

A close-up, blue-tinted photograph of a microscope's objective lens and eyepiece, serving as the background for the document.

CHILDREN'S TUMOR FOUNDATION

Leading the Way to Ending NF

Strategic Business Model:

- Accelerating the Neurofibromatosis Research Path
- Revolutionizing Rare Disease Research Models
- Transforming the Next Wave in Cancer Treatments

Innovation is in Our DNA

At the Children's Tumor Foundation, we're working to better the lives of over 2.5 million people who live with NF. And we're doing so by focusing on the tagline in our name – "Ending NF Through Research." We envision a day when NF patients can live their lives free of the pain and the difficulties that come with NF, and that day is on the horizon because of our innovative team-based approach to drug development.

Patients are told to "watch and wait" to see if their tumors will grow, and determine later how it might impact their lives with devastating conditions such as malignant cancer. We don't think that's fair, and we don't think that's necessary. By bringing together the brightest minds in research and industry, and revamping the incentive systems that often slow the pathway to treatments, we can change "watch and wait" to "here's what you can do."

TRADITIONAL RARE DISEASE MODEL

PATIENT

TREATMENT

COST:
Hundreds of millions of dollars

Why it takes this long and costs so much?

- Patient manifestations unclear
- Clinical trial recruitment can be slow
- Disconnect between discovery and treatment
- Silos mean that experts are isolated
- Time delays in reporting

NEW CTF RESEARCH MODEL

PATIENT

TREATMENT

GOAL:
Double the speed for half the cost!

Why are we faster?

- NF patient registry accelerates patient recruitment
- Team science connects discovery to treatment
- Open NF datahub for real-time data release
- Preclinical platform speeds up drug testing
- Key opinion leader network speeds up decision making

And the best part? CTF's research model not only benefits NF, but any one of the millions of patients living with cancer, or one of the 7,000 rare diseases in existence.

We're in a rush to find treatments for patients. Join us as we revolutionize how treatments are developed for those who need them most.

Over 70% of enrolled patients with plexiform neurofibromas saw decreased volume of at least **20%** in these inoperable tumors, a first in NF research. This highly successful study is the result of the Children's Tumor Foundation's major investment in the team-science approach of the NF Preclinical Consortium. This MEK trial success has been published in the prestigious New England Journal of Medicine, and is on track to be NF's first approved drug.

Our Goal:
100% tumor reduction in **100%** of all patients.

MEK makes a difference

NF HERO: PHILIP MOSS



BEFORE

AFTER

50%
shrinkage

NF HERO: PAIGE DOANE



BEFORE

AFTER

32%
shrinkage

NF HERO: RYKER BENNETT



BEFORE

AFTER

40%
shrinkage

NF HERO: JANE CONSTABLE



BEFORE

AFTER

30%
shrinkage

Built for NF, Ready for Rare D

Synodos: Team Science

All experts together instead of in silos

Designed and managed by CTF, a diverse team of experts collaborate, and immediately share all raw data in an NF data hub, so as to increase the efficiency of solving complex NF problems.

Key Opinion Leader Network: Involving the Experts

Specialists who help guide drug discovery and development

An expert network for increasing scientific and clinical quality in decision-making.

Rare diseases/disorders are defined as affecting fewer than 200,000 people in the United States. Within the Orphan Drug Act, rare disorders can benefit from some regulatory and exclusive marketing benefits.

NF Clinic Network: Connecting Doctors to Improve Care

Cultivating relationships between patients and doctors.

A growing network of CTF-affiliated clinics that standardize and improve NF patient care.

Disease: The CTF R&D Sandbox

REINS (Response Evaluation in NF and Schwannomatosis) Consortium:

Approval Criteria

Defining Success for the FDA

A worldwide consortium that develops new clinical trial designs, better endpoints, and consensus measures.

NF Data Hub: Open Data for All

Ensuring data is available and ready to use

A centralized data repository managed by specialists who collect, analyze, and release integrated data, to accelerate the understanding of NF and the identification of "druggable" targets.

NF Preclinical Initiative: Novel Targets for Clinical Trials

Early testing of innovative concepts

A team of top laboratories with NF-relevant animal models, bringing promising drug treatments to the clinic quickly and efficiently.

NF Biobank: To Provide Tissue for Research

Removing the scarcity of relevant tissue roadblock

A centralized library of openly available samples for biomarker discovery and development, to support all aspects of drug research.

