NF Patients United

2021

Activities 2021

LTF & NF Patients United & CTT

NF Young Adult International Summit

April 10. -11, 2021

- participants from all over the world
- 18-30 years old
- mix of presentations and interactive sessions
- good feedback from the participants
- great working relationship with LTF

NFPU Awareness Campaign 2021
by NF Patients United

„I can say

Neurofibromatosis.

Can you?“


14 MEMBER ORGANISATIONS FROM ACROSS 12 COUNTRIES

ARE COMING TOGETHER TO

RAISE AWARENESS FOR NF

AND WE NEED YOUR HELP!


About

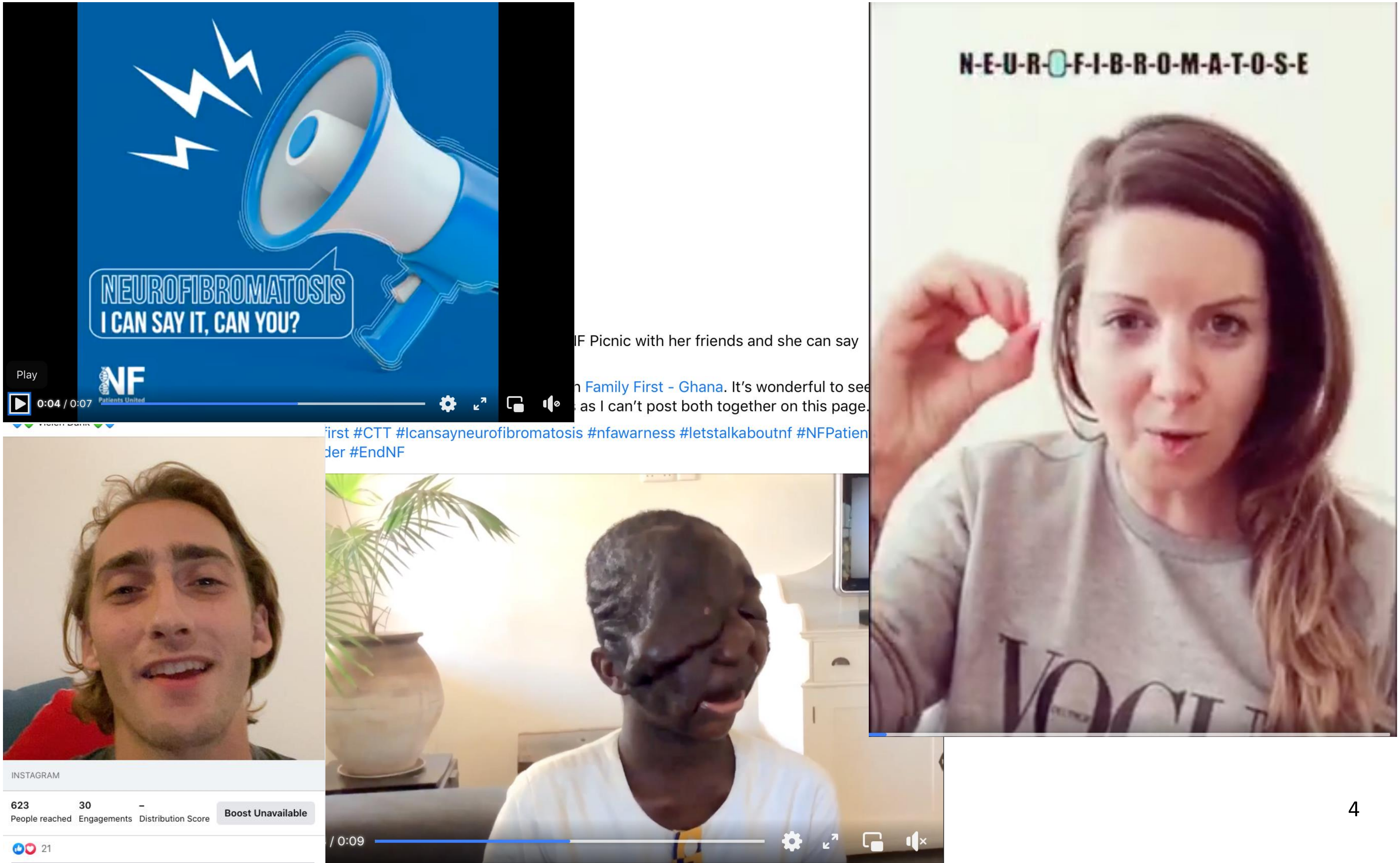
1 OUT OF 25.000

children are born with NF2

FIRST SYMPTOMS MIGHT NOT ARISE BEFORE ADULTHOOD

2021 NF awareness campaign

#IcansayNeurofibromatosis #IcansignNeurofibromatosis



IF Picnic with her friends and she can say

n Family First - Ghana. It’s wonderful to see
as I can’t post both together on this page.

irst #CTT #Icansayneurofibromatosis #nfawarness #letstalkaboutnf #NFPatien
der #EndNF

623 People reached
30 Engagements
– Distribution Score

Activities 2021

EU PEARL

- NFPU as associate member
- Kassandra Walluks and Claas Röhl part of patient advisory group in WP7
- Reviewing master protocol
- Regular calls with European Patients Forum and other PAG members
- Working on patient engagement platform

Activities 2021

ERN GENTURIS

Completion Schwannomatosis guidelines

The Schwannomatosis clinical guidelines have been published in the meantime: **in the European Journal of Human Genetics: https://www.nature.com/articles/s41431-022-01086-x.**

It is the first stand-alone guideline on schwannomatosis!

