

NF NEWS

CHILDREN'S
TUMOR
FOUNDATION
ENDING NF
THROUGH RESEARCH

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
FALL 2021

INSIDE:

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This summer NF awareness got back into the 'real world' with a call for photos in CTF's Make NF Visible Summer Photo Contest. We know how hard the NF community works to raise awareness, so we got to see individuals and families making NF visible "From Sea to Shining Sea!" Photo Contest participants got to show off their Make NF Visible and I Know A Fighter spirit through their travels, both around the world and across the street.



"Since we have property near the flight path of Orlando International Airport, we decided to spread awareness by moving 'END NF' into the yard, 'Making NF Visible' for the millions of airline passengers and the boaters who pass by every day!"

— Lilly Ann Brooks, 2021 National Ambassador, pictured here with her father Bill Brooks

FROM the President

Annette Bakker, PhD



Throughout 2021, as we have continued to isolate due to the global COVID-19 pandemic, the Children's Tumor Foundation (CTF) community of patients and families living with neurofibromatosis (NF) remains united in our common cause, to find treatments for all those living with NF. Our 2021 theme, Make NF Visible, has been evident this year as we are actively telling the world more about NF, and the ways in which this disorder affects individuals in both visible and invisible ways.

We enthusiastically continue to celebrate the 2020 announcement of Koselugo (selumetinib) as the first-ever FDA-approved treatment for NF, which has now also been approved in Europe and Brazil, as you will read about in this newsletter. This announcement has instilled more confidence in CTF-funded research than any other time in our history.

Over the summer, researchers, clinicians, and experts joined CTF's Virtual NF Conference, a

scientific meeting that lasted for two days. An extensive recap of the research that was presented is included in this newsletter.

Likewise, patient events have continued to thrive both in-person and virtually, with families and caregivers logging in to the NF Forum and the Volunteer Leadership Conference, and young people joining a virtual NF Camp. Our Shine A Light NF Walk and NF Endurance programs have volunteer organizers, participants, and athletes joining us both online and in person.

Throughout the month of May, volunteers spread NF awareness from home in support of NF Awareness Month. On World NF Awareness Day, actors Jonathan Sadowski and James Snyder hosted a beautiful World NF Day Live event, which featured incredible musical talents and so many inspirational stories. We have more virtual events in progress, which I hope you will attend with us during the fall months.

You, our family of CTF donors, are funding life-changing research—and continue to instill hope and promise in a time of uncertainty. As individuals we can make a difference, but together – whether in person, in spirit, or online – we stand strong in the fight to end NF.

Sincerely,

Annette Bakker, PhD
President

UPDATE:

CTF President Annette Bakker Published on Fortune.com

CTF President Annette Bakker was once again published in *FORTUNE*, making the case that the repurposing of discontinued (or shelved) drugs for certain indications could have tremendous impact for rare diseases. Arguing that “giving new life to discarded drugs could save patients’ lives,” Dr. Bakker cites examples across disease areas, and also points out the potential for improved ESG (environmental, social governance) scores, a metric that is increasingly noticed by investors looking to partner with companies dedicated to improving society.

Dr. Bakker writes, “We have the preclinical data proving the value of specific shelved drugs for patients with NF. It is infuriating when companies don’t make them available to us, but we won’t give up!”

Find the full article at [ctf.org/news](https://www.fortune.com).

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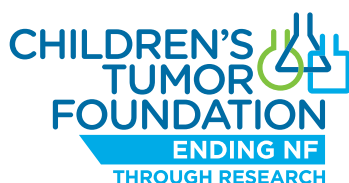
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Virtual NF Forum

The NF Forum is a patient education and family gathering providing people worldwide living with NF the opportunity to connect and learn from NF experts. This year's Forum was held virtually on June 25-26, 2021, and featured experts from around the world presenting the most current information on NF. The meeting had relevant content for everyone living with or affected by all types of NF including NF1, NF2, or schwannomatosis. The Friday evening keynote presentation was from Dana A. Schinasi, MD, and discussed Telehealth for the NF Community, followed by practical tips for coping during a pandemic.

There were some important talks for the NF2 and schwannomatosis community, beginning with a summary of the proposed changes to the diagnostic criteria in a presentation entitled, NF2 and Schwannomatosis Clinical Diagnostic Criteria. Following that presentation were talks on Clinical Care Guidelines for NF2, Communication Strategies in NF2, and Complementary Techniques for Coping with Pain. Researchers presented promising research on various treatments for hearing loss related to NF2, and for schwannomatosis-related pain.

For adults living with NF, there was a comprehensive review of the clinical care guidelines for adults with NF1 and a discussion of the impact of NF1

on the individual, followed by surgical interventions in NF1.

We know that living with NF1 can impact learning in a variety of ways. What parents should watch for and how to work with your child's school was covered during the NF1 Pediatric session, followed by a discussion of transitioning from pediatric to adult care.

There were several updates on clinical research and how patients can get involved in different initiatives, such as Response Evaluation in Neurofibromatosis & Schwannomatosis (REiNS), NF Hackathon, and the NF2 Accelerator INTUITT-NF2 Trial. There was also an overview of the Department of Defense Clinical Trials Consortium, Optic Pathway Glioma Consortium, and research updates on cutaneous neurofibromas.

The 2021 Children's Tumor Foundation Virtual NF Forum was held in collaboration with Johns Hopkins Medical Center and Robert H. Lurie Children's Hospital of Chicago. The meeting was co-chaired by Verena Stadke, MD, PhD, Robert Listernick, MD, and patient advocate Danielle Bonadies.

The meeting was made available in both English and Spanish. To watch the captioned recordings from the meeting, visit ctf.org/nfforum.

CTF Volunteer Leaders Recognition Awards

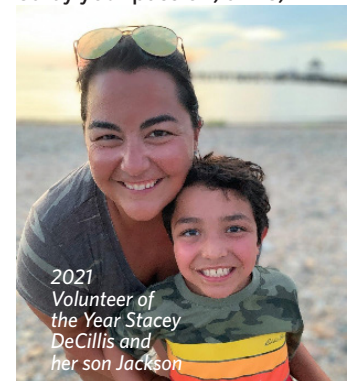
The Children's Tumor Foundation Volunteer Recognition Awards are one of the many ways that CTF recognizes and honors our incredible volunteers each year.

There are nearly 150 men and women nationwide who make up the Volunteer Leadership Council (VLC). They are CTF's public ambassadors for patient education and community outreach, our connectors, our community leaders, our fundraisers, our event organizers, our strategic partners, and our champions of advocacy and awareness.

This year an amazing group of Volunteer Leaders were eligible to receive awards. This form of recognition highlights only a select group of volunteer leaders, who were voted on by their peers.

Congratulations to our 2021 nominees and thank you to everyone who identifies as a Children's Tumor Foundation Volunteer in the present or in the past. We are awed by your passion, drive, and dedication to the NF cause.

For more information about how to become a CTF volunteer, please email volunteer@ctf.org.



