUNCTION: Integration of multidiscipline analysis from DNA sequencing, RNA sequencing, as well as the previous knowledge about the gene interactions, to identify new target genes for further drug discoveries.

TEAM QIAGEN: Inferring regulators and pathways involved in NF1 and NF2 tumors originating from Schwann cells using gene expression data.

TEAM STIR: Automated segmentation of whole-body MRI using deep learning segmentation algorithms.

TEAM DERMAVIZ: Comprehensive Research Suite for integrated Phenotyping, Genotyping and 3D Modeling for NF patients.



Volunteer Leadership Training Conference



Over 50 Volunteer Leaders, from all over the country, gathered with CTF staff in San Francisco for an annual daylong conference. They shared in a day of networking, leadership training, camaraderie, and skill building to further enhance the NF community-building work that they partner in together across the country. As part of the weekend, each of these dedicated volunteers attended the NF Family Forum as CTF ambassadors and made connections with Forum attendees from around the world.

“I was feeling tired in my event efforts, and lonely in my NF journey. The VLTC re-energized my efforts and sparked my creativity for the upcoming year.”

“It's always re-energizing. I'm thankful for the resources CTF gives us and tools to make us better.”

To learn more about how to get involved with the Volunteer Leadership Council, email Connie Sorman at volunteer@ctf.org.



Dear Friends,

We are proud to announce the CTF Discovery Fund for NF Research, an \$8 million dollar investment over 3-5 years that will fund a minimum of 45 new research studies that will accelerate drug discovery for neurofibromatosis (NF). This initiative is set up to attract and invest in the best and brightest minds, who will advance our goal of bringing new treatments to patients faster and more efficiently.

The urgency of this work was brought to the forefront a few months ago when six brave heroes told their NF stories at the first neurofibromatosis listening session held at the U.S. Food and Drug Administration, also known as the FDA. The FDA is the agency that makes the ultimate decision about whether treatments are approved for patients, and on that day, a room full of FDA staff listened with rapt attention as NF patients and parents, and Children's Tumor Foundation staff and volunteers, spoke of the pain and fear of living with NF.

The FDA staff – all experts in their fields and among the best science has to offer - listened attentively to our assembled NF team because the details shared are those that cannot necessarily be captured in medical charts or scans. These snapshots of living with NF are poignant glimpses into the realities of NF that often go unnoticed or unrecognized because of lack of knowledge, or fear, or both.

And so, one by one – **Aidan Fraser**, **Jesse Sorman**, **Brianna Worden**, **Jack Burke**, **Maureen Hussey** (speaking for her daughter, **Maggie**), and **McKinnon Galloway** – rose before the room and eloquently and impressively shared about

their lives, from when they learned of their diagnosis, to the daily realities, to their hopes for the future. In all its outward and inward diversity, the NF story - both NF1 and NF2 – was well-characterized for the audience, and the NF community was well-represented through these six courageous individuals.

Brave NF Heroes, Brave NF Scientists

A few months later, the NF research community assembled in San Francisco for our annual scientific gathering, called the NF Conference. With over 500 experts in attendance, the buzz in the rooms was palpable. [We are, for the first time in NF history, at the brink of a potential approved treatment for NF!](#)

Clinical trials at the National Institutes of Health (NIH) have shown that the MEK inhibitor selumetinib reduces tumor size from 20-55% in over 70 percent of patients with inoperable



plexiform neurofibromas. This game-changing moment for NF is here because of an early-stage discovery by Children's Tumor Foundation-funded NF researchers who showed that MEK inhibitor drugs have the potential to affect tumor size. This spark of an idea was followed by a concerted team effort among many groups who are working collaboratively to bring this drug to patients so that it is safe and effective. And there are other MEK inhibitors in the clinical pipeline, including mirdametinib, which should bring even more life-changing options to even more patients!

We are at this unique moment because of supporters like you, who took a chance on the Children's Tumor Foundation, and the inspiring scientists we funded, who took a chance in their research. You have stood with our NF community, and believed in the work we are doing to solve the challenges of all forms of NF.

As patients like Aidan, Jesse, Brianna, Jack, Maggie, McKinnon, and millions of others are told to "watch and wait" to see how tumors will impact their lives, we are impatient with that status quo, and obsessed with **discovering** the answers we need to end NF, and **accelerating** the path to that goal.

