

CONNECT4CHILDREN (C4C), SUCCESS STORY FROM A PPP

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TOPICS

What can we offer

- Services and rationale

Public Private Partnerships

- Mechanisms
- Sustainability

C4C STICHTING OBJECTIVE

- To facilitate the development and availability of new drugs for the paediatric population
- Assumption and learning: co-developed, focused interventions generate meaningful benefits



C4C-S / ERN TOUCHPOINTS

Methodology

Medicines used
in trials
(formulations)

Working with
industry

Supporting
sites

Study design

Study conduct

Involvement of
children and
young people

Prioritisation
(multistakeholder
meetings)

Finding sites
outside ERNs

Recognizing site
capabilities

WHAT CAN WE OFFER: C4C STICHTING SERVICES

- Expert Advice
 - Convening and facilitating expert panels to gather important insights for product development
 - Patient and Public Involvement
- Trial Feasibility
 - Conducting fast and efficient feasibility across over 220 paediatric sites in Europe
 - Including site identification
- Trial Support
 - Providing site support that enables recruitment and secures retention

These services build on company capabilities by adding low-cost capacity to address gaps, and can be deployed to support academic projects.

C4C-S STRUCTURE

All paediatric specialties (except Oncology)

>400 experts
Under Master
Consultancy
Agreement

>4000
investigators
>220
sites

Up to 18 National Hubs*

**Always-on access to sites, investigators,
and experts**

Expert
Advice

Feasibility and
Trial Support

*currently 13 NHs under contract

HOW TO WORK WITH US: OPERATING MECHANISM DEVELOPED AND TESTED WITHIN PPP



- Master Service Agreements are executed between c4c-S and each Sponsor
- All experts are under Master Consultancy Agreements
- All sites use CDA templates for feasibility
- Each sponsor enters into clinical trial agreements with each site for trial conduct

NATIONAL HUBS AND SITES

NORWAY
NORPEDMED
(HUS-NORWAY)

conect 4children

NETWORK & AFFILIATIONS

YOU HAVE SELECTED: COUNTRIES: Tutti | HUBS: Tutti | AFFILIATION TO NATIONAL HUBS: Tutti | PART OF SPECIAL NETWORKS: Tutti

CONECT4CHILDREN OVERVIEW

19 AVAILABLE HUBS | 222 AVAILABLE SITES | 158 AVAILABLE CITIES

SITES BY AFFILIATION

TOTAL	PART OF SPECIALTY NETWORK(S)				TOTAL
	YES	NO	I DON'T KNOW / NOT APPLIC.	NO ANSWER	
154	38	19	1	1	222
(73.9%)	(17.1%)	(8.6%)	(0.5%)	(0.5%)	(100.0%)

SITES BY HUB

AT-OKIDS		BE-UGENT	
1	GRAZ	AT-GRAZ-001	14
2	INNSBRUCK	AT-INNSBRUCK-001	14
3	LINZ	AT-LINZ-001	14
4	SALZBURG	AT-SALZBURG-001	14
5	WIEN	AT-MED UNIVERSITY VIENNA-001	14
6	WIEN	AT-WIENNA CHILDRENS HOSPITAL-002	14
BE-UGENT			
1	ANTWERP	BE-ZNA ANTWERPEN-002	14
2	BRUGGE	BE-AZ SINT JAN-001	14
3	BRUSSELS	BE-BRUSSEL-002	14
4	BRUSSELS	BE-HUGESF-001	14
5	BRUSSELS	BE-SAINT LUC-001	14
6	CHARLEROI	BE-CHARLEROI-001	14
7	DE HAAN	BE-ZEEPVENTORUM-001	14
8	EDEGEM	BE-ANTWERPEN-001	14
9	GENT	BE-GENT-001	14
1	HASSELT	BE-JESSA-001	14
1	KORTILIK	BE-GROENINGE-001	14
1	LEUVEN	BE-LEUVEN-001	14

SITES BY COUNTRY

SPAIN	25
NETHERLANDS	21
GERMANY	21
FRANCE	18
GREECE	17
BELGIUM	15
ITALY	13
DENMARK	12
POLAND	11
SWITZERLAND	9
PORTUGAL	9
SWEDEN	8
IRELAND	8
HUNGARY	8
NORWAY	6
FINLAND	6
CZECH-REPUBLIC	6
AUSTRIA	6
ESTONIA	3

