4th of October, 2022

# Cross-border Access to Paediatric Clinical Trials

## Enpr-EMA Working Group

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# WORKING GROUPS MEMBERS:

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CT NH Network Chair
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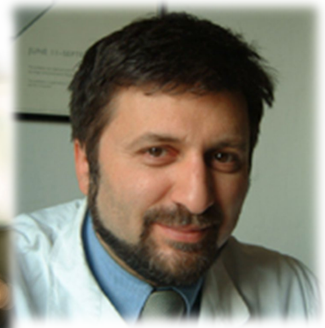

**Dr. Luca Sangiorgi**

ERN Coordinator
(Italy)

**Begonya Nafria (Chair)**
Patient Engagement in Research Senior Expert - Sant Joan d Déu

# Objectives of the working group:

**PRIMARY OBJECTIVE**:

Develop guidance to facilitate the inclusion of paediatric patients living with rare diseases in cross-border clinical trials in Europe avoiding language discrimination.

**SECONDARY OBJECTIVES**:

1. **Identify if there are cases of discrimination** against paediatric patients not being accepted as participants in a clinical trial abroad.
2. **Analyse the reasons for such discrimination** from the scientific and ethical perspectives.
3. To review the **current evidence on cross-border clinical trial access in the EU** and frameworks on its implications ( ie cultural, legal, ethical, operational)
4. Create a consensus **guidance to facilitate the experience of patients in cross-border clinical trials** that will validated by different stakeholders.

# Working plan (Sep 2022- Sep 2024)

## PHASE Ia

- Clinical Trial Sites
  - Survey
    - Use of language as eligibility criteria
    - Country of residence as eligibility criteria
- Patients' Organisations
  - Survey
    - Use of language as eligibility criteria
    - Country of residence as eligibility criteria

Creation a pool of data based in the analysis of the state of the art.

## PHASE Ib

Clinical trial protocol analysis based in the case studies identified:

1. Identification of the scientific (PROMs and QoL scales)

and,

2. Ethical and scientific reasons behind the use of language and country of origin as exclusion criteria

Clinicaltrials.gov

+

Studies identified in the survey phase

## PHASE II

CONSENSUS GUIDANCE. Different steps:

1. Draft of the Guidance. Version 0
2. EnprEMA internal consultation of the version 0:
   - 50 European Clinical Research Networks
3. Guidance Version 0.1
4. Public consultation addressing all stakeholders
5. Guidance Version 0.2
6. Stakeholders' interviews to validate the guidance document
7. Final version 1.0

# Working plan (Sep 2022- Decemer 2022)

**Clinical Trials Sites Survey**

- Sociodemographic data
- Good practices
- Cases of discrimination

- Assent/consent
- PROMs and QoL tools
- Other information to patients

**Focus Groups with Networks & Sites Interviews**

- Sociodemographical data
- Good practices
- Cases of discrimination

- Assent/consent
- PROMs and QoL tools
- Other information to patients

**Patient Organizations Survey**

- Focused in the patient experience:
  - Good practices
  - Cases of discrimination

- **Pending validation with patients organizations across Europe**

**2022**

**November:**

**Ethics Committee submission**

- Open to all the Sites

- Invitations to networks
- Invitations to sites to have European geographical representation

- Open to RRDD organizations
- Translation to European languages
- Invitations to sites to have European geographical representation

# Working plan (2023)

Jan | Feb | Mar | Apr | May | June | July-December

**Clinicaltrials.gov**

Computer analysis program: identification of the studies that accomplish the inclusion criteria (language / country of residence)

**Clinical Trials Sites + Patient Organizations Surveys**

**Focus Groups with Networks & Sites Interviews**

**Data Analysis**

**Scientific and ethics assessment of the cases**

**Scientific publication**

# Working plan – Consensus Guidance (2024)

**Jan** | **Feb** | **Mar** | **Apr** | **May** | **June** | **July-December**

Creation of the Guidance by the Enpr-EMA WG

Internal consultation to the networks of Enpr-EMA

Consolidation version 1

Public consultation to multistakeholders

Interviews to multistakeholders

Consolidation of the final versión

Scientific publication

Thank you