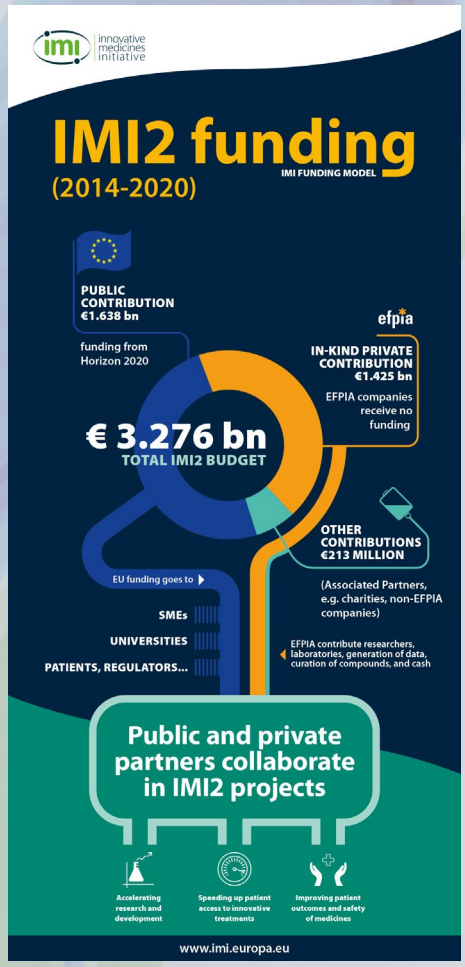
A pan-EU Paediatric Clinical Trial Network

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

EnprEMA September 28th 2021

Private-public partnership between Academia and Pharma

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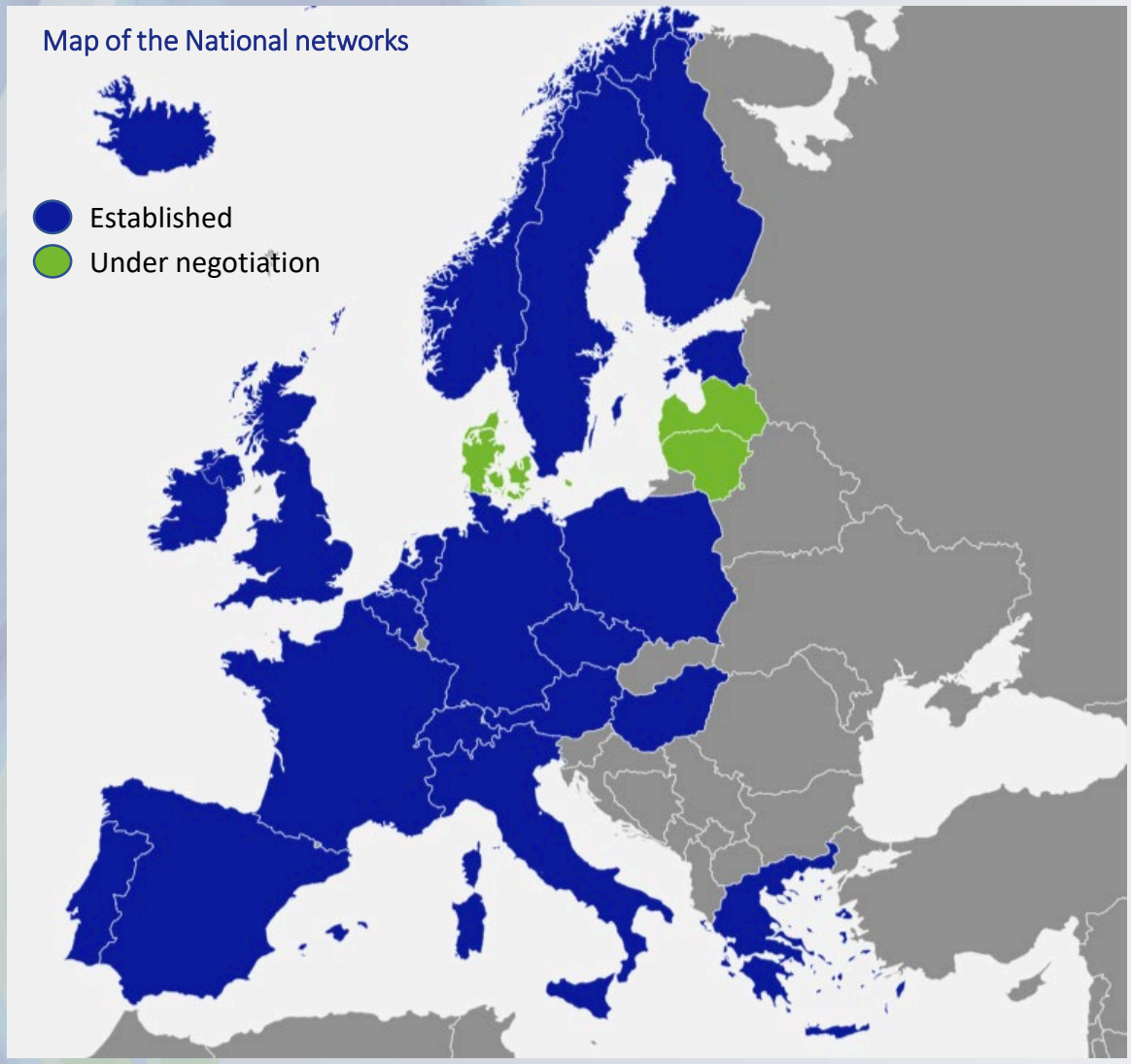
The c4c Consortium members



