Essential requirements before thinking about a clinical trial

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This webinar is an overview about what should be done at the very least before starting a clinical trial in a study site.

For rare diseases, clinical research is important to provide evidence in a field characterised by few and geographically dispersed patients. As for all the other fields, the clinical studies need to be well-conducted both from the methodological and the ethical point of view.

The investigator needs to know

1) What a clinical trial is

A clinical trial is an investigation on one or more investigational medicinal products in humans with specific pharmacological, pharmacodynamic, pharmacokinetic, safety or efficacy aims, as stated in the GCP guideline. The decision on the treatments and diagnostic procedures is taken outside the clinical practice.

2) Which rules apply

• Good Clinical Practice (GCP) – ICH E6(R2), the main International agreed standard;

• EU Clinical Trials Regulation (CTR) 536/2014 (EU), issued to harmonize multi-national trials in Europe and ruling the trial registration, application to competent authorities including the Ethics Committee approval and authorisation, amendments handling, informed consent and assent;

• Good Manufacturing Practice (GMP), regarding the handling of investigational drugs.

These documents include relevant provisions also coming from other key regulatory documents, such as the Helsinki Declaration.

In case of paediatric trials,

• ICH guidance (E11), as recently updated with an addendum including the most recent innovative methodologies;

• European Commission Ethical considerations on trials conducted with minors (2017)

also apply.

3) How to set up the clinical trial protocol

The protocol should have a strong rationale and explain the available supporting evidence. Its contents are structured according to ICH-GCP.

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4) **Roles and responsibilities**

In the framework of a clinical trial, the investigator could act as

- **Sponsor**:
  responsible for the site identification, registration the trial in the relevant databases, authorisation and the ethics approval, investigational medicines handling, pharmacovigilance and monitoring activities, etc.
- **Coordinating Investigator**:
  responsible for the coordination of investigators at different centres in the same country participating in a multi-centre trial
- **Principal investigator (PI)**:
  responsible at each trial site level. The PI should be able to demonstrate adequate time, facilities, staff and potential to recruit patients according to the protocol. He also needs to report pharmacovigilance data and to manage local issues. In case of paediatric trials, the PI needs further competencies and facilities to undertake the informed consent and assent process, to collect and store samples.

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