

Children's Tumor Foundation Europe – Strategy Summary

Background

The Children's Tumor Foundation (CTF), as the largest non-governmental funder of all forms of NF in the world, is committed to supporting the best and most promising research globally, independent of where the research is taking place. As such, in its history, even as a U.S.-based organization, CTF has funded approximately \$2.5 million in NF research conducted by researchers in Europe.

In order to further expand the NF research community globally, the Foundation's U.S. Board of Directors endorsed the establishment of a European-based organization, called Children's Tumor Foundation Europe. CTF Europe is entirely focused on funding and supporting transnational research and clinical programs, as well as accelerating the path from new findings to improved treatments for NF patients.

CTF Europe was launched as a public foundation (with Royal Belgian decree) in November 2018 and obtained approval from the King Baudouin Foundation. This enables CTF Europe to receive donations in Europe through the Transnational Giving Europe network.

Since its founding, CTF Europe has established itself within European networks, and has met with key stakeholders in Europe, including executives from the European Federation of Pharmaceutical Industries and Associations (EFPIA), leaders of the European Commission, European Patient Forum, and the European Organization for Rare Disease (Eurordis), which has enabled CTF to:

- Participate as panelist at the Innovative Medicine Initiative (IMI) stakeholder meeting.
- Become an associated partner of the IMI.
- Become the Work Package Leader for NF in the IMI call for the development of Integrated Research Platforms for 4 diseases, including NF.
- Participate in a trans-European patient engagement initiative, called IMI Paradigm.
- Become a member of the IMI Strategic Governing Group Oncology in Europe.

Overall Long Term Goals of CTF Europe

1. **Build** an NF funding landscape in Europe that is complementary to the one in the United States, by leveraging unique opportunities exclusive to Europe, including:
 - a. Universal health care system – which makes it easier to do natural history studies and population studies
 - b. Unique public-private partnerships in Europe – for example, the above mentioned IMI (Innovative Medicines Initiative) and the EFPIA (European Federation of Pharmaceutical Industries and Associations)
2. **Advocate** for European funds directed towards NF research
3. **Expand** global data sharing efforts
4. **Serve** as a partner to European Pharma companies to enable/support their bringing drugs to patients

Our Principles – How We Will Approach Our Goals

- We aim to raise **new** money for NF research – we do not want to compete with the current European patient associations.
- We aim to collaborate as much as possible with local foundations when there are synergies and alignment of goals; we can play a key role in enabling and catalyzing research because of the connectivity/work we do with top NF researchers.
- We will remain as lean as possible – we will work hand in hand with CTF US in a pragmatic way, and always with the top priority of funding/driving research in mind. To this extent, we will hire staff members only if we attain at some point in the future a substantial base requiring local administration.

Short/Mid/Long Term Milestones

Short term (2019-2020)	Medium term (2021-2024)	Long term (>2025 or as soon as >1M €/ year income)
<ul style="list-style-type: none"> - Fine tune strategy and research/clinical priorities via a workshop with Key Opinion leaders (Academia, Associations, Pharma, regulators) - Work with Horizon Europe (leading EU research program) - Leverage existing CTF donors who reside in the EU - Raise new money - Launch Discovery fund (After having raised approx. 250K €) 	<ul style="list-style-type: none"> - Conduct an EU-specific 'unmet needs assessment' - Build a stable donor base of <u>new</u> money, with a minimum of 3 donors at 1M € to fund promising research 	<p>Expand CTF Europe as a staffed trans-European operation that funds new discoveries and accelerates the translation of those discoveries into better treatments for NF patients.</p>
<p>Participate in IMI Raise NF awareness in Europe Build relationships with pharma & biotech in Europe</p>		