Project Overload? No, actually now we are converging: pieces of the puzzles are coming together

- EJP – EU Joint Programme for Rare Disease
- c4c
- Both offer opportunities to pilot tools and approaches we have and think will work, but need to demonstrate with more certainty
- ERNs offer added value in many ways – we heard about some of their priorities in research
- Discussed some crucial actions to help ERNs reach their potential
  - E.g. Industry engagement, Legal Entity status, registries (come back to this)
- Some of these can only be realised when utilised as pilots- which the above offer, alongside (we hope) dedicated research funding
- In many ways, EJP, c4c, ERNs – these approaches are complimentary.

Methodologies for conducting clinical trials in small populations

- We have the outputs of the 3 projects: IDEAL, ASTERISK, INSPIRE
- Presentations highlighted the POWER tool:
  - **P**atient participation in **O**utcome **M**easure **WE**ighing for **R**are Diseases
  - It became more a tool for involving patient when thinking about trial design - raised perspectives not seen before
- Hoped take this to EMA, potential to undergo qualification, but need to test it more on real setting…
- Luckily…
- EJP has a dedicated WP (20) involving IRDiRC TF experts to review and revise this tool and the other methodologies and the tools which emerged from the 3 funded projects … then
- … apply it, using pilot projects (some with the ERNs).
- Goal is to demonstrate that these models work, and to de-risk it, to overcome the apprehension of sponsors.

Rare Diseases and Paediatric Diseases

- Why are RD and paediatric fields somewhat separate? When they often involve the same patients and experts
  - Healthcare organisation
  - Tendency in Industry to have paediatric representatives and RD departments
- Strong benefit of forging stronger synergies here: c4c, EJP and the ERNs together should help to unite these communities
- How? One way is, much clearer mapping of where expertise exists in Europe, for rare diseases and for paediatric rare diseases
- Patient organisations should also strive to bridge this gap when it is needed.

Registries

- Excellent presentation on CF Registry
- Qualification by EMA seen as huge step FW for RD registries – however, do we need to different approaches and models for the rarer diseases?
- Really complex landscape – what do we do with existing registries?
  - ERN pilots should be illuminating… we are moving towards a really overarching workshop/meeting on this
- People need clarity on who to seek guidance from, re. registries: JRC? EMA Patient Registry Initiatives
  - Proposed perhaps that registry platforms uniting disease combining with modules for specific conditions. Certainly strong for PMS but also for natural history registries, say? Can one type do all?
  - ERNs again have huge potential here – need to think of what type of data we'd want to capture, if we could, at all ca. 1000 centres
    - Particularly, in action at EMA in pre-competitive space