

# 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

Maria Cavaller (Patient Engagement Junior Manager, EURORDIS)  
Gulcin Gumus (Research and Policy Project Manager, EURORDIS)

# PPI team

**Jennifer Preston** (Senior Patient and Public Involvement Manager, University of Liverpool)

**Pamela Dicks** (Network Manager, Scottish Children's Research Network, ScotCRN)

**Begonya Nafria** (Patient Engagement in Research Area Coordinator, FSJD)

**Nuria Noel** (Patient Engagement in Research Area, FSJD)

**Joana Claverol** (Clinical Research Unit Manager, FSJD)

**Eric Vermeulen** (Policy Officer Research, VSOP)

**Mariette Driessens** (Policy Officer Research, VSOP)

**Aisling Walsh** (Senior Project Manager, EFCNI)

**Loïc Notelet** (Trial Operations Program Manager, Sanofi)

**Segolene Gaillard** (Project manager - Methodology adviser – Paediatrics, Hospices Civils de Lyon)

**Katherine Cheng** (Senior Director, Paediatric Drug Development, Janssen)

**Maria Cavaller** (Patient Engagement Junior Manager, EURORDIS)

**Gulcin Gumus** (Research and Policy Project Manager, EURORDIS)

**#conect4children**



# Objectives of the workshop

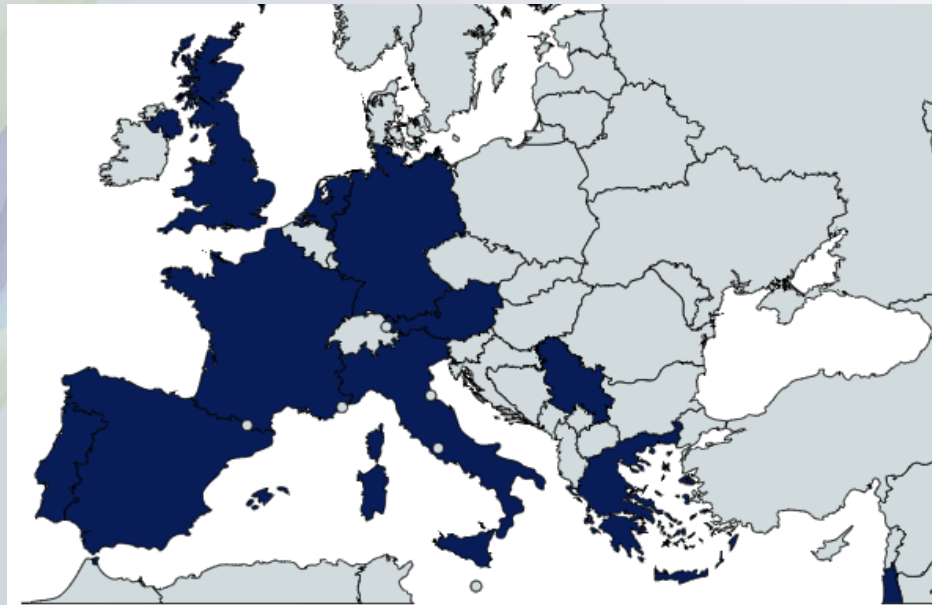
- Building capacity on the life cycle of medicines.
- Educate participants to support patients/parents involvement at the European level for paediatric clinical trials.
- Describe and demonstrate the different ways patients can be engaged within the c4c project.

# 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



Bambino Gesù  
Istituto per la Salute



Penta  
Child Health Research

Radboudumc



ARISTOTLE  
UNIVERSITY  
OF THESSALONIKI



Karolinska  
Institutet



Sant Joan de Déu  
Research Foundation

swiss  
clinical  
trial  
organisation

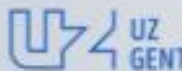
SWISS  
PEDNET

Swiss Research Network of  
Clinical Pediatric Hubs

Inserm



Newcastle  
University



FONDAZIONE  
PER LA RICERCA FARMACOLOGICA  
GIANNI BENZI



Robert Koch Krankenhaus



Neuromuscular Network



UNIVERSITY OF TARTU

UniversitätsKlinikum Heidelberg



SERVIZO  
GALEGO  
de SAÚDE



Organisation Kinderarztnetzforschung  
Initiative for Children Research Organization - AiRiO



THE CHILDREN'S MEMORIAL HEALTH INSTITUTE



HUS



European  
Reference  
Network

for rare or low prevalence  
complex diseases

Network  
Hereditary Metabolic  
Disorders (HerMetNet)



MCRN HUNGARY  
MEDICINE FOR CHILDREN  
RESEARCH NETWORK



IKP  
STUTTGART



University College Cork, Ireland  
Coláiste na hOllscoile Corcaigh



ECRIN  
EUROPEAN CLINICAL RESEARCH  
INFRASTRUCTURE NETWORK

AIDFM CETERA



janssen



Hospital General  
Universitario  
Gregorio Marañón



EURODIS  
RARE DISEASES EUROPE



INCiPiT  
Italian Network for  
Paediatric Clinical Trials



Nasjonalt kompetansenettverk  
for legemidler til barn



efpia

connect  
4children



UNIVERZITA KARLOVA



PRINTO



NOVARTIS



# 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!