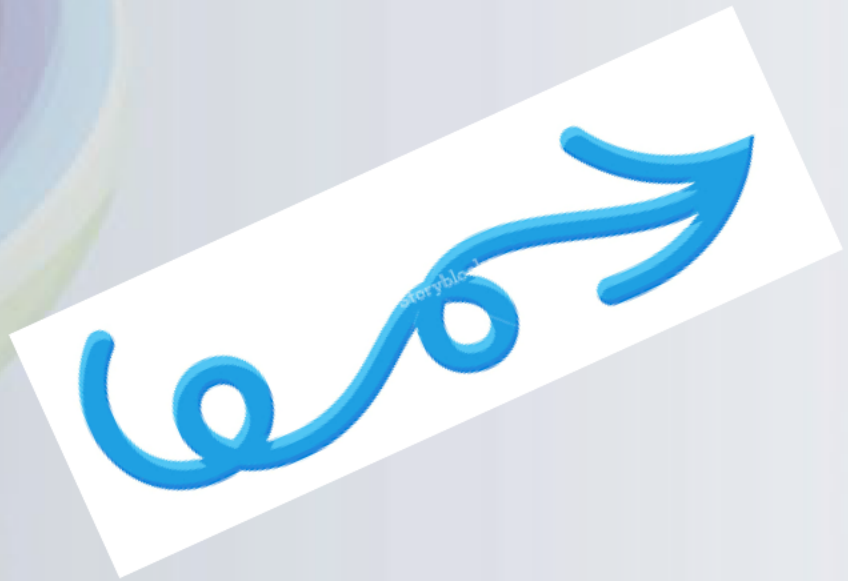
The conect4children journey

Mark Turner (on behalf of c4c)

2023 Annual Meeting of the European network of paediatric research at the EMA (Enpr-EMA)

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.

The journey…



From needs

To success

From Needs to Success

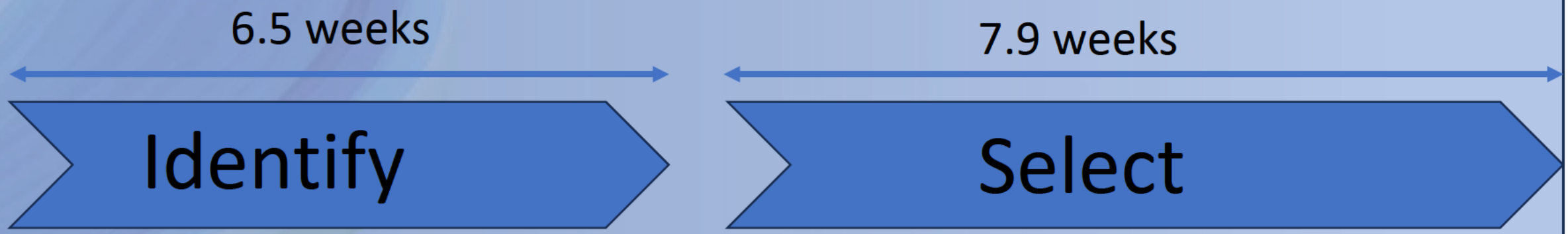
Needs

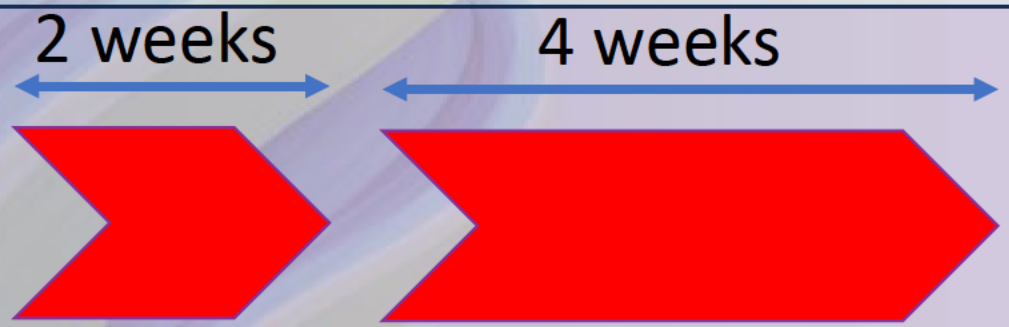
- Rapid access to expert opinion
- Voice of people with lived experience (including patients)
- Rapid and comprehensive access to sites
- Reliable conduct of studies
- Efficient services
- Education and Training
- Paediatric data standards
- Interoperability

