



Research Foundation perspective: How nonprofits can be the changemakers.

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President of Children's Tumor Foundation

Chairperson of CTF Europe

NEUROFIBROMATOSIS

Family of three genetic disorders

NF1; NF2; Schwannomatosis

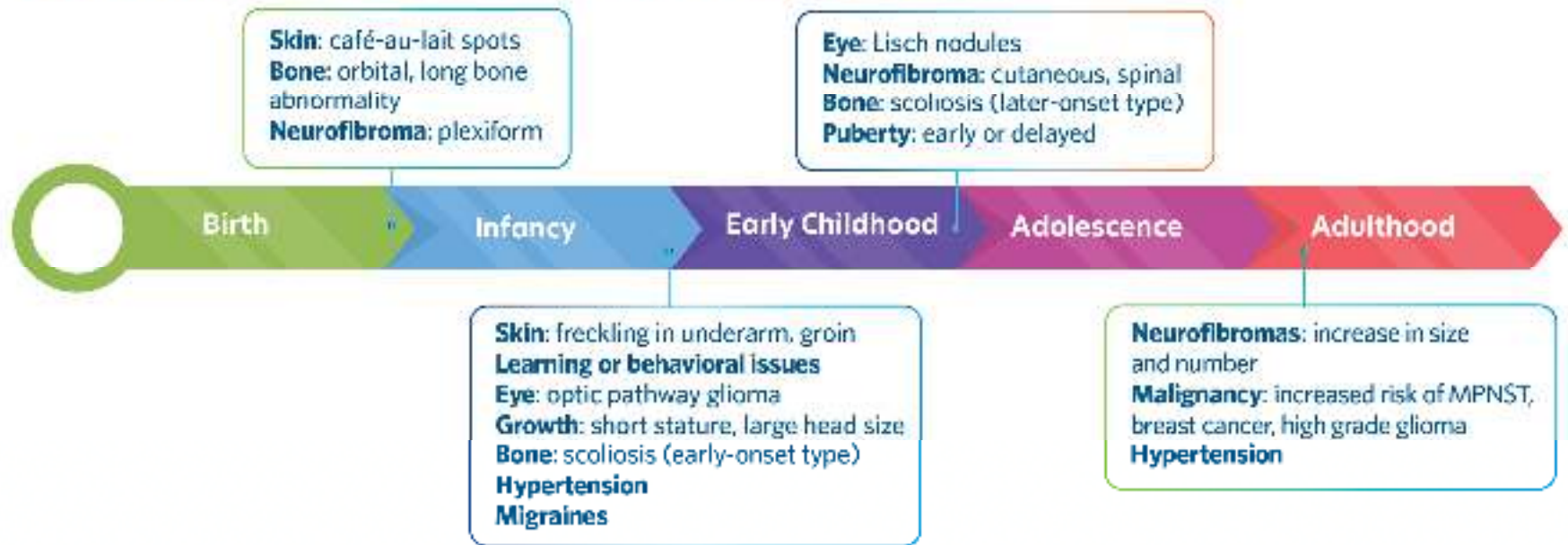
All three disorders:

- Are caused by loss of tumor suppressor genes, mainly leading to activation of oncology pathways
- May result in development of tumours
- Lead to significant morbidity
- Are progressive multisystem disorders



