

NF NEWS

THE NEWSLETTER OF THE CHILDREN'S TUMOR FOUNDATION
SPRING 2023

CHILDREN'S
TUMOR
FOUNDATION
ENDING NF
THROUGH RESEARCH

Make NF Visible

May is NF Awareness Month, and the Children's Tumor Foundation is amplifying our efforts to Make NF Visible.

Often patients with visible signs of NF struggle to be seen as more than just their NF, while patients whose NF is invisible struggle to make others understand. Make NF Visible is about seeing NF, and seeing the person living with it.

Learn more about all the ways you can Make NF Visible this May on page 8.

INSIDE:

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Pictured: Lindsey and her son Bryson both live with NF, and participated in our new Make NF Visible portrait series by photographer Craig Warga. [makenfvisible.org](https://www.makenfvisible.org)



FROM the President

Annette Bakker, PhD



At the Children's Tumor Foundation (CTF), we are passionate about accelerating the development of treatment options for NF, which includes neurofibromatosis type 1 (NF1) and all forms of schwannomatosis (SWN), including NF2-related schwannomatosis (NF2). We are working hard to attract pharma and biotech to the NF space in order to invest and develop treatments that will improve lives. A decade ago, there were no such companies investing in NF; now, there are 10. This is a tremendous development, and we believe the future is promising for even more partners to join us in the fight to end NF.

In support of these endeavors is our incredible CTF Board of Directors, which recently named Gabriel Groisman as Board Chair. Through the efforts of this illustrious Board, CTF has further diversified its investment

portfolio, now including impact investments in companies. NFlection Therapeutics was the first company, and definitely not the last one. It fits our philosophy of accelerating access to treatments for patients.

Thank you for joining us again this May as we Make NF Visible through our awareness month efforts, including our Shine a Light campaign, proclamations, video contest, and more. Throughout this newsletter and on social media, you will see photographs from an all-new Make NF Visible portrait series. Our upcoming World NF Awareness Day Live event will take place on May 17, and I invite you to join me at this beautiful evening of celebration, which will debut short documentaries of NF Heroes.

To all of you who braved the winter cold this February at your local Cupid's Undie Run, we thank you. We also thank the thousands of walkers, runners, swimmers, and donors who join together in our Shine a Light NF Walk and NF Endurance programs. Above all, thank you to our NF patients, for being such amazing partners on our path to ending NF. Your input is essential on our path to finding more treatments, improved clinical care, and an end to NF.


Annette Bakker, PhD
President

IN THE NEWS:



On March 9, 2023, USA Today published an opinion essay written by NF Hero Lilly Simon, who last year was filmed on the NYC subway without her knowledge.

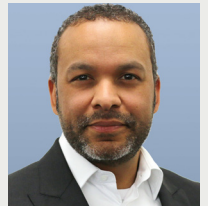
People Magazine featured McKinnon Galloway, who lives with NF2, in their "Real-Life Love" series, with a story about how her relationship has prevailed despite surgeries and hearing loss due to NF2. McKinnon recently announced her engagement to Brandon Kwiatek.

Read more at ctf.org/news

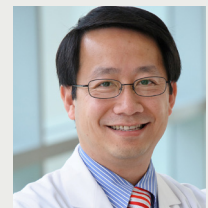
Welcome New Board Members



Terri Rawson



Roger-Ketcha Ngassam



Lu Le, MD, PhD

Board of Directors

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Liz Rodbell
Vice Chair

Randall Stanicky
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Scott Plotkin, MD, PhD
Chair, Clinical Care Advisory Board

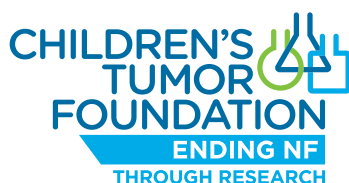
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Ed Stern
Pro Bono Counsel

Allan Rubenstein, MD
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Gabriel Groisman

Elected Chairman of the CTF Board of Directors

The Board of Directors of the Children's Tumor Foundation has unanimously elected Gabriel Groisman of Bal Harbour, Florida, as Chairman of the Board. First elected to the CTF Board in 2016, Mr. Groisman most recently served the Foundation as Vice-Chairman of the Board, and previously as Secretary. He has also served as Chair of the Community Relations and Government Engagement Committee of the Board.

Mr. Groisman is a government affairs consultant and attorney at LSN Partners, LLC in Miami, Florida, where he is a Partner and Chair of the Israel Tech Practice. Mr. Groisman also served his community as Mayor of Bal Harbour, Florida for six years (Nov. 2016 - Nov. 2022). He is a husband and father of five beautiful girls.

In addition, Mr. Groisman is also a sought-

“I am deeply grateful and humbled to have the support of the Board of the Children's Tumor Foundation to serve as its Chair.”

— GABRIEL GROISMAN, CTF BOARD CHAIR



after public speaker and TV and radio analyst on the topics of combating anti-Semitism and Middle East policy. In 2018, Mr. Groisman was awarded the Pursuit of Justice Award by the prestigious American Association of Jewish Lawyers and Jurists. Mr. Groisman often advises on and litigates cases around the world related to protecting the Jewish community from discriminatory conduct by governments and private individuals alike.

Mr. Groisman has a BA in Philosophy from the University of Michigan, Ann Arbor, and a JD from the American University-Washington College of Law.

“I am deeply grateful and humbled to have

the support of the Board of the Children's Tumor Foundation to serve as its Chair,” said Gabriel Groisman.