Sustainable services

Rapid and comprehensive access to sites

Average timelines for studies*





- c4c site identification
- c4c protocol specific feasibility

From Needs to Success

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- Paediatric data standards
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Sustainable services

Success

Strategic feasibility advice and patient/parent involvement

Single Point of Contact during feasibility and setup

c4c Training Academy

Paediatric Data Dictionary & Therapeutic Area User Guide

Private-public partnership between Academia and Pharma

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

The c4c services

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Strategic feasibility advice

- **Expert advice** on all aspects of paediatric clinical trial design
- **400+ experts** in innovative methodology, clinical trials, and patient and public involvement

> 50 requests so far

3 months for completion

Based on standard agreements between the network and Sponsors and experts

Multi-Stakeholder Meetings (MSMs)

Facilitate pre-competitive discussions and interactions among relevant stakeholders to define unmet medical needs and how best to address those needs and to facilitate development of innovative medicines for children

Legislation ready

Support and coordination of clinical trial conduct

- **Single Point of Contract** (SPoC)
- **20 NHs** and **+240 clinical sites**
- Site **identification** & feasibility, **site set-up**, **recruitment** and site **engagement**
- **Proof of Viability** (PoV) **Studies** to test the performance and viability of the c4c network

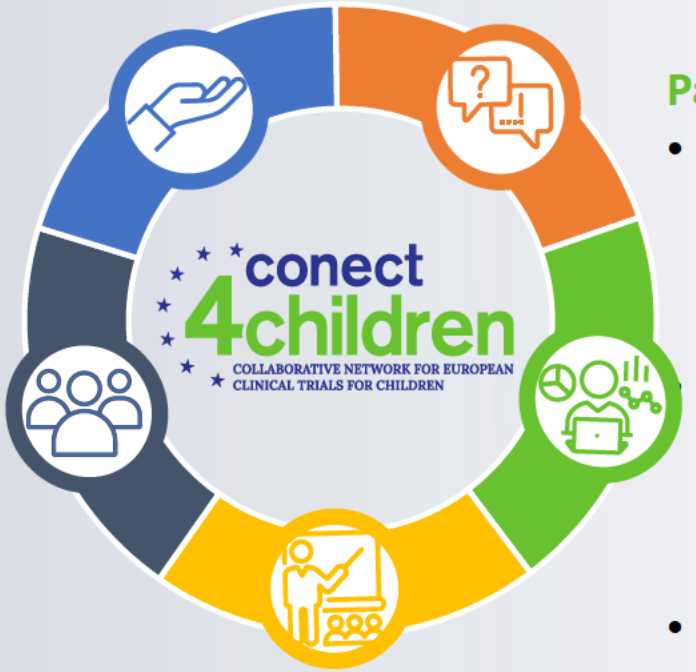
Information System

IT platform, compliant with industry standards, set up to support services and measure network performance

Training Academy

- **Education and Training** to all study sites and personnel
- **Short and advanced courses** in paediatric drug development for heath professionals

E.g., Trial Start-up, Innovative Trial Design, Developmental Pharmacology, Gene Therapy Readiness



Paediatric data standards

- **Data harmonization and standardization**: Optimization of use and reuse of paediatric trial data
  Cross-cutting paediatric **data dictionary** to harmonize and standardize data collection across paediatric studies
- CDISC Paediatric **User guide** in place to support data standardization

