

Welcome!

We will be starting shortly...



Use your real name



Mute yourself when not speaking



Turn your camera on, if comfortable



Use the chat box for questions



Or raise your hand to use your microphone

Please note that this webinar will be recorded.

Workshop for patient representatives *'Train the trainers'*

16 & 17 September 2020

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PPI team

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Maria Cavaller (Patient Engagement Junior Manager, EURORDIS)

Gulcin Gumus (Research and Policy Project Manager, EURORDIS)

#conect4children



Objectives of the workshop

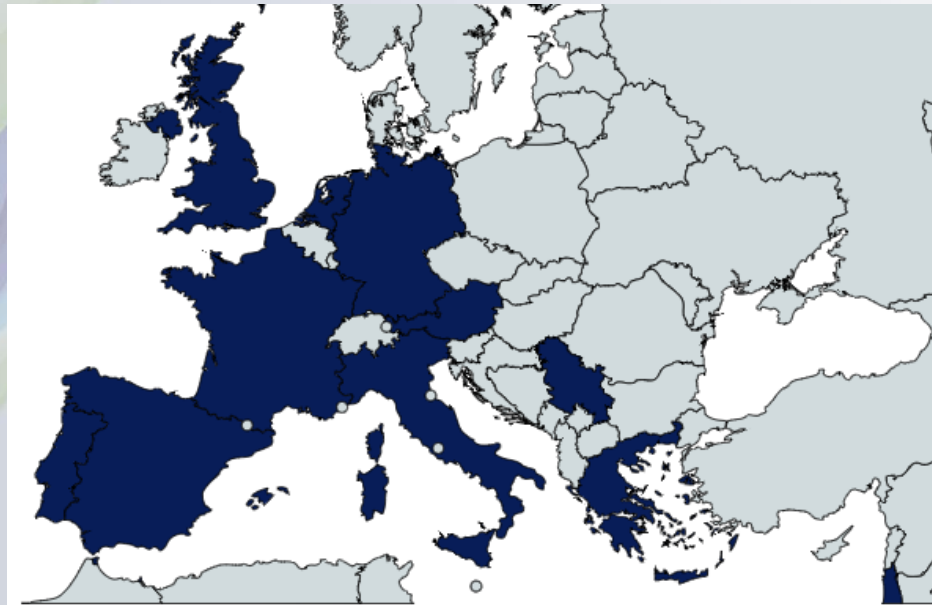
- Building capacity on the life cycle of medicines.
- Educate participants to support patients/parents involvement at the European level for paediatric clinical trials.
- Describe and demonstrate the different ways patients can be engaged within the c4c project.

Today's programme

- 9-9.30 am: *Welcome, Introduction c4c project and Objectives*
- 9.30-11am: *Children and Young Patients and families (CYP) Involvement: Why? and How?*
- 11-11.30 am: *Comfort break*
- 11.30-12.30 pm: Ethics session *'Ensuring the ethical acceptability of a research proposal involving children - a framework for all'*
- 12.30-13.30 pm: *Regulatory session*

36 patient representatives & 2 of our c4c colleagues

From 12 countries, 20+ diseases



Sparkup Word cloud

<https://eurordis.sparkup.live>

Code: EEEKZ





conect4children: Collaborative network for European Clinical Trials for Children

Vision

- Better medicines for babies, children and young people through a pan-European clinical trial network
- **c4c** is using a coordinated approach to deliver high quality “regulatory grade” clinical trials in:
 - Multiple countries
 - Multiple sites
 - All paediatric age groups



Planning, set-up & conduct of a Paediatric Development Program

A multifaceted challenge...

Defining the medical need

Right indication and population

Preparing and agreeing a Paediatric Development Plan

Small patient populations – competing developments

Use/acceptance of innovative study designs

Insufficient trial infrastructure

Divergent view of Ethic Committees

Contradictory local regulations

Diverse standard of care across Europe

Impact on daily lives of patients and families

Dose, route of administration, application device

Acceptance of Paediatric research in society

Key Objectives

- a) a **single point of contact** for all sponsors, sites and investigators
- b) efficient **implementation** of trials, adopting consistent approaches, aligned quality standards and coordination of sites at national and international level;
- c) collaboration with **specialist** and national networks;
- d) high quality input into study design and preparation, through rigorous strategic and operational **feasibility** assessment;
- e) the promotion of **innovative** trial design and quantitative scientific methods;
- f) an **education** and training platform to shape the future leaders of paediatric drug development;
- g) the development of **sustainable** support for all these activities.

Private-public partnership between Academia and Pharma



Penta
Child Health Research

Radboudumc



Karolinska Institutet



ARISTOTLE UNIVERSITY OF THESSALONIKI

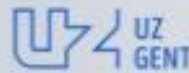


Sant Joan de Déu
Research Foundation

swiss clinical trial organisation



Swiss Research Network of Clinical Pediatric Trials



Newcastle University



UNIVERSITY OF TARTU

UniversitätsKlinikum Heidelberg



Organisation Kindererzweiforschung
Initiative for Children Research Organization - o.k.ids



European Reference Network
for rare or low prevalence complex diseases



SERVIZO GALEGO de SAUDE



MCRN HUNGARY
MEDICINE FOR CHILDREN RESEARCH NETWORK



Network Hereditary Metabolic Disorders (HerMetD)

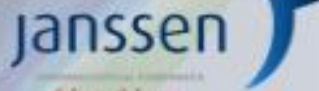


University College Cork, Ireland
Coláiste na hOllscoile Corcaigh



EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK

AIDFM CETERA



Hospital General Universitario Gregorio Marañón
Universidad de Madrid



EURORDIS
RARE DISEASES EUROPE



INCiPiT
Italian Network for Paediatric Clinical Trials

Nasjonalt kompetansenettverk for legemidler til barn



UNIVERZITA KARLOVA



Benefits to the **paediatric community**

- **Harmonized, streamlined procedures** across the trial lifecycle
- Opportunities to **build economies of scale** at site and national level
- Reducing barriers to entry and so making paediatric research more attractive and **competitive**
- Access to a wide range of study sponsors through a **transparent, evidence-based, network-wide vetting procedure**
- Input from **relevant specialty networks** and methodologists on study design, implementation and assessment
- The specific **medical needs of children** at the foreground

Sparkup poll

<https://eurordis.sparkup.live>

Code: EEEKZ



Let's start!



innovative
medicines
initiative

