The c4c project: a comprehensive overview

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Vision

Better medicines for babies, children and young people through a pan-European clinical trial network
A pan-EU Paediatric Clinical Trial Network  
A project under the EU Innovative Medicines Initiative (IMI)

- Ensure **efficacy, safety & quality** of health products
- **Reduce time** to clinical proof of concept
- Improve the current **drug development process**
- Develop **new therapies** for diseases with **high unmet need & limited market incentives**
- Allow **engagement** in a cross-sector, multi-disciplinary consortium at the forefront of cutting-edge research
Private-public partnership between Academia and Pharma
6 years to deliver

<table>
<thead>
<tr>
<th>Type of study</th>
<th>Industry/non-industry</th>
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</thead>
<tbody>
<tr>
<td>Intervention</td>
<td>Drug, biologics, devices</td>
</tr>
<tr>
<td>Geography</td>
<td>Europe</td>
</tr>
<tr>
<td>Phase of study</td>
<td>Ph 1- 4; registry studies, non-interventional</td>
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<tr>
<td>Endpoints</td>
<td>PK/PD, efficacy, safety</td>
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<tr>
<td>Responsibilities</td>
<td>The <strong>c4c</strong> network will provide some <strong>central services for trials</strong>, for example, trial feasibility, pharmacovigilance activities and commissioning of trial supplies. Other operations to be supplied by the sponsor</td>
</tr>
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Version 1.0
**Mission**

**c4c** will use a coordinated approach to deliver high quality “regulatory grade” clinical trials in:

- Multiple countries
- Multiple sites
- All paediatric age groups

by supporting:

- **Trial implementation** using resources shared between studies
- **Trial design** through a combination of information about natural history, feasibility, appropriate innovation, and expert opinion
- **Education and awareness** within and beyond the network

**Relevant to ERNs**

Version 1.0
The c4c consortium members

- 10 EFPIA companies
- 18 pediatric national networks
- 2 large patient advocacy groups
- 8 EU Multinational sub-specialty Networks
- 200 large children’s hospitals
Challenges when conducting paediatric trials

Lack of experience in designing & conducting Paediatric studies by (industry) sponsor

Assessment of site capability, patient availability, and feasibility of trials is often inaccurate

Most new paediatric trials require a new network of sites to be built

Most sites negotiate their own CDAs, contracts, budget templates, and IRBs/EC approvals to prepare for studies

Many sites are inexperienced, poorly trained, and under-resourced

Poor study design
Poor feasibility
Poor site engagement
Inefficiency

Poor/delayed study delivery

Relevant to ERNs
Project Concept

1. Use resources and funding of the IMI project to setup the network and its processes
   1. National
   2. International

2. Demonstrate the value of the network approach with selected
   1. Studies
   2. Sites

3. Generalise from the demonstration projects to the broader network

Avoiding duplication
Project Concept

1. Use resources and funding of the IMI project to setup the network and its processes
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   2. Sites

3. Generalise from the demonstration projects to the broader network
   From
   • Specific
   • Selected

   To
   • Generalised
   • Sustainable

From 2019
To 2024
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

Relevant to ERNs
Key Objectives

• More efficient trial implementation through the set-up of **national hubs** and qualified sites
• Input in clinical trial design and implementation from **pilot expert advisory groups** and other fora
• **Educational programme** for health professionals and **awareness raising campaigns** for the general public
• Identification of **Data standards** and performance metrics
• Business cases for **sustainability** beyond IMI funding
Key features

• **International network** with lean central coordination
• A single point of contact (**One-stop-shop**)
• Efficient **implementation** of trials
• Consistent procedures **across sites**
• Strategic and operational **feasibility assessment**
• Involvement of experts to develop **innovative trial designs and methodology**
• **Multi-KEY stakeholder collaboration**
Global Paediatric clinical trial networks

The c4c network will collaborate with other existing networks

- DCRI-GPN (Global Pediatric Network – Duke)
- i-ACT
- Kids CAN
- Japan
  - Japanese Pediatric Network for Drug Development
- Australia
  - Pediatric Trials Network of Australia (PTNA)
- Europe
  - c4c
  - PENTA-ID /PRINTO/CF/SIOPE/ECRIN...
Benefits for sponsors in placing a study with c4c

- High quality input in study design and preparation through rigorous strategic and operational feasibility assessment
- **Efficient implementation** by adopting consistent approaches, aligned quality standards and coordination of sites at national and international level
- A **single point of contact** for all sponsors, sites and investigators
- **Collaboration** with EU pediatric specialty networks
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
Expected long term impact of c4c

- **Access to new experimental therapies** for children in well-designed clinical trials
- Better training for research personnel and **improved trial readiness** at all participating sites
- **Improved efficiency** in executing trials (faster, cheaper)
- **Improved data quality** for labelling of next generation medicines for children
- Enhanced role of **clinicians and patient/parent advocacy groups** in planning and designing studies
- **Broadening the access** of academic medical centers and clinical faculty across Europe to new experimental therapies

Version 1.0
How c4c will be put to the test

- **Proof-of-viability trials**
  - Industry sponsored
  - Non-industry-sponsored

- Selecting and comparing metrics about studies’ start-up and conduct