Mr. Groisman succeeds the previous Board Chair, Tracy Galloway, who is now Chair Emeritus and a member of the Executive Committee. The CTF Board of Directors expresses its profound appreciation to Ms. Galloway for her many and continued years of service to CTF and the NF cause.

The Board has also selected Liz Rodbell as Vice Chair, RB Harrison as Secretary, and Randall Stanicky as Treasurer.

To read the entire announcement, go to ctf.org/board

NF2 MEETUPS

Join us for NF2 Information, Community, and Advocacy

Did you know that CTF's NF2 Accelerator Initiative hosts virtual monthly meetups for NF2 patients and patient advocates? In January of this year, CTF Foundation President Annette Bakker, along with Drs. Leia Nghiemphu and Marco Giovannini, offered a recap of the research updates shared at the 2023 NF2 and Schwannomatosis State of the Art Meeting that took place early this year in Los Angeles, California.

On February 16, Vikram Chakravarthy, MD of Ohio State University Wexner Medical Center presented about spine manifestations in NF2-related schwannomatosis and his approach to treatment options.

To view recordings of these and future NF2 meetups, go to ctf.org/nf2 and click the link to our NF2 Education playlist.

To join the NF2 Accelerators and receive news and information specifically related to NF2 from CTF, email us at NF2@ctf.org or subscribe using the newsletter form on our website at ctf.org. To learn more, go to ctf.org/endNF2



Lauren Gottlieb-Mora and her daughter Alex

“The NF2 Accelerator Meetups are meaningful to me because they foster hope, and hope is the only thing stronger than fear. The meetings empower me to face the future and to continue to support my daughter, who is impacted by NF2 on a daily basis,”

— Lauren Gottlieb-Mora

Major Biomarker Project to Help Identify Cancer Predisposition in NF1 Patients

The Children's Tumor Foundation has announced a groundbreaking three-year study, which it will fund for nearly \$2 million, to determine if a DNA-based blood test can offer better understanding and ultimately earlier diagnosis of cancer predisposition in neurofibromatosis type 1 (NF1) patients.

Approximately 7-15% of NF1 patients may develop malignant peripheral nerve sheath tumors (MPNSTs), which currently have no cure. Earlier diagnosis could offer patients more options and a better chance at combating these deadly tumors—whether surgically or through other treatment opportunities. Preliminary data has demonstrated that blood testing can improve the predictability of tumor progression, and major players across the NF research funding spectrum have agreed that this forthcoming study shows great promise and deserves to be funded.

It is this alignment and collaboration across the NF funding spectrum that is making this research opportunity possible. The Principal Investigators (PIs) of this project are Angela Hirbe, MD, PhD of Washington University and John Shern, MD of the National Cancer Institute (NCI) at the National Institutes of Health (NIH), in PI-level collaboration with Adela Chaudhuri MD, PhD at Washington University.

In their initial proposal to the Congressionally Directed Medical Research Programs (CDMRP), the Principal Investigators outlined their plan to offer a relatively simple blood test that has minimal risk to patients and may offer the ability to diagnose cancer earlier, which will likely improve outcomes for cancer patients. If the study is successful, the goal is to move it into clinical trials.

Their proposal requested funding as a CDMRP Synergistic Idea Award, aligning with the program's funding of potential high-impact research opportunities that have historically been neglected. Although the proposal received a very favorable and excellent review, the level of funding needed for the entire study could not be supported.

Fortunately, and somewhat uniquely in the medical research ecosystem, major NF funders have been working together, sharing information about projects that have received funding, those that haven't, project end goals, and results. This information-sharing ensures transparency across federal agencies and private funders, and it also allows for outcomes and products to be shared across the research spectrum.

In recent years, the effectiveness of this collaborative model resulted in the first FDA-approved treatment, Koselugo, (selumetinib), approved for NF patients with inoperable plexiform neurofibromas.

In the instance of this new biomarker study, CTF has committed its support to fund the study in its entirety. The project format is inspired by CTF's highly effective Synodos model, which brings top researchers together to solve complex problems in an open and collaborative environment. The researchers commit to the open sharing of data, and an NF patient representative is an essential part of the planning team. In addition to the successful path for selumetinib, CTF's Synodos model also identified and advanced the testing timeline for brigatinib as a potential



“I want to see the day when we can cure all cancers for our patients through early detection.”

— ANGELA HIRBE, MD, PHD

treatment for NF2 patients. Brigatinib is currently in clinical trial, in a funding partnership with Takeda.

“We believe that the most effective way to bring treatments to NF patients more quickly is through collaboration and information sharing,” said Annette Bakker, PhD, President of the Children's Tumor Foundation. “Patients are told to ‘watch and wait’ to see if the tumors grow, which is scary and unfair, so we are highly motivated that the biomarker study proposed by Drs. Hirbe, Shern, and Chaudhuri can potentially flip that scenario when it comes to the deadliest MPNST tumors. We also believe that great science should never stop, and we are proud of our partnership with the CDMRP, the National Institutes of Health, and other NF funders that projects like this will receive the support they deserve. We have all agreed to communicate openly with each other to benefit our common priority—the health of our NF patients.”

Further details will be announced through CTF's website and social media channels. Patients may also join the NF Registry to receive information on clinical studies taking place for the various forms and manifestations of NF.

To read more, go to ctf.org/news

CTF EXPANDS INTO A NEW STRATEGIC CHAPTER AS IMPACT INVESTOR IN NF RESEARCH



The Children's Tumor Foundation entered a new phase in its strategic NF research model by announcing a significant impact investment in a Phase 2b clinical trial at NFlection Therapeutics, a biotechnology company focused on the discovery and development of effective, targeted therapies for rare conditions. The trial involves NFX-179, a topical (on the skin) treatment which has successfully passed a Phase 1/2a (safety and first signs of efficacy) trial. The current Phase 2b study will more rigorously test the drug's safety and effectiveness.