2021 Volunteer of the Year Stacey DeCillis and her son Jackson

2021

VOLUNTEER RECOGNITION AWARD WINNERS

Mission Driver Award	Lynne Black, VA
Mission Driver Award	Kelly Carpenter, UT
Mission Driver Award	Renie Moss, AL
Go-Getter Award	Emily Tseffos, WI
Make NF Visible Award	Rachel Mindrup, NE
Leadership Award	Teresa Williams, VA
Leadership Award	Lara Mukabenov, NJ
Leadership Award	John Schafer, NE
Innovator Award	Ginger Marshall AR
Innovator Award	Teresa Williams, VA
Team Player Award	Stephanie Jaramillo, CO
Shining Star Award	Lynne Black, VA
Shining Star Award	Jessica Contreras, CA
Volunteer of the Year	Stacey DeCillis, NY

2021 Virtual NF Conference Highlights

Held on June 14-16, the 2021 Virtual NF Conference brought together nearly 750 attendees from 35 countries, including members of the NF research and clinical communities, patients, advocacy groups, and industry representatives. Attendees were presented with talks given by an international group of speakers on the latest developments in research and clinical care for NF1, NF2 and schwannomatosis.

In addition to the core agenda, attendees were able to view and listen to recorded poster presentations while a panel of judges selected the top three submissions from clinical and basic science entries. The meeting wrapped up with the presentation of the 2021 Friedrich von Recklinghausen Award, given to Marco Giovannini, MD, PhD of UCLA, accompanied by tributes to Dr. Giovannini from his colleagues. Read more about the poster winners and the von Recklinghausen award on page 5.

NF Clinical Care Symposium

The Clinical Care Symposium kicked off the NF Conference focusing on clinically relevant topics and projects of the CTF Clinical Care Advisory Boards (CCAB), led in the U.S. by Scott Plotkin, MD, PhD and in Europe by Pierre Wolkenstein MD, PhD and Rosalie Ferner, MD.

CCAB – United States

The U.S. CCAB launched a virtual case conference in March 2021 for NF Clinic Network (NFCN) members to expand knowledge of best practices and optimize care for specific manifestations of NF. Highlights included the continued expansion of new clinics into the NF Clinic Network (now open to Canadian clinics), a publication and patient resource about the use of MEK inhibitors for NF1-associated tumors, and a survey studying the effects of COVID-19 on NF care along with research of rapid adoption of telehealth during the pandemic (with publication).

Progress has been made in the multi-year project to identify consensus guidelines for the care of NF patients, assess the level of compliance, create an educational campaign to inform clinicians, and monitor changes in guideline-concordant behavior over time. The first step was a clinician survey assessing the awareness of and agreement with the published guidelines. Results were presented by Justin Jordan, MD, MPH (Massachusetts General Hospital) at the 2020 NF Conference. The second phase of the project involved a survey within the NF Registry (May 2021). Preliminary data was presented by Vanessa Merker, PhD (Harvard Medical School) indicating that almost 15% of the 392 respondents delayed or did not get medical care in the past 12 months because of costs. Findings suggest although most patients get recommended care for many of the guidelines, there are a significant portion that had not received recommended

monitoring and surveillance or relevant cancer screenings.

Patient Access to Novel NF Treatments

Laura Klesse, MD, PhD (UT Southwestern Medical Center) presented results of a survey of NFCN clinics evaluating comfort levels and identifying barriers to prescribing new NF treatments, including on-label FDA approved and off-label FDA approved medications. Approximately 93% of clinics had complete or high comfort levels of providing FDA on-label approved Koselugo (selumetinib), most often prescribed within oncology or neuro-oncology departments. The most significant impact reported was lack of familiarity with treatment options, followed by insurance barriers and patient concerns about side effects. A complementary patient survey using the NF Registry to determine patient experiences, barriers, and concerns is planned.

Continued on page 16

FRIEDRICH VON RECKLINGHAUSEN AWARD WINNER

Marco Giovannini, MD, PhD



During the virtual NF Conference the Children's Tumor Foundation announced the recipient of the 2021 Friedrich von Recklinghausen Award, Marco Giovannini MD, PhD, of the University of California, Los Angeles (UCLA). Dr. Giovannini is Professor-in-Residence at the Head and Neck Surgery at UCLA, Director of the Neural Tumor Research Laboratory, and a member of the Signal Transduction and Therapeutics Program at the UCLA Jonsson Comprehensive Cancer Center. He is also co-director of the UCLA Multidisciplinary NF Clinic.

The Children's Tumor Foundation's Friedrich von Recklinghausen Award is given to individuals in the professional neurofibromatosis community who have made significant contributions to neurofibromatosis research or clinical care. It is named after Friedrich Daniel von Recklinghausen (1833-1910), the German physician who first described 'von Recklinghausen's disease' – what we now know as neurofibromatosis type 1.

As a researcher, Dr. Giovannini has had a long-term interest in the genetics and biology of human cancer predisposition and progression. His lab has made significant contributions to the characterization of many major NF tumor suppressors especially *NF2*, and *SMARCB1*. The overarching goal of his lab is to effectively translate the discoveries into medicines that will improve the quality and long-term survival of *NF2* and schwannomatosis patients. He

recognized that the lack of preclinical *NF2* and schwannomatosis models present a real roadblock to developing better treatments for patients.

Not only did his lab develop a variety of innovative cell and animal models that faithfully replicate human tumorigenesis, but he is also known to openly share the models with the community and test novel discovery paradigms swiftly.

He is creative, always open to collaborating, and is now in his third year acting as the *NF2*/Schwannomatosis Mentor to the CTF Hackathon participants. This is emblematic of his enthusiasm to share his vast knowledge, not just within the community, but outside of *NF* to help educate and raise awareness.

We are very proud and delighted to recognize Dr. Giovannini's significant achievements with the 2021 Friedrich von Recklinghausen Award. Please join us in congratulating Dr. Giovannini for this well-deserved honor.

2021 NF Conference Poster Session Winners

Poster sessions are an opportunity for researchers to showcase their work in the basic and clinical sciences to an audience of *NF* researchers by creating and displaying a poster that summarizes their research. A panel of judges then select the top posters, and these investigators are invited to deliver a presentation about their work in front of the full conference.

Below are the winning posters from the 2021 *NF* Conference in clinical and basic sciences.

Read the complete abstracts on the CTF newsfeed at ctf.org/news.

CLINICAL SCIENCE

1. Kimberly Marrs, Graduate Student, California State University, Sacramento
Survey of Communication, Swallowing, and Hearing of Individuals with NF1: A Pilot Project

2. Edgar Creus, IDIBELL, Spain
A High-Throughput Screening Identifies the Combination of MK-1775 And Doxorubicin as a New Therapeutic Approach for MPNST

3. Priya Chan, MD, Fellow, Children's Hospital Colorado
Post-Operative use of MEK Inhibitors to Prevent Rebound Growth Following Partial Resection of Plexiform Neurofibromas

BASIC SCIENCE

1. Jennifer Patrilli Cram, PhD Student, Cincinnati Children's Hospital
P2RY14 Modulates Schwann Cell Precursor Self-Renewal and Tumor Initiation in Mouse Model of Neurofibromatosis Type 1

2. Myriam Mansour, PhD Student, France
Exploring Mechanisms Driving Initiation and Progression of Plexiform Neurofibromas from Prss56Cre, Nf1fl/FI Mouse Model

3. Garrett Draper, PhD Student, University of Minnesota
Induced Pluripotent Stem Cell Derived Schwann Cells Harboring MPNST-Associated Mutations Fail to Escape Senescence In Vitro

YOUNG INVESTIGATOR AWARDS 2021

The Children's Tumor Foundation is pleased to announce the funding of six Young Investigator Awards (YIA) for the 2021-2023 cycle.