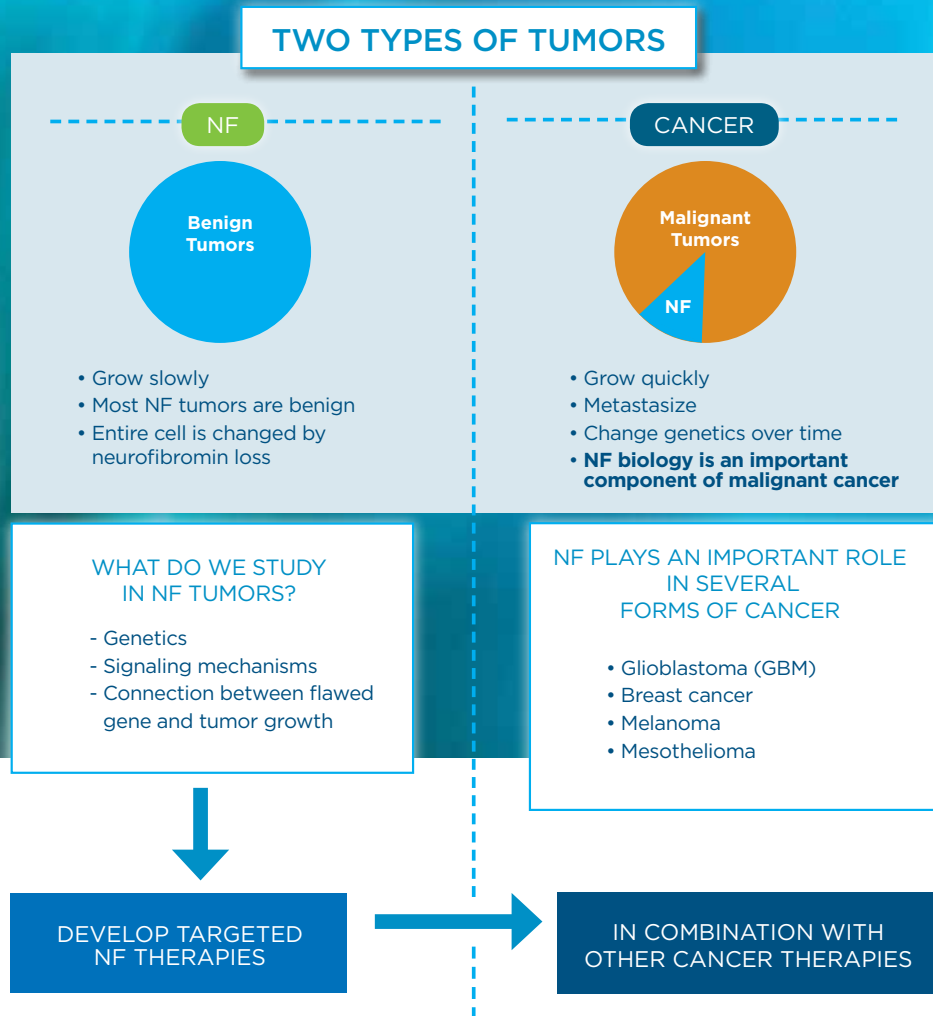
**The CTF Model
is Expandable to
All Rare Diseases**

Patient Registry: Because the Patients are our Partners

Patient data fuels knowledge of all NF manifestations

A patient-entered registry structured to accelerate clinical trial recruitment, and a better understanding of the diversity of NF manifestations.

Why investing in NF can help



The Children's Tumor Foundation's business model is a new approach in several areas, as well as for

Children's Tumor Foundation Innovation:

Rare benign tumor conditions offer safe effective combination therapies to cancer patients

In malignant cancer, the growth and metastasis of the tumors are driven by many different biological pathways and show genetic instability. That means that a pathway that drives the growth of a tumor today may not be the same as the growth of the tumor tomorrow. In essence, they adapt. In such a complex biological system, one immediately understands that cancer can never be "cured" when targeting only one molecular mechanism. That is why combination treatments are needed—to hit different mechanisms at the same time, or one right after the other.

In recent times, treatments have gotten better at specifically hitting certain mechanisms. As recently as the

1970's, cancer patients were treated with heavily toxic treatments that would only kill cells that were dividing. Today there are "targeted therapies" that specifically aim at certain mechanisms. However, these currently used drugs can also affect "off target" mechanisms, and/or cause unacceptable side effects.

