EURORDIS
Rare Diseases Europe

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EURORDIS CHARTER FOR CLINICAL TRIALS IN RARE DISEASES

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Why regulate patient-sponsor interaction?

Actors (sponsors and POs) involved in clinical development face highly variable situations in rare diseases:

- clinical aspects of the disease,
- varying skills and competences,
- size,
- experience,
- scientific commitment,
- and logistics.

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

Common objective

Production of high-quality knowledge on diseases and development of effective and safe treatments.

POs can collaborate with sponsors on all aspects of clinical trials:

- **Adapting the design of the study to patients' expectations**
  - **Inclusion/exclusion, concomitant meds, length of trial, can a pt leave?**
- **Providing early information to potential participants**
  - **As results are known to investigators, they are made known to participants**
- **Supporting patients during the study**
  - **Supporting families during study**
- **Taking quality of life into consideration and discussing trial results**
  - **QOL questionnaires, formal letters of thanks and results**

General Principles

- **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 documents and consent form.**
  - Newsletters?
- **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.** (Published on websites)
- **Financial relationships between sponsors and POs should be made transparent.**

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General Principles

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

- **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.**

Eurordis Charter in Practice

Eurordis has put forward this Charter as a set of general principles.

- **Implementation of the Charter is based on the willingness of both sponsors and Patient Organisations (all Eurordis members)**
- **The Charter and its outcome will be reviewed after an initial trial period**
- **Eurordis is committed to making public the list of sponsors having adopted the Charter (7 companies so far)**
- **Any collaboration between a Sponsor and a Patient Organisation, according to the Charter, agreed to in the "Agreement of Understanding", will be made public on the Eurordis Website.**

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Eurordis Charter in Practice

Eurordis is committed to facilitating the use of the Charter

- **Eurordis will help the sponsor identify European POs interested in collaborating with them**
- **Eurordis may also assist in the setting-up of the collaboration between sponsors and POs, without interfering in the study itself.**
- **Eurordis develops training sessions aimed at helping PO representatives to better contribute to clinical trials.**
- **Additional documents aimed at these collaborations - glossary, specimens of agreement, "fiches de collaboration" - are available on Eurordis website.**

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Agreement of Understanding

- **Non-confidential, not legal, but keeps things transparent**

- **The Sponsors**
- **The design (objectives, endpoints, etc)**
- **Implementation (sites, countries, DSMB, etc)**
- **Conduct (PO as information source during trial, financial support)**
- **Analysis and dissemination**
- **Financial aspects**

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