

# ICORD 3<sup>rd</sup> International Conference on Rare Diseases and Orphan Drugs

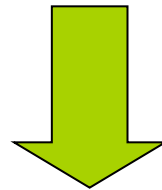
## WG4

### Recruiting Patients for Clinical Research Studies and the Value of International Collaboration

**Domenica Taruscio**  
**National Centre Rare Diseases, Italy**

# Introduction to the discussion

Recruiting patients vs different approaches  
for small trials



Recruiting patients **AND** different approaches  
for small trials

# Recruiting patients vs approaches for clinical trials with small sample size

## Panel discussion topics:

- Small clinical trials/studies can be useful?
- Small clinical trials are ethical?
- Can different statistical approaches help?
- Is the Industry using alternative approaches to orphan drugs research & development?
- Regulatory Authority views on level of evidence and approaches

# Outline of the discussion

- **Discussion on small Clinical Trials: brief overview**

**Annalisa Trama**

- **Methodological Issues on Clinical Trials with Small Sample Size**

**J. Gerß, Wolfgang Köpcke**

- **Balancing the importance of getting some information vs the importance of getting good quality information**

**Simon Day**

- **ECRIN Network**

**Arrigo Schieppati**

- **Comments**

- **Rumen Stefanof**

- **Plenary discussion; additional WG4 members interventions**

- **Conclusions**