

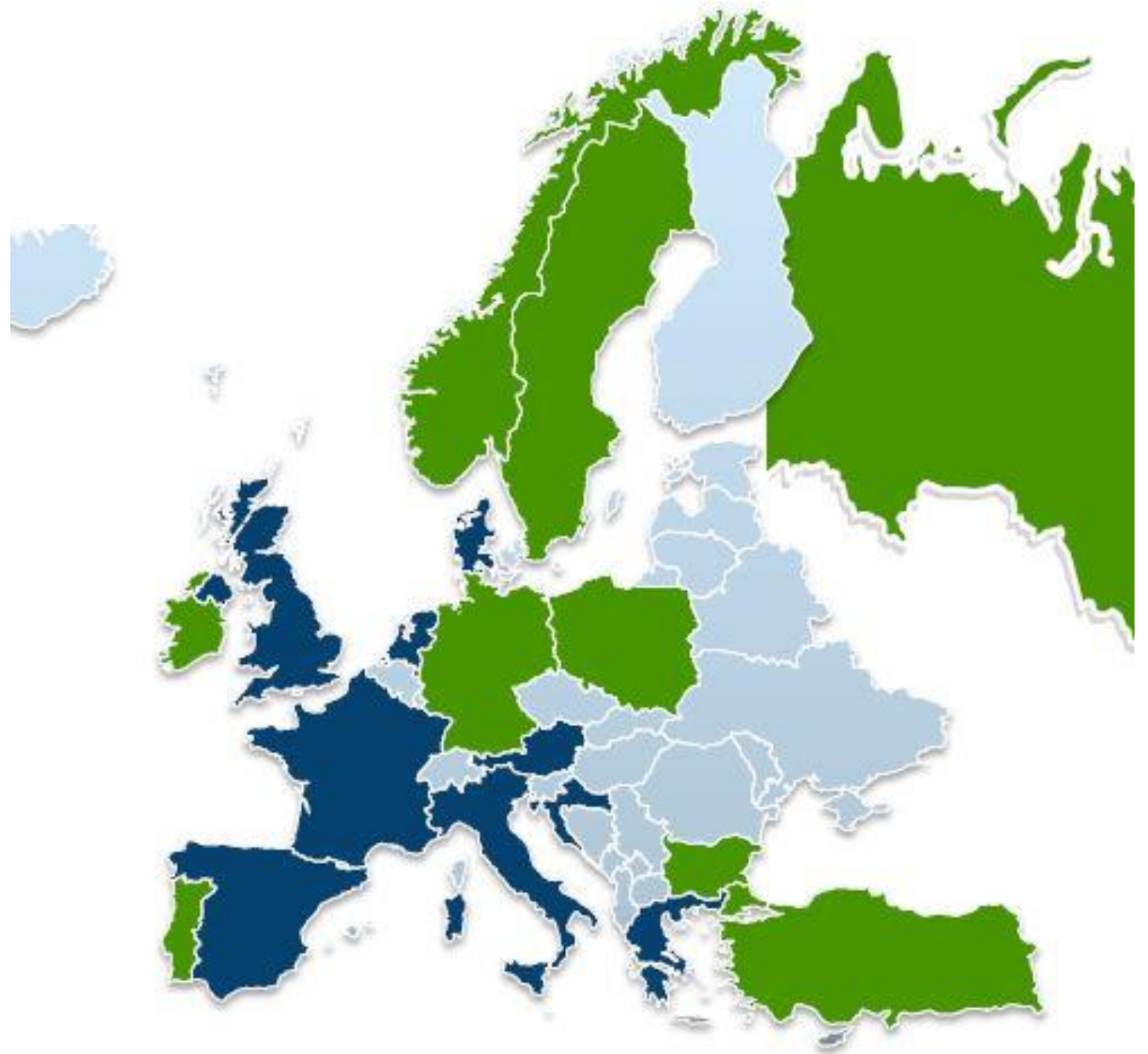


# NF Patients United

2019

# NFPU members 2019

- **NFPU Members (blue)**
- **Associatiated or interested members (green)**
- **New candidates for NFPU-membership: Portugal, Turkey**



