TOP TEN ADVANCEMENTS IN NF RESEARCH 2021



Masterclasses in NF (INFER), bilingual NF Conference / Investigators Forum, Virtual Case Conferences.

(OPG) multi-year natural history study expands to further understand tumor impact on vision loss,

NF2 Accelerator fully funded at \$2.3M, expanding NF2 drug pipeline, bringing treatments to patients faster, plus gene therapies for better options, helping NF2 patients today and tomorrow.

DRIVING PESE

CTF's NF Clinic Network continues to grow, increasing accessibility to more than 15,000 patients each year. There are now 67 clinics, with the first NFCN clinic recognized in Canada.

NF Registry has over 10,000 participants, and is a critical resource to researchers for all forms of NF. Now also available in multiple languages to gain insights across diverse populations.

> CTF brings NF experts together to align on clinical trial design and criteria (EU-PEARL), for more effective global platform trials for all forms of NF, that will bring treatments to NF patients faster.

WHAP WOING KNOWLEDGE

PUANCING CARE **Expanded resources for**

patients transitioning from pediatric to adult care, including publications from CTF and the NF Collective, plus adult NF clinics currently in development.

Increased number of pharma/ biotech firms investing in NF, with 10 companies testing drugs in clinical trials. CTF's R&D enabling platform attracts firms to enter the NF field.

Global collaborative of 90 NF experts update diagnostic criteria for NF1 and Legius Syndrome, improving accuracy, earlier diagnosis, and care for NF1. Updates for NF2 and schwannomatosis coming in 2022.

Following FDA approval of first-ever treatment for NF, Koselugo (selumetinib) now approved in 12 countries, including European Medicines **Agency. More countries** expected in 2022, and additional MEK inhibitors showing promise in trials.

At the Children's Tumor

Foundation, we're dedicated

to bettering the lives of the over 2.5 million people living with neurofibromatosis (NF). We want to end NF as fast as we can by connecting the unconnected, leading the way with a strategy that applies innovative and inventive approaches to scientific advancement and improved patient care.

This pioneering approach attracts the brightest minds in research and industry to NF, revamps systems so as to accelerate the pathway from discovery to treatment, and includes the voice of the patient every step of the way.

Read about the important things that happened in NF research in 2021, which are even now laying the groundwork for even greater scientific advancements in the near future.

Stay informed with NF research updates throughout the year at ctf.org/news.

ctf.org