INTRODUCTION TO c4c: A PAN-EUROPEAN CLINICAL TRIALS NETWORK

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This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.

Vision

Better medicines for babies, children and young people through a pan-European clinical trial network

MISSION

c4c is using a coordinated approach to deliver high quality “regulatory grade” clinical trials in:

- Multiple countries
- Multiple sites
- All paediatric age groups

by supporting:

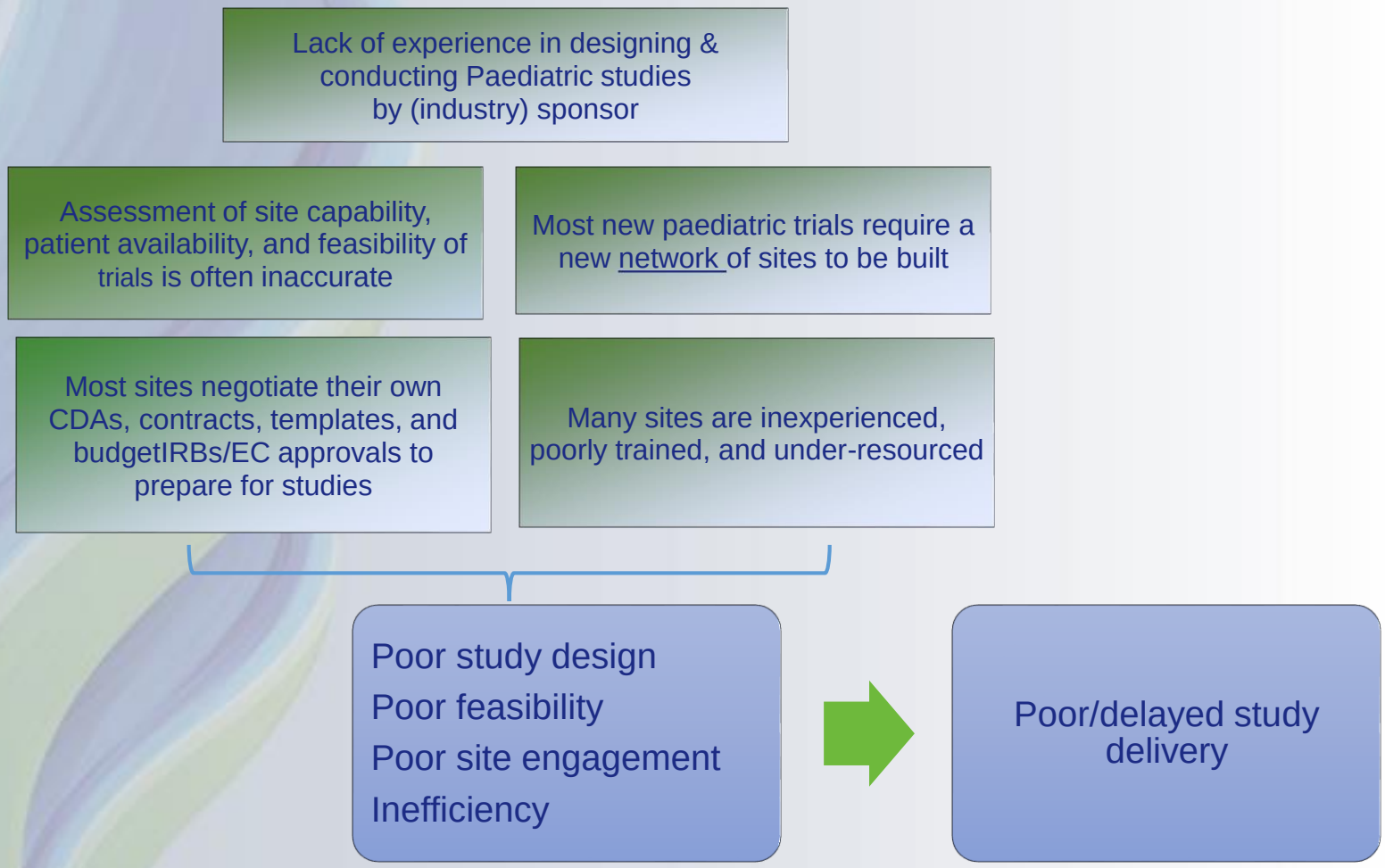
- Trial implementation using resources shared between studies
- Trial design through a combination of information about natural history, feasibility, appropriate innovation, and expert opinion
- Education and awareness within and beyond the network