# Activities 2019

- Start of the **monthly joint webinars** (10 webinars) and **Working Group webinars** (12 webinars)
- First joint project of NFPU: **Shine a light campaign**
- Organization of 3 Webinars with Scott Plotkin and Gareth Evans about **new diagnostic criteria and name change** in July 2019
- Several monthly calls with GENTURIS including 2 face2face meetings
- Several monthly calls with REiNS, including 2 face2face meetings

# NFPU awareness campaign 2019

## **Best practice - Shine a light Austria**

- Webinar about Shine a light experiences after 2 years in Austria
- Manual for Shine a light was shared
- Draft letter for contacting sites was shared
- Draft for press release was shared

# NFPU awareness campaign 2019

## SHINE A LIGHT ON NF 2019

*number of buildings*

AUSTRIA	33
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GREECE	2
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ITALY	47
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ENGLAND	71
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NORTHERN IRELAND	13
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SCOTLAND	13
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WALES	6
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ISLE OF MAN	3
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SPAIN	10
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PORTUGAL	4
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<i>Total</i>	202
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### Media:

Press releases, Social Media publishing, Press Conferences and further activities have been made in:

- Greece
- Croatia
- Austria

# NFPU awareness campaign 2019

## SHINE A LIGHT ON NF 2019

*number of buildings:*

United States	102
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Canada	20
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New Zealand	1
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Europe (total)	202
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• NFPU–member organisations	93
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• Great Britain	109
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<i>Total (worldwide)</i>	325
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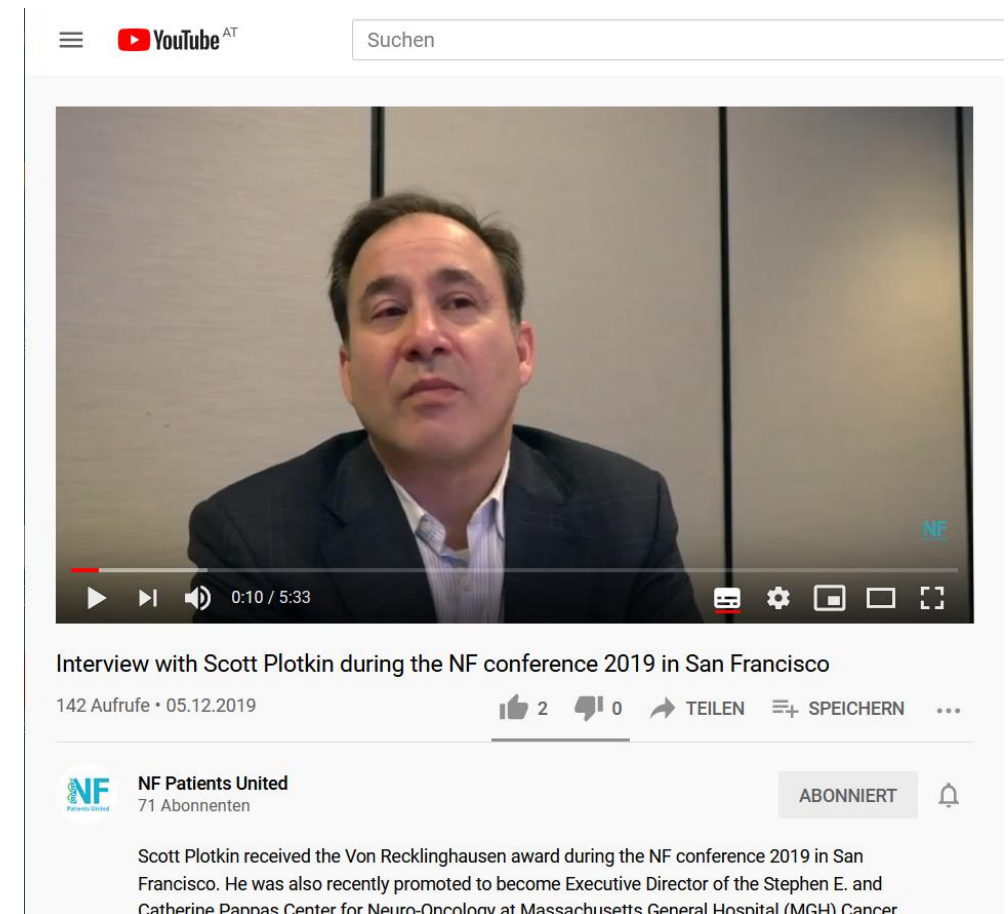
# Research 2019

