

conect4children (c4c) Stichting

From IHI initiative to sustainable paediatric research infrastructure

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conect4children (c4c)

Problem

Public
Private
Partnership

Solutions

Sustainability

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efpia



- Underpinning work
 - “Preclinical”
 - Mechanism / Safety
 - Natural history
 - Planning
 - Extrapolation
- Clinical
 - Design
 - Execution
- Access / Availability
 - Health Technology Assessment
 - Knowledge Translation

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ESSENTIALS OF TRANSLATIONAL PEDIATRIC DRUG DEVELOPMENT

From past needs to future opportunities



Edited by
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Lien Dossche, Mark Turner



We know how to do this!

Essentials of Translational Pediatric Drug Development: From Past Needs to Future Opportunities

<https://shop.elsevier.com/books/essentials-of-translational-pediatric-drug-development/gasthuys/978-0-323-88459-4>



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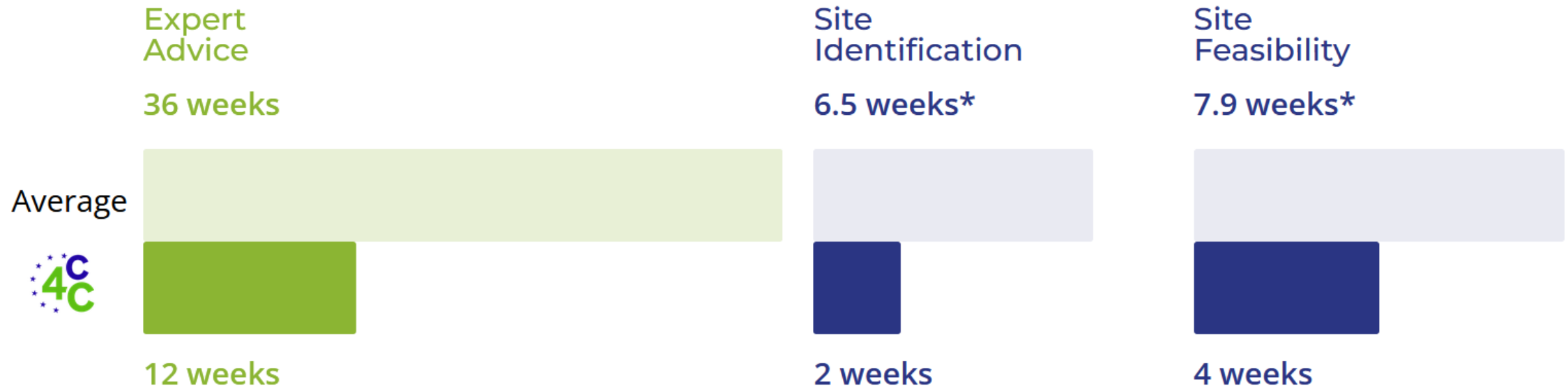


OPERATING MECHANISM



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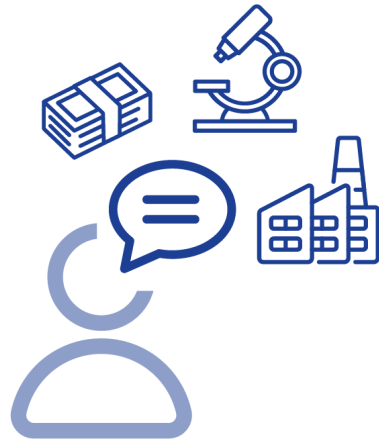
THE BENEFIT OF WORKING WITH c4c - MORE EFFICIENT PROCESSES, DECREASED TIMELINES



*For all studies, not only paediatrics Adapted from Tufts CSDD:

<https://blogs.oracle.com/life-sciences/post/parallelization-imperative-to-the-compression-of-clinical-trial-cycle-time>

EXPERT ADVICE PROCESS



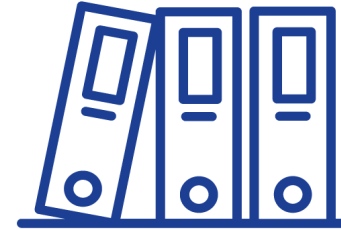
REQUESTS



ADVISORY BOARD

- Experts in innovative methodology
- Clinical experts
- Experts in PPI

Setup 2 weeks



ADVICE

- Single drug
- Single/multiple PIP & PSP development
- Clinical
- Methodology
- Feasibility assessments

Report 3 months

EXPERT ADVICE: 62 COMPLETED ENGAGEMENTS

| 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 |
| Gastroenterology & 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 | |




BACKGROUND

- Client is one of the top 10 global pharma companies and wanted to develop a multinational guidance tool on end-to-end instructions for a paediatric treatment intervention.