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

A multifaceted challenge…

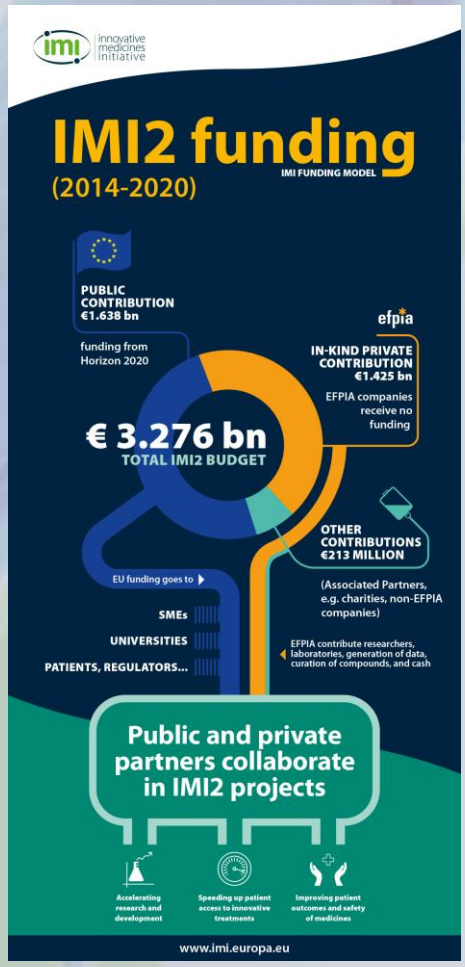
Defining the medical need	Right indication and population	Preparing and agreeing a Paediatric Development Plan	Small patient populations – competing developments
Use/acceptance of innovative study designs	Insufficient trial infrastructure	Divergent view of Ethic Committees	Contradictory local regulations
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

Challenges when conducting paediatric trials



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
- Ensure the **voice of patients** is heard to safeguard better treatments for children

