

THE PATH FORWARD: TEN STEPS IN NF RESEARCH 2014

1. CTF INVESTMENTS LEAD TO KEY DISCOVERIES

NF2 drug targets validated due to CTF-initiated partnership. CTF data has informed over 70% of NF clinical drug trials. CTF fosters consensus: first standardized sample and clinical data collection for biobank.

2. SYNODOS FOR NF2 IN ACTION

This first-of-its-kind multidisciplinary collaboration of top scientists from leading institutions was initiated, sponsored, and managed by the Children's Tumor Foundation, to accelerate development of NF2 treatments.

3. NF REGISTRY IS DELIVERING

The number of registrants in the NF Registry has doubled in the last 15 months. With over 4,500 participants, the NF Registry is attracting increased interest in NF from pharmaceutical companies. To learn more, visit nfregistry.org

4. CTF IN ACTION FOR PATIENTS

Children's Tumor Foundation now supports 47 NF clinics nationwide to provide the best NF care, and our collaboration with REINS (Response Evaluation in Neurofibromatosis and Schwannomatosis) paves the pathway to FDA approval.

5. NF BIOBANK EXPANDS

The Foundation's NF Biobank includes the first-ever protocol in place to include post-mortem tissue, thereby increasing our understanding of NF in all its manifestations.

6. NF AWARENESS GROWS ACROSS ALL CHANNELS

National and community media coverage has resulted in billions of impressions in print, broadcast, and online media coverage. CTF receives coverage in highly influential journals. Active Foundation participation on influential panels and think tanks.

7. FIRST-IN-CLASS DATA SHARING PLATFORM

In partnership with Sage Bionetworks, the Children's Tumor Foundation launched the first ever data sharing platform, to provide information in real-time and improve understanding of research across NF.

8. FOUNDATION PARTNERSHIPS

Children's Tumor Foundation expanded its collaborations to accelerate the drug discovery process, as well as relationships with other NF patient organizations. We are all on the same team.

9. CTF STRATEGIC BUSINESS MODEL TRANSFORMS NF RESEARCH & DEVELOPMENT

The Foundation's detailed plan forges a path of support to every NF stakeholder: researchers, medical centers, bio-industry - and most importantly, NF patients.

10. LARGEST NF RESEARCH GATHERING EVER

The collaborative setting of the joint NF Forum and NF Conference brought doctors and patients together. A groundbreaking "Business Opportunities in NF" forum attracted significant attention.

2014 WAS A BIG YEAR IN THE FIGHT AGAINST NF. TOGETHER WITH YOU, 2015 WILL BE EVEN BIGGER.

Children's Tumor Foundation

FIVE THINGS TO LOOK FORWARD TO IN 2015

1. EXPANDED COLLABORATIONS

Launch of Synodos for NF1. With Synodos for NF2 under way, we are bringing that model to NF1. Additionally, launch of biggest OPG (optic pathway glioma) clinical consortium.

2. NEW MODELS OF PATIENT SUPPORT

CTF to deliver expanded psychosocial services through telemedicine.

3. FIRST TREND ANALYSIS OF NF REGISTRY DATA

New initiatives to further grow the NF Registry.

4. OMICS INITIATIVE

You'll hear a lot about 'omics', which is short for fields of study in biology such as genomics and proteomics. CTF will fully characterize all tissue in the NF Biobank, further increasing our knowledge of NF.

5. NEW ANNOUNCEMENTS OF CRITICAL RESEARCH DEVELOPMENTS

New collaborations across NF and across disease areas.