conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

c4c aims to enhance the development of Better Medicines for babies, children and young people through a pan-European clinical trial network

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How things have developed...

Since the first implementation of the Paediatric Regulation, we have seen...

- An ever-increasing number of Paediatric Investigation Plans
- Continuous increase in Paediatric clinical trials and the number of children to be involved
- A growing number of new treatment options being approved for use in children

But also:

- About 40% of PIPs are not completed as planned
- Increased competition between studies about shared resources (Investigators, sites, patients)

A multi-faceted challenge…

Finding the right indication and population	Lack of sufficient trial infrastructure	Diverse standard of care across Europe
Use/acceptance of innovative study designs	Impact on daily lives of patients and families	Divergent Ethical standards
Lack of appreciation of need for clinical research in children in society	Contradictory local regulations	Small patient populations – competing developments

A pan-EU Paediatric Clinical Trial Network

A project under the EU Innovative Medicines Initiative (IMI2)

- 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

CONECT4CHILDREN

COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

Why PPP

The **paediatric clinical trial infrastructure in the EU is fragmented and not sufficiently developed**.
A broad **multidisciplinary public-private collaboration is required to meet the challenges** and to be transformative and to **collectively address children's needs for better medicines**.

Impact

Improved paediatric development plans and study designs
More efficient implementation and conduct of Paediatric clinical trials
Improved data quality, better trial feasibility and faster enrollment

Status & Value

Expert advice and patient/parent involvement
Access to over 400 Clinical and methodological paediatric experts
Inclusion of YPAGs, patients and parent groups in advice meetings; Single contracting structure, coordination of Expert Advice

Single Point of Contact
Access to local networks in 21 European countries and over 250 clinical sites
Aligned processes across the entire network increase efficiency and quality

c4c Training Academy
Providing standardized training to all study sites and site personal, Master courses on Pediatric Drug Development

Paediatric Data Dictionary & CDISC TAUG
1st Pediatric Data Dictionary established to allow standardization of data collection across Paediatric studies

c4c makes a difference

Areas of highest impact

Design and planning of studies

Advice requests

- Outcomes directly impacting studies designed and conduct
- Reports supporting discussion with Regulatory authorities

Trial feasibility & opening sites

Significant decrease in time to sign CDAs
Increase in number of high quality sites available for site selection and feasibility

Data standards

Cross-Cutting Paediatric Data Dictionary as basis for CDISC TAUG

- Supporting sharing and interoperability of data

Education

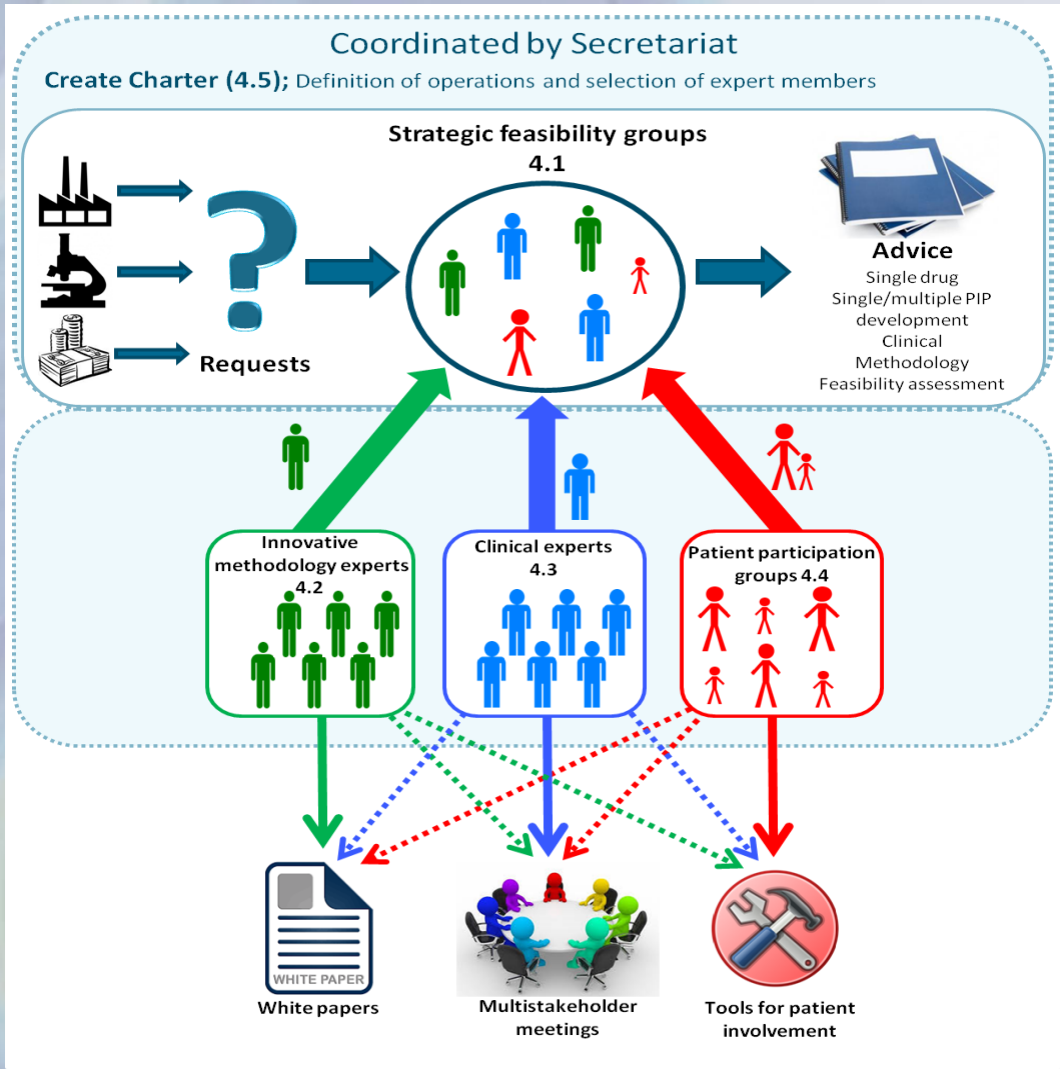
Multiple short courses
Advanced Course in Paediatric Clinical Trials and Drug Development is in progress