The NFX-179 treatment is for cutaneous neurofibromas (cNF), which are tumors that grow in the skin or right underneath the skin, and result in severe disfiguring bumps on the skin. The vast majority of NF1 patients have cNF that are responsible for significant negative effects on their quality of life, including pain, itching, and emotional difficulties due to appearance concerns.

potential to shrink tumors. This new use of MEK is as a 'soft' topical inhibitor designed specifically for cutaneous neurofibromas, for which there is a high unmet need for treatment. CTF is making a landmark impact investment in next phase topical studies to help accelerate the development of this promising treatment.

CTF's investment strategy in NF drug development is a diversified portfolio approach to tackling all forms of NF, from early-stage investments in young investigators to established academic initiatives in the Discovery Fund, to large-scale open data collaboratives such as Synodos. CTF has operated as a strategic partner to all stakeholders who can help bring effective treatments to the patients. This first-in-its-history impact investment further establishes CTF as a co-investor with pharma/biotech in innovative NF

research, expanding opportunities to increase the NF drug development pipeline.

"We're thrilled to be working with NFlection on this incredible opportunity to bring life-changing treatments to NF patients," said Annette Bakker, President of the Children's Tumor Foundation. "CTF is mainly known as a seed funder, willing

to take risks, and willing to put in a lot of sweat equity to advance the science and the treatments. With our recent investments in science and pharma, we are sending a clear message that if we want to accelerate treatments, NF deserves serious funding."

"We're thankful to the Children's Tumor Foundation for this investment in our work,"

"We're thankful to the Children's Tumor Foundation for this investment in our work."

—WILLIAM HODDER,
CEO OF NFLECTION THERAPEUTICS

said William Hodder, CEO of NFlection Therapeutics. "We believe—with good reason, based on our previous results—that this treatment has the potential to help so many. We're also grateful for the connectivity CTF brings with this partnership to NF patients, clinics, and experts. It is known from other disease foundations that investments from medical research foundations like CTF may convince other investors that supporting NFlection is the right path forward."

Over the past decade, CTF has worked to grow the NF field, which in addition to expanding partnerships with researchers and funders, now has a growing number of pharma/biotech companies in the space. In addition to NFlection, they include Alexion/AstraZeneca, Takeda, SpringWorks, Novartis, Vivace, Pfizer, Healx, and NF2 Therapeutics.

Gabriel Groisman, Chairman of CTF's Board of Directors said: "The evolution of NF research has come so far in recent years, thanks in large part to the herculean efforts of the Children's Tumor Foundation. With this investment, we are taking the first step in our new approach to ending NF. The Foundation's Board of Directors and the Business Advisory Council have made the bold decision that it's time to start directly investing in select impact opportunities in the private sector that have the potential of bringing relief to NF patients as quickly as possible. Pharma and biotech can now look to CTF as a true partner, not just for knowledge and access to stakeholders, but also as a strategic investor. We at CTF are all in to find treatments and ultimately a cure for NF."

"CTF is mainly known as a seed funder, willing to take risks, and willing to put in a lot of sweat equity to advance the science and the treatments. With our recent investments in science and pharma, we are sending a clear message that if we want to accelerate treatments, NF deserves serious funding."

—ANNETTE BAKKER,
PRESIDENT OF THE CHILDREN'S TUMOR
FOUNDATION

NFX-179 is a topical, metabolically labile, MEK inhibitor, which is the same mechanism used in the drug Koselugo (selumetinib), which has previously been approved for inoperable plexiform neurofibromas. Koselugo is an oral drug that has systemic impact on the body. CTF was an early-stage funder of both animal and in-human studies that led to the discovery that MEK has the

Clinical Care Advisory Board Annual Retreat in Dallas

The Clinical Care Advisory Board (CCAB) met in February for its annual retreat. The CCAB consists of a group of committed NF clinicians and patient representatives collaborating with CTF to identify ways to improve NF clinical care, educate patients and clinicians, and research relevant clinical care topics. Scott Plotkin, MD, PhD is the CCAB Chair. The retreat reviewed the previous year's accomplishments and set new goals for 2023.

Among the 2022 accomplishments were two publications: "Awareness and agreement with neurofibromatosis care guidelines among U.S. neurofibromatosis specialists," published in the *Orphanet Journal of Rare Diseases*, and "COVID-19 in people with neurofibromatosis 1, neurofibromatosis 2, or schwannomatosis," in the *Genetics in Medicine* journal.

In addition, the CCAB approved the addition of three new clinics into the NF Clinic Network: two adult-focused clinics at the University of Washington in Seattle and Toronto General Hospital in Ontario, Canada, and a pediatric clinic at Akron Children's Hospital in Ohio.



As part of a multi-year project, the CCAB and colleagues continue efforts to assess the delivery of guideline-driven care. The first part of the study surveyed clinicians on their awareness and agreement with current NF care guidelines. A patient survey is available every May through the NF Registry and asks patients and caregivers about their experiences with receiving NF care. This kind of input from the NF community is essential to understanding how CTF can improve clinical care for NF patients. Read more about this important survey on page 14, and please consider joining the NF Registry or visiting your account each year in May to update your health profile and participate in the survey at nfregistry.org.

New CCAB projects for 2023 discussed at the February retreat include assessing

ways to improve health equity and developing an approach to establishing best care practices.