OUR APPROACH

- Co-created a questionnaire in line with the client's request.
- Identified relevant experts, National Hubs and sites throughout Europe and across clinical areas, to complete questionnaire.
- Compiled insights for the client to use.

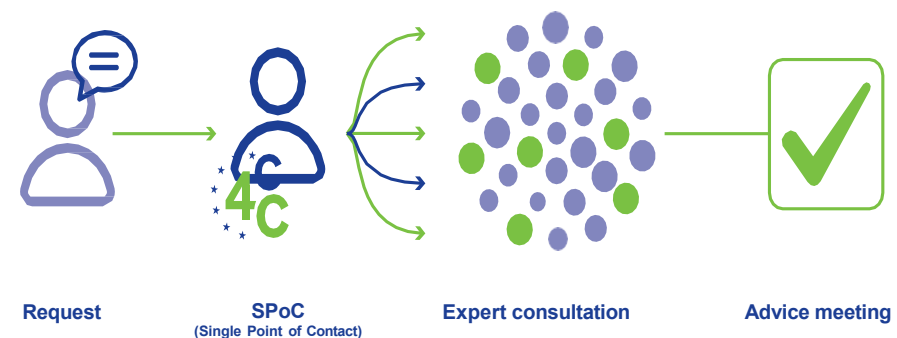
KEY ACHIEVEMENTS

-  Cross- functional and multinational view.
-  Comprehensive overview of the types and brands of materials used, and methods of administration.
-  Additional information gathered on the intervention that is useful for the future.

Stay informed about c4c-S:

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BACKGROUND

- Client's local team was facing challenges with patient recruitment (6m – 18y) for a chronic kidney disease study in Spain, due to disengaged sites.

OUR APPROACH

- Worked with the national sponsor representative to define discussion points with specific sites.
- Met with specific sites and PIs to understand the reasons for disengagement and encouraging joint action for reengagement.
- Followed-up with specific sites and PIs to ensure continued engagement. Provided a platform for sites and PIs to flag new or persistent issues or concerns affecting the client's study.

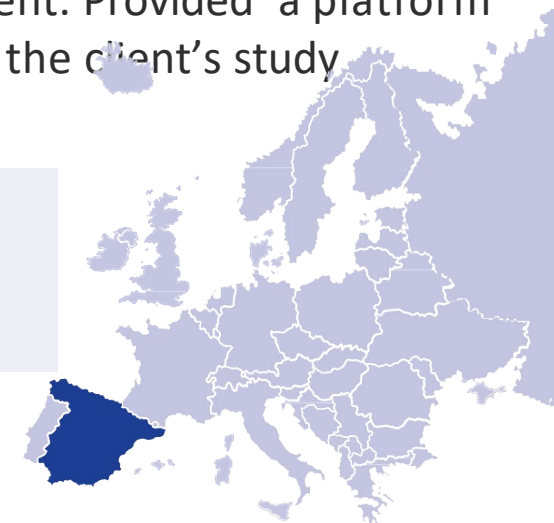
KEY ACHIEVEMENTS



Accelerated pre-screening of patients by specific PIs (within one week).



Recruited an additional 7 Spanish patients within 12 months.



BACKGROUND

- Client's local team was facing challenges with patient recruitment (6m – 18y) for a chronic kidney disease study in Spain, due to disengaged sites.

OUR APPROACH

“In combination with support from the local team, the National Hub (NH) has assisted with motivating sites to pre-screen patients for our study. This has had an impact on recruitment, and we are grateful for the support that the NH has provided.”

– Sponsor Trial Team

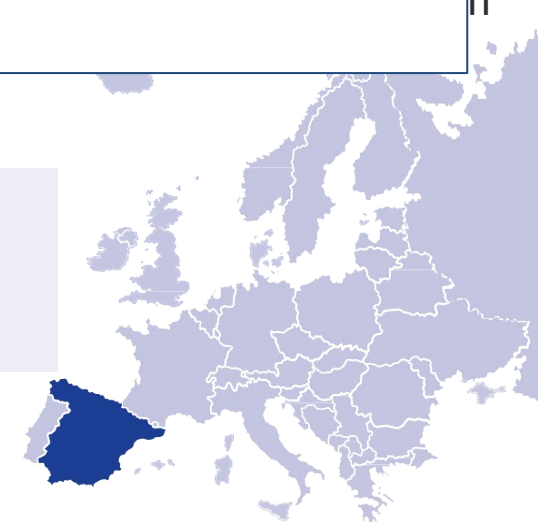
KEY ACHIEVEMENTS



Accelerated pre-screening of patients by specific PIs (within one week).



