CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

CHILDREN'S TUMOR FOUNDATION

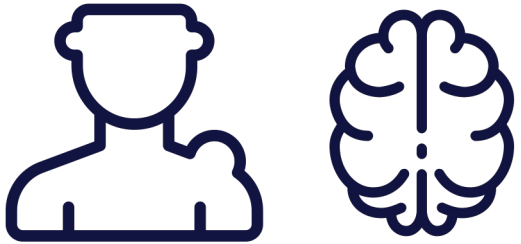
Your Partner and Innovation Catalyst in Neurofibromatosis and Schwannomatosis

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.



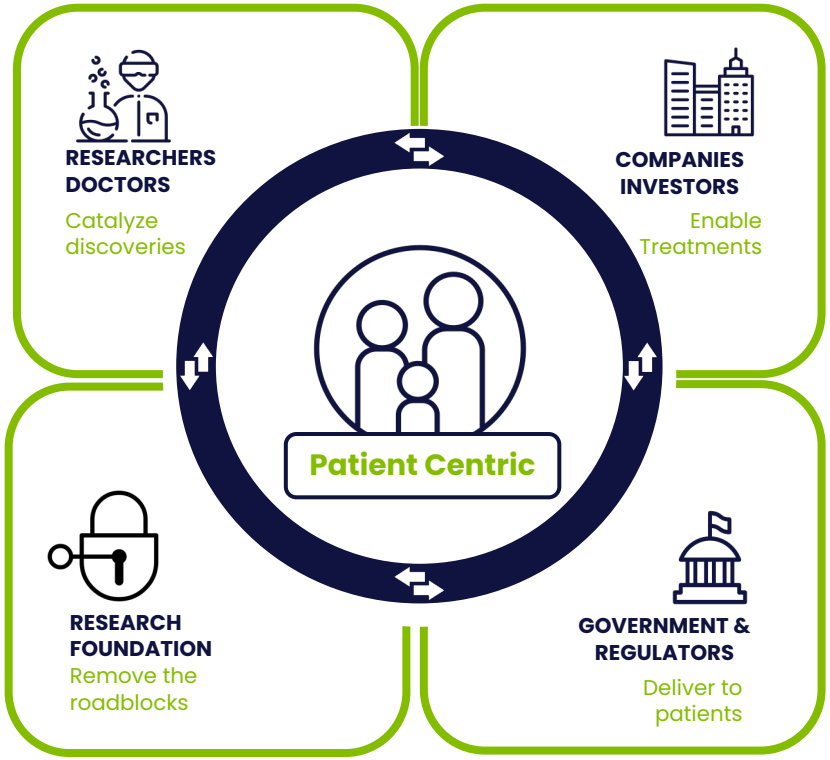
Schwannomatosis (SWN) causes tumors on nerves throughout the body causing excruciating pain. Est Incidence: 1 in 70,000.

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.



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EUROPE

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!

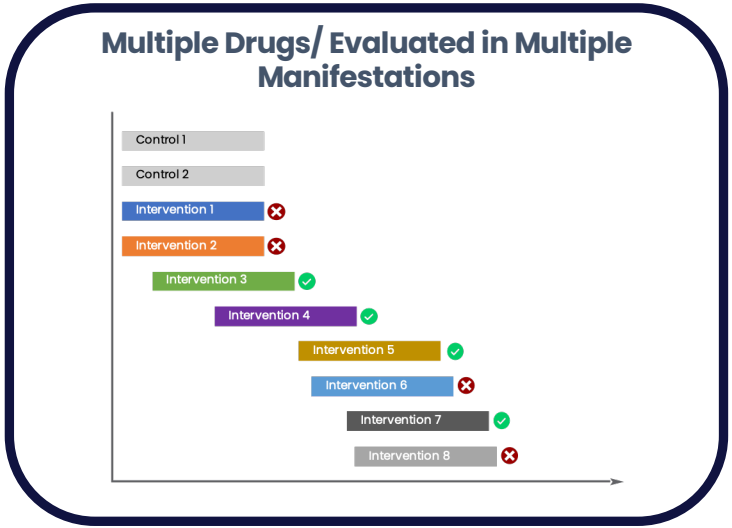
Philip Moss
2015
Unable to turn his head, in danger of losing his ability to swallow or talk.

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

PRECLINICAL HUB

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

GCAR
Global Coalition for Adaptive Research

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

The global leader in neurofibromatosis/ schwannomatosis research.

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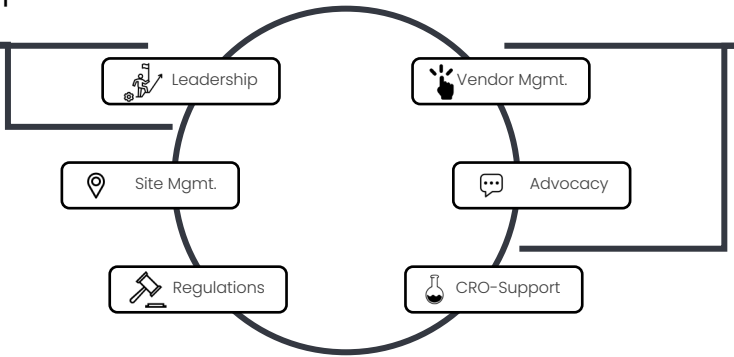

EU-PEARL
EU PATIENT-CENTRIC CLINICAL TRIAL PLATFORMS

The funder of the clinical trial design

* Track record in building self-sustainable platform trials

CHILDREN'S TUMOR FOUNDATION

ENDING NF THROUGH RESEARCH

Partner, Connector, Advocate & Investor

CTF AS A PARTNER

Diagnose

Dx Criteria
Connect to Experts

Quality Care

Educate & Raise Awareness
Clinical care guidelines
Expand NF Registry

Research

Discovery
Bench to Bedside
Multi-disciplinary Consortia

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