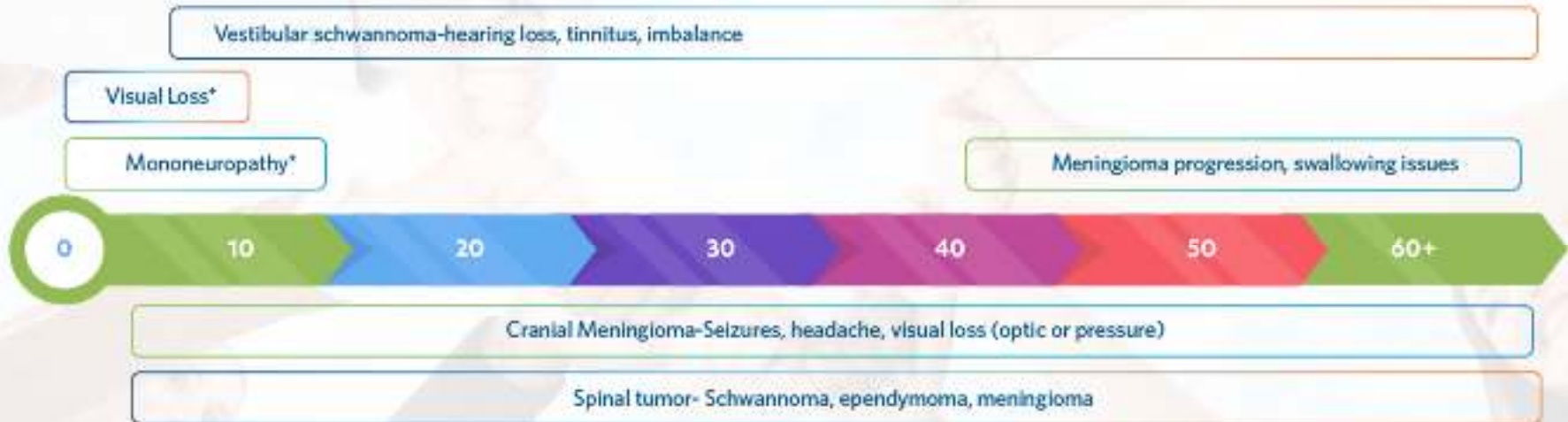
PROGRESSION OF NF1

Approximate timing of possible NF1 manifestations



PROGRESSION OF NF2

Approximate timing of possible NF2 manifestations



*Facial, 3rd nerve, foot drop, wrist drop
+ amblyopia, congenital cataract, hamartoma



Unpublished, Kindly provided by Dr. G. Evans

PROGRESSION OF SCHWANNOMATOSIS



Diagnosis

Surgeries

Excruciating Pain



NF1 is about real people



Julie

Ken

Aiden

Dan



Aiden



Yaniri



Sandy



Alex

Make NF Visible

ctf.org/makenfvisible



I KNOW A FIGHTER. CTF.ORG



#EndNF

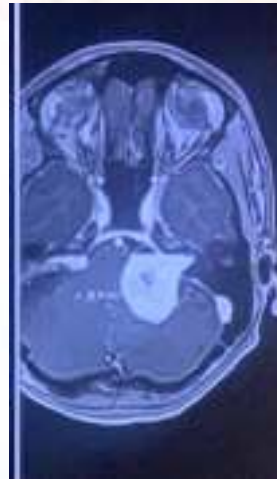
BORN A FIGHTER. CTF.ORG



THE NF2 & SCHWANNOMATOSIS PATIENTS: SUFFER IN SILENCE



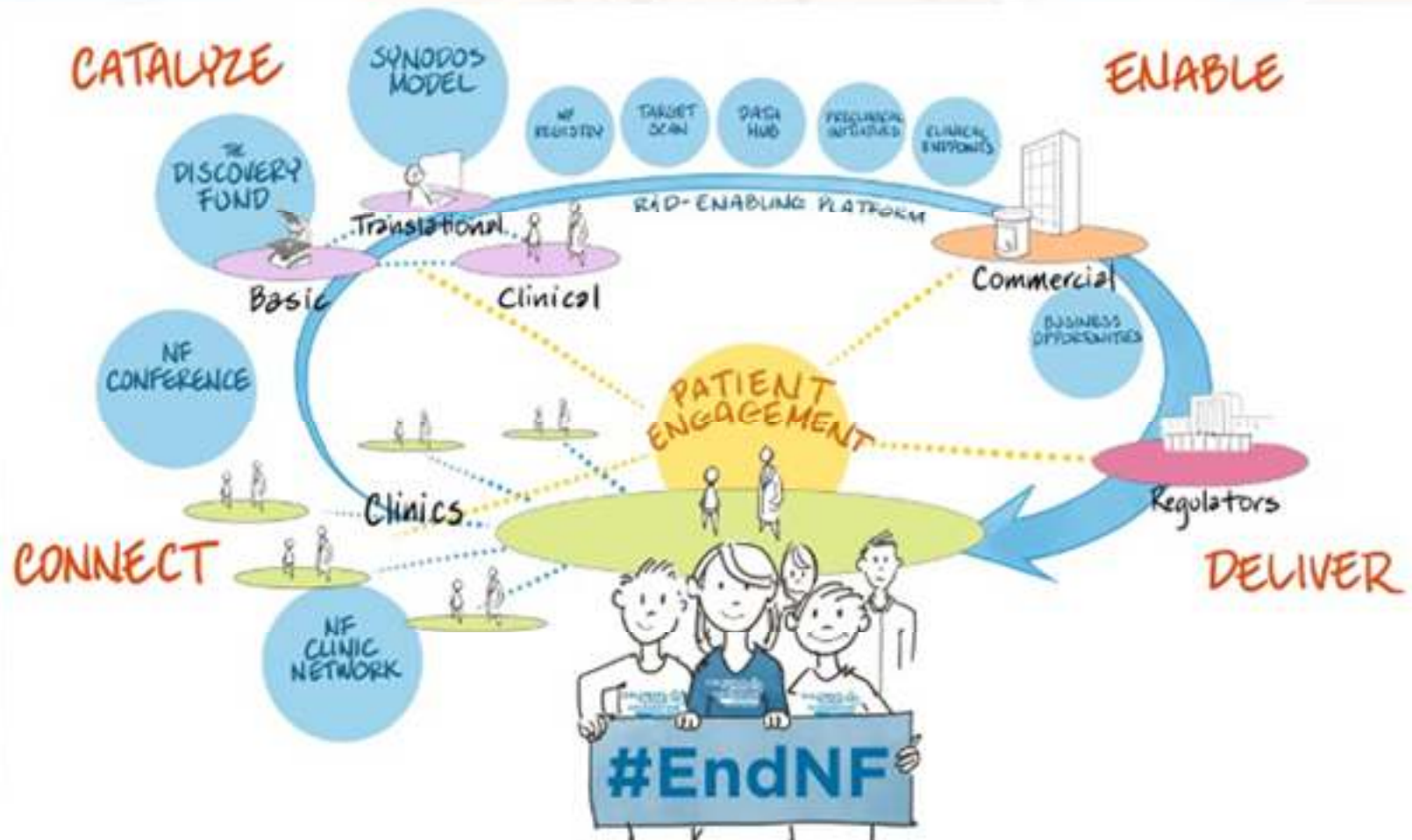
McKinnon Galloway
27 Years Old, diagnosed at 16
Profession: Public Relations Manager
Many tumours, multiple clinical trials!
At risk of completely losing her hearing!