Steps

1. Vision
2. Connect
3. Community
4. Focus
5. Detail
6. Test
7. Sustain
8. Scale up
9. Adapt

Vision

National networks

- Account for language, culture and context

Experts

- Diverse
- PPI

Data standards

- FAIR

Education and Training

- Core skills
- Support drug development

Connect

National networks

- With input from EnprEMA and PDCO

Need for sustainable national networks

NETWORK MATURITY MATRIX

	PURPOSE AND DIRECTION	GOVERNANCE AND STRUCTURE	LEADERSHIP AND FACILITATION	KNOWLEDGE CAPTURE AND REUSE	INTEGRITY AND VITALITY	LEARNING AND IMPROVEMENT
FIVE	The network continually reviews its strategic focus, spawning additional groups to cover specific topics or actions as appropriate. Members share the same ambition for the network. They fully buy into the strategy and plans for the network, and are personally committed to its future. External drivers and influences on the network are fully understood.	Membership coverage is complete, providing well-balanced representation. Diversity and cultural/ regional differences are well handled. Governance is fully effective, demonstrating a genuine strategic interest in the success of the network. Sponsors are proactive advocates who champion the cause and promote successes externally.	Leadership is shared seamlessly between several members, who have time and support to carry out the role effectively. There is good understanding of dynamic social processes (e.g. bridges and brokers, connectors and mavens.) and how to facilitate the network to get the best from these. There is a virtuous circle of credibility and confidence in the network to respond and deliver.	Members bring new insights, analysis and content for inclusion as a matter of course. Discussions are regularly distilled into valued knowledge assets. They become essential reading for all members, and may spawn other products, guides and checklists for wider use. Mechanisms for capturing and sharing are well established, including live and virtual events.	High levels of trust and mutual respect enable passionate discussions. People are able to discuss their feelings. Conflict is handled professionally, openly and positively. People honour commitments to participate and deliver. Good range of contributions and unsolicited offers. Members regularly inter-act on a peer-to-peer basis is well as with the network as a whole. Where appropriate, interaction extends well beyond the boundaries (e.g. suppliers, partners, other networks).	The network regularly engages in formal and informal learning, (e.g. guest speakers, internal and external bench-marking, project reviews and visits) with strong participation. The network models reflective practice and seeks ways to improve its effectiveness through evaluation and feedback. Members openly share their learning from failures as well as successes.
FOUR	All members are clear about the purpose of the network and its role in convening, amplification, and advocacy or building community. Deliverables for the community are well known and plans to achieve them are underway. The network 'charter' is accessible to all, and used to induct new members	Network membership is well rounded, with actions in place to fill any gaps. Relationships with other networks are clear. They work to share and learn beyond the boundaries and with external stakeholders wherever appropriate. Governance is fully effective and is valued. Healthy membership turnover – few 'passengers'.	Leaders are engaged and have the requisite skills and dedicated time to fulfil the role. The network appreciates and values their input. A core team of committed participants supports the facilitation and leadership activities. Members have an expectation that questions and contributions will receive considered responses.	A dedicated portal provides a gateway to well managed information resources. The network has tangible products which go beyond FAQs to include, for example: top tips, examples, case studies, expertise, tools and templates. Examples of sharing and reusing knowledge are easily found and members regularly provide new material.	Leaders ensure regular, effective, animated virtual meetings and 'events'. People make this a priority and participation levels are high. Contributions come from the full of members. Members know about each other's expertise and experience. Diversity and cultural differences are well utilised. Leaders ensure that interactions stay focused and forward thinking.	Network members regularly share their insights and lessons learned without the prompting of the facilitator. Members make full use of the network to ensure that their projects learn from others, e.g. via Peer Assists. Plagiarism (with accreditation) is seen a positive - "steal with pride". Curiosity levels are high: "Not invented here" is not observed here!
THREE	The network has an agreed charter, clearly stating purpose, scope, and ways of working. Most members have a good understanding of the purpose of the network and could articulate it to others. There is an agreed plan for developing the network for the next year.	Good coverage of potential membership and awareness of any gaps in representation. Sponsor is in place, understands what is required of them and is regularly active in the role. Governance has been considered and is in place at the appropriate level. Sub-groups may evolve around specialist subjects.	The network has a credible leader/ facilitator in place, with dedicated time available for the role. Other members of the network support the leader informally. The network responds positively when the leader requests participation in an event or response to a challenge or question.	Members pool and validate their most useful documents, and make use of the available material. Experienced members or subject experts regularly summarise discussion threads into FAQs, but largely out of goodwill. Information resources are simplified, well structured and kept up to date.	The network makes use of voice, data-sharing and social media tools where possible. Contributions come from a wide range of members and people's expertise is appreciated. Most questions receive responses, but some go unanswered. Leaders sometimes work 'behind the scenes' to find responses to unanswered questions.	The network leader encourages members to reflect and share lessons. Members demonstrate an interest in learning from their peers and are willing to ask for help.

