Research and clinical trials
what are the rights of patients?

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## Disclosures

<table>
<thead>
<tr>
<th>Company name</th>
<th>Research support</th>
<th>Employee</th>
<th>Consultant</th>
<th>Stockholder</th>
<th>Speakers bureau</th>
<th>Advisory board</th>
<th>Other</th>
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<tr>
<td>University of the Basque Country</td>
<td>Enerca Project</td>
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### This talk is applicable for:

<table>
<thead>
<tr>
<th>Condition</th>
<th>Definite</th>
<th>Probable</th>
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</thead>
<tbody>
<tr>
<td>Thalassemia’s</td>
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<td>Sickle cell disease</td>
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<td>Membrane disorders (e.g. sferocytosis)</td>
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<td>Enzym defects (e.g. PKD, G6PD)</td>
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<td>PNH</td>
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<tr>
<td>Other forms of hemolytic disease</td>
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THE LEGAL FRAMEWORK


Direct effect in national legal systems.

Transparency and information.

Speed procedures (one position by country and coordination between countries).

Entry into force: when the EU Portal and the EU Data Base achieve full functionally. Not earlier than 28 May 2016.

EU Portal
EU Database. Notification of the recruitment, of the conditions of the trial, of the results.
DEFINITIONS AND PRINCIPLES

A clinical trial is a clinical study related to the effect of a medicinal product in humans in addition to normal clinical practice.

• The rights, safety, dignity and well-being of subjects should be protected.

• The data generated should be reliable and robust.

• The interests of the subjects should always take priority over all other interests.

• Clinical trials should be subject to prior authorisation
  Administrative authorisation.
  Ethics review.

• Clinical trials for the development of orphan medicinal products and of medicinal products addressed to diseases affecting no more than one person in 50,000 in the Union (ultra-rare diseases) should be fostered.
THE PROCEDURE

Sponsor                   Submits the application to the European Portal
                          Selects a reporting MS

Reporting MS             Initial assessment

Other MS involved        Revision

(Recital 4: future clinical trials will target more specific patients populations, such subgroups identified through genomic information. In order to include a sufficient number of patients it may be necessary to involve many or all MS)

Reporting MS             Consideration of all the revisions
NATIONAL REVISION

• Compliance with the requirements for **informed consent**.

• Compliance of the arrangements for **rewarding or compensating** subjects.

• Compliance of the arrangements for **recruitment** of subjects.

• Compliance with **data** protection rules.

• **Qualification** of persons involved in conducting the trial.

• **Sustainability** of the clinical trial sites.

• Compliance with the applicables rules for the collection, storage and future use of biological **samples**.
THE PROTECTION OF SUBJECTS’ RIGHTS

NATIONAL REVISION

• Information and consent
• Data protection
• Rights related to the samples
• Compensation and rewarding

(There is no a right to participate in a clinical trial as a right to medical treatment)

PARTICIPATION IN THE EVALUATION AND ACCESS TO RESULTS

• The national body in charge of the ethical evaluation should involve lay persons, in particular patients or patients’ organisations
• The sponsor should submit a summary of the results understandable to a layperson.

FREE ACCESS TO THE PRODUCTS IN THE TRIAL

• Subjects should not have to pay for medicinal products and procedures required in the protocol.
THE PROTECTION OF SUBJECTS’ RIGHTS

Information and consent

• Comprehensive, concise, clear, relevant and understandable to a lay person information
  Nature, objectives, benefits, implications and risks
  Possible treatments alternatives
  Protection of rights
  Conditions of the clinical trials (place, duration…)
  (of course in the language of the patient)

• Prior interview

• Free decision
THE PROTECTION OF SUBJECTS’ RIGHTS

Data and samples

• The withdrawal of the consent shall not affect the activities already carried out and the use of data obtained.

• Data can be used for other research if the subject consents (broad consent. Art. 28.2).

• Directive 95/46/EC: Proposal of a new Regulation. Includes samples in its scope (but this could be problematic taking into account national regulations).

• Eurordis Statement on the EP report on the Regulation for the protection of personal data (2013): it is vital that the EU strikes an approriate balance between facilitating the safe and secure use of patient data for health research and the rights and interests of all individuals.
THE PROTECTION OF SUBJECTS’ RIGHTS

Genetic data

• No **binding** international regulation:
  Recomendation of the CE on research with human material, 2006.
  Protocol to the CE Biomedicine Convention on genetic analysis in the healthcare system, 2008 (nhas not entered into force).

• Confidentiality (also regarding family members) and non discrimination.

• Right to know / to ignore (very relevant regarding NGS).

• Right to genetic counselling (accredited professional and center, assessment about treatments or other decisions).
THE PROTECTION OF SUBJECTS’ RIGHTS

Minors

• The subjects should represent the population groups that are likely to use the product investigated.

• The clinical trial is intended to investigate conditions related to minors or is essential to validate data.

• A direct benefit or a benefit for the population represented by the minor is expected.

• The rules concerning the determination of the legal representation are national.

• The minor has the right to receive information adapted to his/her age and mental maturity.

• The explicit opinion of a minor capable of forming an opinion should be respected.

• The minor should consent when reaches the age of legal competence.
CONCLUSIONS

Moving forward…

• Harmonization.

• Transparency.

• Efficiency.

• Recognition of the relevance of some particular conditions in the legal instruments.
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THANKS!