Rare diseases such as NF, Familial Adenomatous Polyposis (FAP), Gorlin, and others are monogenic and genetically stable. These pre-malignant conditions are therefore uniquely positioned to develop safe, precise, and efficacious treatments that specifically affect one of the pathways that also drive malignant cancers. Expanding

cure cancer

or Foundation's innovative
prototype for other rare disease
cancer.

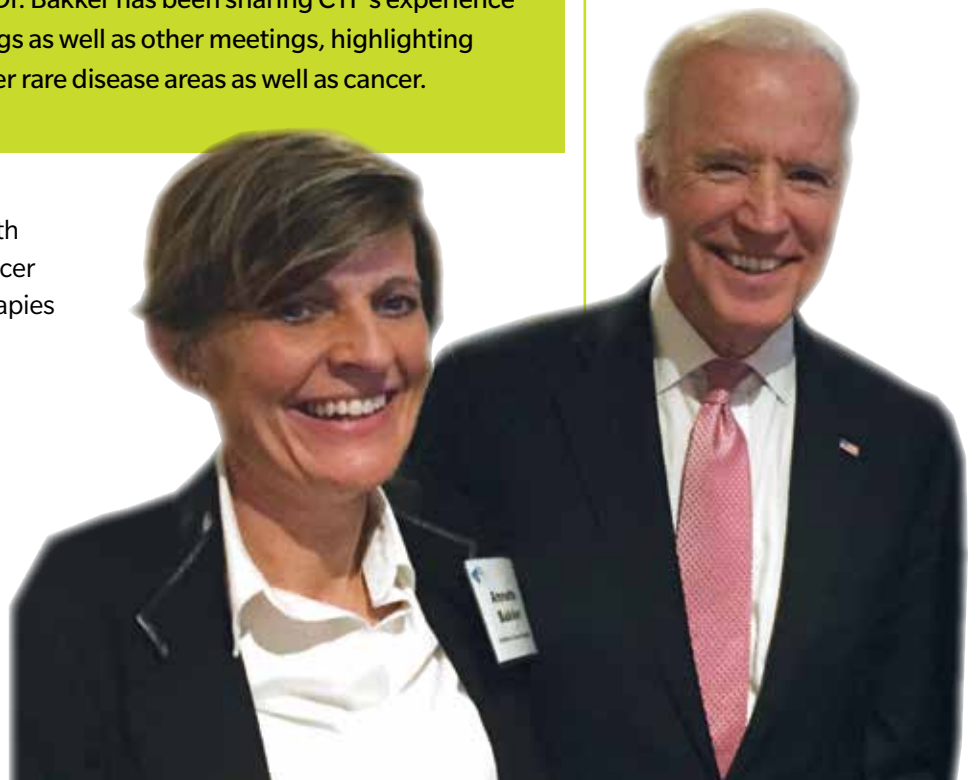
Cancer Moonshot

At the invitation of former Vice President Joseph Biden, Children's Tumor Foundation President and Chief Scientific Officer Annette Bakker, PhD, attended the Cancer Moonshot Summit in Washington, DC. This meeting of esteemed scientific leaders was dedicated to shortening the length of time it takes to develop effective treatments for cancer in particular, and disease in general. The first-of-its-kind summit was focused on promoting collaboration among researchers, industry, and patients, and to breaking down the barriers that hamper progress. CTF's collaborative research model is a successful example of this approach, and Dr. Bakker has been sharing CTF's experience at subsequent Cancer Moonshot gatherings as well as other meetings, highlighting CTF's business model as applicable to other rare disease areas as well as cancer.

this approach to other monogenic disorders with pathways that are deregulated in malignant cancer will allow us to finally develop combination therapies that are effective and safe.

The Opportunity: Offer safe effective combination therapies to cancer patients

by developing effective, tolerable drugs that massively shrink NF tumors. At the same time, identify other rare benign tumor conditions that are monogenic (e.g., NF, Gorlin, FAP) and combine safe effective drugs for those conditions.