Connect

National networks

- With input from EnprEMA and PDCO

Need for sustainable national networks

Expert community building

- Advice
- Data
- Education and training

Industry partners

Community

Alignment on:

Goals

Culture

Assumptions

Terminology

- Concepts
- Definition

Methods

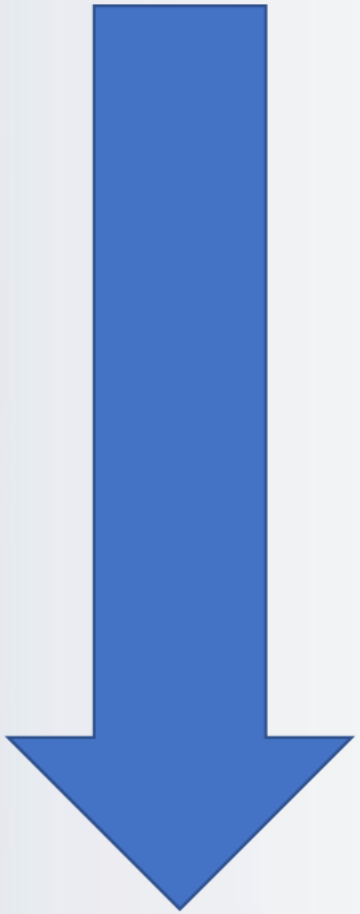
Process

Develop:

Familiarity

Consistency

Trust



6 – 18 months

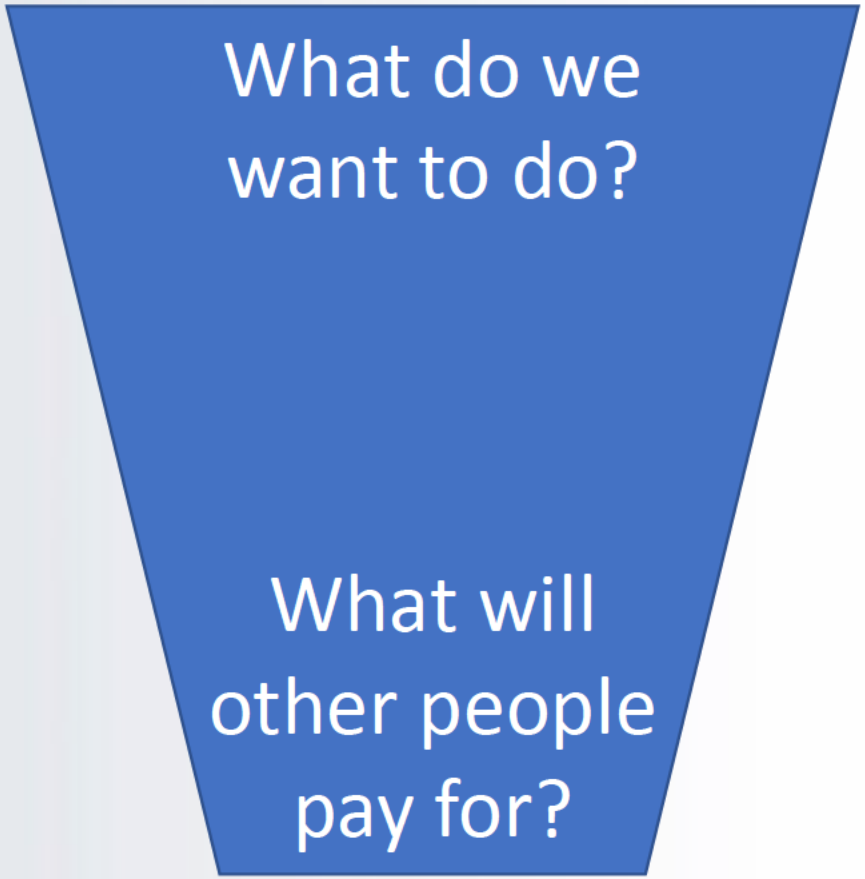
Focus

Select limited number of activities

Priorities

Sustainable:

- Demand
- Supply
- Revenue



Detail

Develop services

- What do the user / customer need?
- How can we meet that need?

Business processes

- Inputs
- Outputs
- Steps

Buy-in

People

Costs

Benefits

Standard contracts with experts
Standards CDAs with sites

Needs dedicated expertise (not academics)

Test

Use of services

1. Advice requests
2. Proof of viability studies
3. Courses
4. Data standards

Fenna Mahler

Advice requests

55 scoping interviews

(7 industry partners & 8 academic sponsors)

11 did not proceed

34 completed

10 ongoing

Network of > 400 clinical and innovative methodology Experts
Patient and Public Involvement database

~86% of 400 Experts have a fully signed Master Consultancy Agreement

Multistakeholder Meetings

Gilles Vassal, Andrea Demadonna

Expand from cancer and NMD model to:

