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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Gulcin Gumus (Research and Policy Project Manager, EURORDIS)
PPI team

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#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...*

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<tr>
<th>Defining the medical need</th>
<th>Right indication and population</th>
<th>Preparing and agreeing a Paediatric Development Plan</th>
<th>Small patient populations – competing developments</th>
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<tbody>
<tr>
<td>Use/acceptance of innovative study designs</td>
<td>Insufficient trial infrastructure</td>
<td>Divergent view of Ethic Committees</td>
<td>Contradictory local regulations</td>
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<tr>
<td>Diverse standard of care across Europe</td>
<td>Impact on daily lives of patients and families</td>
<td>Dose, route of administration, application device</td>
<td>Acceptance of Paediatric research in society</td>
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Version 2.0 dd-mm-yyyy
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

[Logos of various academic and pharmaceutical institutions]
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!