The CTF Discovery Fund for NF Research

We are launching a mission critical Discovery Fund for NF Research. This multi-year, \$8 million initiative will drive and fund the best and most promising NF research, wherever it may take place. **The Discovery Fund will speed the process, and bring treatments to people with NF faster. We need your help to make this idea a reality.**

Discovery Fund researchers will focus on all stages of NF drug development, from basic research to translational research to clinical trials. The ultimate goal is the development of new treatments and cures.

The everyday goal for our NF patients is an improved life free of the complications of NF. No more "watch and wait." Instead: "here's what you can do." We are enthusiastic about 20-55% tumor reduction in 70% of patients. But we want 100% tumor reduction in 100% of patients!



The Answer is in Discovery ... and Acceleration

That is our Children's Tumor Foundation focus – to reach the day when "End NF" means something for all NF patients.

And so as we drive, fund, and

catalyze the best research, we are determined to break through the barriers that stand in the way. In the last 5 years alone, we have seen the following results:

- **Tripled the number of NF clinical trials from 19 to 65**
- **Generated promising drug candidates for both NF1 and NF2, and are leading a worldwide consortium focused on pain, for schwannomatosis**
- **Expanded the NF Clinic Network by 30%, so more patients receive quality care**
- **Formed a worldwide collaborative (Synodos) of over 20 leading institutions focused on NF**
- **Attracted follow-on funding that has tripled the impact of your support of CTF**
- **Doubled the size of the NF research community, meaning more researchers are searching for a cure**
- **Innovated new efforts in gene therapy, and more**

Help Accelerate Our Goal of Ending NF by Making a Gift Today

Our work is not done. As you know, NF is not easy, and neither is the path to effective treatments. More than ever, the NF community needs your help. Please join the brave patients I described above, and the brave researchers working hard to discover and accelerate to a cure. Make a donation to the Discovery Fund today.

Learn more, and give online at ctf.org/discovery

Or if you prefer, you can also mail your donation in the enclosed envelope. Either way, be a part of the innovation behind the Discovery Fund for NF Research, so that future groups of NF patients can visit the FDA too, but this time, in celebration of their approved treatments for NF.

Thank you for your support.

Sincerely yours,

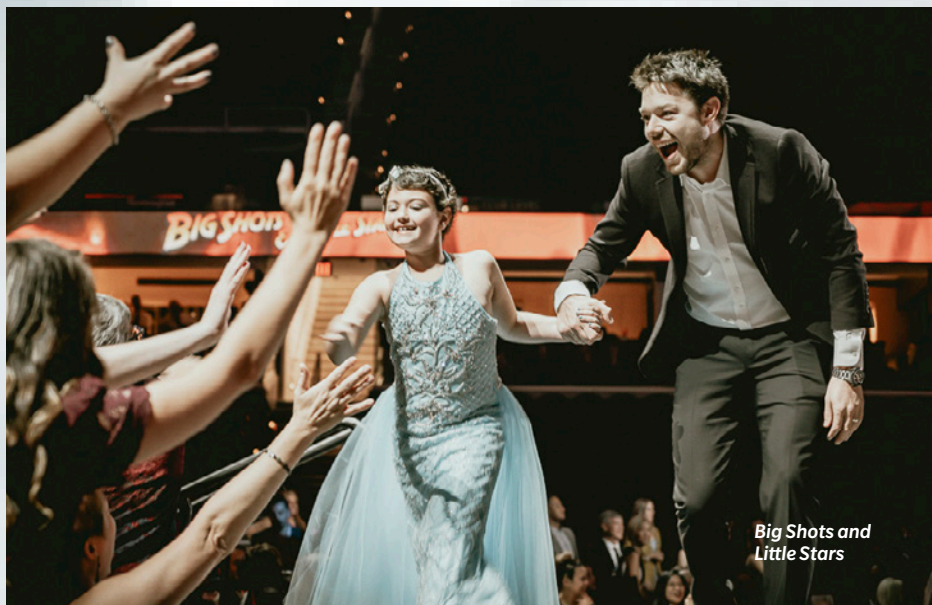
A handwritten signature in blue ink, appearing to read 'Annette'.