CCAB members are committed to clinician education and networking by organizing monthly case conferences for NF Clinic Network members and organizing a half-day clinical session at the NF Conference. CTF also involves CCAB members with writing and/or approval of CTF patient resources.

The CCAB team and its committees continue to meet at least monthly to collaborate on how to continue to improve clinical care. CTF has extreme gratitude to our CCAB members who volunteer their time and expertise to improve care for the NF community.



Children's Tumor Foundation MOBILE APPS FOR NF

NF CARE APP
for patients & families

NF & SWN DIAGNOSIS APP
for healthcare providers who are not NF experts

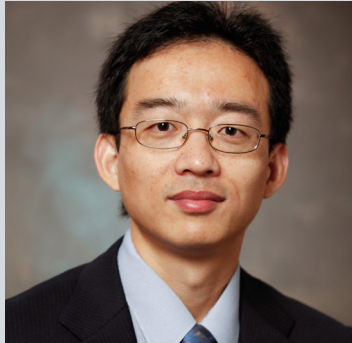
For more information, including links to download these free apps for Apple and Android users, go to

ctf.org/nfapp



2023 NF1 Gene Therapy Awardees

The Children's Tumor Foundation is pleased to announce the funding of three 2023 awards as part of the NF1 Gene Therapy Initiative, a research program focused on gene-based therapeutic approaches for the treatment of NF1.



Jiangbing Zhou, PhD
Yale University

Targeted Delivery of Gene Replacement Therapy for NF1 Plexiform Neurofibromas

Award amount: \$323,375.00 for a duration of two years

The goal of this project is to develop next-generation nanoparticles designed for targeted delivery of full-length human *NF1* cDNA preferentially to plexiform neurofibromas (pNF). This study will also characterize the therapy in animal models and target specific human pNF-relevant pathogenic variants. Successful completion of the study will result in novel therapeutic regimens for improved treatment of pNFs.



Harish Vasudevan, MD, PhD

University of California, San Francisco

Too Much of a GAP: Full-length NF1 Reconstitution in Neurofibroma and MPNST

Award amount: \$329,445.00 for a duration of two years

This project aims to define the mechanistic effects, functional requirement, and anti-tumor efficacy of NF1 gene therapy in the peripheral nervous system. The researcher will study how full-length neurofibromin restoration differs from that of GAP-related domain (GRD) alone or an arginine finger mutant (R1276P) incapable of inactivating Ras. This study will be critical to define the parts of the *NF1* gene required for successful gene therapy for NF1.



Nicholas Boulis, MD
Emory University

Patient-derived Plexiform Neurofibromas Organoid Model for Drug Repositioning in Precision Medicine

Award amount: \$164,817.00 for a duration of one year

This study aims to develop a patient-derived pNF and MPNST organoid system that preserves tumor heterogeneity and microenvironmental features and can be used for both high-throughput pharmacological screening (HTS) as well as transplantation in patient-derived xenograft (PDX) models. Using a peripheral nerve tumor bank and an existing FDA-approved compound library, the study will identify candidates for translational therapy and demonstrate the proof of concept of this methodology in pNF and MPNST.

CTF and American Academy of Pain Medicine Partner to Expand Field of Pain Studies in NF



The Children's Tumor Foundation and the American Academy of Pain Medicine (AAPM), a leader in pain study, prevention and care, have partnered to expand the number of investigators dedicated to the study of pain affecting the 2.5 million patients worldwide living with NF. The partnership announcement was made at the AAPM's Annual Meeting this March.

Driven by the critical need to attract more researchers into the study of NF pain, CTF announced a request for applications that will provide up to \$200,000 over 2 years to investigators new to NF, while also matching the researchers with CTF's experts for collaboration and mentorship. Learn more at ctf.org/aapm

MAKE NF VISIBLE

Throughout the year and especially during NF Awareness Month, the Children's Tumor Foundation is passionate about working with you, our dedicated donors and friends, to Make NF Visible. We want to make sure the world sees NF, and sees each person living with it.

We count on your donations each year to fund the vital CTF programs that are making a difference for the 2.5 million patients worldwide who live with some type of NF. We work to make a difference for patients like **Christine (pictured here) who live with NF2-related schwannomatosis, or NF2.**

Christine began having seizures when she was just seven-years-old. At nine, she was diagnosed with NF2 after an MRI revealed that she had bilateral vestibular schwannomas, tumors that are the hallmark sign of NF2.

About a year later, Christine developed a tumor over her eyelid, which had to be removed five times. Later Christine went on a clinical trial when she was 14, and stayed on it, despite harrowing side effects. Unfortunately, her tumor grew so much that she needed brain surgery which led to a number of complications. That surgery was followed by another and then another.

“By my junior year of college, I needed yet another surgery. And so, as an almost deaf individual with no hearing in my left ear and very little in my right ear, I’m trying to embrace the fact that I’m going to be deaf soon. And yet, this is another challenge, but I am going to overcome it,” she told us in January of this year.

In March, Christine underwent the surgery she referred to, which robbed her of her hearing. But with a fighter’s spirit, Christine is not letting that stop her from pursuing her goals and ambitions in life.



Christine, who lives with NF2

“I want to show others that just because I have NF2 and I’ve had 27 surgeries... Despite these challenges, I’m still pursuing my dreams. Right now, I’m in grad school. And I hope to pursue my PhD in the future. And that was my goal throughout my life, and I’m not letting NF2 stop me.”

Together with Christine, we envision a day when NF patients can pursue their dreams without surgeries and hearing loss due to NF. We want adults and children living with all types of neurofibromatosis and schwannomatosis to be free of the pain and fear that come with these conditions.

