



**Including families in protocol design, protocol simulation,  
identification of meaningful clinical aspects, with patients**

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# The Experience of the Children's Tumor Foundation

## **GOAL**

To accelerate the drug development process for neurofibromatosis (NF) by empowering patients and catalyzing their involvement in the research process.

## **Context/Vision**

....built for NF, designed for rare diseases and R&D stakeholders

# Background

1. Created a culture of **patient centrality** across Children's Tumor Foundation and with external collaborators
2. Established a Patient Training program that equipped and **empowered patients** to participate in R&D
3. Became a **connector** for patients between researchers, clinicians, industry and regulators

# Top 5 wins / Last 3 years



Established a Patient Engagement Training Program



Held the first [FDA Listening Session for Neurofibromatosis \(NF\)](#)



Patients are active members of Pharma Advisory Board. Now the first FDA approved drug for NF1 plexiform in pediatric patients (Koselugo, selumetinib)



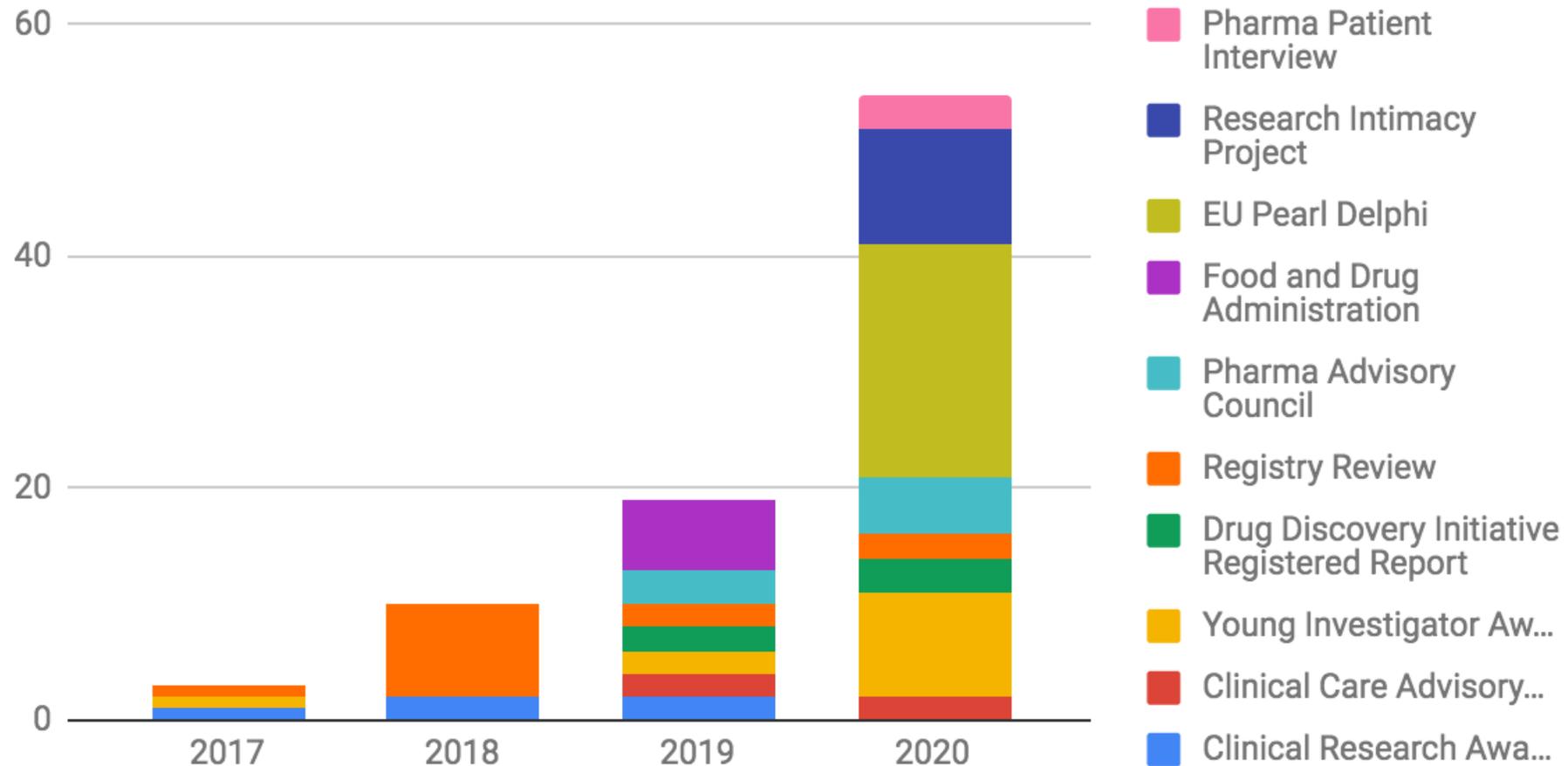
Patients are evaluators of CTF grant applications alongside scientific experts



