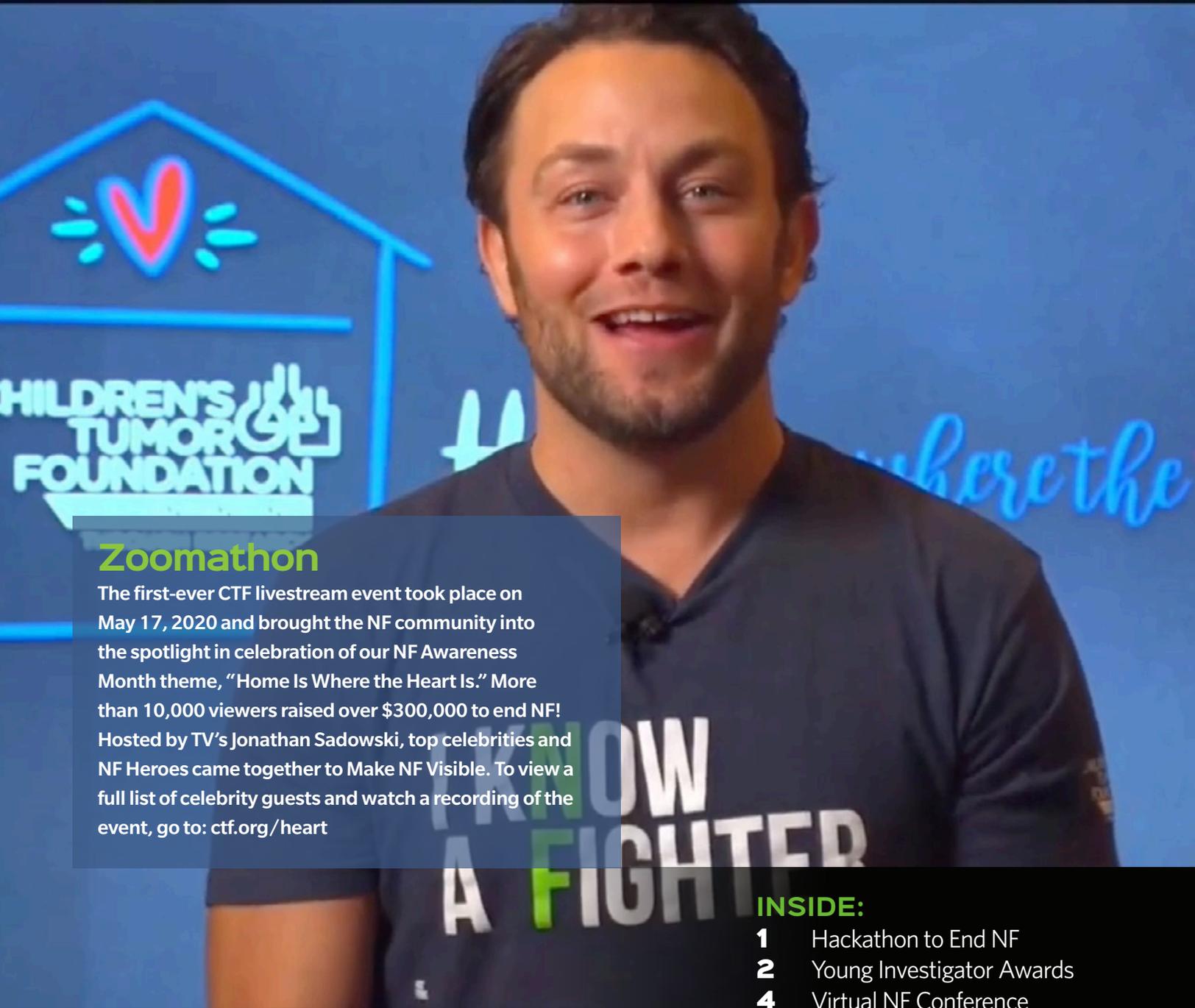


NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
FALL 2020



Zoomathon

The first-ever CTF livestream event took place on May 17, 2020 and brought the NF community into the spotlight in celebration of our NF Awareness Month theme, "Home Is Where the Heart Is." More than 10,000 viewers raised over \$300,000 to end NF! Hosted by TV's Jonathan Sadowski, top celebrities and NF Heroes came together to Make NF Visible. To view a full list of celebrity guests and watch a recording of the event, go to: ctf.org/heart

INSIDE:

- 1** Hackathon to End NF
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- 7** Brigatinib for NF2
- 10** NF Awareness Month Highlights

You are invited to join our biggest event of the year
The National Celebration Concert
to End NF on November 16 (see back cover)

FROM the President

Annette Bakker, PhD



As summer moves into fall, we joyfully continue the Children's Tumor Foundation (CTF) celebration of the 2020 announcement of Koselugo (selumetinib) as the first-ever FDA-approved treatment for neurofibromatosis (NF). Even in a global pandemic, which has brought so much uncertainty, this news has instilled more confidence than any other time in NF history that we will one day end NF.

Over the summer we celebrated another monumental moment in NF history - the 30 year anniversary of the discovery of the NF gene in the labs of the late Ray White, PhD, and Francis S. Collins, MD, PhD, who currently serves as the Director of the National Institutes of Health. Dr. Collins delivered the keynote address at CTF's Virtual NF Conference, where he kicked off a scientific meeting that lasted for two days - virtually joined by NF experts, clinicians, and researchers who normally attend the meeting in person.

Likewise, patient events have gone virtual as well, with families and caregivers logging in to the NF Forum and the Volunteer Leadership Conference, and young people joining NF Camp. Our Shine A Light NF Walk and NF Endurance programs have also risen to the occasion, as volunteer organizers, participants, athletes, and our family of donors have participated with us online.

Throughout the month of May, volunteers spread NF awareness from home in support of our NF Awareness Month campaign, Make NF Visible. On World NF Awareness Day, my friend, actor Jonathan Sadowski hosted the first "Home is Where the Heart Is Zoomathon to End NF," which then sparked the planning of additional events, many of which I hope you will join this fall.

In this newsletter we recap important work in the field of NF research, clinical trials, and our research awards, as well as the wonderful work of our NF community. Our donors this year are funding life-changing research—and instilling 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 all play a part in the fight to end NF.

UPDATE:

Newsweek



CTF President Annette Bakker, PhD was recently published in Newsweek, writing about the speed and collaborative spirit by which the scientific community is working together to find treatments and vaccines for Covid-19. In this important op-ed, Annette writes about the NF community experience that led to the FDA approval of Koselugo (selumetinib), the first-ever approved drug for neurofibromatosis, and calls for more of this spirited collaboration, urgency, and funding across all diseases. To find a link to the article, please go to ctf.org/newsweek2020

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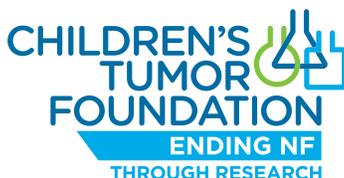
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CTF Hosts the World's Largest Virtual Hackathon to End NF

The Children's Tumor Foundation will host "Hack for NF," the world's largest virtual hackathon to end NF from October 2 to November 13, 2020. This event will mobilize the scientific community across the globe to accelerate research progress for NF and launch the first NF Incubator.

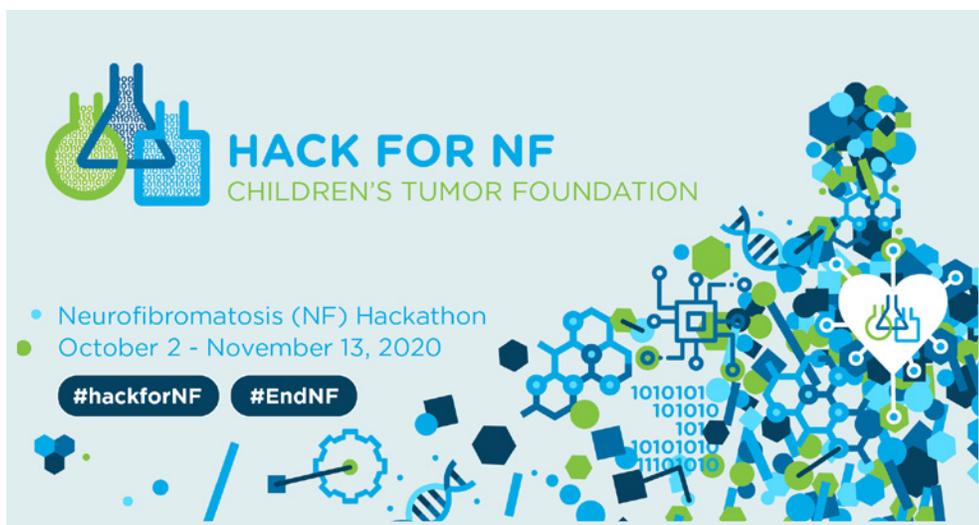
Healthcare startups, developers, solutions architects, hackathon enthusiasts, but also NF patients and NF researchers are being called upon to drive innovation and improve the lives of patients living with neurofibromatosis and other rare diseases.