Patient and Public Involvement (PPI)

Improving PPI plan`s of sponsors, ensuring systematic involvement
Impact design and planning of studies

Strategic Feasibility Advice

Improving the way paediatric studies are planned and designed



25 Expert Groups – over 400 registered experts

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

Implementation of the advice

Impacting the design of Paediatric Investigational Plans (PIPs)

As of 17 Aug2022

34 scoping interviews
(7 industry partners & 3 academic sponsors)

7 did not proceed

25 completed

2 ongoing

11 include PPI

23 include one or more Clinical Expert Groups

14 include one or more Innovative Methodology Groups

 # advice requests per group:

- Adolescent medicine (4)
- Cardiology (2)
- Developmental Pharmacology (3)
- Ethics (7)
- Formulations (2)
- HTA (1)
- Infectious diseases & Vaccinology (3)
- Intensive Care (2)
- Neonatology (3)
- Nephrology (3)
- Neuroscience & Epilepsy (4)
- Oncology/Haematology (4)
- Pharmacogenomics (2)
- Pharmacovigilance (1)
- Psychiatry (2)
- Respiratory (5)
- RSV (1)
- Study design and Clinical trial methodology (8)
- Other; dermatology (1)

Advice Reports support Regulatory discussions & submissions
Master Consultancy Agreements in place to allow easy contracting

c4c Multi-Stakeholder Meetings (MSM)

1st MSM: Paediatric Inflammatory Bowel Disease

- ✓ Virtual event held 14-15 April 2021 with more than 100 participants representing academia, patients and advocates, regulators (EMA, PDCO, FDA) and industry
- ✓ Very positive post-meeting feedback
- ✓ Publication in scientific journal accepted
- ✓ A multi-stakeholder approach enables the identification of solutions to accelerate drug development in paediatrics
- ✓ Initiative fully endorsed by EMA* and FDA