Annette Bakker, PhD

President, Children's Tumor Foundation

P.S. We need your help to reach this \$8 million goal, which will fund a minimum of 45 research studies. Please consider making your year-end donation in honor of the young patients who testified at the FDA, and indeed all NF patients, who need new treatments NOW.

Galas TO END NF



Big Shots and Little Stars



Brianna Worden

The Children's Tumor Foundation's annual **National Gala** took place at Cipriani, at 25 Broadway in New York City on Wednesday, November 20, 2019. The NF patient community, joined by leading philanthropists, medical professionals, civic leaders, and businesses came together for a lovely evening to celebrate important achievements in the fight to end NF. A new CTF initiative was launched at the Gala called the CTF Discovery Fund for Research, an \$8 million campaign investing mission critical support over 3-5 years in our bench to bedside research awards program.

The founding members of the Discovery Fund were recognized, which include dedicated CTF donors **Frank and Shelley Houghton**, **Richard Horvitz and Erica Hartman-Horvitz**, and **Jim Bob and Laurée Moffett**, whose combined investment in the program reaches \$6 million. **Peggy Wallace, PhD**, was named the 2019 CTF Medical Humanitarian Awardee for her many years of groundbreaking research and commitment to NF. Colorado Rockies baseball player **Ian Desmond** was also recognized as our NF Champion (read more about Ian Desmond on page 16).

NF Hero **Brianna Worden** was named the 2020 National Ambassador, an honor passed to her by **McKinnon Galloway**, the 2019 Ambassador. Now 22 years old, Brianna's role as an ambassador for neurofibromatosis awareness began many years ago, at the age of eight, when she first began advocating for NF

with the Children's Tumor Foundation. She traveled with her mother to Washington DC, New York City, and Las Vegas to symposiums and forums to help others with neurofibromatosis and share her story.

This newsletter must go to print before the evening of the Gala, so please go to ctf.org/news to view photos, videos, and an update on the event, which was emceed by Emmy Award-winning journalist **Raina Seitel**, and included appearances by Pro Football Hall of Famer **Darrell Green**, and actor, writer, producer, and friend to the NF cause **Alec Baldwin**.

The 11th annual **Big Shots & Little Stars** event took place on Saturday, October 19, 2019, hosted by the Cleveland Cavaliers Basketball Team and Flashes of Hope. For one adventure-filled night, forty very special children experienced a night to remember. The bravery of these 'Little Stars' was celebrated as they walked the runway with Cleveland Cavaliers basketball players and Northeast Ohio business leaders. The evening featured Flashes of Hope founder **Allison Clarke**, a live auction, live entertainment and more, with the proceeds going to benefit Flashes of Hope and the Children's Tumor Foundation.

Hosted each year by **Dan and Jennifer Gilbert**, the **beNeFit VII** took place on Friday, November 15 at TCF Center in Detroit, Michigan. Over the past six years, the beNeFit has raised more than \$25 million to end neurofibromatosis thanks to thousands of amazing supporters who have broken all fundraising records to advance research toward a cure for NF.

This year's 'Strength and Honor Award' was presented to **Aidan Fraser**, who is 19 years old and lives with NF1. Aidan has a large plexiform neurofibroma on his neck that extends into his brainstem, down his back to his lungs and down his left arm. He's had nearly two dozen surgeries, which he journaled about last year



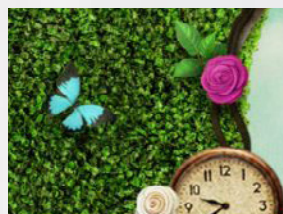
Dancing With Our Stars



McKinnon Galloway and Leslie Kates at Boston Cocktails for a Cure



Aidan Fraser



Dan and Jennifer Gilbert, with their son, Nick

for the CTF newsfeed. Aidan has been an advocate for NF and the Children's Tumor Foundation and has spoken numerous times to the U.S. Food and Drug Administration (FDA) to advocate for approved treatments for NF. Along with McKinnon Galloway, he stars in the CTF YouTube series, *Tumor Talk*.

This newsletter must go to print before the evening of the beNeFit VII, so please go to ctf.org/news to view photos and read more about this wonderful, yearly event.

