



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Enpr-EMA Annual Report 2021-2022

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4October2022


An agency of the European Union






 **Meetings (Virtual)** 03-March 22 (CG+Networks), 30-June 22 (CG), 04-October 22 (CG+Networks)

 **CG Members & Networks** **Members of Coordinating Group:** No changes since 07-2021

 **Networks status 09-2022;**

- **NETSTAP** (Germany): upgraded to category 1.
- **PolPedNet** (Poland): A new application for national network – Under assessment
- **HELNET** (Greece): A new application for national network – Under assessment
- **ECAPN** (Spain): A revised application for national network – Under assessment
- **PEDSTART** (France): A revised application for national network – Under assessment
- **Japanese network /NCCHD**: A revised application for national network – Under assessment
- **Neocirculation**: closed.

 **Queries from Industry & PDCO**

1. Paediatric product development for fibrodysplasia ossificans progressiva (FOP) – November 2021
2. Paediatric product development for Spinocerebellar Ataxia (SCA) and a feasibility study to understand the viability of recruiting and conducting a clinical trial in paediatric patients with SCA – December 2021
3. Paediatric product development for heart failure – March 2022
4. 2 queries from the PDCO regarding general questions related to paediatric unmet needs, and feasibility of studies.

    **4 main challenges;**

**COVID-19, Ukraine war, “Only Virtual Life”, several ongoing initiatives (overlapping human resources)**



# Enpr-EMA Secretariat information sharing



EUROPEAN MEDICINES AGENCY



## Regulatory News



## Conferences



## Workshops



## Initiatives



## Webinars



## Hearings



## Trainings



## Reports



## Plans

1. Workshop with the title “Advancing the Development of Pediatric Therapeutics (ADEPT 7) – 1st, 2nd of September
2. MRCT Webinar: Advancing International Pediatric Clinical Research. Part 1: INFORMING THE FUTURE FROM COVID-19 LESSONS LEARNED – 6 & 7 October 2021
3. Training modules regarding CTR and CTIS – 14 December 2021 (as part of Enpr-EMA newsletter)
4. TREAT-NMD DMD Early Diagnosis Training Seminar - Spring 2022
5. Invitation to take part in beta-testing of a decision tree for inclusion of adolescents in adult trials, which has been developed by the European Forum for Good Clinical Practice (EFGCP) Children’s Medicines Working Party (CMWP) – December 2021
6. Invitation to a virtual event on ‘Research in regulatory science – addressing the needs’ - 18 January 2022
7. MRCT Webinar: Advancing International Pediatric Clinical Research. Part 2: Time to Listen: Hearing from Young People in Clinical Research – 2 February 2022
8. Go live of the Clinical Trials Information System (CTIS) – 31 January 2022
9. Information shared on the c4c external course catalogue – 02 February 2022
10. The European Commission Directorate General for Health and Food Safety conference on “Health Technology Assessment – What’s next” - 22nd June 2022.
11. MRCT Webinar: Advancing International Pediatric Clinical Research. Part 3: Assent and consent in the field: culture, context, and respect. – 28-29 June 2022
12. TEDDY Hearing within the Council of Europe (COE) regarding children participation in the decision making in the biomedical field - 7th of July 2022
13. EMA’s Annual Report 2021 together with the accompanying message from the EMA’s Executive Director shared on July 2022.
14. The 2022-2026 workplan of the Accelerate Clinical Trials in the EU (ACT EU) initiative shared on 30 August 2022.
15. EFGCP & DIA Better Medicines for Children virtual Conference, October 18-19, 2021
16. EFGCP Better Medicines for Children, October 18-19, 2022, and Pre-conference workshop, on the 17th of October focusing on the Fundamentals about Paediatric Extrapolation. (*AOB in this meeting*)
17. MICYRN and CHEER webinar entitled “Research Ethics Review Streamlining Initiatives” - 29th of November 2021
18. International collaboration between stakeholders for promoting paediatric drug development at the PMDA-ATC & U.S. FDA Pediatric Review Webinar, September 2021
19. Congress of the European Society for Developmental, Perinatal, and Paediatric Pharmacology – 27th to 30th June 2022
20. Enpr-EMA-c4c Worksp on Site Standards / Quality Criteria on October 3, 2022