Make your donation now at ctf.org/hope to help fund more treatment options for all types of neurofibromatosis and schwannomatosis. We are counting on your help to continue the life-changing progress underway for patients with NF. **There is so much more work to be done — and we need your help today.**



Scan the code to donate, or watch a video featuring **Christine** along with NF Heroes **Joel**, who lives with schwannomatosis, and **Selena and Tyler**, who live with NF1.



MAY is NF Awareness Month!

Visit makenvisible.org to learn more ways YOU can **Make NF Visible!**



NYC's Empire State Building

LIGHT

up a building or visit a lit-up landmark in your area to help Shine a Light on NF



WEAR

our exclusive CTF blue and green gear all month, especially on May 17 & 22



CHANGE

your profile picture to one that celebrates NF Awareness



JOIN

the NF Registry if you or your child has NF



PROCLAIM

NF Awareness Month by securing a proclamation in your city or state



LIKE

Children's Tumor Foundation on social media, and tag your posts with #EndNF



SHARE

our exclusive photo series, Make NF Visible



VIDEO

yourself and submit at ctf.org/myvideo to be included in our May Recap



WATCH

NF Awareness Month videos on the CTF and Make NF Visible YouTube channels



EDUCATE

using informative fact sheets and brochures



NATIONAL BILLBOARD CAMPAIGN

Our thanks to Lamar Advertising who again this year is helping the Children's Tumor Foundation Make NF Visible through a national PSA digital billboard campaign to celebrate NF Awareness Month. Between the Lamar billboards, those secured by local NF families, and visibility opportunities provided by NFlection, Colbeck Capital Management, and BuzzTime Media, we're Making NF Visible in grander ways than ever before!

Download NF Awareness Month resources at: makenvisible.org

A World NF Day LIVE Event

to benefit the Children's Tumor Foundation

MAY 17
8:00 PM ET
5:00 PM PT

MakeNF Visible

Featuring the world premiere of two short documentaries about two young NF Heroes

HOSTED BY:



Melissa Brunner
News Anchor, WIBW-TV



Michele Holbrook
NF Ambassador 2023

Register and watch at: ctf.org/live

Extraordinary Spirit: **SUSIE MORGAN**



Susie Morgan performing in Dancing with Our Stars where she took home the Best Performance Mirror Ball Trophy.

The Children's Tumor Foundation met Susie Morgan in 2021 on the dance floor in Little Rock, Arkansas, for our annual Dancing with Our Stars Gala when she took home the Best Performance Mirror Ball Trophy. A born philanthropist and native Arkansan, Susie joined Children's Tumor Foundation efforts locally and became the Campaign Chair of the Foundation's Adult Pilot NF Clinic at the Winthrop P. Rockefeller Cancer Institute at the University of Arkansas Medical Sciences (UAMS).

With a goal of raising \$500,000 over two years, Susie has helped the Foundation raise more than \$250,000 in committed funds for this pilot project bringing multidisciplinary care to adults in the Arkansas area and outer states. This is a first-of-its-kind program being piloted in Arkansas and at UCLA in Los Angeles. More than 75% of the NF population are adults who aged out of pediatric care when they turned 18, and often have difficulty finding the multidisciplinary care they need.

Adult NF care is more complex and, at present, there is limited research or knowledge providing a standard of care for clinicians who treat adult NF patients. Adults with NF face an increased risk of cancer, insurance issues, job challenges, disability, and many other health and quality of life

issues, including the inability to travel to care, not having access to care in their local community, or traveling out of state to receive care which became even more difficult during the global pandemic.

The Foundation is honored and grateful to be working with Susie, a long-time philanthropist in Arkansas who is beyond passionate about causes involving children. Susie serves on the board of the Ronald McDonald House Charities of Arkansas and helped to raise \$9 million for their capital campaign. She chairs the Miss Arkansas Scholarship Foundation, having been a former Miss Arkansas herself. She has also been involved in the American Heart Association and is a lifetime Board member of the Arkansas Symphony Orchestra.

We sat down with Susie to ask her a few questions about her involvement with the Children's Tumor Foundation.

What did you know about the Children's Tumor Foundation before you became a DWOS Champion?

That's a great question as, in fact, I got involved when someone asked me to dance, and I love to dance! But, once I got to know the mission of the Foundation and met some of the children and young adults living with NF, I knew I had to get involved and help.

What drew you to support the Adult Pilot Clinic Project?

It's just incredible to me that when a child with NF becomes 18, they have nowhere to go for their treatment. This is a serious unmet need not just in our Arkansas community but across the United States. With 75% of the entire NF population being adults, we MUST provide them with the clinical and multidisciplinary care they need and deserve.

What has been your proudest moment in chairing the campaign?

The fact that we have already raised \$250,000 towards \$500,000 is incredible, but we have a long way to go. NF is not well known and the opportunity for us to be in front of our donors and prospects to tell them just how their dollars will support the community has made me so proud to be the Chair of the campaign and involved with the Children's Tumor Foundation.

It is with much gratitude that we offer our thanks to Susie Morgan for her commitment to this important CTF initiative and for her Extraordinary Spirit.

To learn more about how you can support the Adult NF Clinic Pilot Program, you can reach out to Michele Przepyszny, Chief Advancement Officer for the Foundation, at mprzepyszny@ctf.org or at 917-742-5977.

stories

OF NF



Allyson Miles NF1

I was diagnosed with NF1 at birth. Living with NF has impacted my career goals and influenced what path I can take. It impacts my ability to do daily living activities and makes me need a little extra help.