- Several monthly calls with GENTURIS including 2 face2face meetings (Claas)  
Taskforce: Research, ePAG-steering committee, chair of the patient council
- Several monthly calls with [REiNS](#), including 2 face2face meetings (Claas)  
Working groups: cutaneous neurofibromas, patient reported outcomes, patient representatives/ patient education  
REiNS call for new patient representatives will start 2021!
- Speaking at national and international events and raising awareness about NFPU, including the NF conference in San Francisco 2019
- Claas joined the Scientific Committee of the IMI (Innovative Medicine Initiative) - goal to push for Rare Disease specific IMI calls

# Activities 2019

## CTF conference 2019 San Francisco

- Summary of the conference
- Videointerview Scott Plotkin
- Side meetings with AstraZeneca, Gilbert Foundation, REiNS meeting



<https://www.youtube.com/watch?v=rC940woHm7k>



# Activities 2019

## **Kick off meeting EU PEARL (IMI project)**

21.-22.11.2019 in Barcelona

## **EU-PEARL mission**

EU-PEARL aims to transform the current approach of clinical development into a more patient-centric and cross-company collaborative Integrated Research Platform, aiming to bring new innovative medicines faster to patients.

## **NFPU as associated partner**

Workpackage 7 is dedicated to NF

# NFPU working groups 2019

## NFPU PROJECTS 2019

**01**

### Joint Materials

Collection of Manuals/  
Handbooks/ Pictures /  
Videos and to provide  
Materials

**Lead:** Vera Lipkowskaya  
(NF Russia)

**02**

### Organisation of Care

Body Map  
Classification of  
Europ. NF Center

**Lead:** Vanessa  
Martin (CTT)

**03**

### Awareness & Fundraising

Shine A Light on NF  
Rare Disease Day  
NF Walks

**Lead:** Claas Roehl  
(NF Kinder)

**04**

### Research

NF Patient Registry  
Research Projects

**Lead:** Federica Chiara  
(LINFA)

**05**

### Psychological Activities

NF Youth Camps  
in Europe

# Lecture for members

## **Online-lecture on Fundraising for patient organisations by Claas Röhl**

- Best practice NF Kinder
- Recording was shared via download link

# Activities 2019

## TRADITIONAL VS. PLATFORM CLINICAL TRIALS

CHARACTERISTIC	TRADITIONAL TRIAL	PLATFORM TRIAL
SCOPE	Efficacy of a single agent in a homogeneous population	Evaluating efficacy of multiple agents in a heterogeneous population; explicitly assumes treatment effects may be heterogeneous
DURATION	Finite, based on time requires to answer the single primary question	Potentially long-term, as long as there are suitable treatments requiring evaluation
No. OF TREATMENT GROUPS	Prespecified and generally limited	Multiple treatment groups; the number of treatment groups and the specific treatments may change over time
STOPPING RULES	The entire trial may be stopped early for success or futility or harm, based on the apparent efficacy of the single experimental treatment	Individual treatment groups may be removed from the trial, based on demonstrated efficacy or futility or harm, but the trial continues, perhaps with the addition of new experimental treatment(s)
ALLOCATION STRATEGY	Fixed randomisation	Responsive-adaptive randomisation
SPONSOR SUPPORT	Supported by a single federal or industrial sponsor	The trial infrastructure may be supported by multiple federal or industrial sponsors or a combination