Dancing with Our Stars was a huge success this year, and took place on September 5, 2019 at the Robinson Center Ballroom in Little Rock, Arkansas. The Stars for the event were the highest group of fundraisers in the event's history, which brought this year's fundraising total to \$290,000 overall. NF Ambassador **Hannah Elliott** and her mother spoke about her experience dealing with NF, and their testimonial helped raise more than \$20,000 during the

Research Auction. Dazzling dance performances followed, and a silent auction was held throughout the evening. While the votes were being tallied, the Star Alumni stole the stage to perform a wonderfully choreographed dance. Pete Tanguay was the winner of the Mirror Ball Trophy for the highest fundraiser, and Alisha Curtis won the trophy for Best Performance. With over 400 in attendance, it was a fun-filled, exciting night that brought awareness to NF and raised much-needed funds for research.

Boston Cocktails for a Cure was held on September 14, 2019, with approximately 80 attendees at the Needham Golf Club in Needham, Massachusetts. The volunteer committee, headed by longtime donor Leslie Kates, hosted a lively event with a new look and feel. The event raised more than \$100,000 with over \$40,000 earmarked for the new CTF Discovery Fund. Speakers included Scott Plotkin, MD, PhD, and 2019 National Ambassador McKinnon Galloway, who passionately reminded donors of the importance of their role in the fight to end NF. **Leslie and Dick Kates** were presented with a Thank You Award for over 40 years of service to the NF Community.

The Children's Tumor Foundation is tremendously grateful to the many individuals who attended these unique events, and to the donors and organizers who made them so delightful.

SHINE A LIGHT NF WALK

FALL HIGHLIGHTS



Denver Shine a Light NF Walk

The Denver Shine A Light NF Walk had a record-breaking year and was the top overall fundraising Walk, raising more than \$96,000! The event took place in the evening for the first time and included more than 300 participants. Organizer Stephanie Jaramillo and her amazing committee, Bonnie Bates, Mike Lynn, Shelly Pesta and their families, rallied the entire Denver NF Community, including the local NF Clinic, Cupid's Undie Run, and many End NF with Ian Desmond supporters, to come together to raise awareness and funds for NF research. A successful kickoff event was also hosted in August to introduce new families to the NF community and CTF staff.

Michigan Shine a Light NF Walk

Organizer Christy Wheaton along with Ashley Adamski, Kristen Glazer, Brian Eastman, and Kelly Eastman embraced CTF's new Shine A Light on NF evening concept and moved the Walk from Addison Oaks to downtown Rochester, Michigan, where more than 300 people attended. Main Street Billiards hosted the September event and the participants strolled through the scenic streets of downtown Rochester, shining a light on NF. Special thanks to John Sammut and his colleagues at Firstronic for hosting another successful golf outing to support the Walk, which helped it surpass its \$60,000 goal to raise more than \$77,000 this year. Michigan is the second-highest fundraising Walk of 2019 thanks to the dedicated organizers, donors, and attendees!



Kansas City Shine a Light NF Walk

The Shine a Light NF Walk in Kansas City continues to grow as a must-attend event in this community! Over 380 Walkers came together in October to bring NF out of the shadows, despite the grey skies. This community continues to raise the bar, and this year, the Shine a Light NF Walk in Kansas City reached their goal of raising \$35,000 to help CTF continue to fund important research to improve the quality of life for all NF patients. Hannah Duby, the fierce Volunteer Organizer for the Kansas City Walk, is a true example of determination and leadership.



Anne Noble Chicago Marathon

Last March, Anne Noble was in the midst of training for her fourth Boston Marathon when she went in for a scheduled surgery to remove a 3cm schwannoma tumor. She was told it went very well, and was discharged the next day. Unexpected complications, however, sent her back to the hospital, and in the weeks that followed she underwent two major throat surgeries and an emergency midnight tracheotomy. Her medical crisis kept her from the start line of the 2019 Boston Marathon, but she was determined to race. Less than two months after leaving the hospital, she ran eight miles and set her sights on running her 20th marathon in Chicago with CTF's NF Endurance Team.



Portrait of Anne Noble painted by Rachel Mindrup

"When I was 7 years old, I was diagnosed with NF2. I've had surgeries for tumors in my brain, spine, ears, leg, eyes, and most recently my throat. When I was 19, I became totally deaf, but I am fortunate to do very well with my Auditory Brainstem Implant. Having NF2 has been a battle throughout my life, but I'm mostly healthy, thanks to my CTF family, as they support research that goes into clinical trials, and more!"