I stay motivated by looking at my strengths rather than my limitations. I am not suffering from NF but living with it. Although life is not always easy, I can manage with the support of my loved ones and friends. Living with NF has also helped me realize that sometimes people will stare and have comments that are not so nice, but the only comments that matter are my own. I have learned to be comfortable with who I am. I am proud of who I am, and that's the only opinion that matters.

I just graduated with my Master of Social Work degree. I specialized in aging and completed a thesis on using robotic pets with older adults enrolled in hospice or receiving palliative care. I have a passion for helping others. I have worked as a crisis counselor with a non-profit organization for the past five years.

Outside of school, I am part of the Wild West Working Dogs, a dog club that's part of the United Schutzhund Clubs of America. In this dog club which participates in RH, which is search and rescue competition, our dog Nox competes, and she is learning footstep tracking. Nox is also a therapy dog, and we take her to different agencies to support their clients.

My love for supporting my community is not only in my work but also in my everyday life.

"I stay motivated by looking at my strengths rather than my limitations. I am not suffering from NF but living with it. I have learned to be comfortable with who I am. I am proud of who I am, and that's the only opinion that matters."

—ALLYSON MILES, WHO LIVES WITH NF1



Mya Rodgers NF1

I was given the NF1 diagnosis at the age of 6 months. My struggle with NF began with a few cutaneous neurofibromas and café au lait spots. I was put in a hardback brace for 22 hours a day when I was 4 years old—and hardly ever complained. I've had a lot of operations throughout the course of my life.

My daily life is impacted by NF since it hurts all over my body. I detest whining, and I never do unless I'm in excruciating pain, but it does affect how I live my life every day. There are so many events, especially now that I'm a college student, that I wish I could go to, but occasionally my body is in so much agony at the end of the day that I simply can't. But I always give it my best shot and go to as many activities as I can, especially basketball games at my school!

I participated in a variety of sports, but my three favorites are cheering, competitive dancing, and flag football.

I am a college student with a major in Sports Communications and a minor in Marketing and Entrepreneurship, with a concentration in sports marketing. Something that makes me happy is watching the New York Giants play football and when I attend a game, which is a great experience.

SHINE A LIGHT NF WALK

The 2023 Shine a Light NF Walks are presented by **ALEXION**
AstraZeneca Rare Disease

It's time to register for the 2023 Shine a Light NF Walks!

We can't wait to see old friends and meet new ones. In 2022, 5,000 participants raised more than \$1.6 million to fund critical NF research projects and improve diagnosis and treatment for NF patients. We are setting our sights even higher this year.



Join an upcoming Shine A Light NF Walk!
Learn more at shinealightwalk.org

6/3/23	Cincinnati
8/26/23	Utah
9/9/23	Minnesota
9/9/23	Virginia
9/10/23	Seattle
9/16/23	Nashville
9/16/23	South Dakota
9/17/23	Michigan
9/23/23	Denver
9/23/23	Iowa
9/23/23	Kansas City
9/23/23	New Jersey
9/30/23	Chicagoland
10/7/23	Carolinas
10/14/23	Philadelphia
10/14/23	Dallas
10/14/23	New York
10/21/23	Atlanta
10/21/23	Southern California
11/4/23	Houston
11/12/23	Florida
11/12/23	Arizona

CUPID'S UNDIE RUN

In February, over 6,000 Cupid's Undie Runners hit the streets, sidewalks, and beaches in 29 different cities for a one mile-ish run. The 2023 season was bigger and brighter than ever, with more participants and nearly **\$1.7 million** raised!

Many Cupid's Undie Run cities reached or exceeded their participant and fundraising goals, including Boston, Cincinnati, Denver, Durham, Los Angeles, Phoenix, San Francisco, and Washington, D.C.

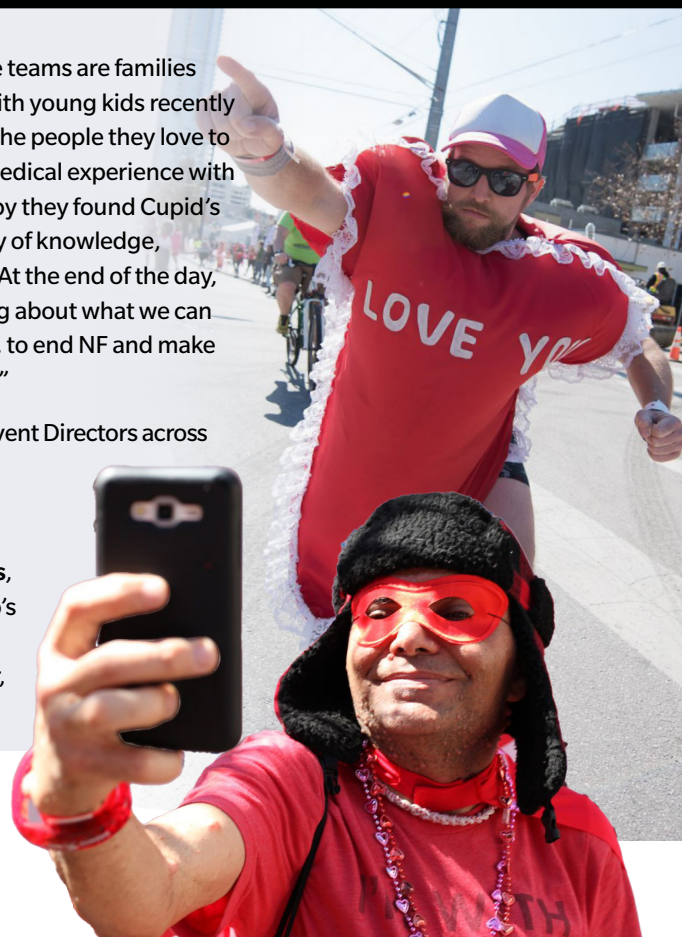
Christine Gallager, Cupid's Undie Run Volunteer Event Director in Denver, said:

"I'm just really blown away by how the community in Denver has shown up this year. It's our biggest fundraising year yet, and we have an incredible group of top teams and fundraisers who make it happen. Each of our top 6 teams has a direct connection to NF, and together they are bringing in more than \$110k this year. What's even more

amazing is that two of those teams are families newer to our community, with young kids recently diagnosed. Nobody wants the people they love to go through a challenging medical experience with their kids, but we're so happy they found Cupid's and CTF and the community of knowledge, support, and care we have. At the end of the day, I'm really emotional thinking about what we can do, and are doing, together, to end NF and make life better for these families."

Thank you to our amazing Event Directors across the country, our local committees and volunteers, local partners, and to our National Partner, **Love, Tito's**, the philanthropic arm of Tito's Handmade Vodka, and our National Underwear Partner, **MeUndies**.

Registration for the 2024 Cupid's Undie Run season will open soon!
Check cupids.org for details.



United Airlines NYC Half

This March, the NF Endurance team hit the ground running at the 2023 United Airlines NYC Half, raising more than \$43,000 for NF research. The NYC Half team included new-to-CTF participant Leanna Scaglione, who lives with NF2 and found CTF while running last year's NYC Half. She saw a runner pass her in a blue CTF jersey with END NF on the back and knew she had to join the team. Leanna's story caught the attention of local media, including PIX11 News and WCBS Newsradio 880, who broadcast interviews with her the week of the race, further making NF visible.

Two fundraising teams created by CTF Junior Board Members also made a strong showing. Former CTF Ambassador Sarah Rodbell, who lives with NF1, launched A Hero and Her Sidekicks, which also included her sister Hannah and Hannah's boyfriend Mac Novich (see below). Meanwhile, Team Julia Perfetti - Running for a Cure was the event's top fundraising team, achieving their goal of \$15,000. Julia, who also lives with NF1, was joined by her brother AJ Perfetti, uncle John Perfetti, and aunt Meg Blank.

The NYC Half team also included CTF Board Chair Gabriel Groisman, who finished his first NF Endurance event, along with NFE alumni Cody Eaves, Anne Mary Orr, Sasha Drebskaya, Kristy Rath, and Jerome Falic, as well as CTF's Chief Advancement Officer, Michele Przepyszny, who was the team's fastest finisher! All were cheered on by the NYC Half team's NF Hero, Alex Owens, and his mom, Diane Owens, whose boisterous cheers, clanging cowbells, and colorful signs supported these incredible NF Endurance athletes.



NF Hero Sarah Rodbell

NF ENDURANCE ATHLETE PROFILE:

Mac Novich

What makes running with NF Endurance different from running a race outside of the team?

Over the past three years, I have run one full marathon and three half marathons. I was proud of these accomplishments but often found myself thinking about incorporating a platform to make these races more meaningful. With NF Endurance, I have found this meaning. Now I have the added motivation and pride of knowing that each step means more. I am also running with some inspiring teammates who have NF.

How do you see the impact of your efforts?

First, you could say, 'I feel the love.' Whenever I mention to my friends or coworkers that I am raising money for NF research, I am celebrated. Those who ask, 'What is NF?' get enrolled in NF 101, and spreading awareness has an immediate impact. Second, I know my effort leads to progress. I understand the immense expense associated with research for rare conditions. That's why it takes every dollar to make a difference. I know that there will be breakthroughs down the road, and I will take pride in knowing that the dollars I raised will have contributed to them.

Mac ran the United Airlines NYC Half in honor of his girlfriend Hannah's sister, Sarah Rodbell, who lives with NF. He will run with NF Endurance in the Berlin Marathon this fall.



Hannah Rodbell and Mac Novich



EL TOUR DE TUCSON

November 18, 2023

NF Endurance's cycling program is growing! Situated in Arizona's beautiful desert, El Tour de Tucson is a family-friendly event offering multiple distances, including a 102-, 63- or 32-mile course for seasoned cyclists, as well as several shorter rides for those just beginning their cycling journey. Join over 7,500 riders for the 40th year of El Tour de Tucson, a renowned event that spotlights charity, community, health, and wellness. For more information, contact Lydia Vanderloo at lvanderloo@ctf.org.

2023 NF ENDURANCE UPCOMING EVENTS

Rock 'n' Roll Salt Lake City
Aug. 18-19, 2023

BMW Berlin-Marathon
Sept. 24, 2023

Rock 'n' Roll San Jose
Sept. 30 - Oct. 1, 2023

Rock 'n' Roll Clearwater
Oct. 7-8, 2023

Bank of America Chicago Marathon
October 8, 2023

TCS New York City Marathon
November 5, 2023

El Tour de Tucson
November 18, 2023

Rock 'n' Roll San Antonio
Dec. 2-3, 2023

More information at nfendurance.org

MAKE A DIFFERENCE

CALLING ALL NF REGISTRY MEMBERS:

Please Complete the Healthcare Access Survey this May

Existing NF Registry members are encouraged to complete the **Healthcare Access Survey** this May. This once-a-year survey, conducted by researchers at Massachusetts General Hospital, asks about your or your child's NF care during the last year (from June 2021 to May 2022).