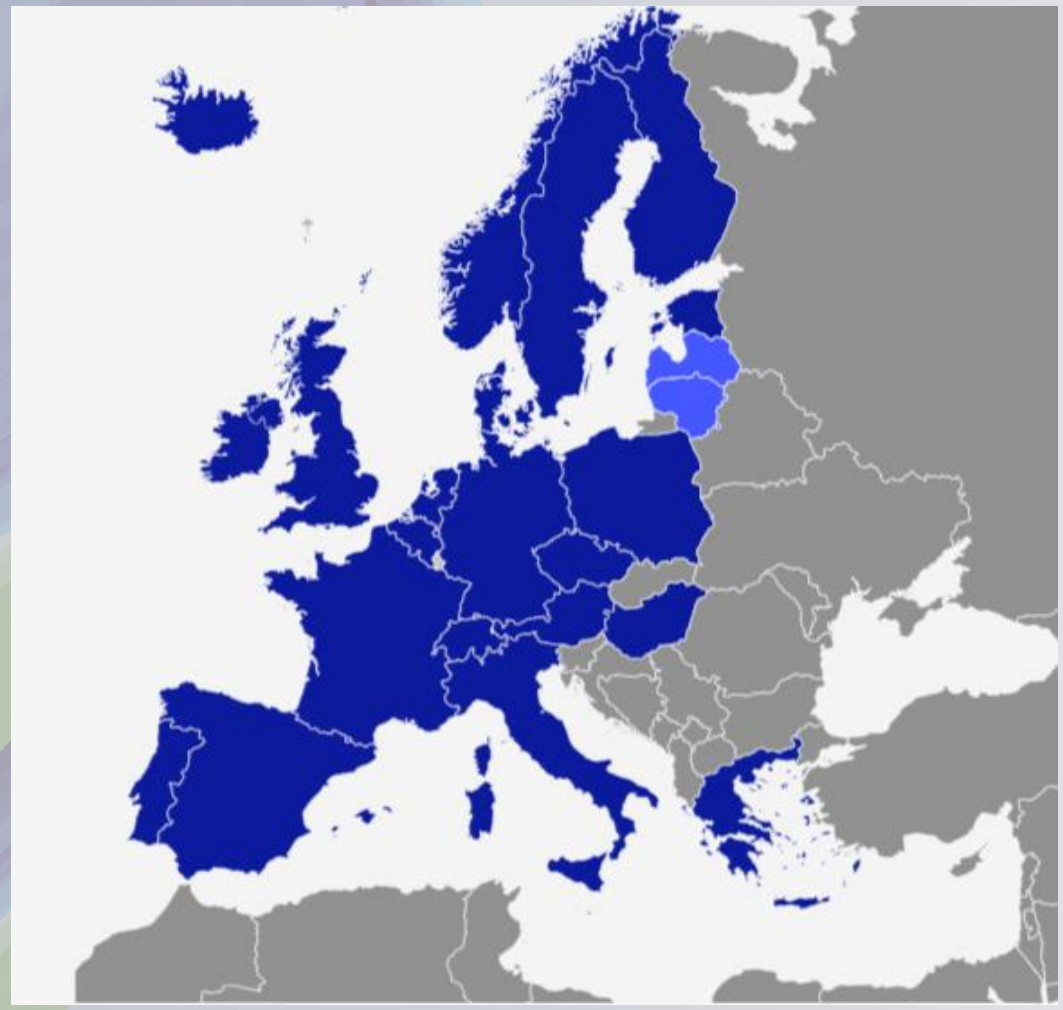
2nd MSM: Atopic Dermatitis

- ✓ 1-2 March 2022 (Virtual)
- ✓ participants representing academia, patients and advocates, regulators (EMA, PDCO, FDA) and industry
- ✓ Publication in scientific journal planned

