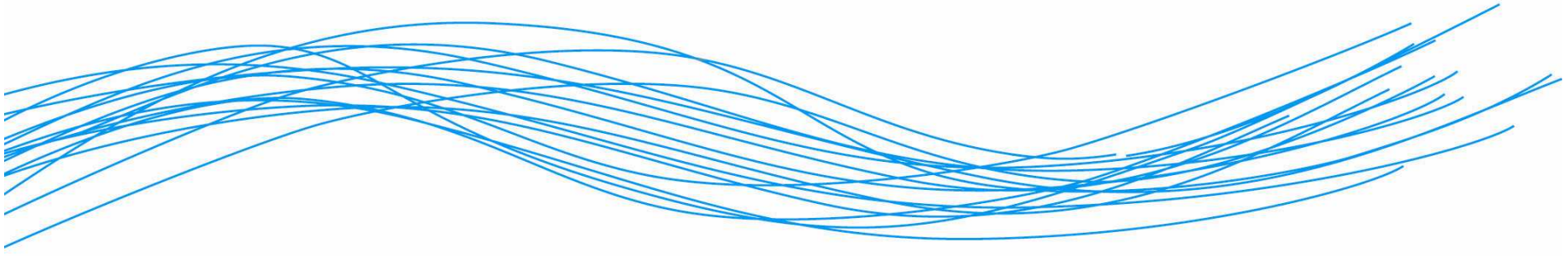
Expectations for Enpr-EMA from Patient Perspective



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A highly variable situations in the field of rare diseases:

- clinical aspects of the disease,
- varying skills and competences.
- size,
- experience,
- scientific commitment,
- and logistical means of both sponsors and POs.

Certain rules are required to maintain a trust relationship between the various stakeholders (sponsors, POs, patients, and investigators), which is essential for a fruitful collaboration.

Eurordis charter for clinical trials in rare diseases I.

(www.eurordis.org)

- *Patient organisations (POs) should be informed on all aspects of the clinical study protocol before committing to collaborate.*
- *POs should actively contribute to the documents aimed at patients - information document and consent form.*
- *Domains and extent of collaboration should be declared in a document called "Agreement of Understanding", available for all stakeholders: patients, investigators, ethics committees and national competent authorities.*

Eurordis charter for clinical trials in rare diseases II.

(www.eurordis.org)

- *Financial relationships between sponsors and POs should be made transparent.*

- *Study results should be published, even in case of negative outcomes, non conclusive or abandoned clinical trials.*

Eurordis charter for clinical trials in rare diseases III.

(www.eurordis.org)

- *Data acquired during clinical trials should be made available to the scientific community, with a view to foster scientific progress and avoid unethical duplication of clinical trials.*

- *The commitment of a PO in the design and/or development of a trial does not modify the role and responsibilities of the sponsor, even if the study is financially supported by the PO.*

Enpr-EMA

- link together existing paediatric networks
- create a platform to facilitate industry access to paediatric clinical research centers
- enable CTs requested by the PDCO in the PIPs

Enpr-EMA of existing paediatric networks

-sharing of relevant expertise

-fostering innovation

- 6 main criteria for inclusion

-Enpr-EMA must ensure that these high standards are implemented across all paediatric CT research in Europe

Enpr-EMA Criterion 6 : Public Involvement

- Added value of patients/ PO involvement
- How to identify relevant PO / patients
- How to ensure the systematic involvement of patients
- How to promote public involvement in paediatric networks

Communicate !

Break out session 3

How patients can be involved in networks (trial design etc) and in trials