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

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

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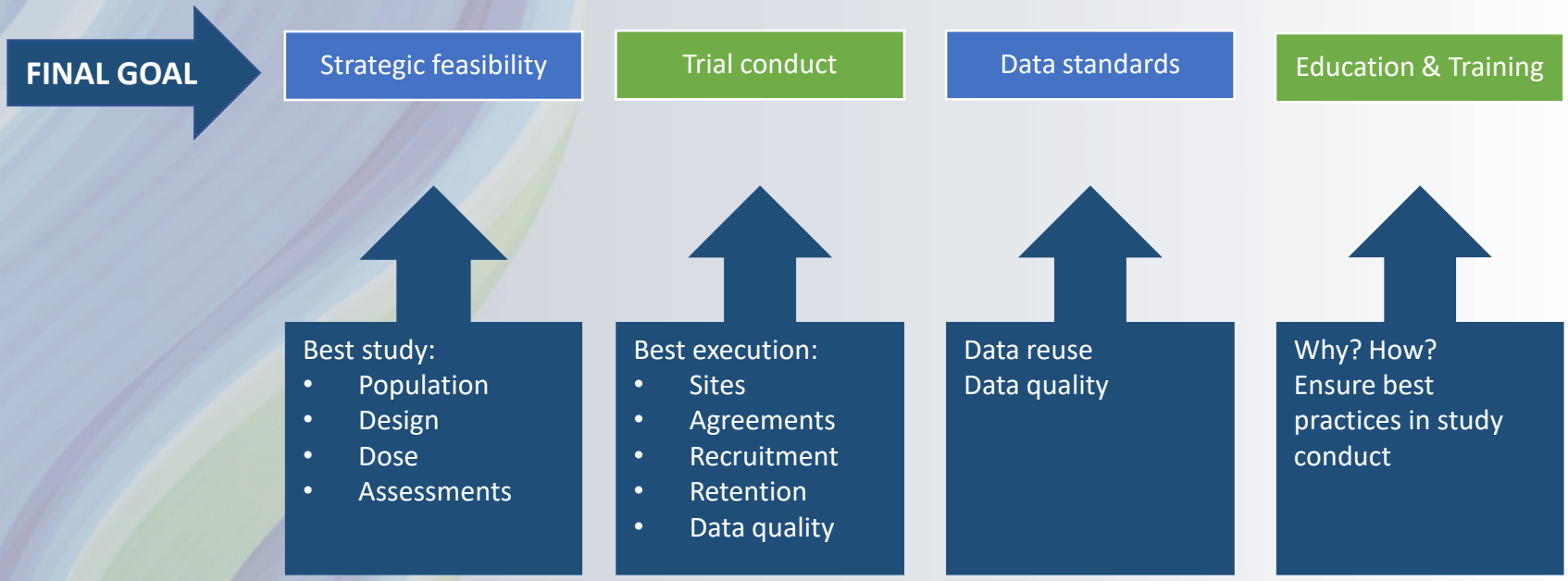
c4c services