*2020 progress report on Joint EMA/EC action plan on paediatrics

20 National Hubs serving 21 countries across Europe

Providing access to over 250 clinical sites



* Finland & Iceland is a joint network

c4c established

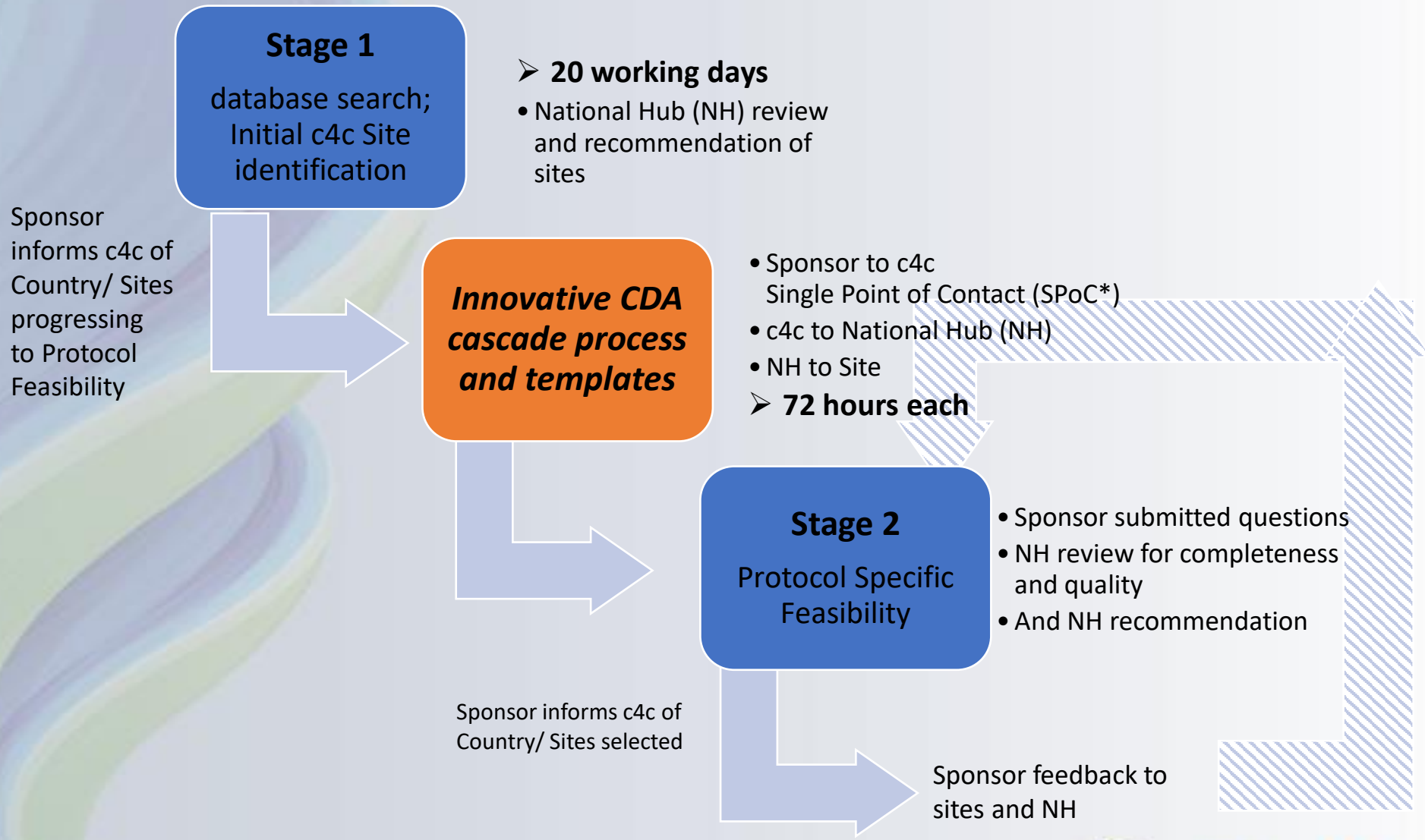
- 20 paediatric national networks in 21 countries*

Closely cooperating with

- 8 European multinational specialty networks
- 3 global research networks

c4c Site Feasibility Services

Increased efficiency through unique CDA cascade process



c4c Site Identification and Feasibility Service

Fast identification of high number of high quality sites

Stage 1- Initial sites identified by c4c

Within 20 working days

Trial	Number of c4c sites identified
Sponsor A	101
Sponsor B	142
Sponsor C_a	161
Sponsor C_b	160
Sponsor D	171

Stage 2- Protocol specific feasibility

	Number of sites	Mean Time to complete*
Sponsor A	8	15 days
Sponsor B	74	9 days
Sponsor C_a	65	16 days
Sponsor C_b	ongoing	
Sponsor D	ongoing	

- *Minimum time 2 days;
- *Maximum time 38 days

c4c work supporting Data Harmonisation and standardisation

Paving the way for better data quality and re-usability

Cross Cutting Paediatric Data Dictionary

IMPACT: *More harmonised paediatric data = More efficient and effective trials*

Data Recommendations

IMPACT: *Higher quality more interoperable data = increased scientific knowledge*

Therapeutic Area User Guide (TAUG)

IMPACT*: c4c is influencing standards development on a global level = potential to de-risk paediatric trials*

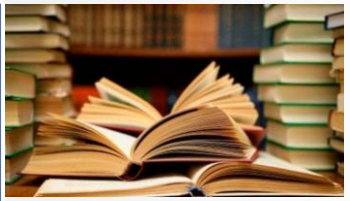
Training and Education

c4c Paediatric Medicine Academy

Teachers from Academic and Pharma Partners

Integrated educational program

to address best practice in paediatric clinical trials and paediatric medicine development



Students/Users

Trainings addressing different professionals roles

c4c Academy Platform

Virtual Learning Environment hosting courses

c4c Academy Management Secretariat

Administrative management of the courses

Education Board (EB)

To provide quality oversight and address educational needs

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

Route to sustainability

To make our vision a reality c4c needs to:

- Transition the c4c network into an independent organization that can function at the end of the IMI funding
- Co-ordinated by a not-for-profit legal entity, likely based in the Netherlands

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Thank you!