

Children's Tumor Foundation



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Mission

The Children's Tumor Foundation (CTF) is a nonprofit medical foundation dedicated to improving the health and well-being of individuals and families affected by neurofibromatosis (NF).

Strategy/Approach

The Children's Tumor Foundation achieves its mission by:

- **Encouraging and supporting research** leading to improved treatments for neurofibromatosis type 1 (NF1) and neurofibromatosis type 2 (NF2), schwannomatosis, and associated medical manifestations
- **Supporting persons with NF**, their families, and caregivers by providing thorough, accurate, current, and readily accessible information.
- **Assisting in the development of clinical centers, best practices, and other patient support mechanisms** (but not including direct medical care) to create better access to quality healthcare for affected individuals.
- **Expanding public awareness of NF** to promote earlier and accurate diagnoses by the medical community, increase the non-affected population's understanding of the challenges facing persons with NF, and encourage financial and other forms of support from public and private sources.

The Foundation's Strategic Plan accelerates NF research by balancing its funding and partnering initiatives with academia, industry, patients, other disease organizations and government towards ending neurofibromatosis through the development of effective therapies. Progress has been made towards this goal through the launch of Foundation strategic initiatives to fill those gaps that hamper the progression from discovery to clinical benefit. The new initiatives include the patient registry, Biobank, preclinical trial consortia, molecular pathway analysis service as well as a compound scouting and negotiation service.

The Foundation's Neurofibromatosis Clinical Network (NFCN) is the first nationwide network dedicated to improving clinical care and establishing best practices for treating those living with NF. The NFCN recognizes clinics that provide NF care through the implementation of current consensus clinical care guidelines for the NF population. CTF invites clinics in the United States that see patients affected with NF to apply for Affiliate Clinic status in the NFCN. By 2014, the foundation devoted over \$1M to building what has become an over 40 member network.

Research Portfolio

The Children's Tumor Foundation is the largest non-government source of NF research funding. We regularly evaluate our programs for effectiveness, and seek to fill gaps that exist in research from other larger funding sources, primarily the National Institutes of Health (NIH) and the Congressionally Directed Medical Research Program-Neurofibromatosis Research Program (CDMRP-NFRP).

The Foundation provides funding through a variety of mechanisms.

Investigator-initiated funding through the following grant mechanisms and initiatives:

- **Young Investigator Award (YIA)**—The YIAs are the Foundation's longest running funding mechanism and a cornerstone of its research portfolio, providing support for pre- and postdoctoral scientists and physicians pursuing careers in NF research. Applications investigating novel ideas in basic, translational and clinical research in all forms of NF and its complications are encouraged. The YIA offers substantial funding over a two-year period.
 - \$32,000 - \$54,000/year over two years (dependent on stage of training)
- **Drug Discovery Initiative (DDI)**—The DDI is designed to fund early stage, high-risk / high-reward research that with relatively modest amounts of funding will provide results that help researchers winnow the large number of potential compounds into those that demonstrate the best potential for treating NF. It is a condition of funding that researchers make their tools (candidate drugs, animal models, cell lines) publicly available for collaboration with other researchers. The DDI funds at three levels:
 - *In vitro (cell line) screens: \$25,000 to fund cell-based preclinical screening*
 - *In vivo (mouse model) screens: \$50,000 to fund animal-based preclinical testing*
 - *Advanced grants will be given to the applicants that can result in the delivery of compelling (typically in vivo) data requiring (up to \$75,000)*
- **Clinical Research Awards (CRA)**—The CRA program funds pilot clinical trials that are expected to generate preliminary data that will lead to further trials of promising compounds. Calls for letters of intent are issued annually, and up to three awards per year are typically granted.

- \$150,000
- **Schwannomatosis Awards**—These are awarded in an effort to accelerate research in unravelling the molecular basis of schwannomatosis, and move toward identifying candidate drug targets and therapeutics for this disorder. These awards are issued upon approval.