This is the second hackathon that the Children's Tumor Foundation has hosted. In September of 2019, data scientists, artificial intelligence experts, and engineers gathered at the Google Launchpad in San Francisco, California to 'hack' genomic, research, and imaging/clinical data from the NF Data Portal, in order to bring their unique insights and experiences to help accelerate NF medical research.

For more information and to learn how you can participate in this year's Hack for NF event, go to: nfhack.bemyapp.com

“ This is an incredible opportunity for the brightest minds in tech and research to work together. We are looking for solutions that will help to analyze data, identify new drugs and targets for NF, engage patients via mobile devices, and create data visualization apps and analytical tools for the NF Data Portal. We are aiming to reach out across the globe and gather the most diverse talent to work on NF data and find solutions to help researchers and clinicians improve our patient's quality of life. ”

— **Salvatore La Rosa**, Chief Scientific Officer, Children's Tumor Foundation



UPDATED NF REGISTRY Unites NF Patients in the Fight to End NF

The NF Registry is a secure resource where patients living with NF can take an active role in the search for better treatments for all forms of NF. With over 9,000 participants, the Registry has already made a tremendous impact on NF research: it has alerted participants to over 50 clinical trials, sped up study recruitments for faster results, focused research to match patient priorities, and attracted pharmaceutical companies to the NF field.

Guided by feedback from the NF community and combined with expert knowledge in analyzing registry data, the Children's Tumor Foundation assessed that

a new platform provider could help even further improve the NF Registry, which is now managed by OpenApp, a company focused exclusively on patient registries.

This change will let users move through the surveys more efficiently and with less repetition, as well as make the registry more engaging and accessible in other languages.

The goals of the NF Registry remain the same, and are essential to the fight to end NF:

- Keeps patients informed about the latest in neurofibromatosis information and current clinical trials (including tests of new treatments, such as MEK)

- Helps NF researchers learn about NF from the patient perspective, by providing a source of anonymized information from thousands of individuals all over the world

Participation remains safe, easy, and secure. For those under age 18, a parent or legal guardian/caretaker fills out the survey. For those already in the NF Registry, there is no need to do anything – you can update your record, as needed, at your discretion. For those who haven't yet joined, this is a great time to join the thousands of others living with NF in a united fight to end NF. Learn more and join the NF Registry at: nfregistry.org

YOUNG INVESTIGATOR AWARDS 2020

The Children's Tumor Foundation is pleased to announce the funding of eight Young Investigator Awards (YIA) for the 2020-2022 cycle. Of the eight applications that were selected for funding, six were focused on NF1, one on NF2, and one on schwannomatosis.

NF1

MATTHEW SALE

University of California,
San Francisco

Unveiling the Molecular Mechanisms of Neurofibromin Regulation for Therapeutic Targeting in Neurofibromatosis Type 1

This proposal is based on the observation that neurofibromin is directly phosphorylated by ERK kinases. Matthew Sale will investigate if ERK-mediated phosphorylation negatively regulates neurofibromin function and if so, the mechanistic details of ERK-neurofibromin interaction and regulation.



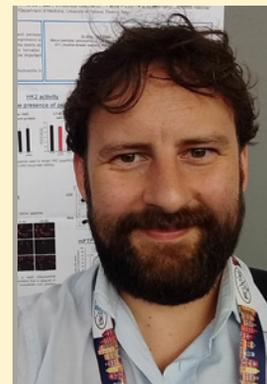
NF1

FRANCESCO CISCATO

University of Padua, Italy

Hexokinase 2 Displacement from Mitochondria-Associated Membranes as a New Antineoplastic Approach in NF1-related tumors

Hexokinase 2 (HK2), a key metabolic enzyme overexpressed in cancers, displays antiapoptotic properties upon its binding to mitochondria. In this project, Francesco Ciscato aims to optimize and test in NF1 model systems a new chemotherapeutic peptide that would specifically displace HK2, thereby promoting tumor cell death.



NF1

KEVIN BRUEMMER

Stanford University

Mapping and Identifying the Roles of Protein Glycosylation in Neurofibromatosis Type 1

Glycosylation, which is the addition of sugar molecules to proteins, is an important post-translational modification that has not been studied in NF1 model systems. This proposal aims to create new chemical biology techniques to better characterize glycosylation using cell models derived from NF1 patients. Studying glycosylation may open up new avenues for therapeutic development by identifying new target pathways.



NF1

GAVIN MCGIVNEY

University of Iowa

Impact of PRC2 Loss on Glutamine Metabolism in MPNSTs

PRC2 mutations occur in 60% of MPNSTs and glutamine metabolism is an important pathway in MPNSTs with PRC2 loss. This study will examine the dysregulation of glutamine metabolism in PRC2-mutant MPNSTs and examine efficacy of the glutaminase inhibitor CB-839, which is currently in Phase II clinical trials for NF1-mutant tumors.





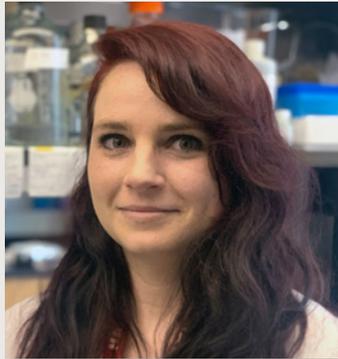
NF1

LAUREL BLACK

Medical University of South Carolina

Malignant Peripheral Nerve Sheath Tumors Achilles Heel: Combinatorial Targeting of ERBB3 and Calcium Signaling

ErbB3 and calmodulin (CaM) are necessary Ras-dependent and -independent signaling proteins required for MPNST survival. These pathways are dysregulated in MPNST cells but not in normal non-cancerous cells. Laurel Black will investigate the role that ErbB3 and calcium-mediated signaling pathways play in regulating cellular proliferation and survival in MPNSTs and the effect of simultaneously inhibiting them.



NF1

JENNIFER PATRITTI CRAM

Cincinnati Medical Center

Understanding the Role of Purinergic Signaling on Tumor Formation in a Mouse Model of Neurofibromatosis Type 1

P2RY14 is a purinergic receptor that is overexpressed in Schwann cell precursor-like tumor initiating cells. The objective of this study is to investigate the role of P2RY14 in driving neurofibroma growth and to use an NF1 mouse model to test if P2RY14 inhibitors can be used as a treatment for NF1 tumors.



NF2

LIYAM LARABA

University of Plymouth

The Use of Novel YAP/TEAD Hippo Pathway Inhibitors to Target Merlin Null Tumors

The Hippo signalling pathway is an important pathway in driving NF2 tumor development. Liyam Laraba, in collaboration with Vivace Therapeutics will investigate the effectiveness of a novel class of YAP-TEAD targeting compounds in animal models of NF2 meningioma and schwannoma. These compounds have already shown to be very effective at blocking non-NF2 tumor growth and with no visible side-effects, and thus hold promise for NF2-related tumors.



SCHWANNOMATOSIS

ARAM KO

Columbia University

Identification and Functional Characterization of the Substrates of LZTR1

LZTR1 is a substrate adaptor protein of the CUL3 ubiquitin ligase complex, which regulates protein turnover by attaching ubiquitin molecules to protein substrates. LZTR1 mutations are identified in a significant number of schwannomatosis patients. This proposal aims to identify and functionally characterize the substrates of the LZTR1 ubiquitin ligase complex and decipher how mechanistically LZTR1 operates to prevent tumor development in normal neural cells.



2020 Virtual NF Conference June 15-16, 2020

So many of CTF's events took a detour this year due to the forces of COVID-19, and the annual NF Conference was no exception. Initially planned for three-and-a-half days in mid-June in Philadelphia, the NF Conference was transformed into a virtual event. The original agenda was shortened, but the attendees were still presented with the best and latest NF research and clinical care practices within a compressed two-day format.

The year 2020 is notable for being the 30th anniversary of the discovery of the *NF1* gene, as well as the first-ever FDA approval of a treatment for NF1, selumetinib (Koselugo). These benchmark events were recognized and celebrated during the course of the meeting.