JORDAN KOHLMAYER

The University of Iowa

Defining the RABL6A-YAP Axis in MPNST Pathogenesis and Therapy

NF1 patients are at increased risk of developing malignant peripheral nerve sheath tumors (MPNSTs) due to the possibility of neurofibroma transformation. This study aims to evaluate two powerful cancer pathways, RABL6A and the Hippo pathway, whose dysregulation promotes MPNST pathogenesis. We will investigate how RABL6A regulates the YAP protein to promote MPNST development, and will develop new combination therapies for MPNSTs that will have reduced toxicity and high efficacy.

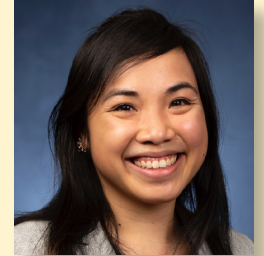


LINDY ZHANG

Johns Hopkins University School of Medicine

The Effects of RAS Signaling Pathway Inhibitors on Tumor Cells and the Tumor Immune Microenvironment in MPNSTs

NF1-associated MPNSTs are resistant to MEK inhibition monotherapy because of activation of alternate cancer signaling pathways. This study will test combinations of MEK inhibitors, SHP2 inhibitors, and CDK inhibitors, which target different signaling pathways, to treat MPNSTs. Additionally, we will investigate the role of the tumor microenvironment and the impact of various inhibitors on immune cells to design trials of drugs.



SARA PARDEJ

University of Wisconsin-Milwaukee

Neural Underpinnings of Attention in Children with NF1

Attention difficulties are a common cognitive phenotype in children with NF1, yet very little is known about the underlying neural mechanisms. This research will test the feasibility of electroencephalography (EEG) approaches in children with NF1 to identify potential biomarkers of attention problems. By studying differences in neural functioning between children with NF1, their peers, and children with ADHD, we hope to find unique functioning patterns that can effectively track the impact of medical and psychosocial interventions affecting attention in NF1.



JAMIE GRIT

Van Andel Research Institute

Targeting inflammatory signaling in cutaneous neurofibromas

Cutaneous neurofibromas (CNF) are a major cause of morbidity in NF1 and clinically behave very differently than plexiform neurofibromas (PNF). Since CNFs rely more on inflammation than the strong MEK signaling that typifies PNFs, they may need different treatment approaches. This study will test diclofenac, an anti-inflammatory COX2 inhibitor ointment, on CNFs, and will determine patient experience and tumor response after treatment.



ISABELLE LOGAN

Oregon State University

Signaling Pathways Regulated by Nitrated Proteins as Novel Therapeutic Targets for NF2

Nitrated proteins are a novel category of NF2 tumor targets as they play a key role in schwannoma growth and are not present in normal cells. The goals of this project are to investigate the regulation of signaling pathways by nitration and to identify the specific nitrated protein(s) that support NF2 tumor cell survival. Besides NF2, these proteins could be new targets in conditions such as glioblastoma, breast cancer, and colon cancer, where protein nitration is involved in proliferation.



FILIPP KULIKOV

Russian National Research Medical University

Exploiting Cytotoxic Role of Nuclear Rac1 to Develop Targeted Antitumor Therapy of NF2-Associated Tumor

There is no specific treatment for NF2 other than non-specific radiotherapy and surgery, which can sometimes be ineffective due to remote localization of tumor. This proposal will determine the mechanism by which statins and bisphosphonates induce Rac1 translocation into the nucleus, thereby causing cell death. We will also investigate the effectiveness of a statin-bisphosphonate combination therapy for NF2-associated tumors.





CTF-Funded Study Shows Promising Treatment for NF2 Hearing Loss

Research funded by the Children's Tumor Foundation and recently published in *Science Translational Medicine* has shown the potential to treat neurofibromatosis type 2 tumors with the blood pressure medication losartan. Vestibular schwannomas, which are noncancerous tumors along the nerves in the brain that are involved with hearing and balance, are a hallmark of NF2, and there are currently no FDA-approved drugs to treat these tumors or their associated hearing loss. Surgery and radiation therapy are currently the only options.

Using an NF2 mouse model, investigators found that losartan had several effects on vestibular schwannomas and the brain, reducing inflammatory signaling and swelling and thus preventing hearing loss, and increasing oxygen delivery to enhance the effectiveness of radiation therapy (which may help lower the radiation dose needed to control tumor growth and limit radiation-associated toxicities). These findings indicate that losartan warrants further study in clinical trials in patients with such tumors.

Through the NF2 Accelerator Initiative and the Drug Discovery Initiative, the Children's Tumor Foundation is committed to expanding the clinical drug pipeline, improving drug selection, and investing in gene therapy for all forms of NF. Learn more at ctf.org/endNF2.

NF1 Clinical Trial from SpringWorks Therapeutics

SpringWorks Therapeutics is currently conducting a clinical trial to study an investigational MEK inhibitor called mirdametininib in patients with NF1-associated plexiform neurofibromas (NF1-PN). The study, called the ReNeu trial, is an open label Phase 2b study in children (2 years or older) and adults with an inoperable NF1-PN. Mirdametininib is offered as an oral capsule or in a pediatric formulation for children who cannot swallow a capsule. Participants will receive mirdametininib, which can be taken without regard to food, dosed at approximately 2mg/m²/dose (up to a maximum of 4mg twice a day) on 3 weeks on, 1 week off intermittent dosing schedule. The primary objective of this study is to evaluate the ability of mirdametininib to shrink the plexiform neurofibromas as measured by magnetic resonance imaging (MRI). Other key objectives include safety and tolerability as well as mirdametininib's effect on physical functioning, quality of life, pain, and disfigurement.

More information on the ReNeu study can be found on the SpringWorks website or on www.clinicaltrials.gov under the identifier NCT03962543.

EU PEARL: Patient Participation in Research Projects and Clinical Trials

Patients need to have a voice and be a part of the research process because scientists and researchers don't always know what patients are experiencing. Patient engagement bridges the gap between setting research priorities for clinical trial development and prioritizing research based on the patient's perspective and priorities. Patients can be valuable collaborators in developing, implementing, and evaluating research. Further, their involvement improves methodology and research outcomes, gives credibility, minimizes cost, and saves time. The need for adequately trained and informed patient representatives is critical to accentuate patient participation in research and assure success.

EU-PEARL is a strategic partnership project between the public and private sectors to shape the future of clinical trials, and through Children's Tumor Foundation involvement, neurofibromatosis is one of four disease groups upon which the international EU-PEARL is focused. The project is developing an Integrated Research Platform for specific diseases, such as neurofibromatosis. Recently EU-PEARL aimed to reach a consensus among 40 experts and 63 patient representatives on the most important manifestations meriting the development of drug trials, using a series of surveys and consensus meetings. The final selection of manifestations was made in a workshop to which CTF, Neurofibromatosis Patients United (NFPU), and EU-PEARL neurofibromatosis clinicians participated.

This approach to clinical trial development is a good example of the impact of patients, patient advocacy groups, and researchers collaborating to determine consensus on research priorities. As we can see from this neurofibromatosis patient collaboration, it is vital to include the patients' voice as early as possible. We must develop studies with patient perspectives and experiences in mind and define endpoints that are important to the patient population.

