CHILDREN’S TUMOR FOUNDATION

Your Partner and Innovation Catalyst in Neurofibromatosis and Schwannomatosis

ctf.org
Neurofibromatosis/Schwannomatosis (NF): Family of Progressive Debilitating Genetic Conditions

**Neurofibromatosis type 1 (NF1)** – tumors grow on nerves, leading to disfigurement, blindness, pain and cancer. NF1 patients may also suffer from cognitive and behavioral challenges and bone manifestations. Est. Incidence: 1 in 2,500. High unmet medical need. Many manifestations

**One approved treatment.**

**Schwannomatosis (SWN)** causes tumors on nerves throughout the body causing excruciating pain. Est Incidence: 1 in 70,000.

**No Approved Treatment.**

**NF2-related schwannomatosis (NF2-SWN)** causes tumors to grow on the hearing nerves, in the spine and in the brain, that can lead to hearing loss, difficulty with balance, tinnitus, mobility issues and death. NF2-SWN also causes painful neuropathies. Est Incidence: 1 in 25,000

**No Approved Treatment.**
Our Mission:
Drive research, expand knowledge, and advance care for the NF community.

Our Vision:
End NF!
CTF Achievements

- Significant increase in number of druggable targets & tripled the number of clinical trials in NF
- Ten companies working on drugs for NF – two dedicated to NF.
- Unique global infrastructure including NF clinic network, NF Registry, BRIDGE.
- First-ever drug approved for NF.
- Co-fund first-ever platform trial with Takeda. One master protocol, multiple indications, multiple treatments
THE IMPACT OF CTF’S LEADERSHIP

CTF funded the critical research for Koselugo’s approval!

Philip Moss
2015
Unable to turn his head, in danger of losing his ability to swallow or talk.

Philip Moss
Today
60% tumor shrinkage, graduating HS and preparing for college.
Preclinical Hub Coupled to Platform—Basket Trials

VISION 2028: Both Self-Sustainable

Accelerate Clinical Trial—Ready Treatments

Increase Efficiency of Clinical Trials

Multiple Drugs/ Evaluated in Multiple Manifestations
IN-VITRO TESTING
Assess which drugs (of many) have a beneficial effect on cells.

BIOMARKERS
Evaluated to help define disease state or a drug's therapeutic effect.

IN-VIVO TESTING
Provides insights into the effects of a set of drugs in a whole, living organism

DATA -> Data Portal
Provides the knowledge to ultimately inform the development of novel therapeutics

PK/PD -> Pig models
Focuses on the effect of the body on a drug (PK) and the drug on the body (PD)

DRUG LIBRARIES
Enable rapid testing of compounds with demonstrated safety profiles in new indications
PLATFORM—BASKET TRIAL FOR ALL NF
In Partnership with GCAR*
Running Trials with Regulatory Intent
Working with all Key Stakeholders from the Start

The leading experts in managing drug-related platforms and processes.

The global leader in neurofibromatosis/schwannomatosis research.

Leadership
Vendor Mgmt.
Site Mgmt.
Advocacy
Regulations
CRO-Support

* Track record in building self-sustainable platform trials

GCAR
Global Coalition for Adaptive Research

EU-PEARL
EU PATIENT-CENTRIC CLINICAL TRIAL PLATFORMS

The funder of the clinical trial design
Partner, Connector, Advocate & Investor
CTF AS A PARTNER

Diagnose
- **Dx Criteria**
  - Connect to Experts
- **Clinical care guidelines**
  - Educate & Raise Awareness
  - Expand NF Registry

Quality Care
- **Discovery**
  - Bench to Bedside
  - Multi-disciplinary Consortia

Research
- **Accelerate Developable Treatments**
  - Focus on Pharma Design and Run Preclinical Hub & Target Scan

Advance Clinical Trials
- **Clinical Trial Infrastructure**
  - Design/ implement Platform Trials
BRINGING TREATMENTS TO NF PATIENTS

- **AstraZeneca**: CTF preclinical funding identified MEK as effective for NF, which later led to FDA approval of Koselugo.
- **Takeda**: CTF’s $2MM Synodos initiative identified Brigatinib for NF2 and inspired the launch of INTUITT-NF2.
- **SpringWorks Therapeutics**: CTF played an instrumental role in the repurposing of Pfizer’s shelved drug mirdametinib, now in Phase 2b.
- **NFlection Therapeutics**: CTF’s investment in NFlection Therapeutics accelerated the development of a topical MEK treatment, now in Phase 2b.
- **Children’s Hospital of Philadelphia**: CTF and CHOP are partnering on a long term natural history study looking at the standard of care for patients with optic pathway glioma (OPG).
Partner with Us!

info@ctf.org