The 2020 Virtual NF Conference was ably moderated throughout via Zoom by this year's co-chairs, Conxi Lazaro, PhD, of Institut Catala d'Oncologia in Barcelona, Spain, Matthias Karajannis, MD, MS, of Memorial Sloan Kettering Cancer Institute, and Nicole Ullrich, MD, PhD, of Boston Children's Hospital.

An inspirational opening address was delivered by NIH Director Francis S. Collins, MD, PhD, one of the two lead researchers whose work led to the discovery of the *NF1* gene. Dr. Collins reflected on the path to this landmark which simultaneously took place in his lab at the University of Michigan, and at the University of Utah, led by Dr. Raymond White. A session focusing on the gene discovery followed later that day, and was presented by Drs. Dave Viskochil and Peggy Wallace, who at that time were CTF Young Investigator Awardees, and whose work in those labs contributed to the landmark discovery.

Dr. Collins also briefly touched on the remarkable story of selumetinib's road to the clinic, as well as recognizing the groundbreaking research currently being conducted, while looking to the future for many more discoveries, treatments, and maybe someday a cure. To hear Dr. Collins' entire address, go to:

ctf.org/nfconference

“As we're entering this conference's idea of a double celebration, the 30-year anniversary of the identification of the *NF1* gene, and just this April, FDA approval of the first effective treatment for NF1...I want to give a lot of credit to the Children's Tumor Foundation, for the role that they have played at every step along the way in making this kind of progress possible.”

**FRANCIS S. COLLINS, MD, PHD,
DIRECTOR OF THE NATIONAL
INSTITUTES OF HEALTH**



Virtual NF Conference Highlights

Clinical Care Symposium

The Clinical Care Symposium, organized by CTF's Clinical Care Advisory Board (CCAB), provided updates to the current guidelines on NF care, a panel discussion on patient studies, clinical monitoring and treatment options, with further focus on topics that are particularly important during the COVID pandemic. This included results of a survey of clinicians on how COVID-19 has impacted NF care and research, and an overview of the use and challenges of telemedicine in the current environment.

The Long Journey to the Clinic: First-Ever FDA Approved Treatment for NF1

The FDA's announcement in April of its approval of selumetinib (Kosulego), the first-ever NF1-specific treatment, has behind it a long and fascinating story of discovery and successful collaboration. From the discovery of the *NF1* gene 30 years ago through today, it took the combined efforts and dedication of researchers, clinicians, government entities, foundations, industry, and most importantly, the NF patients and their families, to finally drive it to the clinic.

Oncolytic Adenovirus as a Therapy for MPNST

Promising early research on certain types of viruses known as oncolytic adenoviruses has demonstrated that these particular viruses may be used as a useful non-toxic therapeutic agent for MPNST. It was found that when these viruses are injected into mice with MPNST, these mice survive longer than those that have not received the treatment.

Supporting NF Education and Parent Advocacy: Advocacy, Pandemic and Beyond

The NF School Liaison Program at



Boston Children's Hospital addresses the psychosocial needs of NF students, and was designed as a resource for school systems to educate their staff and advocate for NF students' special needs. This program is also proving to be particularly helpful during COVID and virtual learning at home, when the parents, some of whom have NF themselves, now face the added responsibility of having to teach their child.

The impact of MEK inhibitor therapy on neurocognitive functioning in children and adults with NF1

A multi-center initial study to measure cognitive function in participants with NF1 in the first year of MEK1 treatment showed no significant decline in cognitive functioning, suggesting no neurotoxicity with this treatment. In fact, there is some evidence of real-world functional improvement in executive functioning by 6 months, and continuing to 12 months of treatment, particularly for patients with pre-treatment cognitive dysfunction.

Liquid biopsy detection of MPNST vs. plexiform neurofibroma in NF1 patients using cell-free DNA ultra-low-pass whole genome sequencing

A study out of Washington University in

collaboration with the NCI is focusing on identification of early indicators of malignancy in the blood of patients with plexiform neurofibromas, using a procedure known as liquid biopsy. It is hypothesized that MPNST patients have higher levels of a certain marker (known as ctDNA) in their blood than PN patients, thereby possibly providing a means of early cancer detection and improved patient outcomes.

Development of topical MEK inhibitor, NFX-179, for treatment of cutaneous neurofibromas in neurofibromatosis type 1

A topical treatment for NF1 cutaneous neurofibromas, which aims to treat cNF without the systemic side effects of the MEK inhibitors, has shown to be effective in penetrating through skin into the neurofibroma and blocking the overactive RAS pathway in pre-clinical testing. NFX-179, a gel formulation of MEK, entered human clinical trials this summer.

Stepwise tumor progression of plexiform neurofibroma to atypical neurofibroma and then MPNST in a mouse model with CDK2a alteration

A new genetically-engineered mouse model has been developed that grows plexiform neurofibromas (benign

Conference Highlights

Continued

tumor), atypical neurofibromas (a pre-cancerous tumor), and MPNSTs (fully malignant tumor). This model is able to recapitulate the transformation of atypical neurofibromas into MPNST tumors similar to what is observed in patients, thereby creating a new tool that will be very useful to quickly and efficiently evaluate treatments for each tumor grade.

Selumetinib in children with clinically asymptomatic inoperable NF1-related plexiform neurofibromas

In a retrospective study of clinical trials at the NIH, researchers tried to determine if certain PN-directed therapies of MEK affected the development of MPNST among a small percentage of patients. However their analysis demonstrated no large difference in risk of MPNST between treatment groups, prompting further research that will examine other risk factors for MPNST development in patients with a history of PN-directed treatment.



2020 FRIEDRICH VON RECKLINGHAUSEN AWARD

It is with great pleasure that the Children's Tumor Foundation announces the 2020 recipient of the Friedrich von Recklinghausen Award, **Professor D. Wade Clapp, MD**, of the Indiana University School of Medicine, where he serves in multiple capacities. He is the Richard L. Schreiner Professor and Chairman of the Department of Pediatrics, physician-in-chief for Riley Hospital for Children and a Professor of Microbiology & Immunology/Biochemistry and Molecular Biology. Dr. Clapp is one of the rare physician-scientists who is making significant breakthrough contributions to all forms of NF. From his contributions to the basic understanding of NF to the creation of key preclinical models, he is considered by many to be the global gold standard in this broad role. Dr. Clapp's list of accomplishments in NF is very long, and he truly stands out in his commitment to unique collaborations. He was a principal investigator in the NF preclinical consortium and Synodos for NF2. These two transformational team science efforts have been successfully building a preclinical pipeline that front-load, accelerate, and diversify the drug candidates for clinical trials. Dr. Clapp also has a long track record as an out-of-the-box thinker when it comes to leveraging previous investments: his most recent endeavor is the National Cancer Institute's Specialized Program of Research Excellence (SPORE), a 5-year, \$12 million national research project, leveraging his work in the NF preclinical consortium and aimed at efficiently bringing new treatments for pediatric cancers to clinics, with the focus being on NF1.

Poster Competition

This year's winners of the annual poster competition were announced during a special session, each having the opportunity to give a brief oral presentation on their work. The poster competition celebrates the work of the young researchers who are new to the field and represent the future of NF research.

BASIC SCIENCE

First place: *Comprehensive Genomic and Epigenomic Characterization of the Spectrum of Peripheral Nerve Sheath Tumors Associated with NF1 Identifies Two Distinct MPNST Subtypes*

- **Suganth Suppiah**, University of Toronto

Second place: *Rescue of NF1 cryptic splice site in exon 13 mRNA with antisense morpholino treatment*

- **Elias Awad**, University of Alabama at Birmingham

Third place: *PTCH1 and APC regulated pathways contribute to malignant peripheral nerve sheath tumorigenesis*

- **Minu Bhunia**, University of Minnesota

CLINICAL SCIENCE

First Place: *Evolution of the circulating DNA landscape during progression from neurofibroma to malignant peripheral nerve sheath tumor (MPNST)*

- **Taylor Sundby**, Oncology Clinical Fellow, NCI, Pediatric Oncology Branch

Second Place (Tie): *Correlation Between Brain Volume Alterations and Neurological Changes in Adults with NF1*

- **Su Wang**, University of British Columbia

Second Place (Tie): *Progression of Contralateral Hearing Loss in Patients with Sporadic Vestibular Schwannoma*

- **Samuel Early and Charlotte Rinnooy Kan**, Research Fellows, Massachusetts Eye and Ear Infirmary

Bringing Brigatinib to Neurofibromatosis Type 2

The Children's Tumor Foundation announced a significant advancement in care for NF2 patients with the launch of a new clinical trial called INTUITT-NF2, an innovative platform trial which will evaluate multiple treatments simultaneously. This initiative is a result of the landmark work of CTF's visionary Synodos for NF2 research collaborative, its NF2 Accelerator Initiative, an investment from Takeda Pharmaceuticals, the participation of scientists at the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH), and the vital Synodos NF2 leadership from Massachusetts General Hospital (MGH), Johns Hopkins University (JHU) and Indiana University (IU). The Principal Investigator of INTUITT-NF2 is Dr. Scott Plotkin from MGH and the five additional participating centers are Johns Hopkins University (JHU), New York University (NYU), University of California at Los Angeles (UCLA), the Mayo Clinic in Minnesota (Mayo), and the University of Miami (UM). This alliance across the academic, pharmaceutical, and patient landscapes has shortened the time from initial research to active trial, thereby bringing promising treatment options to patients who need them.

