CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

Make NF Visible
Children's Tumor Foundation

Dear Friend,

2021 is the year that the Children's Tumor Foundation will Make NF Visible, a year-long campaign about how we see NF, and how we see the person living with it.

Neurofibromatosis, or NF, occurs in 1 of every 3,000 people, affecting more than 2.5 million people around the world. Why don't more people know about NF? Because there is no one way to define NF. Just like there is no one way to define a person living with it.

Please join this vitally important movement by donating today at ctf.org/NF. With your help, together we can make NF known around the world, leading to improved treatments, improved care, and improved lives for people like nine-year-old Jackson (pictured here) who live with NF.

In December of 2020, Jackson underwent a harrowing surgery due to NF-related scoliosis. Diagnosed with NF1 at just 2 months old, Jackson's yearly MRIs tracked the plexiform neurofibroma tumor in his back, and the resulting curvature of his spine. Last year, with the scans showing that his spine was already at an 85 degree curve, they couldn't wait any longer — surgery was the only option. Jackson underwent a partial fusion of his back and his surgeons put in two growing rods.

Thankfully, after a painful recovery, Jackson's surgery was a success. His doctor now wants him to start taking Koselugo (selumetinib) because his plexiform neurofibroma is growing toward his liver. Thanks to the support of donors like you, Koselugo is now an approved, effective treatment for children like Jackson.

Just one year ago, the NF community received the news of a lifetime — the United States Food and Drug Administration (FDA) announced the approval of the MEK-inhibitor drug Koselugo (selumetinib) for inoperable plexiform neurofibromas. **This historical moment marked the first ever FDA-approved drug treatment for people living with NF.**

This is a direct result of Children's Tumor Foundation-funded research that discovered MEK-inhibitor drugs have the potential to affect tumor size. Researchers took that potential and made it a reality: in clinical trials, more than 70% of NF patients taking Koselugo (selumetinib) have had shrinkage of 20 to 60% in the size of their tumors.

turn over ➔

That pioneering research was funded by donors like YOU – whether through participation in an event or a direct gift to the Foundation. It was your donations that led to an approved treatment for NF!

No one understands the importance of giving and being involved with other families affected by NF more than Jackson's parents, Stacey and Rob. They have been active participants, volunteers, and contributors to the Children's Tumor Foundation since Jackson's diagnosis as a baby. They are part of the CTF family!

> "One reason we are so involved with the Children's Tumor Foundation is because it's the only thing we can do. It keeps us going. The difference between nine years ago and now, and how far CTF has come with research and clinical trials and having real effective treatments is just amazing. **That's the only thing we can control — how can we help.** That's it," said Stacey, Jackson's mother.

The DeCillis family is making NF visible, for Jackson and for all the families that they have come to know and love through Children's Tumor Foundation programs. Please join them – because people with NF desperately need more help, more hope, and more treatment options.

Koselugo is approved **only** for NF1 patients with inoperable plexiform neurofibroma tumors. It is an incredible first step. **But the devastating effects of NF go far beyond this type of NF, and far beyond this one type of tumor.** There is still no approved treatment option for NF patients with disfiguring cutaneous neurofibromas, life-threatening meningiomas, or painful schwannomas. **We cannot stop until there are FDA-approved treatments for all forms of neurofibromatosis – NF1, NF2, and schwannomatosis.**

With your help - more treatments are on the horizon!

But we urgently need your help to bring them to FDA approval. I want to see an approved treatment for **patients with NF2, like Sequoyah Daniel-Robinson, and her father Darien,** who recently passed away due to complications from NF2.

> "Even though I don't look ill on the outside, I do wish people better understood that I face many challenges every day. It's hard to sit in a meeting at work and stay focused while my ears are ringing, or feeling pins and needles up and down my body . . . People know that I have NF2, but I know they don't understand it and how it affects me," said Sequoyah.