Anne, now 31, is a longtime NFE athlete. She has completed multiple marathons and IRONMAN triathlons, and qualified for the Boston Marathon four times, all while raising thousands of dollars for CTF. She also works tirelessly to raise NF awareness and recruit runners.



Shane Vicars and his wife, Candie Vicars

Shane Vicars: Schwannomatosis Runner

Not that long ago, a short walk used to exhaust Shane Vicars. Now, just a couple of years later, Shane has completed 15 marathons, and he's not planning to take his running shoes off anytime soon.

In 2016, Shane was tired of feeling uncomfortable in his own body. Weighing 374 pounds, he decided to overhaul his lifestyle and took up running. He set a goal to run the Flying Pig Marathon in 2018, and that accomplishment sparked an unstoppable outlook on the future.

"I joined the NF Endurance Team because I am personally affected by NF," Shane said. "I was diagnosed at age 17 with schwannomatosis and have had 13 surgeries to my left leg. My goal is to bring awareness about NF to help someday find a cure. When I first started running, I could literally only run about 10 feet. I would run 10 feet, walk 20, run another 10—building up my endurance one day at a time."

On October 13, he ran the Bank of America Chicago Marathon, and the Indianapolis Monumental Marathon on November 9, both as a fundraising member of the Children's Tumor Foundation NF Endurance Team.

"NEVER give up! Stay focused and remember your reason for starting this journey. I felt proud raising money and awareness for NF."

UPCOMING NF ENDURANCE EVENTS:

- United Airlines NYC Half** | March 15, 2020
- Silo District Marathon, Half, 10K, 5K** | April 25-26, 2020
- Flying Pig Marathon, Half, 10K, 5K, Relay** | May 3, 2020
- TD Five Boro Bike Tour** | May 3, 2020
- BMW Berlin Marathon** | September 27, 2020
- Bank of America Chicago Marathon** | October 11, 2020
- TCS New York City Marathon** | November 1, 2020
- Rock 'n' Roll Series** | 5Ks, 10Ks, half & full marathons across the US
- Choose Your Own Challenge - Pick the adventure of your choice!**

For more information, go to nfendurance.org or email nfendurance@ctf.org

Around the COUNTRY

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



End NF with **Ian Desmond**

Ian Desmond, Colorado Rockies baseball center-fielder, has been an on-going supporter, donor, and friend to the Children's Tumor Foundation. He is a champion of the NF cause: donating and raising funds each May during NF Awareness Month, dedicating himself to awareness opportunities through media, and taking the time to meet up with patients and families during Rockies home and away games across the country. All of this support came about as a result of a friendship that he developed with NF2 Hero Ethan Brown, of South Carolina.

This year, Ian asked if we would help to organize meet-and-greets at every away game on the Rockies 2019 schedule so that he could gather patients and families to build community and share his personal story. In a true "team" effort between CTF volunteers, staff, the Rockies organization, Ian's camp, and representatives in many of the home team ballparks, there were 18 group events scheduled between Miami, FL and San Francisco, CA! This undertaking resulted in bringing nearly 1,300 CTF community members together so that they could root on their home teams, while meeting up with others in their areas, giving them the chance to thank Ian for his support face-to-face.

With the success of the 2019 baseball season behind us, we are looking forward to even larger gatherings in the coming year. Be on the lookout for a schedule of events in early spring 2020 and join us at a ballpark near you!



6TH ANNUAL #ENDNF WITH TRAVIS CLASSIC CHARITY GOLF TOURNAMENT

Late June brought about the 6th Annual #EndNF with Travis Classic Golf Tournament supporting the Children's Tumor Foundation in Utah. The event was once again hosted by the Carpenter Family and their longtime friend Matt Solum in the Salt Lake Valley. This year's event was the first complete sell-out for the #EndNF with Travis Classic, with 152 golfers attending. The net donation exceeded the tournament's fundraising goal and reached the \$12,000 mark! The entire field of golfers lined the first tee box as Travis Carpenter (age 11) kicked off the day with a ceremonial tee shot after being announced to the tee by The Ridge Golf Club head professional Bryant Boshard. Bryant has repeatedly exclaimed that the Children's Tumor Foundation tournament is his favorite, and his pro shop staff raised an additional \$500 donation throughout NF Awareness Month and beyond.