- IBD
- T1DM
- Perinatal asphyxia
- Irritation

MSMs can address all aspects of paediatric drug development

Sabah Attar, Chloe Bickerstaff, Ricardo Fernandes

Non Industry PoV

3 Non-Industry PoV studies

2 studies ongoing

1 study in close out

PoV = Proof of Viability Study

Industry PoV

5 Industry PoV studies

4 studies ongoing

1 study closed out

Additional Industry Trials

15 Expressions of Interest

6 Sponsors

2 grant applications

cdisc

New to CDISC | Standards | Education | Resources | Events | Membership

Global Paediatric Data Forum (GLOPAD)

Home / Standards / Therapeutic Areas / Pediatrics / Pediatrics User Guide v1.0

Pediatrics User Guide v1.0

Release Information | Files & Links

Becca Leary

Published Date: 22 February 2023

Version 1.0 of the Pediatrics User Guide focuses on cross-cutting clinical concepts related to pediatric research and describes how to use CDISC Standards to collect and structure data used in clinical trials to facilitate the aggregation of information, take advantage of big data and support data sharing. A team consisting of CDISC standards experts, pediatric clinical research experts from **CDISC Member organizations** and pediatric subject matter experts from the **c4c consortium** developed the User Guide following CDISC's consensus-based, standards development process. User Guide Topics include participant and participants' family information (e.g., medical conditions, reproductive, diet and nutrition, body system assessments), pregnancy and birth, study conduct.

Public Review Comments

CDISC posts Public Review comments and resolutions to ensure transparency and show implementers how comments were addressed in the standard development process.

TA Specifications

TA Specifications show how to modify TAUG examples for various versions of the **SDTM** and **SDTMIG**. These specifications assist the FDA and the Japanese PMDA with testing to enable support of the standards and inclusion in their respective Technical Conformance Guides.1,2

https://www.cdisc.org/standards/therapeutic-areas/pediatrics/pediatrics-user-guide-v1-0

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c4c training

Users of c4c Academy Platform

- 2374 users are registered in the c4c Academy platform
  - 1690 (60%) students and 684 (40%) Teachers, course coordinators, NH staff

Courses

- 7 GCP related courses (2 accreditated by Transcelerate)
- 9 short courses
- 12 PoV CTs relatedshort courses
- 1 course for Children, YPAG and parents involvement
- 1 Advanced Course
- 5 under development

https://conect4children.org/c4cacademy/

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Francesca Rocchi
Jussi Mertsola
Gigi Spadoni
Becca Leary

Accredited Courses

Advanced Course on Paediatric Clinical Trials & Paediatric Drug Development

Paediatric GCP Course

Paediatric GCP Refresher Course

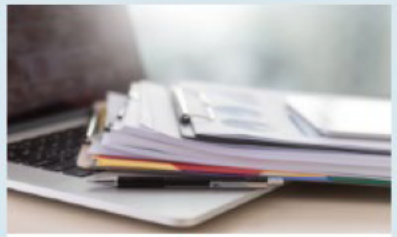
Good Clinical Practice related courses

Audit & Inspection Readiness

This course is on how an inspection (or an audit) should be prepared for and conducted by staff of a clinical trial site.

Enrol: 11th Sept to 8th Oct 2023*

*plus 14 days "extraordinary enrolment" ending on 22nd Oct 2023



Essential Documents

The course is designed to familiarise you with the essential documents needed for a Clinical Trial, following the guidelines of Good Clinical Practice.

Enrol: 21st Aug to 17th Sept 2023*

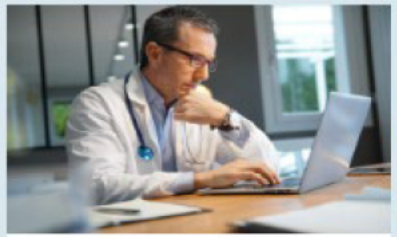
*"extraordinary enrolment phase" from 18th Sept to 1st Oct 2023

Ethics Committee Evaluation

This course focuses on the evaluation of paediatric clinical trials by an Ethics Committee, following the guidelines of Good Clinical Practice.

Enrol: 9th Oct to 29th Oct 2023*

*"extraordinary enrolment phase" from 30th Oct to 12th Nov 2023



Monitoring Paediatrics Trials

This course focuses on the monitoring aspects of paediatric clinical trials, including the role of the monitor (CRA), site staff responsibilities, monitoring plans and study specific procedures (SSP).

Enrol Anytime!