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

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Current status

19 National Hubs operational

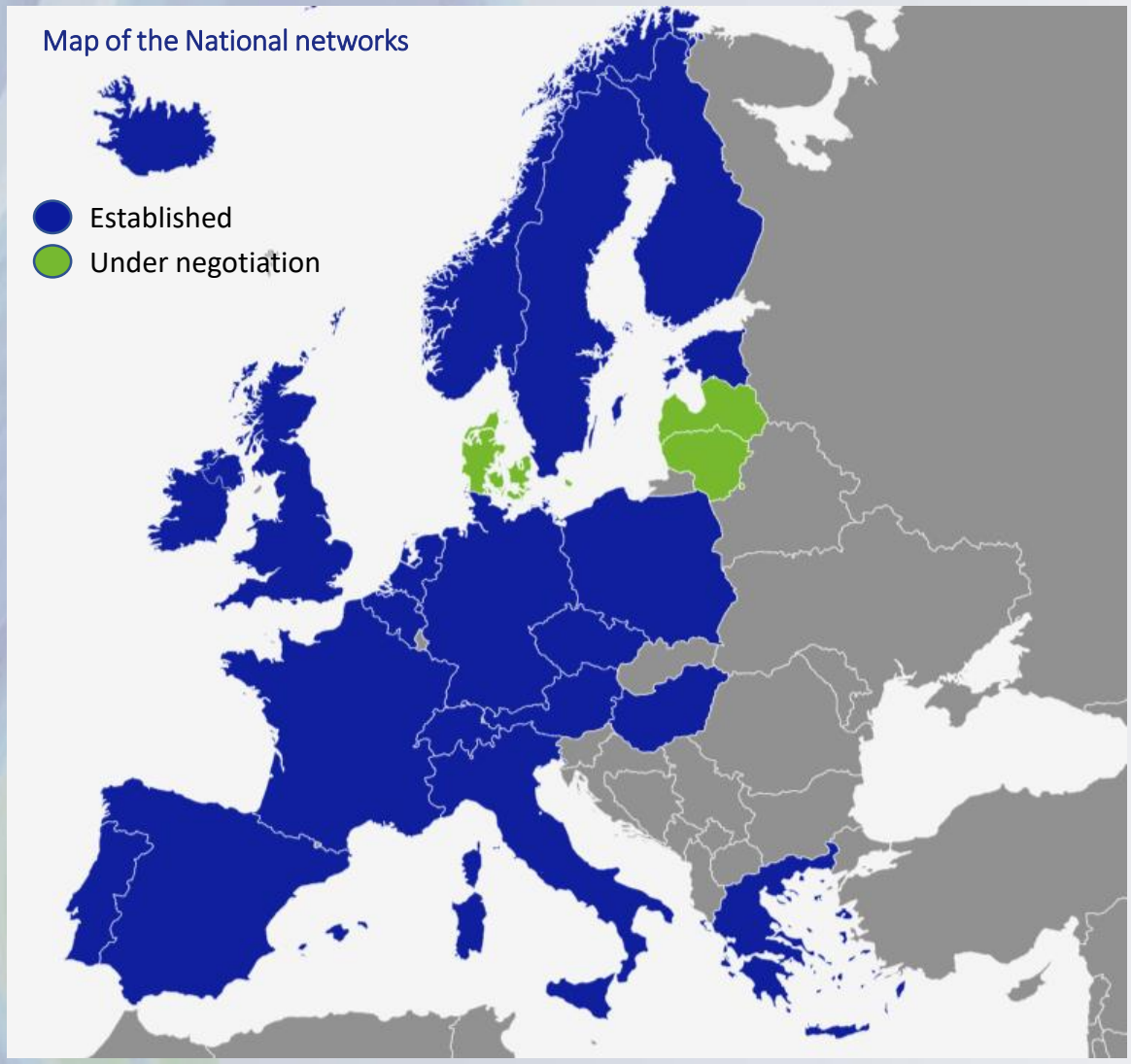
Expert group charter co-produced

Educational portal operational

Standards and metrics in co-production

Business case in co-production

The c4c Consortium members



- 10 EFPIA companies
- 19 paediatric national networks established (Iceland and Finland one single network)
- 3 paediatric national networks under negotiation
- 2 large patient advocacy groups
- 8 EU multinational specialty networks
- 3 global research networks
- 200 large children's hospitals

To know more about the c4c Consortium visit:
www.conect4childen.org

Budget & EFPIA In-kind contributions

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Company
Janssen*
Bayer*
Roche*
Novartis*
GSK
Sanofi
Servier
Pfizer
Lilly
UCB

EFPIA in-kind 73M Euros
IMI Funding 67M Euros

*companies contributing clinical trial(s)

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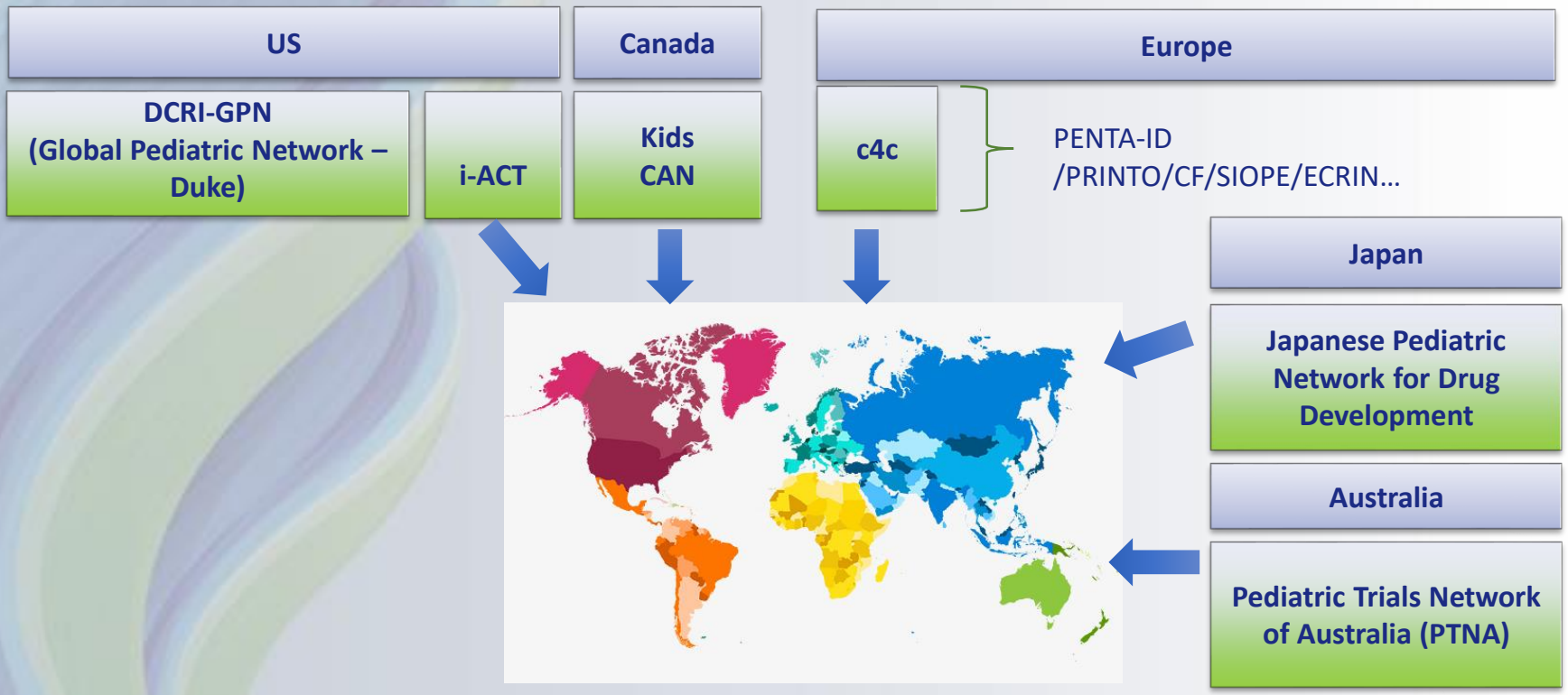
Private-public partnership between Academia and Pharma

