



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH





Enpr-EMA Annual Report 2022-2023

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10October2023

An agency of the European Union



	Meetings	15-February 23 (CG), 6-June 23 (CG+Networks), 9, 10-October 23 (Annual Meeting and Workshop)
	CG Members	New members of the Coordinating Group: <ul style="list-style-type: none">▪ Hettie Janssens, new representative of ECFS-CTN (European Cystic Fibrosis Society – Clinical Trials Network) [member]▪ Bernhard Sandner, representing NETSTAP (Network of National Paediatricians in Germany) [member]▪ Collin Hovinga, representing C-Path Institute, USA [observer]▪ Segolene Gaillard, new representative of RIPPS (Paediatric Investigation Into Health Products Network), France [member]▪ Mariska Mulder, new representative of EUCOPE (European Confederation of Pharmaceutical Entrepreneurs) [observer]
	Member Networks	Networks status changes since 2022 Annual Report: <ul style="list-style-type: none">- ECAPN (Spain): Category 1- PEDSTART (France): Category 1- HELPNET (Greece): Category 2- JPEDNet (Japan): Category 3
	Queries from Industry & PDCO & Networks	Queries from Industry: <ol style="list-style-type: none">1. Paediatric product development for Fabry Disease – January -232. Paediatric product development for Netherton syndrome and Eosinophilic oesophagitis – April -23 Queries from PDCO: <ol style="list-style-type: none">1. Severe sialorrhea clinical advice in diagnosis, unmet need and feasibility of clinical trials for the paediatric population – January 20232. Cystic Fibrosis clinical advice on endpoints and assessments for the paediatric population – January -233. Tardive dyskinesia clinical advice on unmet need for the paediatric population and applicability of extrapolation – April -23 Queries from Networks: <ol style="list-style-type: none">1. Infants-vaccinations in the context to Covid-19, medical needs for combined vaccines with influenza/Covid-19



Regulatory News



Conferences



Workshops



Initiatives



Webinars



Hearings



Trainings










Reports



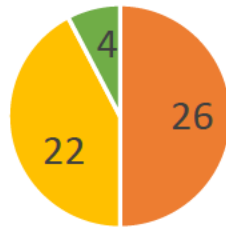
Plans

1. MRCT webinars on advancing international paediatric clinical research – *Facilitating paediatric medicines development: models of global cooperation* - 29, 30 November 2022
2. MRCT webinars on advancing international paediatric clinical research – *Promoting global clinical research in children: informing the future* - 21 March 2023.
3. ACT EU multi-stakeholder meeting on Decentralized Clinical Trials - October 2022
4. ACT EU multi-stakeholder platform kick off workshop - 22, 23 June 2023
5. ACT-EU multi-stakeholder workshop on ICH E6 R3 - 13, 14 July 2023
6. ACT-EU multi-stakeholder methodology workshop as part of ACT EU priority action - 8, 23 November 2023
7. Call for expressions of interest for the EMA PDCO committee – October 2022
8. Call for experts for WHO advisory group on best practices for clinical trials – January 2023
9. Call for experts WHO Technical Advisory Group on Pricing Policies for Medicines – February 2023.
10. Information shared on the MRCT published paper on ‘*Establishing a global regulatory floor for children’s decisions about participation in clinical research*’.
11. Information shared: The closing report of the EMA and EC action plan on paediatrics – February 2023
12. Information shared: The report of the results of the EMA Communication Perception survey conducted in 2022 – August 2022.
13. Information shared: ACT EU recommendation paper on decentralized elements in clinical trials and on the publication of decentralized elements in clinical trials: recommendations from the European Medicines Regulatory Network.
14. **Information shared: The European Commission’s proposal for the revision of the EU pharmaceutical legislation and the open feedback period, from 26 April 2023 to 8 November 2023.**
15. Conference: EFGCP Better Medicines for Children, October 18-19, 2022, and Pre-conference workshop, on the 17th October focusing on the Fundamentals about Paediatric Extrapolation.
16. **Conference: EFGCP Better Medicines for Children, October 17-18, 2023, and pre-Conference on 12th October focusing on pediatric dose optimization.**