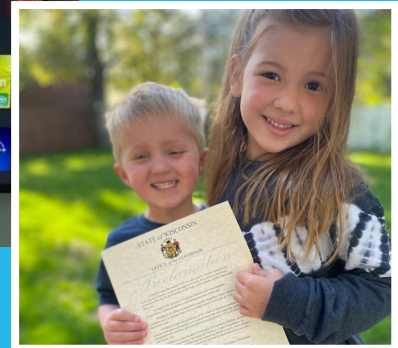
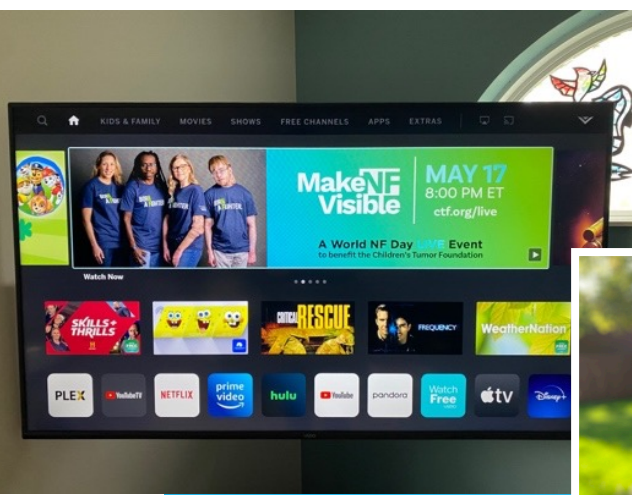
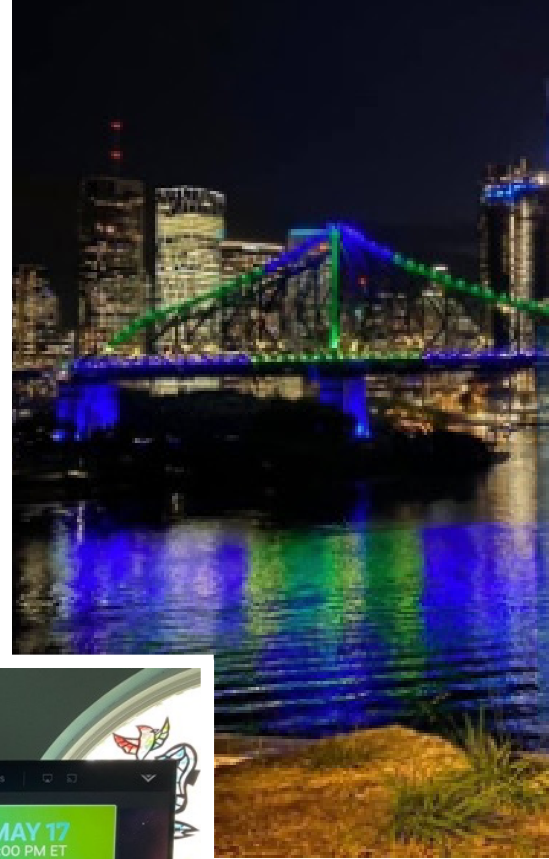
Learn more about CTF's patient engagement and advocacy program, go to ctf.org/patientengagement.

Awareness Month HIGHLIGHTS

MAKE NF VISIBLE

In 2021, the Children's Tumor Foundation took on a new theme for our NF awareness efforts called Make NF Visible, asking the NF community: What is something about your NF that people can't see? And what is something about you that someone can't see because of your NF?

This new focus was evident during May NF Awareness Month, with increased media coverage about the NF community, which included local and national coverage on television, radio, blogs, and podcasts, as well as a PSA campaign. A big thank you to VIZIO for running a nationwide TV promotion in support of World NF Day on May 17, and introducing NF awareness to new audiences.



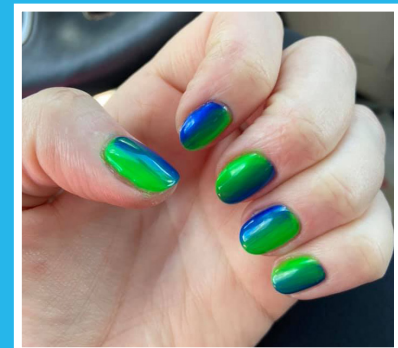
NATIONAL BILLBOARD CAMPAIGN

Our thanks to Lamar Advertising who helped the Children's Tumor Foundation Make NF Visible through a national PSA digital billboard campaign to celebrate NF awareness month. Adding to the billboards secured by local NF families in Virginia, South Dakota, Missouri, and Utah, an additional 122 digital billboards spread NF awareness in 25 markets across the country.

NF AWARENESS AROUND THE WORLD

Volunteers across the country secured proclamations of NF Awareness month in their 18 states and 25 cities. Families and friends joined our efforts on all the @childrenstumor social media channels, and created individual Facebook fundraisers that brought in more than \$66,000 during May to benefit CTF.

Continuing to spread awareness from the safety of their homes and beyond, the NF community was busy all month hanging the End NF flag, putting up yard signs, and painting their nails blue and green.



SHINE A LIGHT

The annual Shine a Light on NF campaign brings NF awareness into the community by lighting up buildings, bridges, and monuments in blue and green. Over the years, this effort has extended to both coasts and around the world, including all over Europe. Together with our partner NF organizations, this year 319 locations in 12 countries worked to Make NF Visible by Shining a Light on NF!

Thank you to our Shine a Light on NF partners:

Nerve Tumours UK (United Kingdom); Children's Tumour Foundation (Australia); LINFA Onlus (Italy); Asociación Catalana de las Neurofibromatosis and Asociación Catalana de las Neurofibromatosis (Spain); Tumour Foundation of British Columbia (British Columbia, Canada); and Dutch NF Foundation/Let's Beat NF (the Netherlands).



Something that people can't see about me because of NF is that

Nearly 50 NF Heroes from around the world responded to our call for individuals to tell their stories of living with NF1, NF2, or schwannomatosis through self-submitted videos. View these powerful video submissions at youtube.com/makenfvisible.

You can continue to grow our gallery and help Make NF Visible by sharing your story. Learn more and submit your video at ctf.org/myvideo.



WEARING BLUE AND GREEN

NF Heroes showed their pride as part of our social media campaigns for two specific days that are important to the NF community, Wear Blue & Green on May Seventeen (for all forms of NF) and Wear Green and Blue on May Twenty-Two (for NF2).

MAKE NF VISIBLE: A WORLD NF DAY LIVE EVENT

On May 17, World NF Awareness Day, we came together to make sure the world knows about NF. Streaming on Zoom and YouTube, this event was hosted by actor/producer Jonathan Sadowski and actor/singer James Snyder. The virtual benefit was filled with celebrity musical performances by Gloria Gaynor, Andy and Aijia Grammer, Jake Clemons and Broadway stars Lena Hall, Denée Benton, Miguel Servantes, and Jessica Vosk. Additional appearances included actor Alec Baldwin, chef Andrew Zimmern, NFL Hall of Famer Darrell Green, Colorado Rockies' Ian Desmond, NFL Kicker Nick Folk, WWE star Roman Reigns, and many more. NF Heroes also joined to tell their stories of NF, combining forces to Make NF Visible and raising more than \$300,000 for NF research.

A World NF Day **LIVE** Event
to benefit the Children's Tumor Foundation

Make NF Visible

Watch the entire event online at ctf.org/live



Tina
NF1

When I was five years old, my parents noticed a tumor on my foot because I was walking in a weird way. They brought me to a doctor, who eventually diagnosed me with NF after a lot of testing.