The name INTUITT-NF2 stands for 'Innovative Trial for Understanding the Impact of Targeted Therapies in NF2', and its innovation is in responding to – and acting against – specific traits unique to NF2 patients. Neurofibromatosis causes tumors to grow on nerves throughout the body, with most NF2 patients affected by vestibular schwannomas on the eighth cranial nerve, which carries sound and balance information to the brain. NF2 affects 1 in 25,000 people of all populations equally, and in addition to schwannomas can develop meningiomas and ependymomas, with patients

suffering hearing loss, severe balance problems, facial weakness/paralysis, and debilitating seizures, among other serious conditions. The INTUITT-NF2 trial will enroll patients with progressive tumors of any type – schwannoma, meningioma, or ependymoma – to allow for the simultaneous study of the various tumor types, rather than one tumor type alone. This approach will accelerate the information gathering and results analysis processes.

This milestone development in NF2 research and care is the result of insights that came out of the Foundation's Synodos for NF2 effort, which launched in 2014 and in collaboration with NCATS identified brigatinib as a promising drug for NF2 patients. The Synodos project brought together a multidisciplinary team of

approach to the disease.

The multidisciplinary Synodos team established the first-ever NF2 preclinical drug pipeline with cell and animal models connected to a sequencing enterprise so as to better understand the biology behind drug response and non-response. This information was shared among all members of the group for phase one of the project, and eventually made public, available to any researcher through the establishment of the NF Data Portal (nfdataportal.org).

The Principal Investigators of the trial are Scott Plotkin of MGH and Jaishri Blakeley of JHU. Participating clinicians include Jeffrey Allen, MD (NYU); Leia Ngiemphu, MD (UCLA); Dusica Babovic-Vuksanovic (Mayo), and Christine Dinh, MD (UMiami).

“First, by focusing on all tumor types within patients, we are able to expand patient eligibility for the trial, meaning we will increase our knowledge pool on how these treatments perform in patients. And secondly, the trial design allows for the addition of drugs to be tested rather quickly, again increasing what we know about their real-life impact on NF2 patients.”

—SCOTT PLOTKIN, MD, PHD, MASSACHUSETTS GENERAL HOSPITAL

scientists from 12 world-class labs at academic and medical centers of excellence to address the confounding problem patients and researchers alike faced, in that research results appeared to contradict each other – with some work showing positive results and others negative results. This siloed approach to the disease was hampering progress, and all agreed that an “audacious new way” was needed. Patients heeded that call and provided funding to the Children's Tumor Foundation to launch a new, and for its time, a somewhat radical

Both the Children's Tumor Foundation (CTF) and Takeda Pharmaceuticals are supporting the financing of the INTUITT-NF2 trial. CTF's investment is through its NF2 Accelerator Initiative, with lead funding from the Thoms Fund and KBF Canada.

To learn about NF2 research and the progress being made to find treatments for neurofibromatosis, please visit: ctf.org/endnf2

FasterCures, Children's Tumor Foundation, and CureSearch Launch New Initiative to Accelerate Promising Drugs for Pediatric Tumors

FasterCures has spent nearly 20 years working toward building a system that is effective, efficient, and driven by a clear vision: patient needs above all else. Much like the Children's Tumor Foundation, FasterCures believes that transformative and life-saving science should be fully realized and deliver better treatments to the people who need them.

But many promising medicines, despite strong safety data and pharmaceutical characteristics, are deprioritized by pharmaceutical companies for non-technical reasons including strategy, organization, and financial changes. Unlocking these drugs – for intended or new indications – is challenging due to informational, operational, and cultural obstacles within those companies. Meanwhile, patients and families suffer waiting for new therapies for their conditions.

To address this challenge and opportunity, FasterCures launched the BRIDGE initiative in 2019, with a mission to enable more dynamic marketplaces for biomedical innovation. Through a series of meetings held during the 2019 Milken Institute Global Conference and Future of Health Summit, a possible path forward was identified: a neutral information and matchmaking forum that can connect discontinued drug assets with partners that have the expertise and capital needed to develop those medicines.

FasterCures is joined in the BRIDGE initiative by the Children's Tumor Foundation and CureSearch for Children's Cancer—two leading nonprofit foundations with missions to accelerate the development of new therapies for treating pediatric cancer and rare disease.

Together, we will take the essential steps to build partnerships to develop discontinued drugs to treat pediatric tumors. FasterCures, CTF, and CureSearch are creating a nonprofit initiative that will approach biopharmaceutical companies, identify promising but discontinued drugs, develop an externalization framework, and match validated drugs to new development and investment partners.

"FasterCures is thrilled to launch a new pilot program with the Children's Tumor Foundation and CureSearch to create a path to development for promising drugs for pediatric tumors," said Esther Krofah, Executive Director of FasterCures. "Through this partnership, we will evaluate the potential for a non-profit approach to accessing and revitalizing discontinued drugs."

"When drug programs are discontinued or not pursued for business reasons, there can be missed opportunities for children," said Kay Koehler, President & CEO of CureSearch. "This innovative project will enable us to offer biopharmaceutical companies an alternative, pediatric-focused path forward for those discontinued drugs to benefit the patients and families who are counting on us."

The Children's Tumor Foundation is proud to partner with FasterCures and CureSearch to further expand the collaborative network of pharmaceutical companies and foundations with the aim of accelerating treatments to patients. The recent FDA approval of the first-ever treatment for neurofibromatosis, Koselugo (selumetinib), shows what is possible through a united effort of all stakeholders, including biopharmaceutical companies, foundations, and patients.

NFlection Therapeutics Initiates Clinical Proof of Concept Study for Cutaneous Neurofibroma Treatment

NFlection Therapeutics, Inc., a biopharma therapeutics developer dedicated to targeting disease pathways to uncover new approaches to fighting rare disease, announced a \$20 million Series A financing round with investment from venBio Partners and F-Prime Capital. The company also announced the initiation of the first clinical trial of its lead product, NFX-179 Gel, a topically applied therapy.

The Phase 2a study, on the heels of the successful submission of their Investigational New Drug Application with the U.S. Food and Drug Administration, will be a safety, tolerability and pharmacokinetic/pharmacodynamic study of NFX-179 Gel in adult patients with cutaneous neurofibromatosis type 1. This study will evaluate three concentrations of NFX-179 Gel compared with its vehicle (placebo) in a randomized, double-blind, placebo-controlled, clinical trial of 48 patients with cutaneous NF1. The study will be conducted at five investigational centers across the United States.

"Almost all adults with neurofibromatosis type 1 develop cutaneous nerve tumors during their lifetime. The tumors form under the skin and manifest as nodules that can be stigmatizing and even painful," said Annette Bakker, PhD, Children's Tumor Foundation President. "We are pleased that NFlection has recognized the unmet need posed by cutaneous neurofibromas, and we are encouraged that topical NFX-179 may hopefully soon provide a life-changing solution for thousands of NF1 patients."

Read more about these partnerships on the CTF newsfeed at ctf.org/news

2020 Virtual NF Forum

UPCOMING EVENTS:

Register today at ctf.org/nfforum

- **October 15th Virtual NF2 Gathering - NF2 Education, Research Updates, and Support.** CTF is excited to partner with The Ohio State University to re-introduce the NF2 Gathering, a flagship event for the NF2 community for many years. Closed captions will be provided for this and all NF Forum sessions and recordings.

