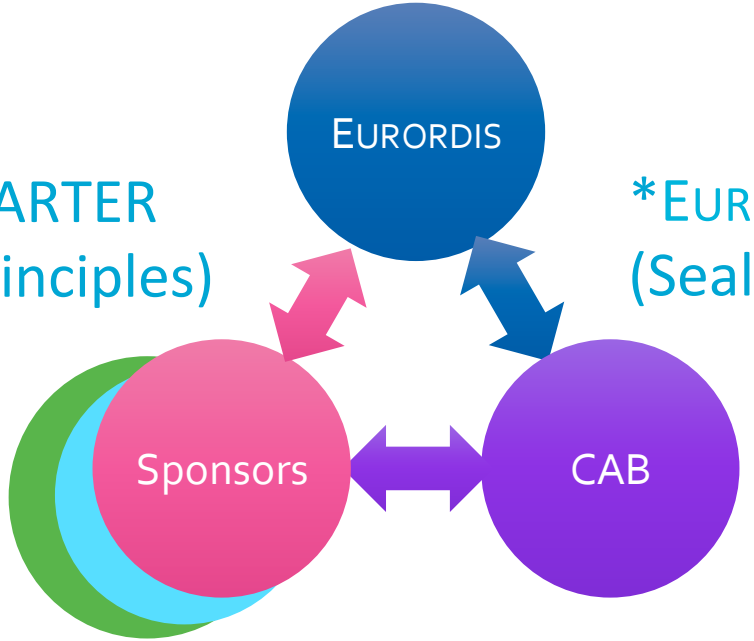
EURORDIS
RARE DISEASES EUROPE

CHARTER & MOU

EURORDIS.ORG

2019 EUROCAB Programme

The CHARTER
(CAB principles)



*EURORDIS CAB status
(Seal, check list)

Agree to an MoU

EURORDIS CHARTER FOR COLLABORATION IN CLINICAL STUDIES IN RARE DISEASES

EURORDIS, the European Organisation for Rare Diseases, represents more than 800 rare disease organisations from 69 countries including all EU Member States, and has emerged as the voice of approximately 30 million patients affected by rare diseases in the European Union. EURORDIS has contributed to the elaboration of the European Regulation on Orphan Drugs and has held seats on the Committee of Orphan Medicinal Products (COMP) at the EMA since its creation in 2000.

EURORDIS launched a process with a number of organisations involved in clinical studies, initially within the Alliance Maladies Rares. This process highlighted the pressing need to define a common framework for collaboration between patients' organisations and sponsors of clinical studies.

This Charter aims at responding to the expectations shared by both patients and sponsors: the rapid

Principles

- This Charter is an expression of mutual intentions and aspirations
- The Charter is not legally binding
- The collaboration is based on respect and is not tokenistic
- The CAB is recognized as an independent body and is not structurally dependent on the sponsor
- The work and the structure is transparent

Principles

- Agendas are cooperatively designed
- The dialogue is meaningful and of high quality
- Collaboration between the sponsors and the CAB is timely, where input can make a difference
- Confidentiality is respected by both sides
- The collaboration is based on trust
- All interactions are considered non-promotional

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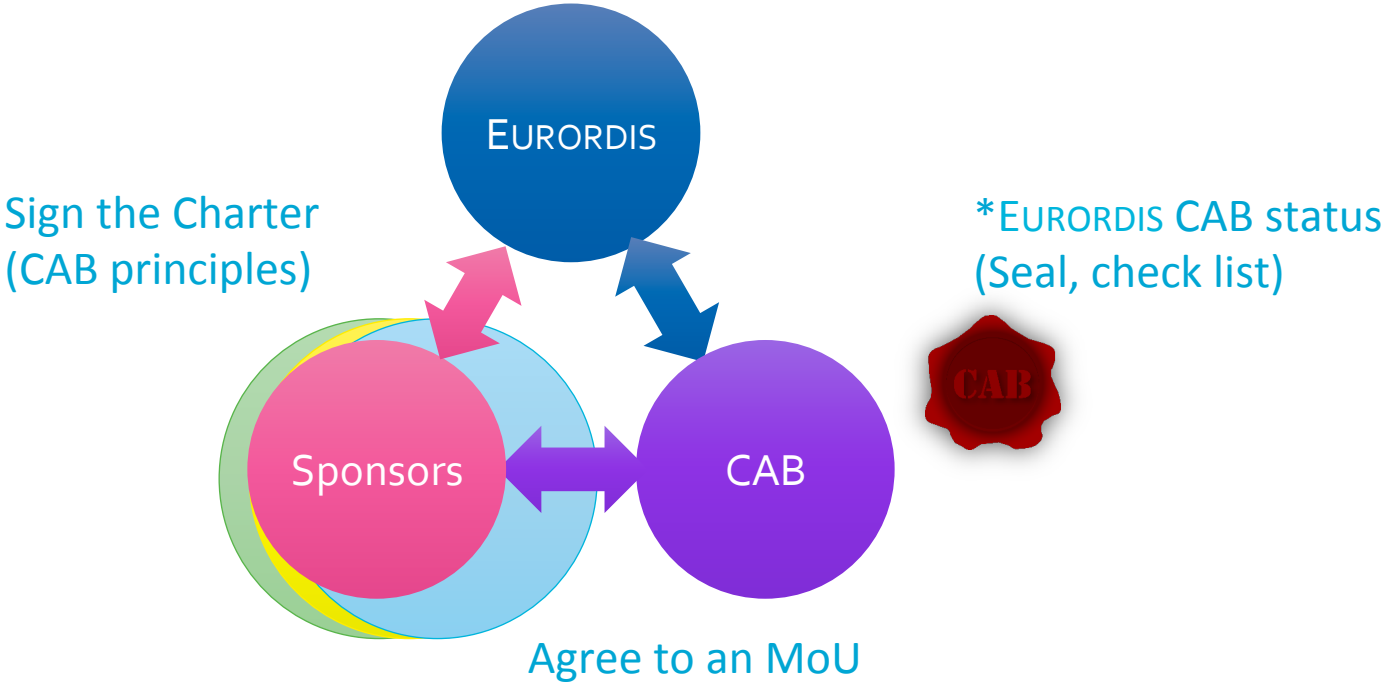