Global Paediatric clinical trial networks

The **c4c** network collaborates with other existing networks



c4c Achievements

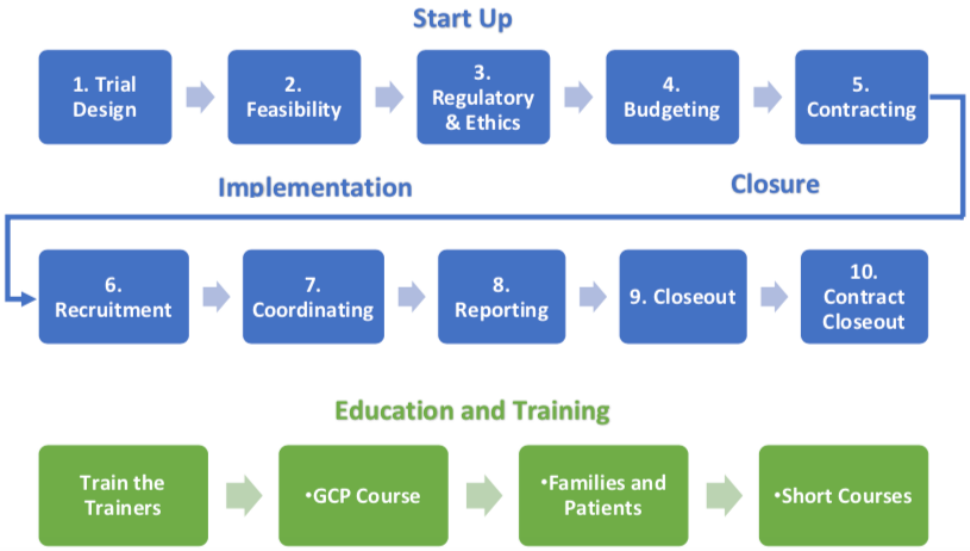
How c4c is being tested

- Evaluating metrics about start-up and conduct of
- Proof-of-viability trials
  - 4 Industry sponsored (opening 2021)
  - Non-industry-sponsored (opening 2020)
    - 57 expressions of interest
    - 27 full applications
    - 11 shortlisted

Support for Proof of viability studies

- Single Point of Contact
- Initial meetings with all trial teams
- Guidance document describes modular approach to trial support
- Modules being rolled out

Figure 1: Trial Stages and corresponding c4c support



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
- The specific **medical needs of children** at the foreground

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

Scope of c4c (at end of year 6)

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

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COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

Project Leadership Team

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imi innovative medicines initiative

efpia

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