

Children's Tumor Foundation Leads the Way:

TEN STEPS FORWARD IN NF RESEARCH 2015

1

PROMISING RESULTS IN TUMOR SHRINKAGE

Over 50% of patients in the MEK inhibitor selumetinib clinical study saw a 20% or more reduction in their inoperable plexiform neurofibromas. Registration trial is ongoing.

2

PROGRESS IN THE PIPELINE

The NF Preclinical and Therapeutic Consortia (NFPC/NFTC) have succeeded in filling the clinical pipeline - 95 preclinical trials, leading to 16 ongoing clinical trials today.

3

SYNODOS LEADS TO INNOVATION

As Synodos for NF2 is delivering with its successful operational / data sharing model, the Synodos for NF1 teams are selected: two groups will develop accelerated compound prescreening models; another will work to discover molecular mechanisms that drive low grade glioma (LGG).

4

POWER OF PATIENT DATA

The NF Registry has grown to over 6,500 registrants, including information on many manifestations of NF, and has recruited patients for 22 different clinical trials. Register today: nregistry.org

5

NF BIOBANK FUELS KNOWLEDGE

In-depth analysis (genomics, proteomics) has been performed on 50 dermal neurofibromas in the NF Biobank - characterized by HudsonAlpha, and data analyzed by Sage Bionetworks

6

SETTING STANDARDS FOR THE FUTURE

Detailed protocols for all NF conditions have been established for body donations to NF research.

7

NETWORK OF NF CARE EXPANDS

The NF Clinic Network has grown to 50 CTF-affiliated clinics nationwide, which provide consistent best-practice approaches to NF patient care.

8

NFPC SUCCESS ATTRACTS FEDERAL FUNDING

The NF Preclinical Consortium generates major follow-on funding as Wade Clapp, MD of Indiana University receives \$12 million from the NIH to study childhood inherited cancers - with NF1 as the focus.

9

ANSWERING THE CALL

"Ask Kate" service gives patients and their caregivers easy access (online or telephone) with an NF expert. Send a note to info@ctf.org or toll-free 1-800-323-7938

10

FIRST-EVER CENTER FOR NF BIOMARKERS

Partnership announced with the National Biomarker Development Alliance (NBDA) to address critical research need for measurable biological indicators for NF.

THREE MAJOR INITIATIVES TO WATCH FOR IN 2016:

1. First results of data delivery from Synodos for NF2, NF Registry, NF2 cell screens, characterization of dermal fibromas, and news from the NF Conference.
2. NF Biomarker Center with NBDA formalized and established.
3. New clinical consortia launched (Optic Pathway Glioma).

CHILDREN'S TUMOR FOUNDATION EARNS SEVENTH CONSECUTIVE FOUR STAR RATING

In 2015, Children's Tumor Foundation received, for the 7th year in a row, a 4-star rating from Charity Navigator. According to their rating service, receiving four out of a possible four stars indicates that CTF adheres to good governance and other best practices, and consistently executes its mission in a fiscally responsible way. Only 2% of the charities Charity Navigator rates have received at least 7 consecutive 4-star evaluations, indicating that Children's Tumor Foundation outperforms most other charities in America.