RECENT EVENTS:

Select NF Forum sessions are available to view at ctf.org/nfforum

- **September 17th Virtual NF1 Forum - The Impact of NF on Relationships, Learning, and School.** Discussion of how NF1 impacts education, including IEPs, learning disabilities, social-emotional issues, and more.
- **August 13th: Clinical Research** - Hear about where we have been and where we are going in NF1 research, as well as a discussion about recent clinical trials.
- **July 16th: Case Conference** - A panel of expert clinicians discussed two unique medical cases that addressed various medical complications seen in NF1.
- **June 25th: Poster Session** - An interactive Q&A with the creators of some of the most exciting posters from this year's virtual NF Conference. Clinical poster session moderated by Bruce Korf, MD, PhD. Science poster session moderated by Verena Staedtke, MD, PhD.

The NF Forum took an exciting and new path this year, going virtual with patient and family meetings. This included four separate events for NF1 and one for NF2.

The 2020 Virtual NF Forum meetings were designed with every patient and family in mind to be relevant, provide important NF research updates, answer questions, and offer support to NF patients, families, and caregivers. Who knew that going virtual would also mean going international; we were able to bring together a wider audience of patients joining from across the United States and from as far as Canada, Costa Rica, England, and Argentina. Over 180 attendees participated in each session.

Financial sponsorship from:  AstraZeneca

In collaboration with:  NYU Langone Health

With planning assistance from:  MSK Kids
Memorial Sloan Kettering  Weill Cornell Medicine

2020 Volunteer Leadership Training Conference

This year's Volunteer Leadership Training Conference, like so many other events, went virtual. The event kicked off on July 23 with a Zoom session called Leading with the Mission, where volunteers learned how to better understand and effectively communicate the mission and research initiatives of the Foundation. Over 90 volunteers were in attendance from all over the United States with many fun and interactive opportunities for all.

After a day of learning together, the annual Volunteer Recognition Awards Ceremony followed. Outstanding volunteer leaders are recognized for their many accomplishments. The highest honor, Volunteer of the Year, is bestowed upon a volunteer who consistently goes above and beyond in their dedication to the NF cause and to the Children's Tumor Foundation. This year's award went to Teresa Williams.

The final session took place on August 1st, with a dynamic guest speaker, fundraising professional, President of Asking Matters, Brian Saber. Participants came away from these sessions with newfound confidence and insight.

Despite the inability to attend in person, attendees joked, laughed, discussed, debated, and celebrated one another in true CTF fighter style. CTF Volunteers are the Foundation's most valuable asset. They rise to every occasion and never allow anything to break their spirit, not even a global pandemic!

Some of the 2020 Volunteer Recognition Award Winners:

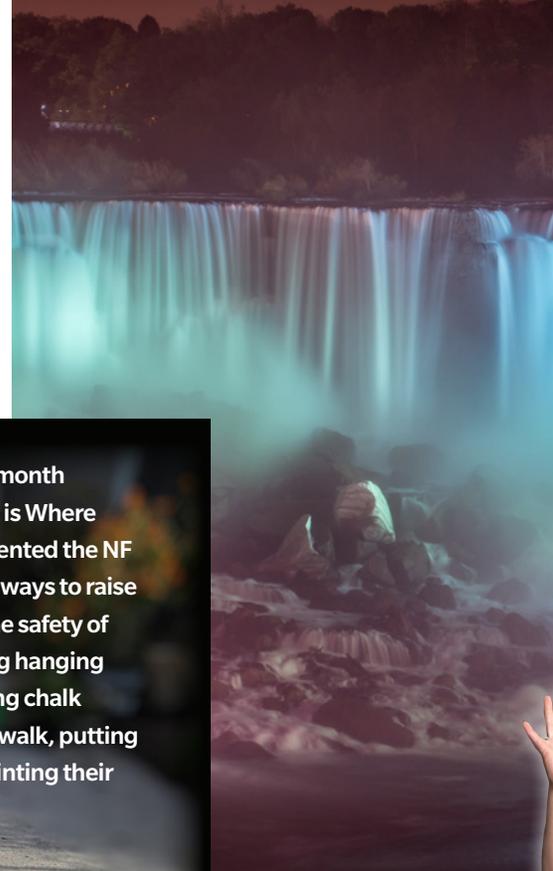
Top row, from left: Diane Owens, Anita Gribben, April Ondis, Teresa Williams; Middle row, from left: Stephanie Jaramillo, Jessica Samblanet, Lara Mukabonov, Hannah Doby; Bottom row, from left: Rachel Mindrup, Jenny Kearschner, Naomi Cuka



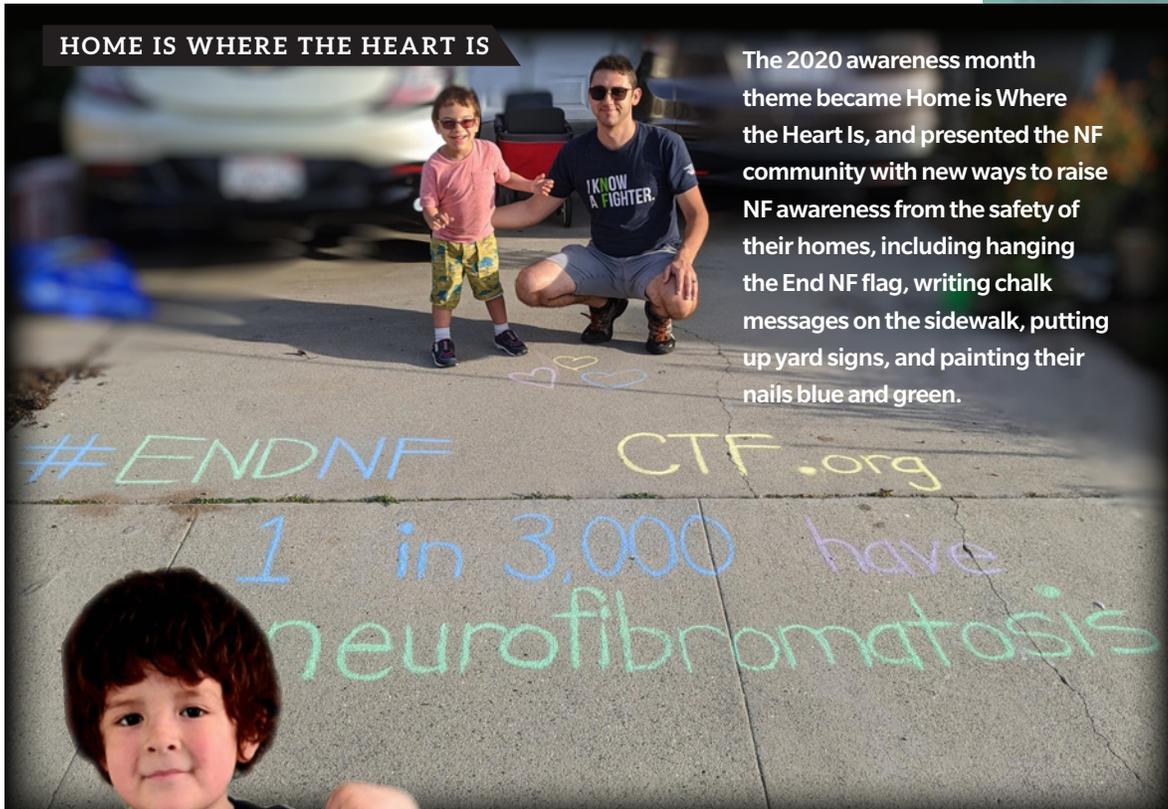
To learn how to become a CTF Volunteer, email volunteer@ctf.org and join others who are making a difference.

Awareness Month HIGHLIGHTS

Each year, May is celebrated as NF Awareness Month, and volunteers and partner organizations draw attention to neurofibromatosis and those living with this disorder in a number of ways.



HOME IS WHERE THE HEART IS



The 2020 awareness month theme became Home is Where the Heart Is, and presented the NF community with new ways to raise NF awareness from the safety of their homes, including hanging the End NF flag, writing chalk messages on the sidewalk, putting up yard signs, and painting their nails blue and green.

ONE STEP CLOSER.

FDA approves 1st ever drug for NF!



“We all have NF2. I honor my father’s legacy by teaching my son what my father taught me: that our illness is nothing to be ashamed of nor will it limit us from living or achieving our dreams. I make NF visible by raising awareness to educate the community about the adversities we, NF fighters, endure and overcome.” —**CHYENNE FUENTES**

PHOTO CONTEST WINNER

For the 2020 Awareness Month Photo Contest, we asked the community to tell us how they make NF visible and why it’s so important. The submissions were beautiful and inspiring, particularly the winning entry from Chyenne Fuentes.