Consortia:

- **NF Preclinical Consortium (NFPC)**—The NFPC performed preclinical testing of existing drugs and small molecule compounds, with the goal to build a pipeline of drugs for clinical trials. The Consortium has collaborated to perform extensive preclinical work and has performed 52 preclinical studies across different models that have led to ten clinical trials. This \$5 million dollar investment in NF1 preclinical testing completed its work in June 2013 and the NF2 NFPC ended in December. Since the Foundation is convinced that this critical effort must go on, and since a thorough SWOT analysis showed that NF1, NF2 and Schwannomatosis need a different approach, the following initiatives have been launched:
 - *Neurofibromatosis Therapeutics Consortium for NF1: A partnership for 3 years (2013-2015) between Children's Tumor Foundation and NTAP (NF-Therapeutics Acceleration Program), continues funding the NF1 preclinical studies.*
 - *The first Synodos consortium for NF2: a \$3 million Children's Tumor Foundation 12 academic center collaboration aimed at bringing new breakthrough treatments to the patients that suffer from NF2. An innovative approach that includes NF2 clinicians, translational scientists, basic researchers and Sage Bionetworks managing the centralized data sharing platform.*
 - *Schwannomatosis. The analysis to address the challenges seen in the Schwannomatosis field is currently ongoing. A Schwannomatosis-specific initiative will be announced soon.*

Partnership Practices

The Children's Tumor Foundation has a long history of promoting collaboration among scientists and clinicians. **In addition the Foundation actively works to build relationships with biotech and pharmaceutical companies to attract further interest in funding treatments and a cure for NF.** Strategically the Foundation is building a business model for NF that accelerates the R&D process significantly.

In 2012, CTF launched the NF Registry (nregistry.org) in order to identify candidates who may be eligible for clinical trials or others research studies being conducted in the field of neurofibromatosis, and to determine the commonality of specific NF characteristics. This comprehensive information from the NF community will assist researchers in better

understanding the different forms NF can take, and importantly, encourage pharma and biotech to invest in NF research.

In a collaborative partnership with **Thompson Reuters**, CTF is offering an extensive panel of services to the NF community. The Foundation also utilizes Thomson Reuters MetaCore platform to support data visualization and enhance the understanding of NF through the development of disease-specific pathways; Thomson Reuters Integrity is used to scout for new compounds and customized Disease Funding Schema are created to offer a better understanding of how its funding impacts research.

Discussions initiated at the Partnering for Cures meeting in 2013 resulted in a very fruitful partnership between the **Alzheimer's Drug Discovery Foundation (ADDF)** and the Children's Tumor Foundation (CTF). The partnership provides CTF's scientific network access to a virtual network of drug discovery experts and contract research organizations (CROs) through the ADDF ACCESS program.

Sage Bionetworks and the Foundation work together to manage the data platform of the Synodos consortia.

Financials

The Children's Tumor Foundation is a 501(c)3 tax-exempt non-profit organization.

The most recent financial information is available from the Foundation's 2013 Annual Report:

- Year ending 12/31/13
- Revenue: \$12,541,283
- Assets: \$ 10,987,913
- Expenditures: \$ 12,022,038

Key Accomplishments

- The Foundation has established the only worldwide NF scientific conference, which continues to grow every year (from a handful to now over 300 participants).
- Since 2012, the Foundation has made the necessary strategic investments to accelerate the path from discovery to clinical benefit.
- The Foundation is participating actively in growing the interest of pharma and biotech in NF by developing innovative business models, creative policies, and economic market models. All agreements are professionally negotiated on a one-by-one basis by CTF personnel.
- The NF Clinic Network is the first nationwide network dedicated to improving clinical care and establishing best practices for treating those living with NF. The NFCN treats over 10,000 patients per year.

Leadership

The foundation is governed by a **Board of Directors** and managed by a New York-based **leadership staff**. Its scientific and clinical grant awards and programs are overseen by a

Medical Advisory Committee, a Clinical Care Advisory Board, and a Research Advisory Board.

- **Chairman of the Board of Directors:** Stuart Match Suna
- **President & Chief Scientific Officer:** Annette Bakker, PhD abakker@ctf.org
- **Chief Financial Officer:** Judith Swartout, jswartout@ctf.org