Involvement in global projects and working groups developing tools for assessing PE practices in the design of clinical trials, developing standardized endpoints for clinical trials and developing metrics for evaluating PE activities ...Paradigm, REiNS, EU-PEARL

# Patient Involvement 2017-2020

2017, 2018, 2019 and 2020



Increase in # Patient Advocates Participating in CTF Projects

# Early engagement with patients can change the course of a study for the better

- Why sponsors maybe hesitant to include patients in research
  - the risk of throwing everything off by learning something counter to what the trial is trying to accomplish
- Examples or how patient advocacy group can help facilitate patient involvement in the research process- FDA, EU Pearl – clinical trial design Delphi, REINS (endpoint development)

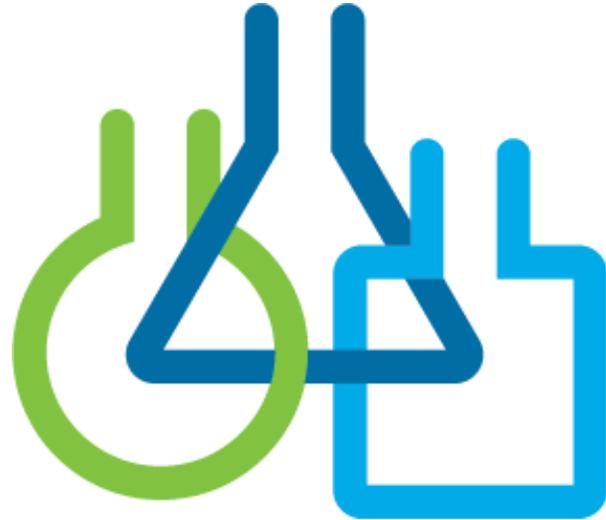
# Lessons from the CTF experience for including patients in protocol design

- Include the patients voice early before the protocol is designed gets it right from the start
- By listening to patients and caregivers, sponsors gain insights they'd find no other way.
- Involving patients is helpful in understanding their Lexicon - you can find out what words resonate with patients and caregivers. Helps sponsors show solidarity with the community that they are going to be involved with for months or possibly years.”
- Gain better understanding who the patients are
- Including patients makes them a partners in research not just the subjects

# Lessons from the CTF experience for including patients in protocol design

- Develop studies with patient perspectives and experiences in mind
- Define endpoints that are relevant to the patient population
- Collaborate effectively with regulatory and legislative groups to advance funding and/or treatments of rare diseases
- Improve recruitment for research studies
- Increase awareness and knowledge of the disease by learning about the patient journey
- Keep costs down by eliminating expensive and time-consuming changes
- Boost retention by improving the trial experience for patients.
- Creative and compliant engagement programs built around the patient experience can make these benefits attainable.

# Thank You!



Learn more about the Children's Tumor Foundation Program  
Patient Engagement Program at  
[www.ctf.org/patientengagement](http://www.ctf.org/patientengagement) or email [trose@ctf.org](mailto:trose@ctf.org)