Watch our 2020 Awareness Month Recap Video at ctf.org/nfawareness



SHINE A LIGHT

The Shine a Light on NF initiative casts a blue and green glow on buildings, bridges, and monuments across the country and around the globe. This year many volunteers spread awareness from home by changing their house lights to blue and green, and hanging up blue Christmas lights. Despite the global pandemic, many landmarks around the world continued the Shine A Light tradition, and lit up in blue and green, including the incredible lighting of Niagara Falls.

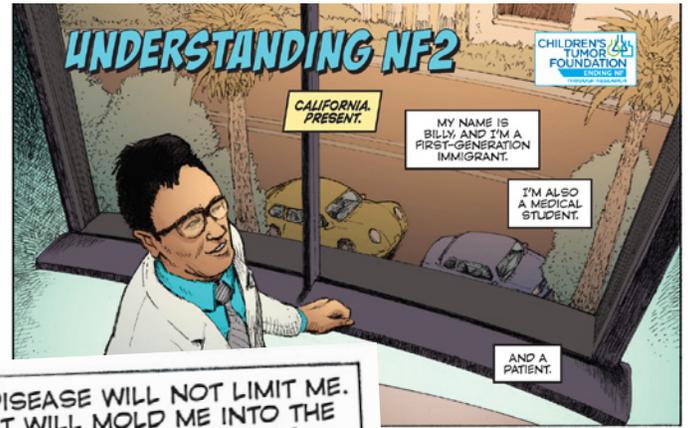


KOSELUGO

April's announcement of the FDA approval of Koselugo (selumetinib) was celebrated throughout the month of May with virtual awareness gatherings and educational webinars. To view videos, webinars, and patient education materials, go to: ctf.org/mek

MAKE NF VISIBLE

Together with photographer Craig Warga, the Make NF Visible photo series looked at the different ways NF can affect a person's life, even when no one else can see, launching a campaign and a movement that carried over into events like the Zoomathon event that took place on World NF Awareness Day (see cover), and beyond. You can see all the photos and read about these NF Heroes at: ctf.org/makenfvisible



CALIFORNIA. PRESENT.

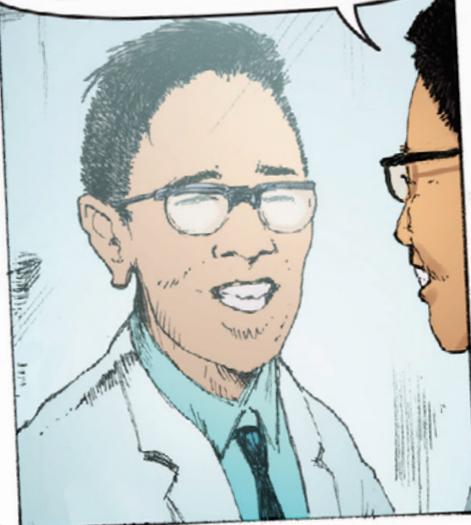
MY NAME IS BILLY, AND I'M A FIRST-GENERATION IMMIGRANT.

I'M ALSO A MEDICAL STUDENT.

AND A PATIENT.



THIS DISEASE WILL NOT LIMIT ME, BUT IT WILL MOLD ME INTO THE BEST PHYSICIAN I CAN BE.



In celebration of NF2 Awareness Day, CTF launched a new NF Hero Comic, *Understanding NF2*. This new comic book tells the story of Billy Nguyen, who lives with NF2, and includes information about an NF2 diagnosis and tumor types. To freely read or download this comic book, go to: ctf.org/billy



stories

OF NF



Kayden Herry

When I first learned about NF when Kayden was about 6 months old, because he had café au lait spots. He was officially diagnosed at 18 months old when he developed a neurofibroma tumor near his temple and right eye.

My almost-5-year-old son is a true fighter! We have so many doctor appointments every month and he is still the happiest boy in the world. Kayden has a severe speech delay that he is in speech therapy for, and has done very well and loves to chat! He loves all his doctors and he is a rock star when it comes to the tests that he does every three months. He takes his chemo pill, selumetinib, like a champ two times a day, every day. He even tells us when it's time to take his medicine! He has had a 25% reduction in his neurofibroma tumor and we are so happy with the progress he has made. Recently, he was diagnosed with level 2 autism. We will be getting ready to get him into school and will be looking at an IEP and aide for him.

Kayden loves to play with cars from the Disney Cars movies. It's his absolute favorite thing in the world! He likes to watch movies and act out scenes with them. He has a very creative imagination! He also loves to be outside and play in the water. He is an absolute daredevil and goes on water slides and fast rides. He LOVES his family. He loves to goof around and wrestle with his Daddy and big Sissy and loves cuddles with his Momma. He is such a strong and kind hearted little boy. His big smile brightens everyone's day!

- Amanda Herry, Kayden's mom



Lindsay Shvetzoff

When I was 21, I was involved in an accident that left me unconscious. I was brought to the emergency room and put into a CT scan. The doctor walked in and showed me a mass in the back of my brain and said it's probably

just a cyst but that an outpatient MRI was recommended. Two weeks later I went in for the MRI and it took five doctors reviewing my scans before they let me go home. Upon my arrival I was met by a neurologist, neuro-surgeon, ENT, and a genetic counselor. I watched as one of them started to write something on a yellow post-it note; he handed it to me and it read "Neurofibromatosis Type 2." That little piece of paper changed the the entire direction of my life.

Trying to explain to my other 21-year-old friends what was happening to me was incredibly difficult and hard for them to understand (rightfully so). I felt extremely alone. I wanted so badly to be able to live a normal young-adult life like my friends. I decided to leave college in order to focus on my health and seek treatments that might be available to me.

In some ways this has been challenging, and in others It has shown me paths that I would have never walked that have changed my life for the better. In 2011, I met Dr. Chris Moertel, a neuro-ophthalmologist at the University of Minnesota Masonic Children's Hospital who approved the drug Avastin for me. Dr. Moertel and his team gave me a chance and have gone out of their way to advocate for me since day one. These people are my heroes and are truly a second family to me. My quality of life and ultimately the devastating impact it would have had on me and my future is not lost on me.

Due to Avastin's long term side effects however, I was told I would never be able to have children. This news was devastating to me, as I had dreamed of being a mother since I was little. So, in 2018, when I found out I was pregnant, I was elated (and shocked!) The human body is amazing in its ability to heal. Now one-year-old, my son Silas has brought so much joy and laughter to my life. I love spending time with our little family at the beach. Building sandcastles and swimming the day away. It is in those moments I am free from NF2.

It is so important to acknowledge your reality. I assign a time to myself to simply be sad, angry, or scared. I let myself rest and do nothing. It's essential to allow yourself this space to feel. Once those assigned days are over, I always get right back up and move forward. No matter who you are there will always be days of emotional or physical pain, but remembering that brighter days are ahead can help keep you going. I would be lying if I didn't say that NF2 has taken a great deal from me, but it can never take away my positive attitude, my life purpose, or my strength. These are the cards I was dealt, and every day that passes I am learning new and more creative ways to play them.



Dear Friends,

For people like me, who are personally impacted by the devastating effects of NF, a gift in support of the Children's Tumor Foundation does more than just fund research – it is an investment in hope. Which is why I am asking you to join me today by making a gift to support the Children's Tumor Foundation and the hope it offers to millions of people who live with NF, like my son Eli.

For the first five years of Eli's life, we thought we were dealing with a rare eye condition. His retina detached when he was just five months old, and all of my thoughts and energy went toward trying to save the vision in his left eye. Even though Eli had a few tumors on his skin, he didn't fit the criteria for NF1, and no one even considered NF2. For 'peace of mind' I took him to a dermatologist to have those tumors checked out, and that appointment led us down the path that resulted in Eli's diagnosis of NF2 at five years old.

The first year and a half after he was diagnosed, we had no one to turn to, and it was an awful feeling. No one I knew or worked with had ever even heard of neurofibromatosis, let alone NF2. Now Eli is 12 years old, and he has already had two spinal surgeries, the last one in January of this year. For Eli, surgery was our ONLY option, and I hate that - I want Eli to have another option, a better choice.

I'm so inspired by the news about Koselugo (selumetinib), the first FDA-approved treatment for NF1! But in truth, that news also hurts, because I want that kind of progress for NF2, and for Eli.

Every day 120 people are born with NF. That means a baby is born with NF every 12 minutes. My dream for Eli, and for anyone born with NF, is to have treatments. I want approved, effective, life-changing NF2

treatments for my son.

To bring us all closer to this goal, the Children's Tumor Foundation has created the NF2 Accelerator Initiative.

This investment needs \$2.3 million to channel toward research that will bring NF2 treatments to patients. It will increase the number of drugs being tested for NF2, improve the testing process, and even develop NF2 gene therapy options.