BAY HILL GOLF TOURNAMENT

On October 7, 2019 the Ehrlri family hosted The Annual Bay Hill Golf Scramble Sponsored by Wireless Zone and Planet Hollywood at Arnold Palmer's Bay Hill Golf Club and Lodge. Over the past 20 years this event has raised nearly \$200,000 to fund research to find a cure for neurofibromatosis. Washington Redskins Pro Football Hall of Famer Darrell Green helped kick off this event, with some inspirational words for the 112 golfers and volunteers. Thank you to the Ehrlri family, Darrell Green, and to all who have made this event such a success over the years.

Arlene Wirjadi

I was about five or six years old when I was diagnosed with NF1. My grandma noticed a lump on my right arm near my elbow. My parents took me to the doctor, who did a biopsy, and from there we learned that I had neurofibromatosis type 1. The way NF impacts my life is that I have trouble learning certain things. In school, I would have to work 10 times harder than other students not living with NF. I learned that I have a learning disability because of my NF and I have to see a few specialists every year, making my medical bills a bit high. I am lucky enough to have a job with health insurance, but I do have to see a lot of doctors each year and those co-payments aren't cheap.

Because of my NF, I don't want to have kids. I don't want to pass down the gene even though it is 50/50 if my partner doesn't have it. I just don't want to risk it and have my children go through what I am going through with medical issues and such. I stay motivated because I have family and friends who support me. They love me for me and don't judge me by how I look. I



stay strong by knowing that there are other NF Fighters/Heroes like myself. I am not the only one out there and there are many events where I can meet people who support each other.

I work with people who have developmental disabilities and because I work with them, I know that I am not alone. My clients and co-workers don't judge me, and they treat me like any other person. They see the good in me and love me for me. My friends and family are what makes me happy, and this includes my fiancé. Knowing that I have a special someone in my life, who loves me for me, makes me happy.

“I stay motivated because I have family and friends who support me. They love me for me and don't judge me by how I look. I stay strong by knowing that there are other NF Fighters/Heroes like myself. I am not the only one out there and there are many events where I can meet people who support each other.”

—ARLENE WIRJADI

Easton Anderson

Our life was turned upside down two weeks after Easton's second birthday. He was diagnosed with an optic glioma that was in the hypothalamic region of his brain. I was about to start nursing school and Jake, Easton's dad, was in the middle of the busy season at work (he is a mechanic). Our lives were put on hold as we spent two and a half weeks admitted at Children's Hospital of Wisconsin-Milwaukee. Easton ended up with failure to thrive, a VP shunt (ventriculoperitoneal shunt) to control the hydrocephalus and then a g-tube (gastrostomy tube) for feeding. He now undergoes chemotherapy every four weeks. We have a whole team of medical professionals that see him now: genetics, neurology, orthopedics, therapists (speech, occupational, hippotherapy, and physical), oncologists, ophthalmologists, and many others.

Easton is such a brave little warrior. He is still smiling, even after all the blood work and scans. After chemo he is sick for a week, but he bounces back so fast. We stay motivated by smiling and laughing at the small things. We take each day as it comes. Easton is our warrior and we hold it together for him. Easton loves Paw Patrol; he is absolutely obsessed with the show. His favorite puppy is Skye. In real life, he has two loyal dogs named Daisy and Ginger.

Easton loves farming (both sets of grandparents have dairy farms). He loves riding in the tractor and sitting in the parlor during milking. He likes to help water the calves and pick the eggs.

- Easton's mom, Molly





“Over the years, I have learned to be ‘zen’ with it. I am now a firm believer that the body is just the shell of the soul.”

—SHEILA DREYANKO

Extraordinary Spirit / SHEILA DREYANKO

Sheila Drevyanko grew up in Chicago, Illinois, one of 10 children but the only one with NF. She was first diagnosed at 14 years old when she had a biopsy of a tumor on her abdomen. “Now, I would have been diagnosed much earlier because I have café au lait spots and an ulnar pseudarthrosis,” said Sheila. “I had many x-rays on my bowed arm and removal of a macule in my armpit when I was six, but they never associated it with NF.”