Recruited an additional 7 Spanish patients within 12 months.



Rapid expert advice to support regulatory agency response

BACKGROUND.

- Client faced a tight deadline to respond to regulatory agency feedback.
- Client requested a quick turnaround in obtaining expert advice, as part of their regulatory agency response.

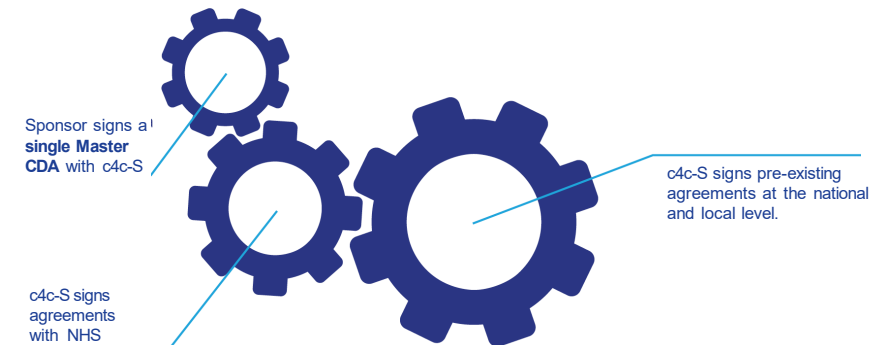
OUR APPROACH

- Use existing Master Service Agreements with the client and with experts.
- Pulled from our dedicated expert network and database, ensuring fluent and rapid communication on client's needs and timelines.
- Accelerated the expert advice process, thereby leveraging our close relationship with the experts.

KEY ACHIEVEMENTS

Provide advice within 8 weeks from the scoping interview:

- ▶▶ 1.5 x faster than c4c-S' aim (12 weeks).
- ▶▶ 4.5 x faster than advice obtained without c4c-S (on average 36 weeks).



Other important work

Education and training

Paediatric data standards

Multistakeholder meetings

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Network coordination and interoperability

Share interest from companies

- **Defined services**
 - US: Real world data + experts
 - Europe/Canada: Experts
 - Tell companies about other countries
- **Defined timelines**

Moving to shared contracts

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Work with learned societies and ERNs

Pilots under development

Education and Training

Expert Advice

- Prepare grant applications
- Allow experts to be paid
- Add methodological expertise

Site finding

- Add to existing networks
- Rapid CDAs



c4c International Symposium
Wednesday 13 – Thursday 14 November 2024
Wicked Grounds HQ, Amsterdam

Please register in advance if you wish to join the International Symposium virtually by Zoom:

Add link

Day 1: Wednesday, November 13, 2024

12:00 – 13:30 Registration & Welcome Lunch

13:30 – 13:35 Welcome to the International Symposium Moderator

Celebrating the Success of c4c: The Journey

13:35 – 13:40 A Message from the Project Officer – *What a successful public private collaboration can achieve* Nathalie Seigneuret

Day 2: Thursday, November 14, 2024

08:30 – 09:00 Registration

Making the Difference: Interactive Workshops Deep dive into c4c-S

09:00 – 09:15 Introducing c4c-S: who we are & how to work with us

Sabah Attar

09:15 -10:45

Workshop 1: How to access and utilize the Expert Advice service to foster use of innovative methods

Topics:

First steps for the advice process
/how to initiate advice
Experts ways of working
Anonymised summary report

**Workshop 4:
Feasibility and site identification**

Topics:

How the site process works
Description of site process findings

Workshop 2: How to access and utilize Trial Support

Topics:

First steps for trial support
Process onboarding
Services available: Feasibility – 2 steps
Impact of c4c support

**Workshop 5:
Interoperability /use of paediatric Data Standards**

Topics:

Standards
GLOPAD

Workshop 3: Education and Training - increase /transfer know how of paediatric drug development

Topics:

Core courses
Advanced courses

**Workshop 6:
Patient and Public Involvement**

Topics:

Impact during all stages of trials and drug development
Key elements to engaging the public
Importance of YPAG structure

Learning points

Identify scope and unique contributions

Focus on sustainability

Bring in relevant experts

Doctors can't do everything

Spend time building culture of collaboration

- Context
- Language

Talk about benefits, as well as capabilities

Build consistency with quality management

Continuous adaptation

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Conclusion

We did what industry, academics, and regulators asked us to do

We are now scaling up our activities to meet the needs of babies, children, young people, and families

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Thank you for your attention!

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