# EU-PEARL 2019

EU-PEARL: EU Patient-cEntric clinicAl tRial pLatforms

## The partners



36 world leading institutions  
26M € budget  
42 months

### Patients Organisations



### European University Hospital Alliance (EUHA) Hospitals



### Other Hospitals



### Universities



### Data, Statistics



### Regulatory



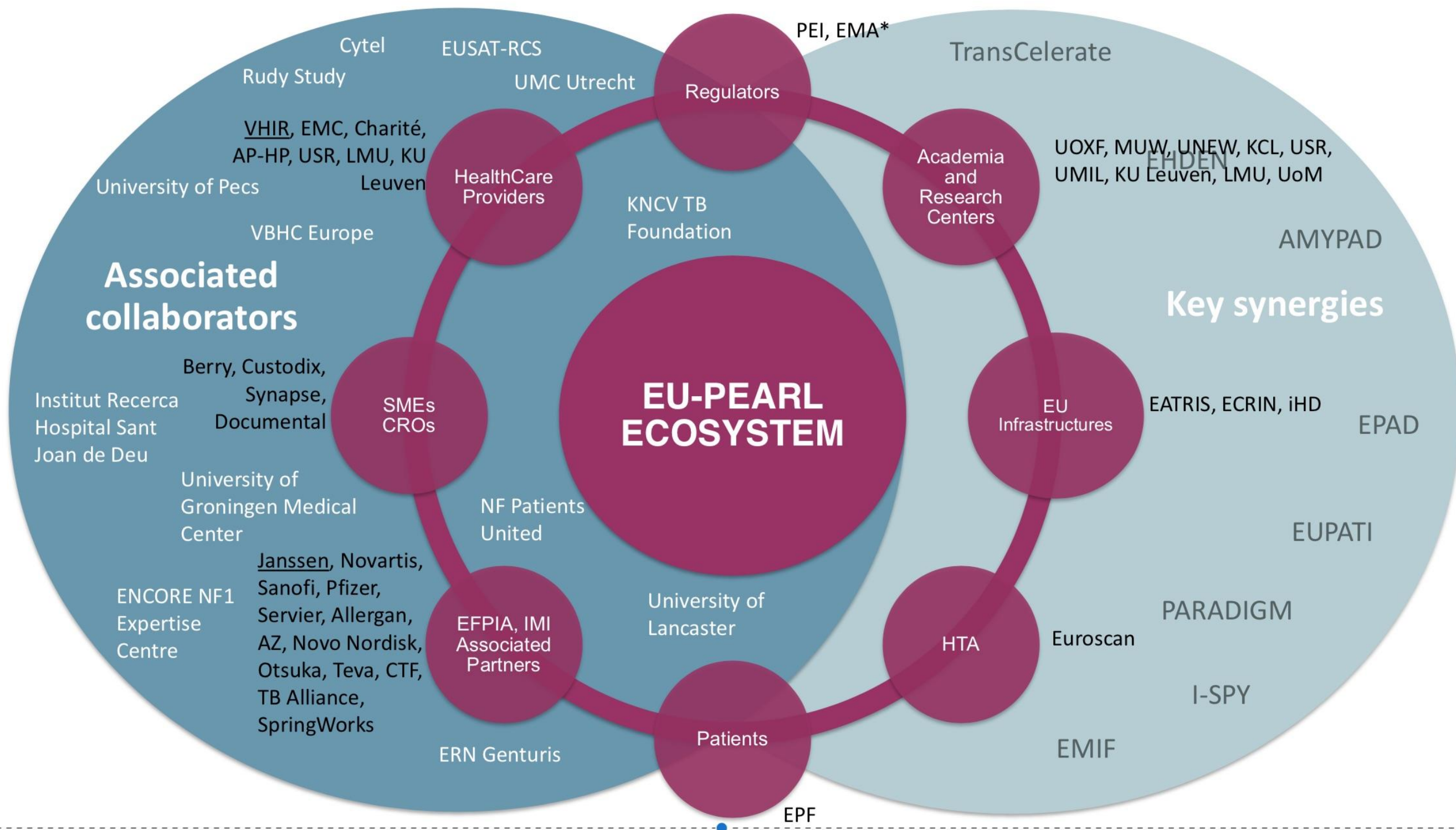
### Project Management



### European Infrastructures

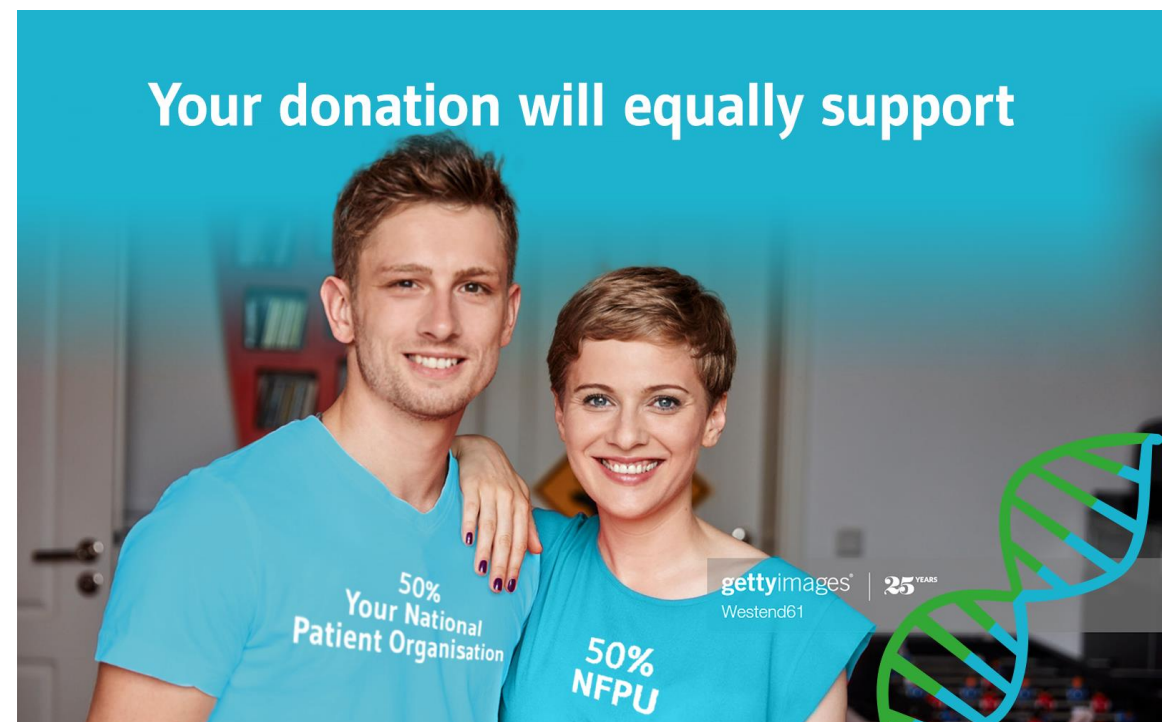


# EU-PEARL 2019



# NFPU Fundraising 2019

- Start with a fundraising tool and webinars
- for to carry out joint projects for NF Patients





# Joint Meeting 2019 in Parma/IT



With workshops on Visions,  
Mission and Goals of NFPU



## Agenda 24.11.2019

9:00-9:30	Explanation of the parallel workshop design
9:30-10:30	Workshop Part 1 - World Cafe - collection of first inputs
10:30-12:30	Workshop Part 2 - Project Plan development lunch break
12:30-13:30	Presentation and Discussion (projects in plenum)
13:30-14:30	Presentation of the results and wrap ups
14:00-15:00	

## Agenda 23.11.2019

12:00	Welcome and lunch
13:00 - 14:00	NFPU general assembly meeting <ul style="list-style-type: none"><li>* Welcome to Parma from ANF president (Corrado Melegari)</li><li>* annual report (Claas Röhl)</li><li>* financial report (Marcel Caubo)</li><li>* election new board members</li><li>* Next NFPU general assembly meeting 2020 Rotterdam</li></ul>
14:00 - 17:00	Workshop „NFPU mission statement & vision“ interactive sessions, group exercises, visualizing and phrasing the mission statement and the vision
17:00 - 17:30	Introduction of NF2 Biosolutions (including Q&A)
17:30 - 18:00	Introduction Infixion (including Q&A)
18:00 - 19:00	Presentation of potential NFPU projects by the project leader (outlining the scope and content of the projects)