The CTF Discovery Fund is an \$8 million investment that will fund more than 45 new research studies, and will speed up the drug discovery process for all types of neurofibromatosis. CTF's approach to NF research is to explore ALL options possible for ALL types of NF.

This research funding will dramatically improve the lives of the thousands of children and adults living with NF today, and the 120 babies who will be born with NF every day until we find a cure.

The time to act is now, and your donation will let each person living with NF know – you are not alone. There is hope. We're all in this together, and we are doing everything we can.

Sincerely yours,

A handwritten signature in blue ink that reads "Hannah DUBY".

Hannah DUBY

PS- [Donate today at ctf.org/donate](https://ctf.org/donate)



ABOUT HANNAH DUBY: Hannah and her husband Lindsey live in Kansas City with their son, Eli and two daughters, Madison and Ava. Hannah has been recognized for her volunteer work with the Children's Tumor Foundation, and her incredible efforts to support families recently diagnosed with NF2. Hannah has organized the Shine a Light on NF Walk in Kansas City for three years, which last fall attracted four new families impacted with NF2.

Around the COUNTRY

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



JEFF MEYER MPNST RESEARCH FUND

Jeffrey Meyer's courage, optimism, perseverance and selflessness were just a few of the qualities which made him an unwavering inspiration to others. The impact Jeff made was long-lasting, although his life was sadly cut short by complications of NF1. His mother, Carolyn, continues to be inspired by her beloved only son's path of altruism. She generously supports MPNST research through CTF and donates her time and resources to the volunteer fire department that Jeff cherished. You can learn more about Jeff at: ctf.org/jeffmeyer



Scott Carpenter, Travis Carpenter, and Matt Solum

#ENDNF WITH TRAVIS

The 7th Annual #EndNF with Travis Classic Golf Tournament occurred on Saturday, June 27, 2020 and was a tremendous success with the largest turnout and funds raised in the event's history. Organizers Scott Carpenter and Matt Solum patiently waited as numerous other summer events were being cancelled during the spring due to the ongoing pandemic, and their patience was rewarded as the host golf club gladly welcomed the return of the event raising funds and awareness for Children's Tumor Foundation. Travis kept the tradition of starting with an honorary tee shot to kick off the day, and Kelly Carpenter and Travis spent the day on the first tee greeting the participants and sharing the news of the recent FDA approval of Koselugo. As the numbers were tallied following the event, an incredible 42 four-person teams played golf, 10 volunteers graciously donated much of their day, and the event raised an estimated \$14,000 in support of CTF.

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

HALLOWEEN Bash

Cocktails & Costumes

October 24, 2020
7:30 - 10:30 PM ET 4:30 - 7:30 PM PT

Registration is free. To donate, register, or organize a watchparty, go to: ctf.org/halloweenbash

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

LOS ANGELES

Art & Cocktails for a Cure

SATURDAY, SEPTEMBER 26, 2020
5:00 - 6:30PM PACIFIC TIME

Hosted by
Jonathan Sadowski

Artwork auction by
NF Heroes

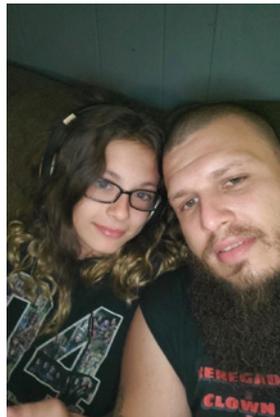
Registration is free. To donate, register, or become a sponsor, go to:
ctf.org/cocktailsla2020



CTF's new opportunity, Connect2Fight is for anyone who wants to raise funds and awareness for NF through live streaming! If you are new to live streaming, some examples include video games, talk shows, board games, yard games, makeup tutorials, live music, or even just chatting. If you have an existing online channel, we would be so thankful to have you educate your community about NF, and use the new tools we have created to help seamlessly raise funds for the Children's Tumor Foundation. Some examples of recent live stream events are below. **Learn more about these stories and this exciting new program at: ctf.org/connect2fight**

The Bearded Guy Live Stream Fundraiser

Joe Downey is a guy with a beard who also happens to be a single dad living with NF2. He's a self-professed single dad chronicler, content creator, influencer, and host of a YouTube channel called Bearded Life. Joe contacted us in April to let us know that he would be hosting a live auction on his channel on May 17, NF Awareness Day and that he wanted to raise money for the Children's Tumor Foundation. CTF's Connect2Fight program gave Joe a link to create a Tiltify page, some logos and graphics, then he did the rest, raising nearly \$5,000 in just 2 hours and 18 minutes.



BRAVE THE SHAVE FOR NF LIVE STREAM

As part of CTF's brand new live stream and gaming program, Sarah Sappington shared her story of living with NF2 through a video campaign on social media, which she called Brave the Shave for NF. She let her audience know that she had decided to shave her head and donate her long, beautiful blonde locks to an organization that makes wigs and gives them to children with hair loss. She professed this in the name of making others aware of the NF cause and set a goal of \$500 to raise. She surpassed her goal in a mere three hours and raised nearly \$3,000 through online donations, with the video garnering 7,500 views.

“ I decided to do my Brave the Shave for NF campaign because I wanted to cut my hair. People have commented on how beautiful it is all my life. Which is so nice, but I realize that so much of feeling pretty or desired as a woman, rests in my hair. So I want to challenge that. . . . Then I realized this would be the perfect opportunity to shave my head and donate so much more hair to a child who needs it. It was NF Awareness month so I thought it would be the perfect opportunity to raise money. ”

—SARAH SAPPINGTON, WHO LIVES WITH NF2

“THANK YOU/I’M SORRY” LIVE STREAM PLAY

Janna Walter and her daughter, NF National Ambassador Brianna Worden wrote a play entitled “Thank You/I’m Sorry,” telling their story of love, pain, and life changes while dealing with neurofibromatosis. The ladies were set to perform the play with SUNY Sullivan’s Performing Arts School for NF Awareness Month, but because of the outbreak of the coronavirus, they performed the production virtually, which they said was actually a silver lining, as they were able to reach a wider audience and raise more than \$800.



END NF CHALLENGE 2020

STRIDE • ROLL • SPLASH • SCULPT • BUILD



Join others to collectively get active to end NF, whether it's yoga, pilates, barre, martial arts or more!



nfendurance.org/challenge

END NF Challenge

In June of 2020, the NF Endurance Team went virtual with the month-long END NF Challenge. In honor of the 1 in every 3,000 people living with NF, our mission was to cover 3,000 miles by working together, and WE DID IT! By June 30, 132 participants across the country walked or ran 3,091 miles, with \$8,000 raised to date. By sharing miles logged, photos, and videos online via Facebook and Strava, the community encouraged and supported one another throughout the Challenge.

With the positive feedback and the success of the June END NF Challenge, we are excited to announce our fall campaign: **the END NF Challenge Virtual Series!** For our Fall Series, we invite everyone to accept the next challenge: A collective goal of 2,500,000 total minutes of any activity until November 15. Everything counts!

Swim | Run | Walk | Stairs | Skate | Scoot | Kayak | Bike | Yoga | Hike | Climb | Trek | Dive | Sail | Ruck | Ski | Row | Crossfit | Surf | Barre | Martial | Paddle

Let's work together! 2020 events and races may be canceled, but our mission is not! Join us and become a part of a global NF Endurance community going the extra mile to END NF.



I was thrilled when I saw the END NF Challenge because it gave me something to focus on, gave me a goal to reach, and I got to meet new people in the NF community. It was a fun challenge and I loved seeing all the hard work people put in for the month of June. So happy and proud to be a part of such an amazing group of athletes!

HOLLY GRIFFIN



NF CAMP

Virtual NF Camp

NF Camp has gone virtual! Over 115 NF Heroes from the USA, Canada, and Europe joined CTF's Virtual NF Camp this July for community and fun. All sessions were hosted by the NF Mentors, a group of young adults living with NF. Activities for virtual campers at home included: online games, pet show and tell, tie-dye, baking, churning ice cream, making slime, and more! A series of sessions were also hosted by members of the medical community, which sparked deeper conversations about stress, bullying, and living with NF in general. The virtual format allowed campers to feel comfortable in their own homes whether they were engaged in thoughtful discussions or just having fun. Keep your eye out for more virtual youth and young adult opportunities later this year.



"My favorite part of NF camp was being able to meet new people and talk about how NF affects us differently, and the art and tie-dye sessions!"