The survey is done during May every year as part of NF Awareness Month. Your participation is very important! It should only take 20 minutes or less to complete. Your responses will be shared with researchers anonymously. You will not be asked about individual doctors that you visit.

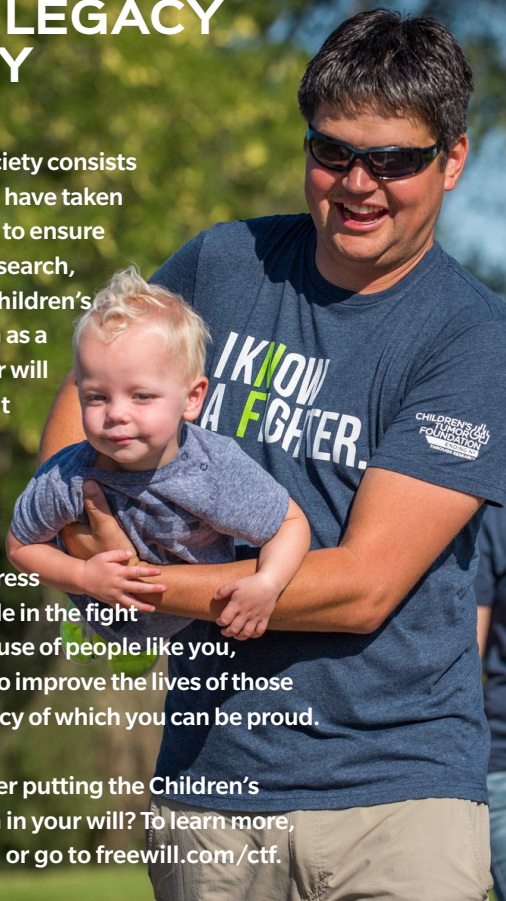
Thank you for helping to improve NF care! To participate, log in to your Registry account. Click on the name of the patient (you or your child) and select "Clinician Performance Surveys." If you have trouble logging in, email us at nfregistry@ctf.org.

THE NF LEGACY SOCIETY

The NF Legacy Society consists of individuals who have taken the extra initiative to ensure the future of NF research, by including the Children's Tumor Foundation as a beneficiary in their will or trust, retirement account, or life insurance policy.

Make no mistake about it –the progress that has been made in the fight against NF is because of people like you, who are working to improve the lives of those with NF. It is a legacy of which you can be proud.

Would you consider putting the Children's Tumor Foundation in your will? To learn more, email info@ctf.org or go to freewill.com/ctf.



Join the **NF Registry**

If you or your child live with any type of NF and have not yet joined the NF Registry, we encourage you to do so this May. NF Registry is a patient-driven resource for accelerating research and finding treatments for all forms of NF. It is the most efficient way to raise awareness for NF, expand the NF community, and connect to help end NF. Joining the NF Registry is easy, and will make an important difference in the fight against NF. Join the NF Registry today at nfregistry.org



"I think everyone should join the NF Registry because first of all, it doesn't hurt. It doesn't cost you anything. It doesn't take any time. You just get emails that tell you about really cool research opportunities . . . and you get to be a part of their research and give them data that can help them develop new drugs, that can help you, and that can help other patients with NF. Even if you don't really feel like it would help you personally, knowing that it'll help someone else with NF, and knowing that they could be a part of a trial that could help you, we're all a family, so whatever you do can help someone else just like yourself."

- ELANA LOFTSPRING, who lives with NF

CTF at European Patient Engagement Forum

The Drug Information Association (DIA) in Europe recently hosted an important session on Patient Engagement in partnership with EFPIA (European Federation of Pharmaceutical Industries and Associations) and EPF (European Patients Forum). On the panel was Annette Bakker, representing CTF Europe, as well as leaders across the drug development spectrum. Titled “Patient Engagement Action Plan – Seven Steps to Move the Needle,” the session explored how to better optimize drug development and clinical trial processes by placing patient input, experiences, and data at the center. You can find a link to a summary of this event by searching for “Seven Steps” at ctf.org/news.

CTF EUROPE TRAVEL GRANTS:

Professional Development Opportunities

Children’s Tumor Foundation Europe is offering travel grants to a selection of multidisciplinary NF centers throughout Europe to further the professional development of clinicians and allied healthcare professionals who see NF patients.

PARTICIPATING CENTERS

- Erasmus Medical Center, Rotterdam, Netherlands
- Hôpital Pitié-Salpêtrière, Paris, France
- Universitätsklinikum Hamburg-Eppendorf, Hamburg, Germany
- Guy’s and St Thomas’ Hospital, London, United Kingdom
- Hôpital Henri-Mondor, Créteil, greater Paris area, France
- UZ Leuven, Leuven, Belgium
- Medical University, Vienna, Austria

For further details, go to ctfeurope.org/research



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.

NF News Editor, Vanessa Younger
NF News Design Director, Susanne Preinfalk

The Children’s Tumor Foundation is the world’s leading nonprofit dedicated to funding and driving innovative research that will result in effective treatments for the millions of people worldwide living with NF, a group of genetic conditions that cause tumors to grow on nerves. These conditions may lead to blindness, deafness, bone abnormalities, disfigurement, learning disabilities, disabling pain, or cancer. NF affects all populations equally, and while there is no cure yet, 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: ctf.org.

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*As of March 30, 2023

MASTERCLASSES IN NF

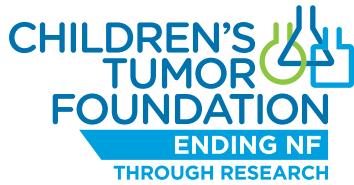
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