The complete guideline and pocket guide can be found on our website as well: https://www.genturis.eu/l=eng/Guidelines-and-pathways/Clinical-practice-guidelines/Schwannomatosis.html

Activities 2021

ERN GENTURIS

Completion Schwannomatosis guidelines

Thank you to Melpo Pittara for her contributions to this groundbreaking work and for representing NFPU in the development of this guideline 💚💚

Activities 2021

ERN GENTURIS

- Clinical guideline on NF1 associated tumour management is completed and currently under review of the journal eClinical Medicine
- Several NFPU members have been involved in the development of these guidelines and will be mentioned as co-authors in the publication
- These guidelines will be another groundbreaking work that NFPU was a part of
- Assessment by focus group meeting with native speakers as final review is planed

Activities 2021

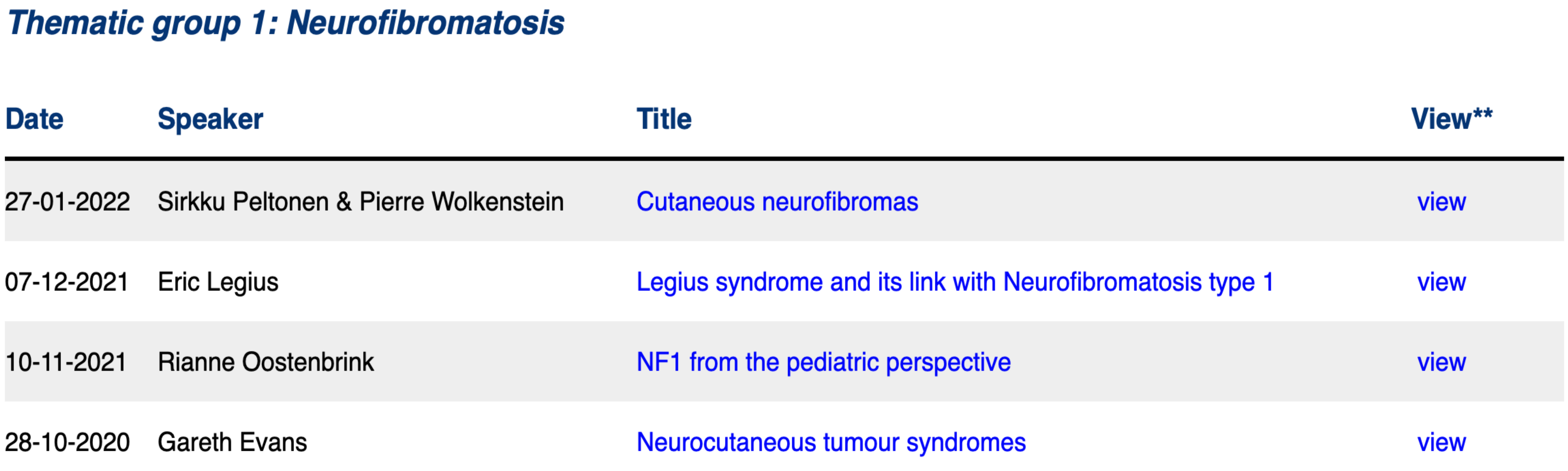
ERN GENTURIS

- After Brexit the ERN GENTURIS partners had to resign from their membership status, but could stay involved in the network as supporting partners
- ERN recommendations on COVID-19 vaccination for patients with rare diseases (Feb 2021)
- The EJP RD ERN Research Mobility Fellowships funding opportunity is now open! It aims to support PhD students, Postdocs & medical doctors in training to undertake scientific visits fostering specialist research training abroad.