There are some days that I am pain-free but most days I live with physical pain. But I wake up each morning thankful to get another chance to make it a good day.

Since I'm the only one in my family with NF, I started Googling and reading medical journals about my condition to better understand it, which helps me stay motivated. Looking for cures and at all the research studies also keeps me motivated. I encourage others by letting them know bad days come and go, but we can hope for the good days.

Lastly, I am stronger because NF gives me a perspective on life that I wouldn't get anywhere else. I don't take life for granted because I may not have a second chance tomorrow; I only have today.

When I am not in college learning about radiography, you can find me spending most nights at a Chick-fil-A, working towards a career where hospitality is important.

What makes me happy is being surrounded by people who want to be there, and being that person that you can hug or just talk to.

stories

OF NF

Barbara
NF2

I was 19-and-a-half years old when I was diagnosed with NF. I had multiple birthmarks, or café-au-lait spots, from birth and I guess my doctors weren't aware of neurofibromatosis. Around Easter, when I was in either 7th or 8th grade, I ended up in the ER due to dehydration and a high fever. I was diagnosed with pneumonia and had a chest x-ray. No one informed my parents of the results that further indicated many symptoms of NF, including rib deformity, scoliosis, and a "thickening in the left upper apex."



When I was in high school, the left side of my neck was fuller than it had been, but even after four minor procedures to remove the growths, no biopsies were performed. My mom even talked to the surgeon who performed the procedures and questioned why there was such a significant difference. He answered that everybody's left and right side are different. This was in 1985.

In late spring 1985, I went into my gynecologist due to the fact that my left breast appeared swollen and the left shoulder blade was even more pronounced. After a breast exam, blood draws, and a series of questions, he referred me to a general/vascular surgeon. After another minor procedure, this doctor actually sent it out for a biopsy and the results came back that I had neurofibromatosis.

The plexiform tumor that I have is inoperable, and even though it was debulked in 1987, it was the size of a pineapple pressing my left lung down. The tumor is in the left shoulder pushing the shoulder blade outward, pushing muscle and nerves, so when I sit "the wrong way" I have tingling in the arms and hands.

I don't want to use NF as an excuse for anything, so whatever job I've ever had I put my all into it. I have been a babysitter, a cashier, data entry, waitress, preschool teacher, and a manufacturer in the semiconductor field. I am currently a caregiver for seniors, people living with Alzheimer's and dementia, and disabled individuals so that they can remain in their homes. I have also worked with people who are in hospice. I enjoy helping others, and it is an honor to be there for families, especially for people who are near the end of life.

I know that there are others out there that are so much worse off than me. I feel that having NF is a challenge and I believe that for whatever reason I have NF, I know it is because God selected me.

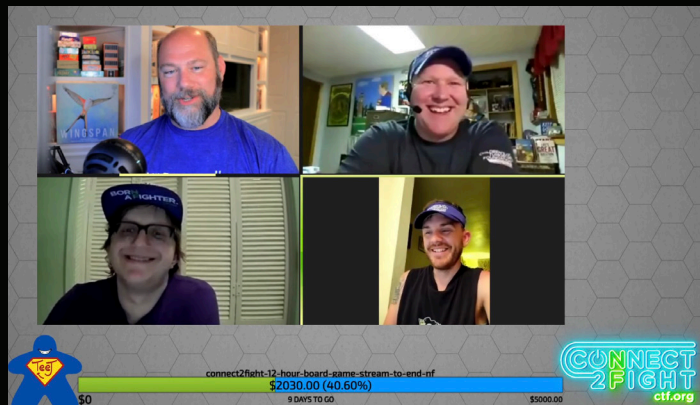
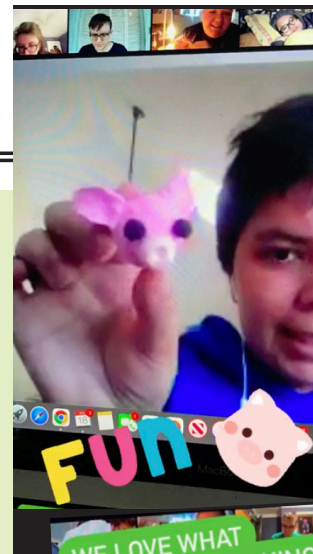
SPECIAL EVENTS

Virtual NF Camp

The Children's Tumor Foundation 2021 Virtual NF Camp took place this July via Zoom in the comfort of the 120 participating NF Heroes' own homes. We hosted our largest group of teens and young adults ever from across the U.S. as well as Canada, the United Kingdom, and even Indonesia. The NF Camp theme this year was "We are all in this together!" NF Mentors led the virtual sessions each day with a variety of activities which included: games such as "Guess the Celebrity Baby"; crafts like making soap; projects like making oobleck;

salsa lessons, and baking cake pops. NF Camp also featured special guest speakers, including: NF Champion, Jonathan Sadowski; singer- songwriter, Damien Horne; TikToker Erin Pettey; Livestreamer Joumie; and 2021 National Ambassador, Lilly Ann Brooks.

A huge thank you to NF Camp sponsors AstraZeneca, RBC Foundation, Bourbon Charity, Bob's Discount Furniture Foundation, and the Jack and Marjorie Schillinger Foundation. Thanks to our 2021 NF Campers and Mentors for the memories! We are already looking forward to July 2022. Learn more at: ctf.org/nfcamp



NF Heroes and Champions Connect2Fight in a 12-hour board-gaming charity livestream

Awareness and charity livestream fundraising were at a high with the Connect2Fight program during May NF Awareness month. The NF community came together in CTF's newest fundraising platform to raise almost \$15,000. NF2 Hero Joe Downey (aka Jersey Joe), held his Benefit Auction Livestream event on YouTube and raised more than \$4,500 for NF research for a second year in a row. We were also fortunate to have a repeat charity

stream by CAG in addition to several newcomers to the Connect2Fight program, including TJ Warren from Iowa and Joumie from Canada. Each have a personal stake in the cause and each raised over \$2,000.

NF Dad TJ Warren and his cousin Chris Holmes shared their love for board gaming with several other NF Heroes and CTF staff in a 12-hour charity livestream event on Twitch, which can be viewed at: www.twitch.tv/board_at_home

“As I reflect back on our experience, I'm blown away by the opportunity to raise funds and spread awareness for CTF through our love of board games!!! These Connect2Fight events serve a greater purpose to make NF visible and benefit the work of the Children's Tumor Foundation. Events like this get us closer to ending the NF fight for so many individuals across the world, including my son.”

—TJ WARREN, NF DAD



SHINE A LIGHT NF WALK

The Shine a Light NF Walk season has returned! We are excited to be together again and are currently offering the opportunity to participate in person as well as virtually while following CDC, state, and local guidelines.

Special thanks to all of our local walk sponsors and to our National Luminary Sponsor, AstraZeneca, for being a part of the events.

For more information on a Shine a Light Walk near you, visit www.shinealightwalk.org.

Michigan kicked off the season on July 25 with their 10th anniversary walk. With a goal of raising \$65,000 to end NF, more than 200 participants gathered at Addison Oaks County Park for a fun and inspiring day of raising awareness and celebrating the NF community.



People walked in their neighborhoods and celebrated with their families for the virtual Utah Shine a Light NF Walk on August 28. Local NF Heroes were given shout outs on the Utah Facebook event page and teams posted photos throughout the day. The Utah Walk community is well on their way to meeting their \$25,000 Walk goal.