— YANIRY LORA, NF HERO AND CAMP PARTICIPANT

shinealightwalk.org

SEPTEMBER 26
Westchester/
Rochester/
Long Island

SEPTEMBER 26
Cincinnati

SEPTEMBER 26
Denver

SEPTEMBER 26
Kansas City

SEPTEMBER 26
Chicagoland

OCTOBER 3
San Antonio

OCTOBER 3
Washington DC

OCTOBER 3
New Jersey

OCTOBER 3
South Dakota

OCTOBER 4
New England

OCTOBER 10
Philadelphia

OCTOBER 10
Idaho & Idaho
Falls

OCTOBER 11
The Carolinas/
Kernersville

OCTOBER 17
Utah

OCTOBER 18
Atlanta

OCTOBER 24
Los Angeles

NOVEMBER 7
Houston

NOVEMBER 8
Alabama

NOVEMBER 8
Phoenix

NOVEMBER 14
San Diego

NOVEMBER 14
Central Florida/
South Florida



The 2020 Shine a Light Walk Season has begun! Michigan, Seattle, Bay Area, Iowa, Virginia, and Jacksonville have already taken place with the remainder of our Walks scheduled between September and November. Our Walks are virtual this year with each event taking place on its originally scheduled date. We are still celebrating our NF Heroes and continuing to reach our goals across the country. Participants can tune in to their Shine a Light Facebook event page that day to see videos that celebrate our heroes and pictures of teams celebrating walk day in their communities. Everyone is encouraged to get out and walk with their friends and family members as well as post photos and videos to their local Facebook event page. Thank you to the amazing committees, families, and the Honorary Chairs from our NF Clinics who have already shared their stories with us at our first four Walks. Join us by registering at www.shinealightwalk.org. Since we are virtual, you can join any walk you choose! Best of all, your fundraising pages remain open through the end of the year. We also want to extend a special thanks to our Luminary Sponsor, AstraZeneca, for supporting our Shine a Light Walk program this year and helping us get closer to our goal of raising \$1.3 million to end NF!

“ Covid has stopped a lot of things, but it can't stop us from continuing the fight to end NF!

—KELLY EASTMAN, MICHIGAN
WALK TEAM CAPTAIN,
HUDSON'S HEROES

“ We will never stop fighting to help find a cure to end NF for our family and thousands of others.

—JILL OSTROM,
SEATTLE WALK TEAM CAPTAIN,
TEAM OWEN O



Children's Tumor Foundation Europe

CTF Europe is committed to developing effective treatments for the over 250,000 European citizens living with neurofibromatosis. With over 2.5 million people living with NF worldwide, we are a global community united to end NF.

A Belgian foundation, CTF Europe is approved as a Public Utility by Royal Decree, and is a partner of the United States-based Children's Tumor Foundation. Complementary to national patient associations in Europe, CTF Europe is focused on raising NF awareness throughout Europe and demonstrating the impact of research for all forms of neurofibromatosis – NF1, NF2, and schwannomatosis – as well as relevance for other rare disease areas, including cancer.

CTF Europe is a partner of the King Baudouin Foundation and a beneficiary of the Transnational Giving Europe network, which facilitates tax efficient cross-border donations in Europe.

Since its launch in the fall of 2018, CTF Europe has been actively working to fund research and raise awareness of NF throughout the world. Highlights of that activity are below, and you can read more at: ctfeurope.org/news

Clinical Care Advisory Board Launched to Help Improve NF Patient Care

CTF Europe has pulled together leading NF Clinicians from the top NF centers across Europe, with the critical goal of improving and standardizing care for NF patients. This newly formed 16-member Clinical Care Advisory Board (CCAB) is a key advancement in NF care. The CCAB will collaborate with the clinicians from EU-PEARL, the European NF group and the European Reference Network (ERN).



NF Registry Welcomes European Patients

The NF Registry is a unique opportunity for NF patients to learn about and take an active role in the development of next phase therapies through information sharing in a confidential, high-impact resource. To learn more and join the NF Registry, go to: nfregistry.org



CTF IS AN ASSOCIATED RESEARCH PARTNER OF THE INNOVATIVE MEDICINE INITIATIVE (IMI)

The Children's Tumor Foundation contributes in-kind to the IMI-funded public-private NF partnerships in coalition with European Federation of Pharmaceutical Industries and Associations (EFPIA) partners.



Putting NF on the World Stage Through Music

CTF Europe launched the first-ever Flying High at the Heart of It: A Musical Journey to End NF, in collaboration with world-renowned musician Sandro Norton, on the 5th of September in Portugal. The event featured beautiful international music and inspiring stories of NF, raising nearly \$28,000 for CTF Europe.

Placing NF on the European Research Funding Landscape

CTF Europe has been greatly involved in introducing NF to high-impact stakeholders throughout the continent, and is in the process of helping shape European Union (EU) policies through its Horizon Europe funding program and Europe's Beating Cancer Plan.



CTF DRIVES FIRST EVER PUBLIC-PRIVATE PARTNERSHIP FOR NF RESEARCH IN EUROPE

The Children's Tumor Foundation co-leads the NF work package in EU-PEARL (EU Patient-Centric Clinical Trial Platforms), a €26 million IMI-funded collaboration among European academic and industry leaders in NF. This exciting multi-stakeholder project will provide the framework for designing and executing cross-company Integrated Research Platforms.



Read more about each of these initiatives at ctfeurope.org/news

Back to SCHOOL



Many of us are faced with a difficult decision about whether or not to send our kids back to school this fall, and how to manage school after months of school closures due to the coronavirus pandemic. Please know that the Children's Tumor Foundation is here to listen, and if there is anything we can do to ease the burden of stress on your entire family, we are here to help.

The NF Parent Guidebook is a free, 160-page resource available to help parents develop tools for the learning issues and anxiety that many children and young adults with NF1 are facing. The guidebook contains information, charts, reminders, and strategies for parents, and each chapter ends with a related activity just for kids.

We hope you will also take advantage of CTF's library of children's resources, including our most recent addition, the *Moxie & Sparx Explain NF1* Motion Comic Video.

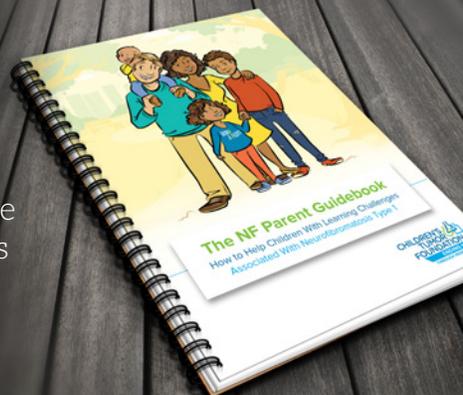
Go to [ctf.org/kids](https://www.ctf.org/kids) to find...

- *Moxie & Sparx NF1* Motion Comic Video
- Coloring pages
- NF Flashcards
- *Moxie & Sparx Explain NF1* comic book
- *Understanding NF2* comic book
- *The Accelerator*, a one-page comic about CTF
- Reading Lists
- Superhero Art Academy



"One of the BEST resources our family has obtained is CTF's NF Parent Guidebook. It summarizes NF in a way that is understandable. I appreciate the table that lists the various traits as well as the approximate age that they usually appear."

—BRITTANY WARREN



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.

FOUNDATION STAFF

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Alissa Marks, Senior Manager, Marketing
Susanne Preinfalk, Director, Design
Vanessa Younger, Director, Communications
Peiyu Zhang, Data Analyst

The CTF National Gala has pivoted to a virtual event.
You are cordially invited to...

The National Celebration Concert to END NF

November 16,
2020 at
7:00 PM ET

HONOREES:



LILLY ANN BROOKS



JACK BURKE



JONATHAN SADOWSKI

AstraZeneca 

Registration is free.

To donate, sponsor, or register, go to ctf.org/celebration
Your support will be 100% tax-deductible.

Or text to join:

Text CTFDONATE to 41444 to donate
Text CTFCELEBRATE to 41444 to register

We hope you can join us for this virtual, star-studded
celebration from the comfort of your home.

Hosted by:

Jonathan Sadowski (Actor/Producer)
Raina Seitel (TV Host/NBC Correspondent)

Honoring:

2020 Scientific Innovation Award: AstraZeneca
2020 Humanitarian Award: Jack Burke | CureNFwithJack
2020 CTF Champion Award: Jonathan Sadowski
Presented by: Ian Desmond of the Colorado Rockies
2021 NF National Ambassador:
Lilly Ann Brooks

Virtual Celebration Co-Chairs:

Erica Hartman-Horvitz
Shelley Haughton
Liz Rodbell

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