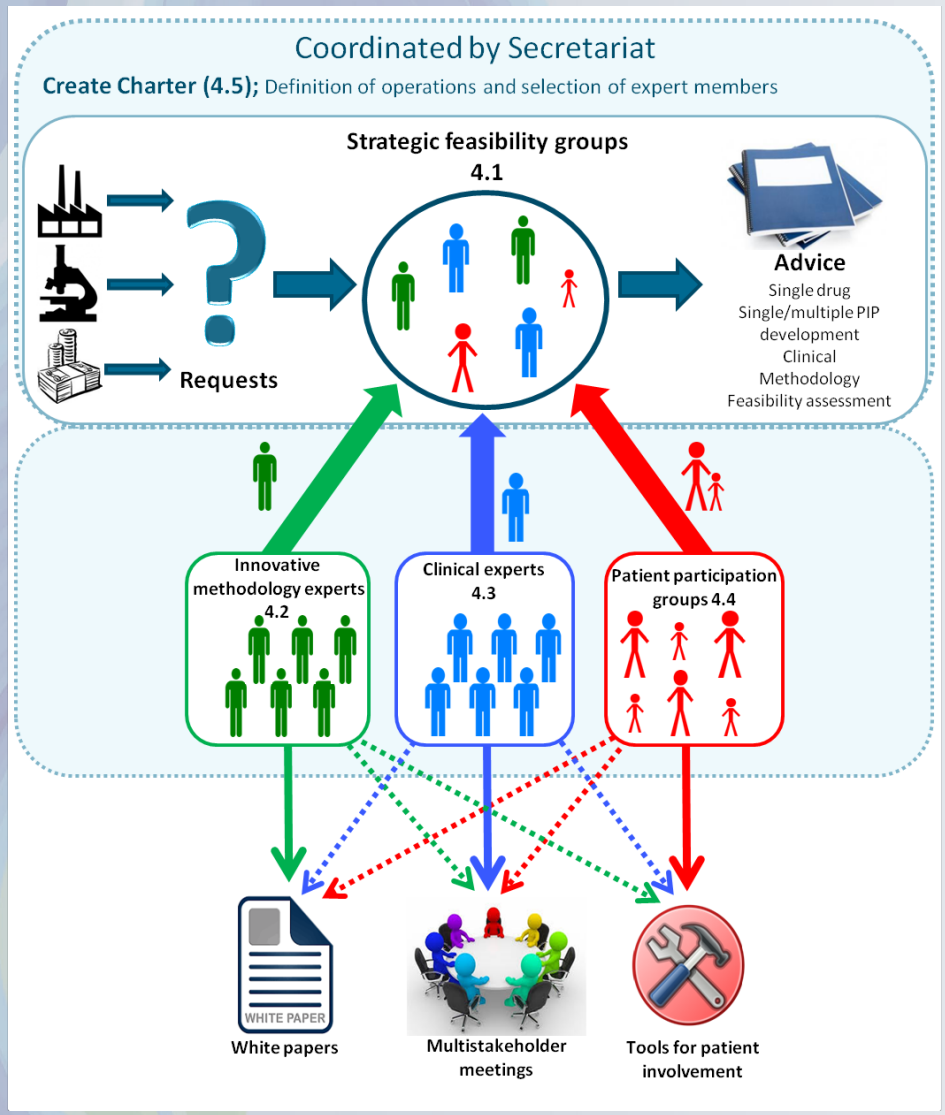


Currently, within the consortium because of the Grant Agreement

c4c services

	Strategic feasibility	Trial conduct	Data standards	Education & Training
FINAL GOAL				
2021	Standing college of experts established 26 requests 1 stakeholder meeting	20 National hubs 240 sites 3 non-industry trials open 5 industry studies in feasibility	Integration with CDISC Paediatric data dictionary	30 courses >1000 users Involving young people in research Advanced course recruiting
2024	Open to all	High-quality sites and data supported by national and international coordination: open to all	Integration across trials Data reusability Integration across sources	Education for Young Patients and Parents Education for hospital administrators Concept for public campaign