Obtaining Informed Consent & Assent

This course focuses on the process to obtain informed consent and assent during paediatric clinical trials.

Enrol: 18th Sept to 15th Oct 2023*

*plus 14 days "extraordinary enrolment phase" ending on 29th Oct 2023

The Role of Research Nurses

The course focuses on the role of Research Nurses in paediatric clinical trials.

Enrol: 6th – 31st March 2023*

*extraordinary enrolment phase ending on 9th April 2023

Trial Start Up

This course provides a general overview of activities required to start paediatric clinical trials in sites, in line with international and European standards, taking into account the specific guidelines for trials involving children.

Enrol Anytime!

https://conect4children.org/c4cacademy/

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Gigi Spadoni
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e-Le Academy - Short Courses

Developmental Pharmacology

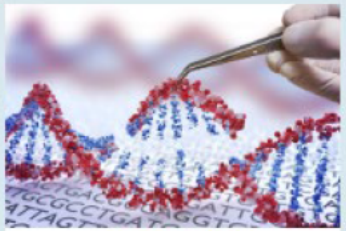
This course will present the key aspects of developmental pharmacology, covering the impact of growth and maturation on drug disposition, effects on dosage selection and evaluation in the different paediatric age groups.

Enrol Anytime!

Elements of Clinical Trial Methodologies

This course provides the basic elements of clinical research methodologies focusing on understanding clinical research results and learning the basics of writing research papers.

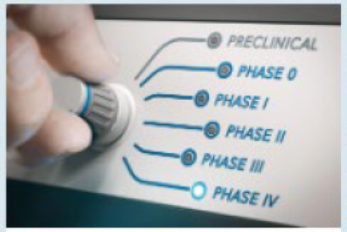
Enrol between 13th & 27th Feb 2023!



Gene Therapy Trials

This self-learning course gives an overview of some key features of gene therapy clinical trials and how the set-up of these trials can differ from other clinical trials.

Enrol Anytime!



Innovative Clinical Trial Design

This course provides an introduction to various types of innovative clinical trial design, including adaptive designs, platform trials & precision medicine approaches.

Enrol Anytime!

Involving Children & Families in Clinical Trial Design

This course aims to enable students to identify activities in the development of clinical trials in which patients can contribute. Students will learn about the design of appropriate methodology and impact assessment.

Enrol Anytime!

Paediatric Investigation Plans

This course provides the learner with knowledge of the key concepts of a Paediatric Investigation Plan (PIP).

Enrol Anytime!

Registries in Paediatric Drug Development

This course will explain how registry data can be used within all phases of paediatric clinical trials, the ethical and legal aspects of using registry data and how regulatory level data can be generated.

Enrol between 19th July & 27th Sept 2023!

https://conect4children.org/c4cacademy/

Heidrun Hildebrand
Ester Montero
Jaana Kallio

Sustain

1. Plan from the start
2. Legal entity
    1. Legal
    2. IS
    3. Accounting
    4. Insurance
3. Financial plan
4. Marketing
    1. World Orphan Diseases Congress, Barcelona, Oct 31st – Nov 2nd 2023
    2. DIA, Europe Meeting, Brussels March 12th- 14th 2024
    3. ANY SUGGESTIONS??
5. National hubs

Public goods need to be paid for

Needs dedicated expertise (not academics)

Scale up

We are ready for business

- Academia
- Industry
- Family groups

Europe

Aiming to work with

- *North America (with partners)*
- *Japan*

Expert Advice (inc PPI), Dec 2023 **Let's start discussions now!**

Site feasibility and study support, April 2024

Data standards, 2024 – 2025

Education and Training, 2024 – 2025

Adapt

Learn from experiences

Become efficient

Expand services

Pharmacovigilance

spoc@conect4children.org <spoc@conect4children.org>

The c4c services

Strategic feasibility advice and patient/parent involvement

Access to over 300 Clinical and Methodological Paediatric Experts

Inclusion of YPAGs, patients and parent groups in advice meetings

Single centralized contracting structure, coordination/organization of Expert advice meetings

Single Point of Contact for site finding and study setup

Access to local networks in 21 European countries

and over 245 clinical sites

The c4c network currently has 20 NHs with 245 sites

c4c Training Academy

Providing standardized training to all study sites and site personal

Master courses on Pediatric Drug Development open for all beneficiaries

Paediatric Data Dictionary & Therapeutic Area User Guide

1st Paediatric Data Dictionary established to allow standardization of

data collection across Paediatric studies