The Seattle community honored more than 20 NF Heroes on August 29 at Magnusson Park. Each NF Hero shared what the walk meant to them, and returning teams were thrilled to be together for the first time since 2019. This community has already raised more than \$34,000 to towards its \$35,000 goal. Incredible job, Seattle!



2021 Fall Schedule SHINE A LIGHT NF WALK

9/11/2021	Denver
9/18/2021	Iowa
9/18/2021	New York
9/18/2021	Virginia
9/19/2021	South Dakota
9/25/2021	Chicagoland
9/25/2021	Kansas City
9/25/2021	Cincinnati
9/26/2021	Minnesota
10/2/2021	New Jersey
10/2/2021	Carolinas
10/9/2021	San Antonio
10/9/2021	Bay Area
10/10/2021	Philadelphia
10/10/2021	Austin
10/16/2021	Washington DC
10/16/2021	Atlanta
10/23/2021	Los Angeles
11/6/2021	Houston
11/7/2021	San Diego
11/14/2021	Florida
11/14/2021	Arizona (previously Phoenix)

Join Us!

The fall Shine A Light NF Walk season is just getting started and we want YOU to join us. To learn more or find your nearest walk, go to shinealightwalk.org and register today!

Racing is BACK! Run or walk with the NF Endurance Team at one of the Children's Tumor Foundation team events below. With distances from 5K to marathon, there's a race for everyone! Whether you stay close to home or treat yourself to a destination race, you will be going the extra mile to help us end NF.

UPCOMING NF ENDURANCE EVENTS:

Rock 'n' Roll SAN JOSE Half, 10K, 5K | Oct. 2-3

Colfax Marathon, Half 10K, 5K, Relay | Oct. 16-17

Rock 'n' Roll SAN DIEGO Marathon, Half, 5K | Oct. 23-24

Indianapolis Monumental Marathon, Half, 5K | Nov. 6

Rock 'n' Roll SAVANNAH Marathon, Half, 5K, 1Mi | Nov. 6-7

Rock 'n' Roll WASHINGTON DC Half, 5K | Nov. 13

Rock 'n' Roll NASHVILLE Marathon, Half, 5K, 1Mi | Nov. 20

Rock 'n' Roll SAN ANTONIO Marathon, Half, 10K, 5K | Dec. 4-5

Rock 'n' Roll LAS VEGAS Half, 10K, 5K | Feb. 26 - 27, 2022

Little Rock Marathon, Half, 10K, 5K | Mar. 5 - 6, 2022*

20TH ANNUAL EVENT & NF ENDURANCE TEAM REUNION RACE WEEKEND!

Register at nfendurance.org or reach out to nfendurance@ctf.org with questions.

Pick an adventure of your choice. Climb a mountain, take an epic hike, swim every day, or do any race not on our NF Endurance team schedule - the challenge is up to you! When you sign up with NF Endurance, you make your challenge more meaningful by fundraising for the Children's Tumor Foundation, supporting our mission to fund NF research. Go to nfendurance.org/cyoc to learn more or register!



Meet these 2021 Choose Your Own Challenge participants!

Cody Eaves

In January, Cody Eaves started training for a big race, IRONMAN 70.3 Lubbock, a 70.3 mile race of swimming, biking, and running. As part of his training, he participated in races throughout the spring and summer, all to raise money and awareness for NF. He completed his first IRONMAN on June 27, 7 days before his 40th birthday. But he's not stopping there! Cody will be running the TCS New York City Marathon for CTF in November.



Ginger Marshall

In 2020, Ginger Marshall fell out of her running habit; but with her 40th birthday on the horizon, and inspired by her 11-year-old daughter, Myleigh, she decided to create a personal challenge, as a springboard back into fitness. Beginning on May 15 and ending on her 40th birthday, she committed to hit the pavement for 40 minutes every day for 40 days straight, and asked for the support of 40 donors at \$40 each to join her in generating awareness and raising money to end NF.

Hear from two more Choose Your Own Challenge participants, Rudy Arietta and George Gaine, in our *Extraordinary Spirit* feature on page 18.

THE CHILDREN'S TUMOR FOUNDATION

2021 NATIONAL GALA

**THE BOARD OF DIRECTORS OF
THE CHILDREN'S TUMOR
FOUNDATION**

Cordially invites you to the

2021 NATIONAL GALA

Monday, November 15

Hosted simultaneously in

NEW YORK CITY

Cipriani 25 Broadway

&

BOSTON

Four Points by Sheraton
Norwood

COCKTAILS

6:30 PM EST

DINNER

7:30 PM EST

PROGRAM LIVESTREAMED GLOBALLY

8:00 PM EST

To purchase tickets, donate, or register to view virtually for free, go to ctf.org/gala

Via text message:

*To donate or to register to view virtually for free, text **ENDNF to 41444**.

*To sponsor or to purchase in person tickets for NYC, text **CTFGALA to 41444**.

*To sponsor or to purchase in person tickets for Boston, text **CTFBOSTON to 41444**.

HONORING

2021 HUMANITARIAN AWARD

Leslie and Dick Kates

2021 CTF CHAMPION AWARD

Raina Seitel

2021 INNOVATION IN MEDICINE AWARD

SpringWorks Therapeutics

2022 NATIONAL AMBASSADOR

Eddie Purtell

GALA CO- HOSTS

JONATHAN SADOWSKI

Actor/Producer

RAINA SEITEL

TV Host/NBC Correspondent

RON DELLA CHIESA

Radio Personality

GALA CO-CHAIRS

Mady Donoff • Erica Hartman-Horvitz

Shelley Haughton

Tila Falic Levi • Liz Rodbell • Clara Wilpon

Marco Nievo, PhD Appointed Chief Scientific Officer of CTF Europe

The Children’s Tumor Foundation Europe is proud to announce the appointment of Marco Nievo, PhD, as Chief Scientific Officer of CTF Europe. Based in Italy and a European at heart, Dr. Nievo has been with the Children’s Tumor Foundation for nine years, working primarily on legal and IP-related matters, compound scouting, and relations with industry. He is also a leadership member of the NF-specific work package of the IMI grant in Europe. He was involved in the inception of CTF Europe, focusing mostly on the creation of the Clinical Advisory Board of CTF Europe.

Dr. Nievo comes to the role with a long history of cross-sector experience. After obtaining his PhD in Biological Chemistry at Imperial College London, Dr. Nievo spent 8 years in the pharmaceutical industry in roles related to intellectual property issues, during which time he qualified as a European patent attorney. Since 2012, he has worked in the intellectual property field in the life sciences, serving both the for-profit and nonprofit sectors.

In this newly established role at CTF Europe, and in support of CTF Europe’s mission, Nievo’s initial focus will be on the consolidation of European clinic networks, the organization and planning of the INFER masterclasses, the expansion of the NF Registry in Europe, advocacy before EU institutions, and fostering Trans-European as well as transatlantic collaborations.



KOSELUGO APPROVED BY THE EU COMMISSION

Koselugo (selumetinib) has been granted conditional approval in the European Union (EU) for the treatment of symptomatic, inoperable plexiform neurofibromas (PN) in pediatric patients with neurofibromatosis type 1 aged three years and above. It follows the recently announced positive recommendation by the European Medicines Agency (Committee for Medicinal Products for Human Use), and last year’s landmark approval by the U.S. Food and Drug Administration.