Strategic Feasibility Advice



- Provide Strategic Feasibility advice
- Provide guidance to assure innovative development plans
- Organize multi-stakeholder strategic discussions
- Integrate children and young people in strategic feasibility

Standing Expert Groups

Clinical		Methodology
Adolescent Medicine	Nephrology	Developmental pharmacology
Cardiology	Neuromuscular diseases	Ethics
Endocrinology & Diabetes	Neuroscience & Epilepsy	Formulations
Gasteroenterology & Hepatology	Oncology (incl. heamatology)	Health Technology Assesment
Infectious diseases & Vaccinology	Psychiatry	Pharmacogenomics and other Omics technologies
Intensive care	Respiratory	Pharmacometrics
Metabolic diseases	Rheumatology & Autoimmune diseases	Pharmacovigilance
Neonatology	RSV	Study design & Clinical trial methodology

PPI	
YPAGs	Parents/caregivers
Patient organisations	Patients

If needed other Experts can be assigned, via our extended network

Achievements

Over 25 advice requests received

7 advice requests with PPI participation

Centralized contracting structure tested

1st (virtual) Multi-Stakeholder Meeting on IBD held

Quality manual developed

Tool to asess ROE & ROI of PPI developed

Time to review the service?

Could c4c share its experience of strategic feasibility advice with PDCO?

How c4c is being tested

Proof-of-viability trials

- 3 non-Industry sponsored (all open)
  - TREOCAPA (prophylaxis of PDA in preterm neonates)
  - KD-CAAP (treatment of Kawasaki disease)
  - cASPerCF (dosing Posaconazole in CF)

Industry proof of viability studies

Janssen: Inflammatory Bowel Disease, RSV

Bayer: Hypertension in CKD

Novartis: Atopic Dermatitis

Roche: MS

Working with others

- EJP RD
- ERICA
- ECRIN
- EUCROF
- EFPIA
- iACT

Sites – location, standards, coordination

Trials – design, implementation

Data sharing

Education and training

Each group needs a specific scope and to match services to that scope

c4c Academy

TRAIN THE TRAINERS COURSE

FAMILIES AND PATIENTS INVOLVEMENT COURSES

POV STUDIES' TRAINING COURSES

GOOD CLINICAL PRACTICE COURSES

SHORT THEMATIC COURSES

ADVANCED COURSE

Next steps towards c4c sustainability

Decisions need to be made…

Selection of Host Country for c4c central organization **M38**

- Desicion process to be aligned with key stakeholders of c4c consortium
- Desicion on host country to be based on **Results of SWOT Analysis, Legal Scenarios as decribed in D3.4 and first iteration of the Business Case**
- Based on the scenarios generated under this task, the **PSC** will select the most suitable successor form and propose to **GA** for final decision

Legal form and organizational structure (service distribution) **M39 - M46**

- Based on outcome of legal evaluation (D3.4), service portfolio and initial BC Subtask 1.10 to develop scenarios for future legal form and organizational structure and discuss possible scenarios with futre service providers and experts for taxation and corporate law of the host country (M41)
- Subtask 1.10 to develop proposal and present to PSC for approval (M46)

Ratification and Implementation **M46 onward**

- PSC to present final proposal to GA for ratification
- Task 1.10 start and initiate implementation of new legal form
- Task 1.10 in close connection with WP3 to develop transition plan and initiate close stakeholder management

WP3 Work Plan towards sustainability

Business model assessment — **M40**

- Comprehensive information from market analysis, network capacity and fee schemes

Feedback from main public/private stakeholders on Business Model — **M42**

- Report on the feedback received from relevant stakeholders (e.g. Trade organisations at national and international level)
- Report will be presented to international, national and regional funding bodies

Stress test of the business model — **M72**

- Initial small pilots of relevant services: include the ROE of the patient centric initiatives; pilots will be executed in order to explore how the business models fits in real settings. How to disseminate and test services behind IMI setting

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

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

Project Leadership Team

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THANKS FOR YOUR ATTENTION!

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