Activities 2021

ERN GENTURIS

- 2 annual meetings were held virtually in 2021
- Monthly meetings with executive board, ePAG group and several group with TF5
- Several education webinars were held



Thematic group 1: Neurofibromatosis

Date	Speaker	Title	View**
27-01-2022	Sirkku Peltonen & Pierre Wolkenstein	Cutaneous neurofibromas	view
07-12-2021	Eric Legius	Legius syndrome and its link with Neurofibromatosis type 1	view
10-11-2021	Rianne Oostenbrink	NF1 from the pediatric perspective	view
28-10-2020	Gareth Evans	Neurocutaneous tumour syndromes	view

Activities 2021

Position paper on orphan and paeditric drugs

- Drafted by Vera Lipkovskaya - reviewed by Claas Röhl

Open Public Consultation on the revision of the general pharmaceutical legislation

- drafted by Pilar Garcia, reviewed by Claas Röhl

Activities 2021

- Vanessa Martin (CTT) joined the REiNS group as a patient representative
- Claas Röhl is working in the working groups for patient reported outcomes, cutaneous neurofibromas, gene therapy, PRO, patient education
- Prof. Pierre Wolkenstein and his group joined the cutaneous neurofibroma working group and works on outcome measures to allow meaningful clinical trials

Activities 2021

IMI (Innovative Medicines Initiative)

- Recommendations for Rare Diseases - article written by Olivier Blin and Claas Röhl https://www.imi.europa.eu/sites/default/files/uploads/documents/About-IMI/Governance/sc/SC_Recs_RareDisv21-08-2020.pdf
- article on the IMI website https://www.imi.europa.eu/news-events/newsroom/how-europe-can-get-better-treating-rare-diseases

Activities 2021

Multistakeholder workshop on complex clinical trials 5-6th October 2021 - https://www.complexclinicaltrials.eu

NFPU was a co-organizer of this meeting, that was initiated by EFPIA. Claas Röhl organized several speakers, organized 2 break out sessions and moderated 2 panel discussions

Meeting report: link

Organising Committee

Josse R. Thomas (BAREC)
Frank Bretz (EFPIA-Novartis)
Olga Kholmanskikh (CTFG-FAMHP)
Solange Corriol-Rohou (EFPIA-AstraZeneca)
Stephane Lejeune (EORTC)
Lucia D'Apote (EFPIA-Amgen)
James McCormick (ACRO-PPD)
Christine Fletcher (EFPIA-GSK)
Pierre Omnes (ACRO-Syneoshealth)

Mireille Muller (EFPIA-Novartis)
Ruediger Pankow (ACRO-Parexel)
Emmanuel Pham (EFPIA)
Claas Roehl (NF Patients United)
Nick Sykes (EFPIA-Pfizer)
Anja Schiel (EMA SAWP, NoMA)
Andreea Iordache (EFPIA Secretariat)
Elke Stahl (CTFG-BfArM)
Silvia Garcia (EFPIA Secretariat)

NF best practice - virtual meeting for patients & caregivers
Conducted by NF Patients United

Saturday - 6.11.2021

12:00 - 13:15	NFPU Annual Meeting 2021 (members only)

Open program (for public)

13:30 - 14:00	Opening Words - Claas Roehl
14:00 - 14:30	NF1 Guidelines - Erik Legius
14:30 - 15:00	NF virtual summit for young patients (U.S) - Gillian Payne
15:00 - 15:30	Break
15:30 - 16:00	NF Young Adult Leadership Program - Littlest Tumor Foundation
16:00 - 16:30	NFPU Awareness Campaign 2021
16:30 - 17:00	REiNS - Vanessa L.Merker
17:00 - 17:30	NF2 Reseach - Scott Plottkin
17:30 - 18:00	Schwannomatosis Guidelines - Ignacio Blanco

AstraZeneca

Sunday - 7.11.2021

Open program (for public)

National member projects & non-member projects

10:00 - 10:30	The meeting spot, A web app for the NF community - Theo Fernandes (PT)
10:30 - 11:00	Rehabilitation of pediatric patients - Claas Roehl (AT)
11:00 - 11:30	Supporting parents with diagnosis on NF1 - Rare Minds (GB)
11:30 - 12:00	Artificial intelligence tools in the work of NF patient organisations: Russian case - Vera Lipovsky (RU)
12:00 - 12:05	Wrap up and Closing Words

Workshop (for members and other patient organisations)

13:00 - 15:00	- New ideas for NFPU projects in the future - Recent developments and how to get the best out of it - Presentation of Outcome

AstraZeneca

Activities 2021

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- article on the IMI website https://www.imi.europa.eu/news-events/newsroom/how-europe-can-get-better-treating-rare-diseases

NF Patients United is:

ACNefi

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ONLUS

NFVN
Neurofibromatose Vereniging Nederland

ANF®
Associazione Neurofibromatosi

Asociación de Afectados de Neurofibromatosis
NF

NF Kinder

22q17

Ζωή με NF
ΠΑΝΕΛΛΗΝΙΟΣ ΣΥΛΛΟΓΟΣ ΑΣΘΕΝΩΝ & ΦΙΛΩΝ ΠΑΣΧΟΝΤΩΝ ΑΠΟ ΝΕΥΡΟΪΝΩΜΑΤΩΣΗ

Childhood Tumour Trust

NF - foreningen

APNF
Associação Portuguesa de Neurofibromatose

NF Turkey

HRVATSKA UDRUGA ZA NEUROFIBROMATOZU
CROATIAN NEUROFIBROMATOSIS ASSOCIATION

Schweizerische Neurofibromatose-Vereinigung

NF Patients United

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