Achievements & Activities

	Activities	1. Enpr-EMA-c4c Workshop on Site Standards / Quality Criteria on October 3, 2022
	Public Consultations & Surveys	<ol style="list-style-type: none"> 1. Public consultation on EUnetHTA 21: deliverable D4.4 Endpoints between 3 October 2022 and 1 November 2022. 2. Public consultation on the ICH M11 guideline, clinical study protocol template and technical specifications; EMA - 31 January 2023 3. Public consultation on the establishment of a multi-stakeholder platform to improve clinical trials in the European Union; EMA, Heads of Medicines Agencies (HMA) and European Commission – 6 February 2023. 4. Public consultation on ICH E6 R3 (Good Clinical Practice)- 26 July 2023. 5. Public consultation on the WHO guidance for best practices for clinical trials- 3 August 2023. 6. Survey on Cross-border access to Clinical Trials to collect good practices and cases of language discrimination; Working group on Patient´s Rights: Survey open between 20 April 2023 -> end of 2023 7. Survey of the Employment, Qualifications and Training of Research Nurses working on Paediatric Studies in European Countries and UK: Survey open between 17 August -> late 2023 (?)
	WG International	<i>2 Publications under submission process for regulatory (CTA) + Ethical (EC) requirements of 6 jurisdictions (EU, UK, USA, Canada, Japan, Australia)</i>
	WG Research Nurses	<i>e-Survey for Research Nurses and Research Nurse Managers. Available in English, French, Italian and Spanish.</i>
	WG Patient´s Rights	<i>e-Survey for Clinical Research Units and Parents. Available in 6 languages and additional 18 languages in process.</i>
	WG Labelling - Evidence	<i>Publication: Article “Off-label is not always off-evidence: authorizing paediatric indications for old medicines ” in The Lancet Child & Adolescent Health – June 2023</i>
	WG Clinical Trial Site Quality Criteria	<i>2 sub-Working Groups: Defining quality of a paediatric clinical trial site and identifying and mapping existing quality criteria and standards for sites -> Aim: Enpr-EMA recommendation on pediatric trial site quality criteria 2024</i>

Network categories 2023 (N=52)



■ Category 1
 ■ Category 2
 ■ Category 3

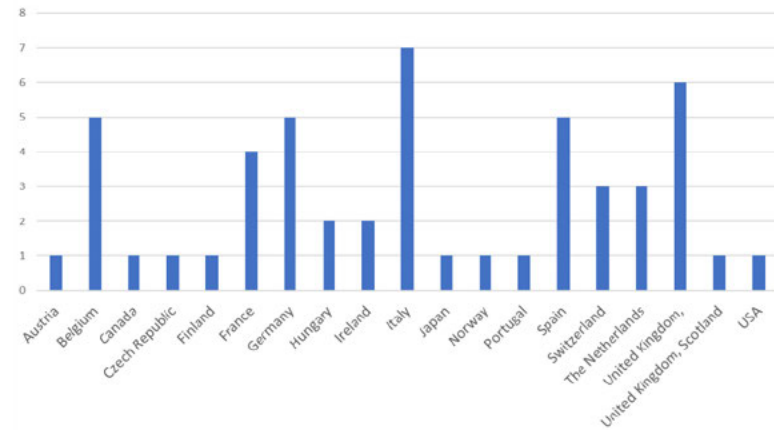
Category 1: Fulfilling all minimum requirements

*Category 1 networks are part of the operational centre of Enpr-EMA – **Coordinating Group**, sharing responsibility for the network's long- and short-term strategy.*

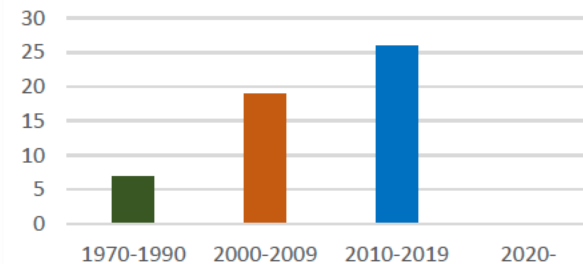
Category 2: Not currently fulfilling all minimum requirements

Category 3: Do not run pediatric clinical trials but have other expertise

Global distribution and number of the member networks 2022



Decade of network establishment



Member networks by therapeutic area and expertise 2023

	multispecialty national	multi-specialty multi-national	Oncology / Haematology/ Malignancies	Allergology / immunology/ Rheumatology	Infectious diseases / Vaccinology	Respiratory / Cystic Fibrosis	Neonatology	Stem Cell/ Organ Transplant/ Haematology	Psychiatry/ Neurology	Intensive Care / Pain / Anaesthesiology / Surgery	Cardiology	Gastroenterology/ Hepatology	Diabetes/ Endocrinology /metabolic Disorders	Gynaecology / Fertility	Special Expertise for CTs	Specialized other Activity
1	OKIDS	c4c	ITOC	PRINTO	PENTA-ID	ECFS-CTN	GNN	EBMT PDWP EPLTN	EUNETHYDIS	ESPNIC	AEPC Task Force	PEDDCreN	-	-	TEDDY	FP-MCRN
2	PCIC-Belgium		Newcastle-CLLG	JSWG of PRES	UKPVG	SPACE	INFANT		ECAPN						EAPRASnet	eYPAGnet
3	FINPEDMED		I-BFM-SG	MIWG-JIA	RESVInet	RESVinet									TREAT-NMD	
4	PEDMED-NL		EORCT-CLG	PIBDnet	RITIP										ESPDDD	
5	MRCN-Hungary		CEPOETA													
6	HUNPEDNET															
7	SWISSPEDNET															
8	PEDSTART															
9	STAND4KIDS															
10	REDSAMID															
11	NORPEDMED															
12	SCOTCRN															
13	RIPPS															
14	IPCRN-NCRC															
15	NETSTAP															
16	RECLIP															
17	NIHR-CRN															
18	DCRI															
19	MICYRN															
20	JPEDNET															
21	HELPNET															