

TEN STEPS FORWARD IN NF RESEARCH 2018

1

Synodos Continues

to Deliver. This tremendous investment in collaborative open science is bringing results, quicker. This year alone Synodos teams identified a promising clinical candidate for NF2; animal models that replicate NF1 in pigs are generating new, applicable knowledge; optic glioma teams are identifying treatment opportunities; and pain mechanisms are being discovered to help schwannomatosis patients.

GLOBAL NF
CONFERENCE
2018 | PARIS



2
A very energetic and largest-ever **NF Conference** in NF history was held in Paris, with nearly 900 attendees from across numerous disciplines, demonstrating the growing significance of NF research, and setting the stage for the 2019 NF Conference in San Francisco.



3
The Foundation's considerable investment in **genotype/phenotype** studies generated results that improve the predictability of NF in patients by connecting specific gene mutations to their associated symptoms. Our goal is to replace "watch and wait" with "here's what you can do."

4
NF knows no boundaries, and as a result, **CTF went global**, launching its partner organization, Children's Tumor Foundation Europe. CTF has also been the NF lead with the European Innovative Medicines Initiative (IMI), and generated new relationships in China, so that all 2.5 million NF patients worldwide will benefit.



5
Each day we learn more about NF, and **Key Workshops** were convened by CTF to update NF diagnostic criteria - so doctors know what to look for when seeing patients. CTF also convened a major animal model workshop - from which patients will see better treatment options.

6
Innovation is in our DNA, and the **pharmaceutical industry** is noticing. Growing interactions with major pharma are bringing attention to NF, and proving NF's critical importance into other disease areas as well.



7
Patients are at the center of everything we do, and CTF generated the tools to increase 'patient power' through our first **Patient Engagement** program, in which 20 new patient advocates graduated - their leadership will increase the patient voice in research priorities.



8
We are all one team, and CTF is a catalyzer of both NF research and NF relationships, bringing both public and private NF funders and organizations together in successful **new partnerships** which will expand the landscape of NF research. We're driving research - for example, new cell lines - and also advancing care, with better-quality expertise for caregiver and patient support.

9
CTF leads the way with the creation of the NF Open Science Initiative and the **NF Data Portal** in partnership with NTAP and Sage, increasing the ability of researchers to access the most current NF knowledge and research results. Our Key Opinion Leader Network brings the best people together in an open source environment, so that we get to a cure for NF faster.



10
MEK is making more of a difference than ever before, as the MEK inhibitor selumetinib was given **Orphan Drug Designation** by both the Federal Drug Administration (FDA) and the European Medicines Agency (EMA). Patients are seeing reduced tumor sizes, and most importantly, their lives are improving dramatically.



Before



After

FIVE THINGS TO LOOK FORWARD TO IN 2019

1.

MEK it happen - we expect that MEK will be the first-ever drug approved by the FDA for NF.

2.

Synodos to launch an NF2 clinical trial, in partnership with major pharma.

3.

NF Hackathon (during the NF Conference) will bring together experts from in and out of NF in a fast-paced, energetic setting.

4.

Updated NF diagnostic criteria, improving how doctors identify and treat NF.

5.

CTF's gene therapy project is launched, cultivating cutting-edge expertise in NF.

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

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