Bill Reiter
> 60 Years Old,
Volunteer with Red Cross
Many tumours, NO trials
Lots of pain!



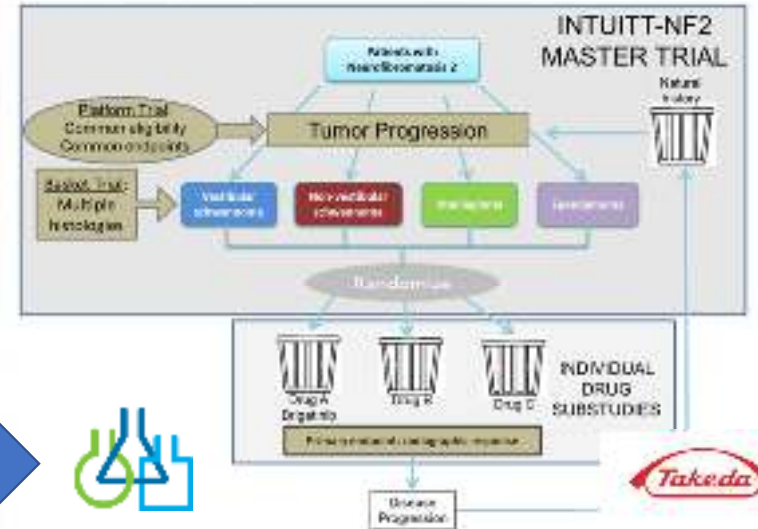
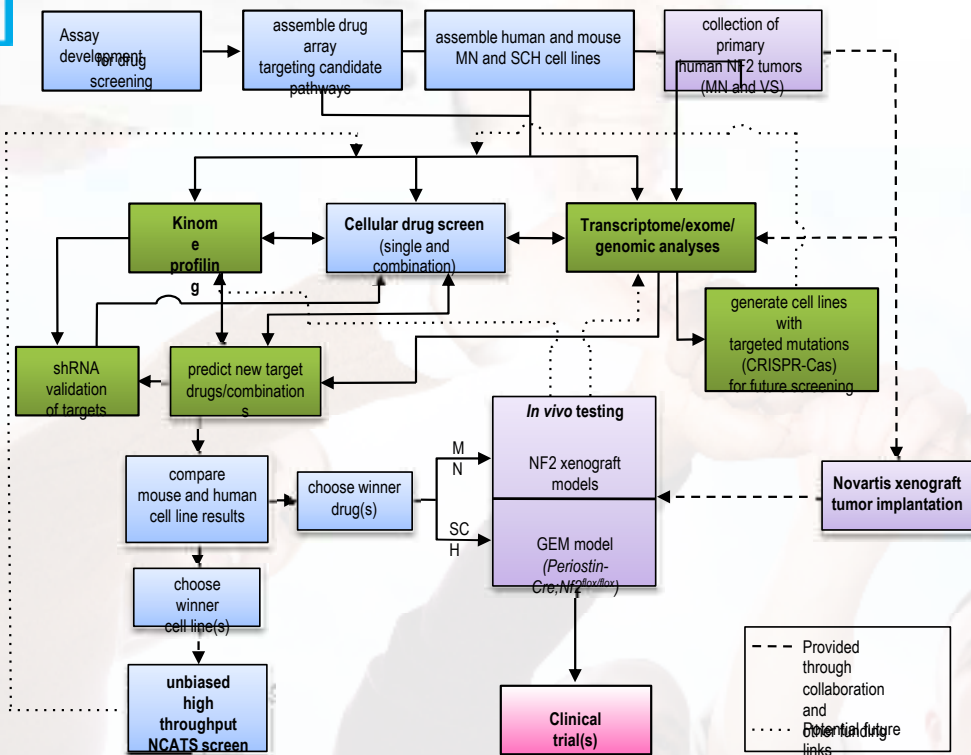
CTF investment philosophy: Connect to Unconnected