Sheila is now 66 years old, but when she was a child very little was known about neurofibromatosis, even in the medical community. Her well-meaning parents did what many in their situation did at that time – they chose to ignore it. “Looking back, I now know that they were dealing with grief for my future, and denial. They didn’t know how to handle it. There was nowhere for them to go for support or information.”

In 1975, while seeking genetic counseling at the University of Iowa Hospital Genetics Clinic, Sheila met Dr. Mary (Wazari) Curtis, the NF Clinic Director, and attended the clinic’s support and educational meetings. In 1978, Dr. Wazari told Sheila about a new foundation being formed, the

National Neurofibromatosis Foundation (NNFF), which is now the Children’s Tumor Foundation. Sheila soon got involved with the NNFF Iowa Chapter and although she wasn’t very active in the beginning, she continued to attend meetings where she met many of her lifelong friends.

“When my husband and I moved to Des Moines in 1985, I decided to become more involved with the Foundation. I organized NF garage sales and local support group meetings. But it wasn’t until 2009 after attending a Chapter Leader’s meeting in Las Vegas with an NF Mom (Kori Ensley) that she and I decided to co-organize the first Iowa NF Walk. We just had our 10th Annual NF Walk on August 10th!”

Several years ago at a National NF Forum, Sondra Solomon, PhD, another visibly affected adult with NF who is now deceased, met with Sheila and the two women discussed the importance of addressing the experience of those who are more visibly affected.

“The general public’s reaction to disfigurement and the unfounded fear that NF is contagious causes feelings of loneliness and social isolation in many adults with NF. Even parents of children

with NF seemed to have difficulty connecting with us. We felt we could help CTF bring this tender issue and difficult dialogue to the forefront. CTF gave us their full support by adding adult-focused topics to symposiums and the NF Forum, and in 2016, the Adults with NF - CTF Facebook Group was formed. It’s a private group, by member request, but we have over 1,100 members. I help moderate the group. So anyone can reach out to me if you would like to join!”

Thanks to Sheila’s ongoing efforts to create awareness and support for adults with NF, many have found comfort and connection online and in person at the Adults with NF meetups at the NF Forum. While the word “Children” is in the Foundation’s name, neurofibromatosis is a lifelong condition, and CTF is passionately committed to research, resources, and connections for adults with NF.

When she is not tirelessly working to provide a safe haven for adults with NF to find connection and support, Sheila and her husband enjoy listening to blues music and traveling together. Her beautiful spirit, wonderful personality, and selfless care for others are just a few of the ways that Sheila is truly extraordinary!

NF knows no boundaries, and neither does our effort to end NF!

Children's Tumor Foundation Europe

One year ago, Children's Tumor Foundation Europe was launched to expand the global fight against neurofibromatosis. It's been a busy year with much progress towards these four goals:

- **BUILD** an NF funding landscape in Europe that is complementary to that in the U.S., by leveraging opportunities unique to Europe, such as collaborations with the Innovative Medicines Initiative (IMI) and the European Association of Pharmaceutical Industries and Associations (EFPIA)
- **ADVOCATE** for European funds at all levels towards NF research
- **EXPAND** global data sharing efforts
- **SERVE** as a partner to European pharma and enable/support to bring their treatments to patients.

Watch this space for more updates in the coming year, and to learn more about CTF Europe, visit: ctfeurope.org.

Children's Tumor Foundation in China

Special thanks to WuXi AppTec for helping raise awareness of NF and CTF in China!



Recently, CTF President Annette Bakker, PhD, and CTF Board Member Richard Soll, Senior Advisor, Strategic Initiatives, WuXi AppTec, had a conversation about the Foundation, the patient journey, and future prospects for those living with NF. A summary of their conversation was published on the WuXi AppTec website and shared throughout the country.

The discussion centered around ongoing clinical trials and the impact CTF has had on available trials through early seed funding. Also discussed was the vital importance of open data, and the multiple ways the Children's Tumor Foundation is evolving scientific research with platforms and collaborations intended to speed up the drug discovery process.

To read the article, please visit: ctf.org/nfchina

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

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

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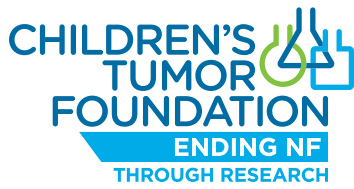
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