This milestone moment in NF is the result of patients, families, researchers, clinicians, doctors, nurses, pharma, biotech, government, donors and friends and so many more working together to end NF. And we promise: we are not done. We will not stop until there are treatments for ALL types of NF!

MASTERCLASSES IN NF

A series of online educational lectures for medical professionals by leading neurofibromatosis experts

Reserve your spot today:

ctfeurope.org/nfmasterclasses

INFER is an initiative of Children’s Tumor Foundation Europe, supported by an educational grant from AstraZeneca.



INFER
INTERNATIONAL
NF
EDUCATIONAL
RESOURCES

Transition from Pediatric to Adult Care

Tena Rosser, MD (Children's Hospital Los Angeles) and David Viskochil, MD, PhD (University of Utah) presented an overview of the importance of an effective transition from childhood to adulthood to improve quality of life. The pediatric and adult healthcare models differ, shifting from a provider and parent-driven process to increased patient responsibility often with multiple providers caring for separate issues. Barriers can lead to fragmented care or unrecognized medical issues.

CCAB - Europe

Pierre Wolkenstein, MD, PhD (University Paris Est Créteil, France) presented updates on the European CCAB, formed in 2020, with a variety of NF specialists and three patient representatives. CCAB chairs from the U.S. and Europe are building bridges by being on each other's respective advisory boards. The CCAB Europe's primary 2021 goal is to provide continuous medical education to physicians, paramedical staff, and lay groups. A virtual format will include basic courses, case reviews, and meet the experts.

Mission Moment Officially Opens the Conference

A panel of four NF patients participated in the Mission Moment Panel at the beginning of the conference, sharing their diverse and unique experiences of living with NF1, NF2 and schwannomatosis, and acknowledged the research and scientific community's contributions to research and improving the quality of life for NF patients.

NF1

Children with NF1 often show weakness in fine and gross motor function. Sara K. Pardej, MS (University of Wisconsin-Madison), used longitudinal measurements of affected children to identify a growing gap in function with age for the NF1 group, in comparison to unaffected siblings. Early intervention is recommended for fine motor, gross motor, and visuospatial skills in order to minimize deficits that could persist into adulthood.

Melissa Perrino, MD (Cincinnati Children's Hospital Medical Center), presented data on a potential path toward adjuvant therapy to increase durability of response in the treatment of plexiform neurofibromas. Dr. Perrino reported on data from experiments in a mouse model of plexiforms, showing that C5aR antagonists (C5aRA), both alone and in combination with the MEK inhibitor Koselugo (selumetinib) caused an increase in tumor cell death. Overall, the combination therapy is tolerable and effective in reducing tumor burden and in altering the immune microenvironment.

The development of optic pathway glioma tumors (OPG) appears to be partly driven by neuronal activity, according to research presented by Yuan Pan, PhD, (Stanford University). In a mouse model of NF1 OPG, stimulation of optic nerve activity through light exposure increased optic glioma growth, while light deprivation prevented OPG formation and maintenance. Additional understanding of the mechanisms involved may lead to better therapeutic strategies for OPG.

MPNST

Christine Pratilas, MD (Johns Hopkins University) spoke on the topic of novel therapeutic strategies for malignant peripheral nerve sheath tumors (MPNSTs). MEK inhibitors show only partial and short-lived efficacy against MPNSTs because of complex signaling pathway adaptations. Understanding these adaptive changes can reveal new therapeutic targets for single or combination therapy. A combination of SHP2 inhibitors and MEK inhibitors showed strong efficacy in preclinical MPNST models. Further, SHP2-CDK4 inhibitor combinations showed more potency than SHP2-MEK inhibitors. These results can be very valuable in informing future clinical trials for NF1 patients with MPNSTs.

Harish Vasudevan, MD, PhD (University of California, San Francisco) presented the results of omics analyses performed to

understand the transformation of benign NF1 tumors into MPNSTs and their resistance to therapy. The present study confirmed that MPNSTs, which lack functional PRC2, were enriched in PRC2-target genes and repressed in Schwann cell differentiation genes. Comparison of MEK inhibitor response between PRC2-intact neurofibroma cells and PRC2-deficient MPNST cells revealed resistant and undifferentiated cells increased over time, while differentiated cells decreased over time.

Kyle Williams, PhD (University of Minnesota) discussed new preclinical models of MPNSTs used to identify therapeutic compounds specifically for MPNSTs. HDAC inhibitors in combination with MEK inhibitors were most efficacious and their synergistic activity was confirmed in *in vivo* models. Activation of specific survival pathways were also observed in drug-treated MPNST models.

NF2

Maria Martinelli from the lab of Cristina Fernandez-Valle at the University of Central Florida, studied a compound first identified within Synodos NF2 called fimepinostat (CUDC-907). From initial studies the compound did not seem to have good activity, but recent studies in cells and animal models show potential to promote NF2 schwannoma regression.

The same group also conducted additional studies with a combination of two compounds: GSK-458 (studied in Synodos NF2) and a compound that inhibits FAK/SCR, two molecules highly regulated in NF2 tumors. The combo GSK-458 and Dasatinib showed mixed yet promising results that warrant further investigation.

Phase 2 study results of Axitinib in NF2 patients were presented by Sheetal Phadnis, MD (University of Alabama at Birmingham). Conclusions are that Axitinib has modest anti-tumor activity in NF2 patients. However, it does not look like a good drug for NF2, as it's more toxic and appears to be less effective compared to bevacizumab.

Schwannomatosis

New evidence of LZTR1 loss in schwannoma development and Noonan syndrome was presented by Dr. Anna Sablina (KU Leuven, Belgium). LZTR1 loss increases RAS and MEK activity, therefore increasing Schwann cell growth and tumor development. In order to explain the two different phenotypes, Dr. Sablina developed animal models where the loss of LZTR1 alone does not produce tumors (but recapitulated somehow the features of Noonan Syndrome), while together with loss of one of the genes involved in NF2, led to tumor (schwannoma) development.

Schwannomatosis is a very rare condition that is caused by pathogenic variants in the *SMARCB1* or *LZTR1* gene. New evidence has been discovered by Barbara Rivera Polo (IDIBEL, Barcelona) for a new tumor susceptibility gene conferring risk for development of peripheral schwannomatosis as well as thyroid abnormalities. The gene *DGCR8* was identified in five tumor samples. More evidence has to be gathered by the researchers to confirm this finding.

Justin Jordan, MD, MPH, (Massachusetts General Hospital) studied a cohort of 37 schwannomatosis patients with genomic and WBMRI (whole-body MRI) data. They identified no significant difference in tumor burden between variant groups (*LZTR1* vs *SMARCB1* vs unidentified), though spinal schwannomas were more prevalent in *LZTR1*-variant patients. Pain scores correlated with total body tumor volume but not with the number of tumors. Pain was higher in *LZTR1*-variant patients, and spinal tumor location did not significantly correlate with pain, suggesting a possible genetic association with pathogenic variants leading to schwannomatosis.

Naomi Ashkenazi (Massachusetts General Hospital) completed a one-year study involving 79 schwannomatosis patients to look at the longitudinal evaluation of pain in this patient population. The study revealed use of opioid or neuropathic medication (but not NSAIDs) was significantly associated

with reductions in pain intensity and use of neuropathic medications or NSAIDs (but not opioids) were associated with reductions in pain interference. In conclusion, pain severely impacts functioning and mental health in people with schwannomatosis, but despite widespread use of pain medications, pain remained high.