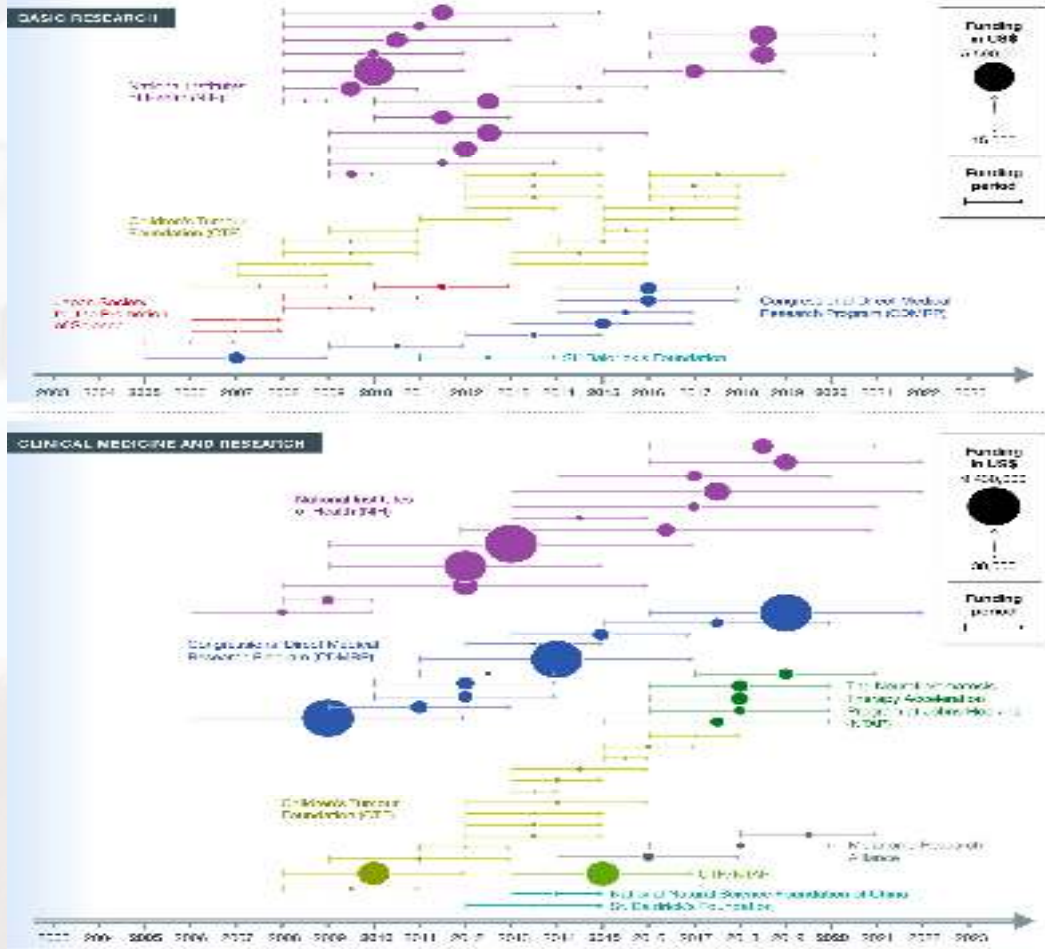
Case study NF2: Synodos to INTUITT TRIAL