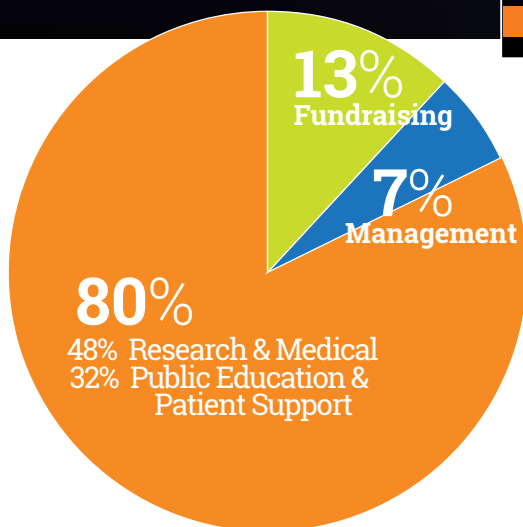


About NF and the Children's

The Children's Tumor Foundation is a highly recognized 501(c)(3) not-for-profit organization dedicated to finding effective treatments for the millions of people worldwide living with neurofibromatosis (NF), a family of 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 as the leading force in the fight to end NF and as a model of innovative research endeavors, the Children's Tumor Foundation's mission of driving research, expanding knowledge, and advancing care for the NF community fosters our vision of one day ending NF.

Revenue and Expenses			
	2016	2015	2014
TOTAL OPERATING REVENUE	\$15,099,970	\$15,041,425	\$15,727,791
TOTAL OPERATING EXPENSES	\$14,837,607	\$15,024,721	\$14,529,405
Change in Net Assets from Operations	\$262,363	\$16,704	\$1,198,386
NON-OPERATING REVENUE	\$304,524	\$73,229	\$327,816
Change in Net Assets	\$566,887	\$89,933	\$1,526,203
NET ASSETS, END OF YEAR	\$11,005,342	\$10,438,455	\$10,348,522

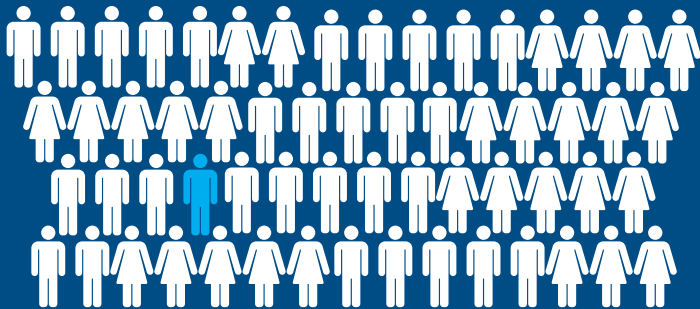


"Only 2% of the charities we evaluate have received at least 8 consecutive 4-star evaluations, indicating that Children's Tumor Foundation outperforms most other charities in America. This exceptional designation from Charity Navigator sets Children's Tumor Foundation apart from its peers and demonstrates to the public its trustworthiness."

Michael Thatcher, Charity Navigator President and CEO



Tumor Foundation



1 IN 3,000
BIRTHS HAS **NF**

THAT'S
2,500,000
WORLDWIDE



NF IS A GENETIC DISORDER
that causes tumors to grow on the nerves

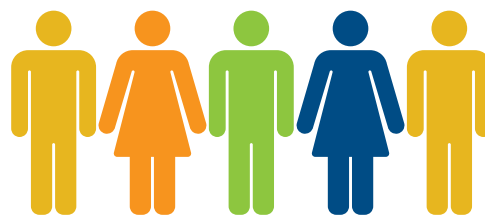
NF affects all
populations,
genders &
ethnicities
equally

1

2

3

There are 3 types of NF:
NF1, NF2, AND SCHWANNOMATOSIS



YOU CAN'T CATCH NF – **YOU'RE BORN WITH IT.**

NF AFFECTS EVERYONE
DIFFERENTLY

The tumors are usually benign, but may lead to cancer and other health issues such as hearing loss, vision loss, bone abnormalities, disfigurement, and extreme pain.

THERE IS NO CURE...**YET**

Join the Children's Tumor Foun

An opportunity to
change lives by
building a future of
effective treatments
for disease.



dation

When you partner with the Children's Tumor Foundation, you have the opportunity to:

- **Provide** strategic help to advance the fight against NF
- **Help** ensure the financial strength of CTF
- **Act** as a connector to build important relationships between CTF and prospective donors, partners, or other potential friends of CTF
- **Work** closely with other CTF friends, including donors, volunteers, the CTF Board of Directors, as well as the CTF staff so as to achieve CTF's mission and vision.



BE A CATALYST OF CHANGE

We need partners like you.

Ground breakers. Business leaders.
Game changers. Thought leaders.
Investors. Influencers. Idea makers.

The Children's Tumor Foundation is leading the way to new discoveries, and you can be a part of it.



OUR MISSION: Drive research, expand knowledge, and advance care for the NF community.

OUR VISION: End NF.

To learn more, please contact:

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**CHILDREN'S
TUMOR
FOUNDATION**
ENDING NF
THROUGH RESEARCH