Paediatric Clinical Trial Site Standards

Work Group 1

Defining what quality of paediatric sites means

Enpr-EMA Annual meeting

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Co-Chairs WG1

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Background Information

- **On Oct. 3, 2022:** Workshop on paediatric site quality requirements co-organised by Enpr-EMA and conect4children (c4c) ➔ Identified Action Points
  - Definition of quality of paediatric trial sites: how can conduct be optimised, what matters to different stakeholders, including children, young people and their families
  - Identification/mapping of existing quality standards
  - Implementation of the recommendations for quality criteria/standards: Roadmap – how to? Publication of a recommendation document.

- **On Jan. 26, 2023:** Follow Up Meeting ➔ Discussion Points
  - Groups aiming to define and identify quality standards for paediatric clinical trials could start their work in parallel, at a later stage merge, and work together in the implementation phase.
  - After the definition/identification of general criteria, specificities for specific cases could be defined.
  - Considerations were given to de-centralised clinical trial elements, innovation and digitalisation
  - Need to avoid duplication of work of other ongoing initiatives

- **Two Enpr-EMA Work Groups formed**
  - WG 1: Defining what quality of paediatric sites means
  - WG 2: Identifying/mapping existing quality criteria/standards for sites

WG1 Members

Name	*Affiliation*
Arianna Bertolani	CVBF
Begonya Nafria Escalera	eYPAGnet
Breanne Stewart	MICYRN
Carmen Rodríguez-Tenreiro	GENVIP
Collin Hovinga	Critical-Path Institute
Ensio Norjavaara	AstraZeneca
Eva Degraeuwe	c4c, BPCRN
Fernando Cabanas	Red Samid, PDCO
Holly Huntington	I-ACT
Ivan Foeldvari	JSWG of PRES - Juvenile Scleroderma Working Group
Jorge Alonso	JNJ
Laura Persijn	C4c, BPCRN
Lionel Tan	Viiv Healthcare
Martine Dehlinger-Kremer	ICON plc (CRO)
Mark Sorrentino	ICON plc (CRO)
Melissa Walsh	c4c, IN4KIDS
Nicola Ruperto	PRINTO
Pavla Pokorna	C4c, Czech PharmNet
Sabrina Pierre	c4c; INSERM
Sarah Zaidi	FDA
Tessa van der Geest	Pedmed-NL
Thierry Lacaze	MICYRN

WG1 Objectives – Defining what does Quality of Paediatric Site mean

- Aims and scope
  - To develop a common understanding of what quality of paediatric sites means with regards to paediatric clinical trial sites and what matters to the different stakeholders involved in the conduct of a clinical trial, including children and their parents/ caregivers.
  - This work addresses paediatric site standards across jurisdictions, paediatric age ranges, and types of sponsor; inclusive to a diversity of types of sites and site involvement; and is focused on sites delivering regulatory-grade clinical trials.
  - The work intends to drive opportunities for rollout of site standards and improvement of sites, with adequate resources.

WG1 Methods – Defining what does Quality of Paediatric Site mean

- Ways of working
  - Five remote meetings with open discussions, moderated by Chairs
  - Main discussion points, topics with agreement and dissent, and distinct perspectives from various stakeholders were captured
  - Offline work helped focus on specific questions, identify and share supporting evidence (environmental scan)
- Liaised with WG2 for shared alignment, synergy and efficiency
- Interim report on our groups' operational approach, plan and first ideas at the (virtual) Enpr-EMA Coordinating Group meeting in June

What has WG1 delivered?

- A document focusing on 4 questions:
  - What is a paediatric site?
  - Why do we need paediatric site standards?
  - What do we mean by quality of a paediatric site?
  - How to identify a fit-for-purpose paediatric site?

- Align with WG2 ➔ joint document with recommendations

Next steps and future directions – for discussion

- To incorporate further input and finalise our WG1 recommendations
- To consolidate with WG2 identified standards and other sources of information
- To plan dissemination and implementation steps, aligned with existing initiatives and target stakeholders