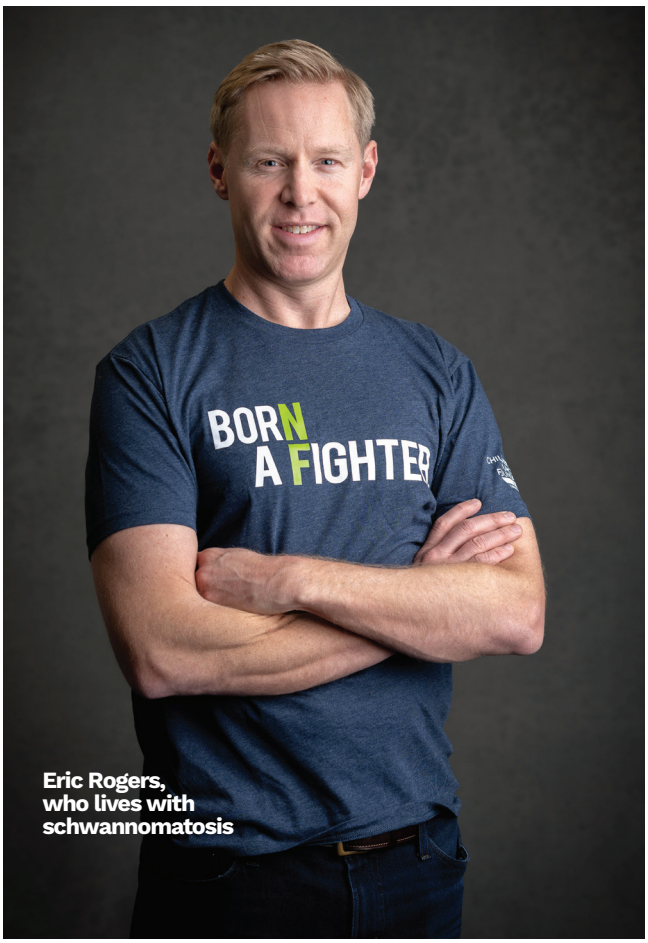
Sequoyah, who lives with NF2, pictured here with her late father Darien, who also lived with NF2.

continued ➔

For patients like Sequoyah, the CTF-spearheaded **NF2 Accelerator Initiative** is speeding research toward treatments for NF2. One of the many promising projects of this initiative is INTUITT-NF2, an innovative platform clinical trial which will evaluate multiple treatments for NF2 simultaneously. Those potential treatments are a direct result of CTF's Synodos for NF2 research, including a promising new drug called brigatinib, the first drug incorporated in the INTUITT-NF2 trial.

Your continued donations to the Children's Tumor Foundation at ctf.org/NF will continue to fund this research. Time after time, year after year - CTF donors like you fund breakthroughs!

But we need even more breakthroughs for people like **Eric** who was diagnosed with **schwannomatosis, the most rare form of NF.** Eric has become an advocate for the Children's Tumor Foundation, running with the NF Endurance team and making a difference for everyone who lives with all types of neurofibromatosis.



Eric Rogers, who lives with schwannomatosis

> "I had my first spinal schwannoma removed 17 years ago, and after recovery, life went on as normal for me. I had no idea that I had schwannomatosis. Only after my second spinal schwannoma was removed did I expand my knowledge of NF and get involved with the Children's Tumor Foundation. It is imperative that we expand awareness and make NF visible so that we can continue the fight for a cure," said Eric.

Eric and all of those suffering from the debilitating pain of schwannomatosis need an approved drug treatment now. They need relief from the pain that no one can see, and few even understand. CTF's Synodos for Schwannomatosis team-science collaboration is speeding the way toward treatments for schwannomatosis, with a specific focus on pain. We are also collaborating with top research institutions on the first-ever schwannomatosis clinical trial.

Thanks to your support, 68 potential drugs for all types of NF are in the pipeline. But without direct funding, those living with NF today will never see those drugs reach FDA approval. The moment we are living in now is pivotal, and we are at this moment because of supporters like you, who took a chance on the Children's Tumor Foundation, and the inspiring scientists we've funded.

turn over ➔

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

Make NF Visible
Children's Tumor Foundation

In March of 2020, just weeks before each of us isolated at home due to COVID-19, these NF Heroes gathered together to take these beautiful photographs, courtesy of photographer and NF dad, Craig Warga. As you see here, NF affects all ages, all genders, all races of people, in myriad visible and invisible ways. Please join these courageous NF Heroes and help to Make NF Visible.

Back row from left: Owen, Alexandra, Ken, Eric, Robert, Aiden
Middle: Sandy, Dan, Julia, McKinnon
Front: El, Amaya

Thank you so much for your past support. There is so much more work to be done — and we need your help now more than ever.

Please return the enclosed envelope, or donate at **ctf.org/NF** to help develop further treatments that will change the lives of millions of people around the world living with neurofibromatosis.

Sincerely,

Annette Bakker, PhD
Children's Tumor Foundation President

PS. Please don't wait. Your gift will make a difference for patients suffering with all types of neurofibromatosis. Donate today by returning the enclosed card, or donate online at **ctf.org/NF**