Site profile

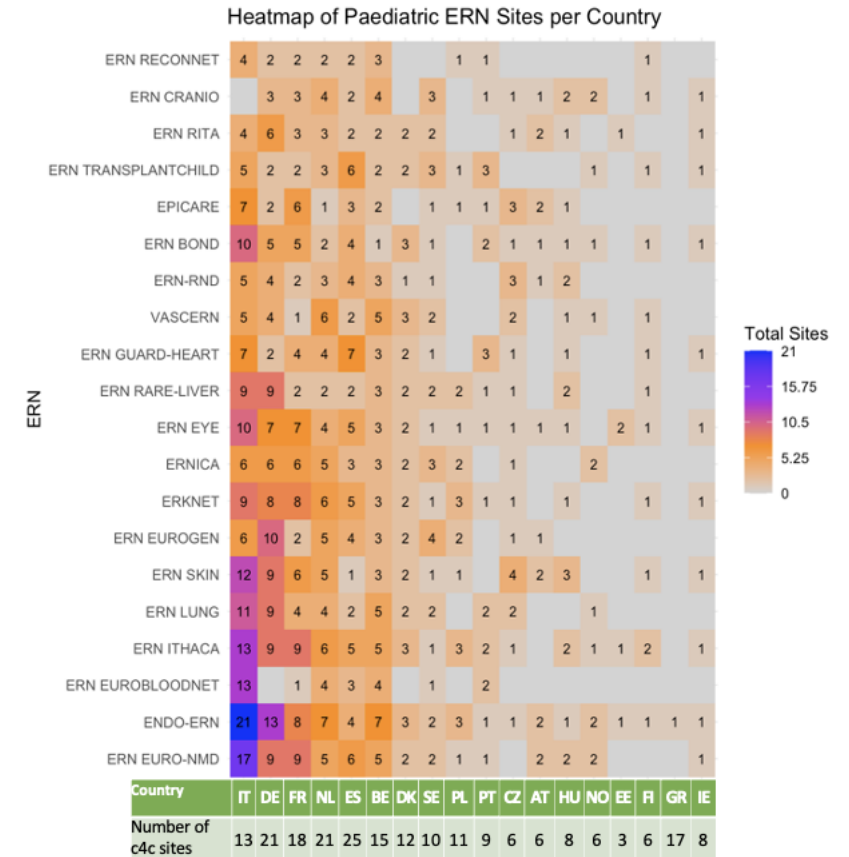
STAND4KIDS (AIDFM)

SWITZERLAND SWISSPEDNET (SCTO)

ITALY INCIPIT

Site mapping

While some countries have significant overlap between ERNs and c4c paediatric sites, there is poor overlap in many countries



c4c has access to many sites outside ERNs that provide health care to babies, children, and young people living with RDs

C4C SITE STANDARDS

A core set of pre-agreed norms and criteria against which the sites involved in clinical trials can be evaluated.

Minimum standards have been defined as a baseline criterion for all c4c sites to meet.

Aspirational standards are designed to be used to drive quality improvement of the sites (can be metric driven, focused around demonstrable performance)

8 Core Domains



Report from the 2022 Enpr-EMA/c4c workshop on quality criteria/standards of paediatric clinical trial sites.

Co-organised by the European network of paediatric research at the EMA (Enpr-EMA) and conect4children (c4c)¹

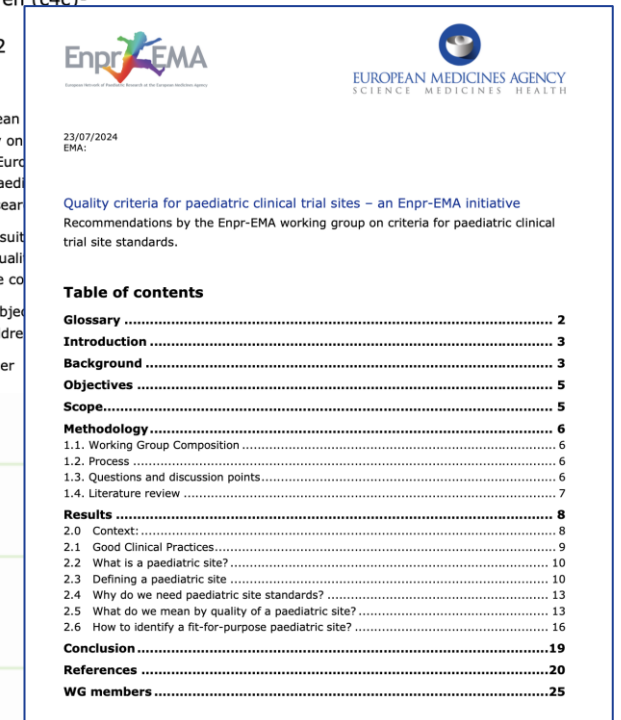
Date: Monday 3 October 2022

A workshop co-organised by the European conect4children (c4c) was held virtually on Enpr-EMA and of c4c, members of the Eurord (PDCO) as well as members of EMA's Paediatric pharmaceutical industry and clinical research

The workshop covered the topic of site suitability need to identify a standardised set of quality sites and to support site selection in the context of

