Supporting Paediatric Trials through c4c National Hubs and National Networks

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Hubs, networks and sites

20 NHs, over 250 sites on CFS

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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**
- Access to local networks in 21 European countries and over 150 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 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

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NH services for PoV trials

Feasibility
- Early identification of sites
- Protocol specific feasibility at Sites

Contracts and agreements
- Budget support
- Contracting and collaboration agreements support

Regulatory services (local not global)*
- Clinical Trial Authorisation application
- Recruitment planning
- Informed consent (& other) form support (ICF)

Site setup
- NH oversight of c4c trial support activities across sites

Recruitment
- NH oversight of c4c trial support activities across sites

Ad hoc support and issue resolution

*Some NHs or related third parties can provide additional trial-specific regulatory services if delegated by Sponsors/CRO, including CTA submissions to NCA/EC and monitoring services

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Where have NHs been able to influence?

- Translations of trial documents or emails
- Budget discussions and negotiation
- Non responsive sites
- Contract negotiation and local law requirements
- Cultural differences
- Navigating regulatory submissions and queries
- Site identification and PI identification
- Resolving issues or bottlenecks at sites
- Advising on site suitability

NHs achieve this through expert knowledge and experience at a national level

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Testing and learning from PoV trials

NHs as active partners
- Site support
- Reviews, informs, advises, anticipates, supports and mediates
- Flexible responders in-country with local knowledge of research landscape and trial environment

Prototyping c4c trial services
- Learning by doing

Principles of the c4c approach
- Collaboration between partners
- Continuous cycle of improvement
- Comprehensive evaluation

A NI trial sponsor perspective:
“NH team greatly involved. Great work and nice collaboration since the very beginning”

A site perspective:
“It has been a pleasure working with the National Hub. The personal approach and broad clinical trial support has substantially helped our site with its pediatric clinical trials”
What makes a successful collaboration?

NIO and NHs working together with sponsor/CRO teams (academic/industry, central/local) to deliver the study:

- Establishing contact early on – build good relationships
- Regular, responsive and aligned communication
- Specifying requirements, defining roles and responsibilities
- Sharing information: study progress, timelines
- Informing, reviewing, advising on actionable potential hurdles or bottle necks at local level
- Identify and collaborate with key partners

PoV trials: learning curve of NHs, NIO and sponsors
From PoV trials to a trial portfolio

Growing number of trials across sponsors, conditions, NHs and sites
Continuity and scaling up, experience and lessons

PoV trials → New trials → NH

Sponsor A
Sponsor B
Sponsor C
Sponsor D

Sites

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Beyond trials: NHs/NNs in action

National Hubs and Networks

- C4c/NNN organization activities
- Network building and stakeholder engagement
- Site relationship, development, and support
- Education and training
- Community & PPI
- Continuous improvement of c4/NN processes and tools
- Organisation and governance
- Dissemination

NH Forum

Networking of NN

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Beyond trials: NHs/NNs in action

- STAND4Kids & HELPNet NIO roles
- BPCRN & PEDMED-NL work with other networks
- OKIDS 10-year experience
- INCIPIT legal entity
- In4Kids new model
- Communication ambassadors across NHs

National Hubs and Networks

- Network building and stakeholder engagement
  - PEDSTART in specialty networks & rare diseases
  - POLPEDNET involvement in Ukraine
- Site relationship, development, and support
  - NorPedMed Study Nurse Network
  - DanPedMed & NordicPedMed work on trial preparedness
- Education and training
  - CzechPharmNet student involvement
  - MCRN Hungary cooperation with NHRA
- Community & PPI
  - NH-supported YPAGs
- Dissemination
  - PEDMED-NL & GERMANNETPAET budget tools
  - RECLIP & FINPEDMED work on business plans
- C4c / NNN organization activities
- Organisation and governance
- Continuous improvement of c4/NN processes and tools

- Communication ambassadors across NHs

- STAND4Kids & HELPNet NIO roles
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Impact of c4c at national level

Development and/or support to national paediatric research infrastructure

- New national funding for integrated research infrastructure: Ireland, Poland
- New sustainable legal entity for integrated research infrastructure: Italy
- New national networks: Spain, Portugal, Sweden, Czech Republic, Hungary, Greece, Belgium, Germany
- Reinforced existing networks: Finland, Norway, Austria, Estonia, Netherlands
Sites: destinations of c4c

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Road to (and beyond) standards

- High quality of NHs is a key element as NHs play a central role in the consortium and the service delivery.

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<th>NETWORK ORGANIZATION</th>
<th>RESOURCES AND STAFF</th>
<th>QUALITY MANAGEMENT</th>
<th>RESEARCH EXPERIENCE AND ABILITY</th>
<th>SCIENTIFIC COMPETENCIES AND EXPERT CAPACITY</th>
<th>TRAINING AND EDUCATIONAL CAPACITY TO BUILD</th>
<th>IMPACT</th>
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- High-quality of NHs will foster the excellence of sites and facilitates the site standards implementation.

Gold-standards sites

Accredited sites
Opportunities in the road to pediatric innovation

- Pediatric devices
- Drug repurposing
- Accelerated approvals and revised HTA
- Use of RWE
- Innovative methods, including platform and adaptive trials
- Dedicated clinical networks & sites
- Involvement of children and young adults
- Bridges between translational & clinical research and personalized approaches

Increasing pipeline of new interventions

Data for all ages; BioMedTracker 2020

- EU regulation
- Decentralized clinical trials
- Big data and AI
Learning for sustainability

• Experiencing how to undertake work in the ‘real’ world

• Focus on critical success factors

• Ready for scale up when the new legal entity is opened

• Collaboration and trust key to a sustainable future

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Kiitos! Tak! Takk!