

# The Eurordis Charter in practice

- Eurordis is putting forward this Charter as a set of general principles. Contrarily to a regulation, the implementation of the Charter is only based on the goodwill of both sponsors and Patient Organisations, who share these principles.

*It is anticipated that this Charter will be reviewed and adapted if needed after an initial phase of implementation and trial.*

- Any sponsor agreeing with the principles underlying this Charter may adopt them. Eurordis commits itself to make public the list of sponsors having adopted the Charter.
- According to the Charter and for a given clinical trial, the collaboration between the sponsor and the PO will be testified by the ad hoc “Agreement of Understanding”, made public to all stakeholders in particular on Eurordis’ Website.
- Eurordis commits to facilitating the implementation of the Charter:
  - For a given clinical trial and upon request, Eurordis will help the sponsor identify European POs interested in collaborating
  - Eurordis may assist in the setting-up of the collaboration between sponsors and POs without interfering in the study itself
  - Eurordis is developing training sessions aimed at helping PO representatives to better contribute to clinical trials (pilot sessions already developed at national level and in development at European level through EC-supported program)
  - Additional documents regarding collaboration - glossary, examples of agreements of understanding –will be available on Eurordis’ website.
  - For any question regarding the Charter, sponsors and POs may contact Eurordis at [clinicaltrials@eurordis.org](mailto:clinicaltrials@eurordis.org)



## About EURORDIS

The European Organisation for Rare Diseases (EURORDIS) represents more than 260 rare disease organisations in over 30 different countries, covering more than 1,000 rare diseases. It is therefore the voice of 30 million patients affected by rare diseases throughout Europe. Further details concerning EURORDIS and rare diseases are available at: <http://www.eurordis.org>



## Clinical Trials for Rare Diseases

### *The need for a Charter*

Sponsors and patient organisations (POs) share common objectives: production of high-quality knowledge on diseases and development of effective and safe treatments. Both sponsors and POs recognise that their collaboration in the development of clinical trials on rare diseases - based on sharing complementary skills and competences - is effective. The interest of such collaboration is also made clear by the participation of PO representatives in the EMEA committees (COMP, Protocol assistance, CPWP).

Because of the poor scientific knowledge on rare diseases and the deep expertise of POs on their disease, POs may fruitfully collaborate with sponsors in all phases and on several aspects of clinical trials:

- Adapting the design of the study to patients’ expectations facilitates their adhesion to the trial;
- Providing early information to potential participants ensures and speeds up their inclusion in the trial;
- Supporting patients during the study reduces number of drop-outs and incomplete files;
- Taking quality of life into consideration and discussing trial results with sponsors contribute to the assessment of clinical and day-to-day benefits of the treatment.

Actors involved in clinical development in the field of rare diseases face highly variable situations. A universal regulation fitting all situations would therefore be unrealistic and ineffective. However, certain rules are required to maintain a relationship of trust between the various stakeholders (sponsors, POs, patients, and investigators), essential for a fruitful collaboration. A clear definition of the conditions and fields of collaboration is a necessary basis for the confidence of patients volunteering in the trial. Transparency may also avoid unjustified fears or hopes from both sponsors and patients.

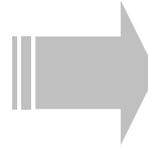
For the above-mentioned reasons, Eurordis launched a reflection process with POs - initially with the French “Alliance Maladies Rares”, later with a European group of experts and the members of the Eurordis Round Table of Companies. The process led to a Charter for collaboration between sponsors and POs. The aim of this Charter is to improve the quality of clinical research in rare diseases and to enhance a transparent and effective dialogue between interested parties.

# Eurordis Charter for Collaboration between Sponsors and Patient Organisations for Clinical Trials in Rare Diseases.



## General Principles

- 1) Patient organisations (POs) should be informed on all aspects of the clinical study protocol before committing to collaborate. This would provide legitimacy to the inclusion of patients in the study. For the same reasons, any substantial amendment to the protocol should be communicated to the Pos in real time.
- 2) POs should actively contribute to the documents aimed at patients - information document and consent form - to ensure their content and format can be understood by lay people, thus allowing truly informed consent by patients.
- 3) Domains and extent of collaboration should be declared in a document called "**Agreement of Understanding**" available for all stakeholders: patients, investigator, ethics committees and national competent authorities. The agreement, co-established on a voluntary basis by sponsors and POs for a given clinical study, describes the fields and potential limitations of the collaboration, without detailing its content.
- 4) Financial relationships between sponsors and POs should be made transparent.
- 5) Study results should be published, even in case of negative outcomes, non conclusive or abandoned clinical trials.
- 6) Patients participate in clinical studies to improve their knowledge of the disease and help develop adapted treatments. To fully respect patients' collective commitment, the 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.
- 7) In any case, the commitment of a PO in the design and/or development of a trial do not modify the role and responsibilities of the sponsor, even if the study is financially supported by the PO.



The **Agreement of Understanding** should define the following elements:

- What is included, or excluded, from the collaboration (e.g. study design, patient information and support, patients' inclusion in the trial, discussion of results, clinical benefit assessment, participation in independent monitoring committees etc).
- In the study design, the fields included or excluded from the collaboration (e.g. inclusion criteria, main and secondary objectives, quality of life criteria, number of patients included, duration of the study, use of control treatments...).
- POs' commitment (e.g. contribution to identification of study centres, communication on the clinical trial, support to the inclusion of patients, patient support during the trial, phone line dedicated to the trial).
- Points potentially considered confidential by the sponsor (e.g. production processes, clinical development plan...).
- Names of PO signatories of the confidentiality agreement in case of restricted dissemination of information. Similarly, any potential conflict of interest should be declared by each signing member.
- Date or event-related clause in case of temporary restriction of access to data (marketing authorisation, scientific communication...).
- Any type of financial relationship between POs and Sponsors: purpose, amount, and type of support, including in-kind donations.

*In case of a multinational study including several national POs, a common agreement for all involved POs should preferably be adopted. However, if necessary, the agreement may be adapted to fit each national specific situation.*