MEMO OF UNDERSTANDING

- A/ Initiators of the research project
- B/ Study design
- C/ Implementation of the study
- D/ Conduct of the Research Study
- E/ Analysis and Dissemination of results
- F/ Financial aspects and commitments

B. Protocol design

The title of the project

The project design, including the type of possible control

The objectives

Endpoints

Constraints for participants

OR NOT

The number of planned participants

Feasibility

The inclusion/exclusion criteria

The evaluation criteria

Information available to patients and when

C. Implementation of the research

The project announcement

Writing patient information documents

Writing / adapting the Informed Consent form

The choice of trial sites

The setting up of a Data & Safety Monitoring Committee, including (or not) a CAB representative

Adverse Events

Discussion of possible interim analyses

OR NOT

D. Analysis and Dissemination of results

The analysis of results

The evaluation of possible benefits, including those based on secondary endpoints and Quality of Life criteria

PRO & PRO measures

Writing scientific papers

Dissemination of results to the patient community

Dissemination of results to the general public

The date(s) of dissemination of results to the different target audiences

OR NOT

E. Financial aspects and respective commitments

- The Sponsor provides financial support to the CAB for:
  - The publication of an information leaflet on the project in all necessary languages (amount)
  - The setting up of a web site (amount), a phone line dedicated to the study (amount), other means of information (amount)
  - Travel expenses for (detailed information) (amount)
  - The publication of a leaflet for the dissemination of results (amount)
  - Other (amount)

OR NOT

Other aspects

Reference for participants during study

In case of negative results

Post-trial access

Compassionate Use

MoU Commitments…

- Collaboration in Systemic Sclerosis and nintedanib

  - FESCA/SSC will announce the study in its Newsletter, on its website, with reference to this MoU
  - FESCA/SSC will support the participants (members or not of FESCA/SSC) during the study
  - FESCA/SSC will contribute to the lay dissemination of the results of the study even in case of negative results

  - Future/ need a Mentor/Admin, they want to make agenda, call meetings, they can find 11 people, want to get in at an earlier stage (than Ph III), how to negotiate better

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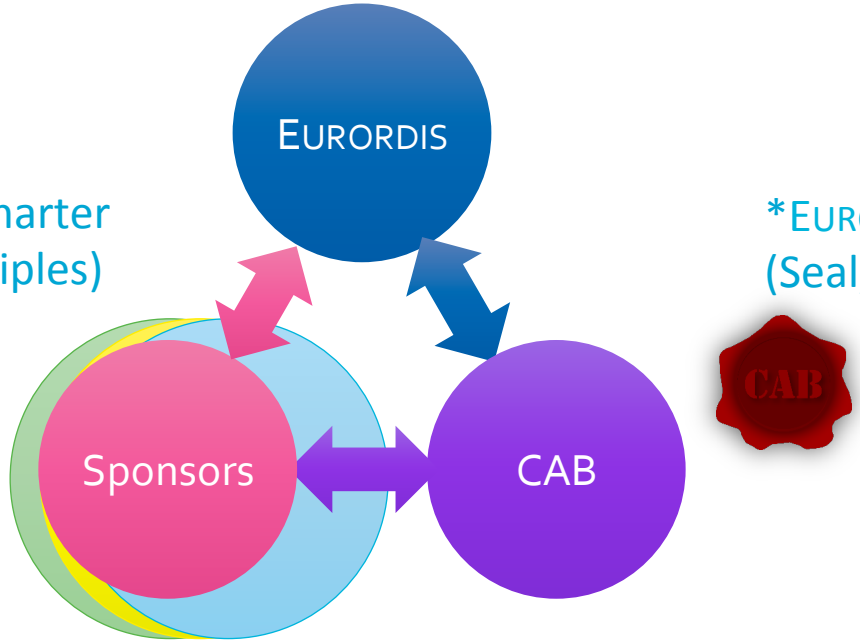

Sign the Charter
(CAB principles)

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