This workshop also contributes to the objective initiative for better clinical trials that address

Chairpersons: Pirkko Lepola, Mark Turner



WORKING IN AND WITH THE PEDIATRIC RESEARCH ECOSYSTEM



Clinical and Research Infrastructures



Rare Diseases



Regulatory Science

International networks

Devices, Medtech



Specialty networks and Learned Societies



C4C PROJECT TOWARDS SUSTAINABILITY

Y1

- Call for NI-PoV studies
- 4 NI-PoV studies selected



Y2



Y4

- Execution of 3 NI-PoV studies
- Execution of 5 I-PoV studies



Y5

- Execution of 2 NI-PoV studies
- Execution of 4 I-PoV studies



c4c beyond
IMI2 funding

Y3

Execution of 3
NI-PoV studies

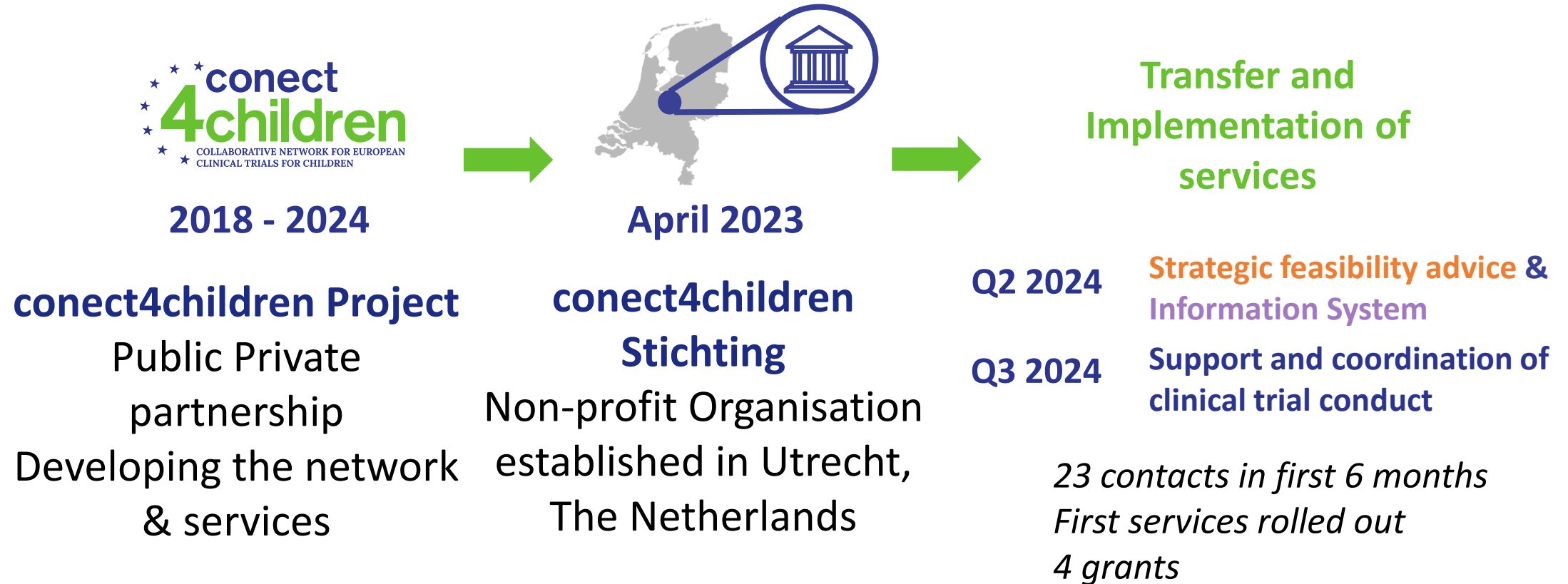


Y6

- Execution of 2 NI-PoV studies
- Execution of I-PoV studies



FROM VISION TO ACTION: HOW THE C4C STICHTING BECAME A REALITY



PUBLIC PRIVATE PARTNERSHIPS: SUCCESS FACTORS

- Broad and deep participation in partnership planning and conduct
- Shared goals
 - After extensive discussions, only commit to shared goals
- Trust
- Time
- Skilled project management
- Appropriate legal framework

KEY LESSONS FOR SUSTAINABILITY

- Find out what people will pay for
- Deliver what people will pay for
- Test
- Market

KEY LESSONS FOR SUSTAINABILITY

- Plan sustainability from the very beginning (before a grant is submitted)
- Promise 100%
 - 60% will be delivered of which half (or less) will be sustainable
 - Less than a third of a project will be sustainable
- Not all good science is sustainable
- Bring in appropriate professionals
 - Legal, finance, HR, marketing, business development etc.
- Start focused and lean, build as resources allow

THE END