Studies by Larry Sherman, PhD and Kim Ostrow, PhD showed evidence that substances secreted by *LZTR1* and *SMARCB1*-related tumors differ and influence neuronal sensitization (specific chemokines and cytokines that influence pain signaling in sensory neurons).

Miriam Smith, PhD (Manchester University) looked at 1500 patients with a *22q11.2* deletion syndrome with a large deletion of chromosome 22 including the entire *LZTR1* gene. These patients report a range of clinical signs and symptoms, but schwannomas have not been reported as a clinical feature. The conclusion is that people with a large germline *22q11.2* deletion may have a reduced risk of developing a schwannoma compared to the general population. The hypothesis is that biallelic deletion of this large region, containing multiple genes, might be cell-lethal, thus preventing tumor development, or that complete loss of *LZTR1* protein does not have the same pathogenic effect as a partially functional protein.

Gene Therapy

Jiangbing Zhou, PhD (Yale University) presented the keynote lecture on non-viral gene therapy for neurofibromatosis. Synthetic non-viral vectors, such as lipid- or polymer-based nanoparticles, are safer due to their limited immunogenicity, can accommodate larger genetic materials, and are easier to engineer for successful targeting. Dr. Zhou briefly reviewed the recent progress in non-viral vector development and progress in developing polymeric nanoparticles for *NF1* gene therapy. He showed that injecting *NF1* cDNA nanoparticles into various *NF1* mouse models restored *NF1* protein expression

and increased survival. Dr. Zhou's team is conducting additional experiments to further validate these preliminary results.

Matthieu Drouyer, PhD (Children's Medical Research Institute, Sydney) presented results of their team's effort to develop novel clinically relevant adeno-associated virus (AAV) vectors for targeting human primary Schwann cells. They screened a panel of AAVs in multiple human Schwann cells and performed directed evolution selection of novel AAV. One novel AAV variant showed the highest efficiency and has been selected for further studies. This study is supported by the CTF *NF1* Gene Therapy initiative.

Stephanie Bouley, PhD (Massachusetts General Hospital) presented on the feasibility of genome editing as a therapeutic approach to correct *NF1* mutations. These proof of principle studies are being tested in patient-derived cell lines and *de novo* Schwann cell models. Three *NF1* variants have been selected for this pilot study and the cell models are being used to test different gene editing strategies. This study is supported by the CTF *NF1* Gene Therapy initiative.

Núria Catasús, MS, (Germans Trias i Pujol Research Institute, Spain) presented results of their studies on using antisense oligonucleotides, specifically Phosphorodiamidate Morpholino Oligomers (PMOs), to reduce the severity of *NF2* pathogenic variants. Catasús treated *NF2* variant fibroblasts with PMOs targeting four independent splicing variants and a pair of PMOs to induce exon skipping. They observed that the former PMOs prevented correct splicing in both wildtype and pathogenically variant cells. The latter PMOs resulted in complete Merlin loss. Complementary analyses are being performed to confirm these results before ruling out PMOs as tools for correcting specific *NF2* pathogenic variants.

To learn more about the NF Conference please go to nfconference.org.



At left, George Gaine's daughter NF Hero Brielle at a basketball game.

Below, Coach George Gaine at the 2018 Two Counties, One Cause tournament.



“It’s humbling to see so much support, and people rallying around a cause. It’s a nice indication of the amazing community that we live and work in – that they will step up even in hard times.”

RUDY ARIETTA

is an event called 29029 Everesting, in which participants repeatedly climb a mountain over the course of 36 hours until they have reached 29,029 feet – the vertical equivalent of Mount Everest. In Arietta’s case, he will climb up Utah’s Wasatch Mountains, taking a gondola down, and repeating for 36 hours straight until he reaches the “summit.” Originally a personal goal to challenge himself, Arietta quickly realized that he could use the event as a fundraising substitute for the Two Counties, One Cause tournament.

With the blessing of Coach Gaine, Arietta set his fundraising goal to \$29,029 – one dollar for NF research per foot of elevation. As of mid-August, he was well over halfway toward that goal and in the midst of an intense training regime. Arietta takes 9-hour hikes on the weekend, bikes, steps or runs for 2 hours, and takes 60 to 90-minute hikes during the week. “Training is challenging,” he says, “but it pales in comparison to the challenges faced every day by NF patients and their families.” He’s been joined by members of the basketball team on some of his hikes, and has seen donations come in from former students and other surprising corners.

“It’s humbling to see so much support, and people rallying around a cause. It’s a nice indication of the amazing community that we live and work in – that they will step up even in hard times,” says Arietta.

Gaine echoes the sentiment on behalf of himself, his family, and his daughter: “It’s humbling, amazing, I don’t know how to keep saying thank you to people. It shows you what’s good out there.”

Extraordinary Spirit / **RUDY ARIETTA & GEORGE GAINE**

When Rudy Arietta came on as principal at Tappan Zee High School three years ago, he was inspired and humbled by the community spirit that had been built there, particularly around the annual charity basketball tournament Two Counties, One Cause. The tournament was the brainchild of Tappan Zee’s basketball coach of 18 years, George Gaine, whose 9-year-old daughter Brielle has NF1-related optic nerve glioma. What started as a small series of games has grown into an annual highlight for everyone involved and a huge part of the community’s culture. All money raised goes to the Children’s Tumor Foundation.

Of his idea for the tournament, Gaine says, “I thought about how I spend all my free time wrapped up in basketball, and I wondered, how can I get basketball involved with the Children’s Tumor Foundation?” In January of 2020, with ten teams from New York’s Rockland and Westchester Counties participating, the event raised more than \$30,000.

But just as the event was booming, it was ground to a screeching halt in 2021. As the COVID pandemic continued to put regular life on hold everywhere, Tappan Zee High School experienced an abbreviated winter basketball season. Coach Gaine knew there was no way they could gather crowds for the annual charity tournament. “You could really feel the absence,” added Arietta. That’s when this high school principal came up with an alternative idea—he would climb the equivalent of Mount Everest.

“COVID was probably the biggest challenge a lot of us had ever faced,” says Arietta. “All of us, no matter who we are, at some point complained and felt bad for ourselves. I wanted to shift my mindset and get back to the idea that we should embrace challenges, because we come out of them better and stronger, and learn something from them.”

Arietta decided to join CTF’s Choose Your Own Challenge initiative with the NF Endurance team. The challenge he chose to embrace

CTF Publications

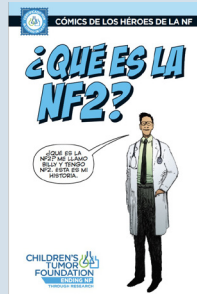
The Children's Tumor Foundation is committed to providing educational brochures in various languages free of charge to patients and families living with NF. To read or download any of our materials, please visit ctf.org/education, where you can also find a link to request a print copy. New additions to our library of resources include:



NF1 Parent Guidebook now available in English, French, and Spanish.



Living with NF1 now available in English and coming soon in Spanish.



Understanding NF2 now available in English and Spanish.



Moxie & Sparx Explain NF1 now available in English, French, and Spanish.

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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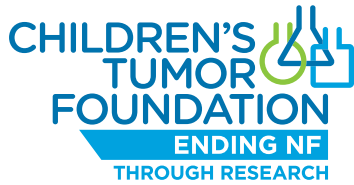
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