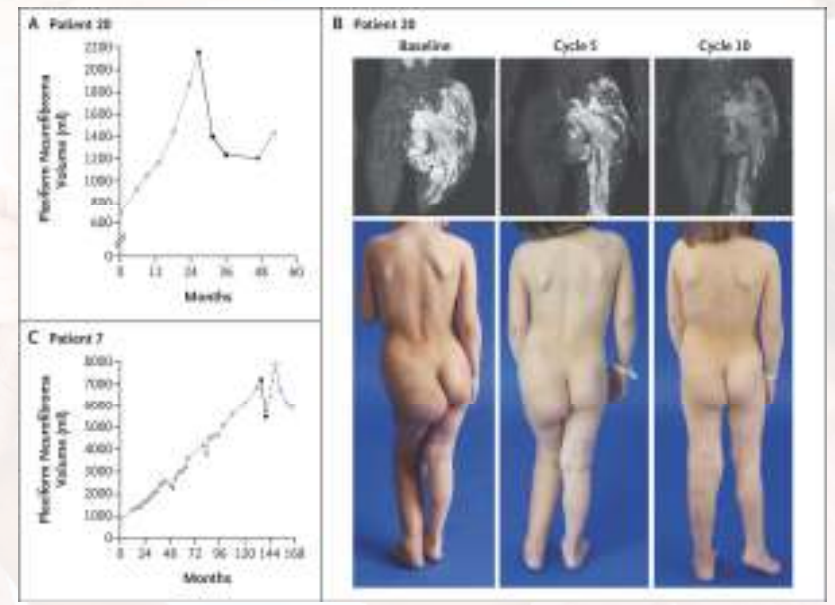
Preclinical pipeline NF2 - \$3m/3 years



Case study NF1: Koselugo - co-fund the path from Discovery to approval



Children's Tumor Foundation funded critical research to get to the Koselugo approval!



N Engl J Med 2016; 375:2550-2560



Other Examples

Advocacy efforts led to:

- NF funding from CDMRP since 1996 (over \$ 300 million)
- Participation in numerous public consultations in Europe
- Inclusion of NF in the call text of IMI2 – Now: NF WP leaders as IMI ass. partner

Partnership with PfizerCures led to Spin-out of Mirdametinib into Springworks

Investment in training led to:

- Masterclass program in NF by Nf experts in Europe – iNFER in 6 languages (Europe)
- Virtual Case conferences (USA)
- First-ever NF training class in South America

Investment in dissemination led to

- CTF's World's largest NF conference since 1988
- Open publication of mission-critical publications

Investment in open Science led to:

- Launch of first -ever NF data portal
- First-ever NF hackathons

And: Resources for NF patients globally

The screenshot displays the Children's Tumor Foundation website. At the top left, the logo for the Children's Tumor Foundation is shown, with the tagline "ENDING NF THROUGH RESEARCH". The main navigation menu includes "UNDERSTANDING NF", "WHO WE ARE", "RESEARCH", "GET INVOLVED", "EVENTS", and "NEWS & RESOURCES". A search bar is located in the top right corner, with a "SEARCH" button and a "DONATE" button. Below the navigation menu, there are three columns of links. The first column, under "NEUROFIBROSIS", includes "Intro to NF", "NF", "PFC", "Schwannoma", and "Genetics of NF". The second column, under "LIVE WITH NF", includes "NF Registry", "NF Rates", "CTF MAGNUM", "TMS & NF Photo Series", and "Patient Engagement Program". The third column, under "GET SUPPORT", includes "Newly Diagnosed", "Family Doctor", "NF Forum", "Symptoms", "NF Camp", "Tools for Kids", and "Contact Us". Below the navigation menu, there are two columns of links. The first column, under "MAKE A DONATION", includes "Make a Donation", "2018 Donation Match", and "Support CTF at Work". The second column, under "FUNDRAISE", includes "Shine a Light NF Walk", "NF Endurance", "Fight NF Your Way", and "Classrooms that Care Youth Development". On the right side of the page, there is a "Search Healthcare Providers" section with a search bar and a "Map" button. Below this, there is a map of the United States with numerous red location pins indicating healthcare providers. A smaller map of Europe is also visible in the bottom right corner.

THANK YOU.

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