# Achievements & Activities



## Working Group's Deliverables

1. Publication: Article “ Informed consent and assent guide for paediatric clinical trials in Europe” in Archives of Disease in Childhood – December 2021



## Public consultations & Surveys

1. EU Commission: EU Pharma Strategy Public Consultation- from 28 September 2021 to 21 December 2021
2. EU Commission: Unauthorised medicinal products used in clinical trials (labelling rules)” – 02 June 2022
3. The International Society for Health Technology Assessment (HTAi), the European Patients Forum (EPF) and the European Patients’ Academy on Therapeutic Innovation (EUPATI) survey among HTA stakeholders as part of their research into patient involvement in HTA interactions – 2 June 2022
4. ACCELERATE and the ALADDIN consortium survey regarding a new educational program on strategic and regulatory science in paediatric oncology drug development to foster expertise and strengthen true multi-stakeholder collaboration September 2022
5. Survey developed at Radboud University Medical Centre in the Netherlands, that will inform the design of an evidence-based pregnancy formulary as part of project MADAM (Model Adjusted Doses for All Mothers) – September 2022.
6. ICH GCP: Consultation on GCP principles (2022) –(AOB in this meeting)



## WG International

- 2 Publications in writing process for CTA + EC
- 1 Survey to Industry partners completed
- 1 workshop organized with c4c



## WG Research Nurses

Continues → re-started. European Survey for Research Nurses planned.



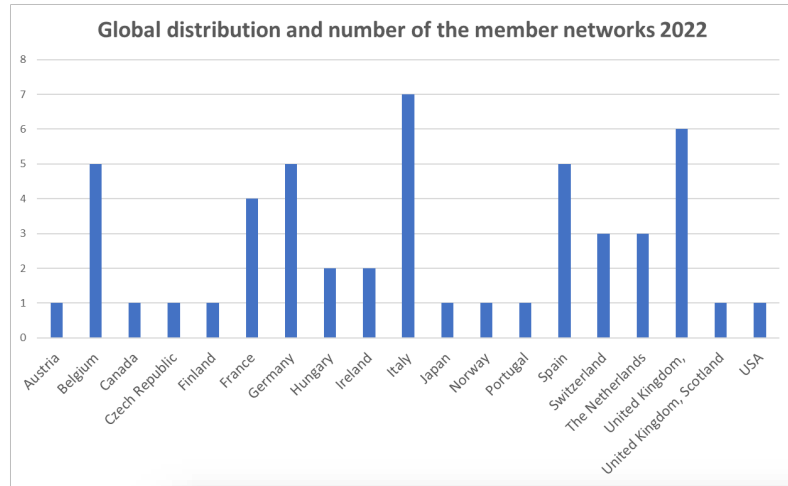
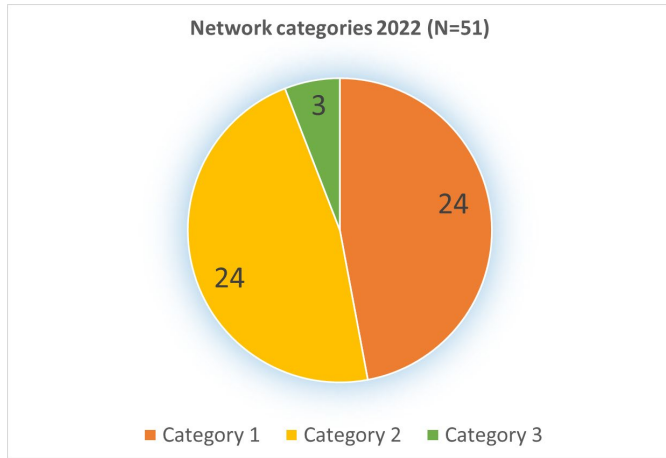
## WG Patient's Rights

New WG for supporting P&P awareness, Patient's Rights and Cross-border access to CTs



## WG Labelling - Evidence

New plans – article in writing process

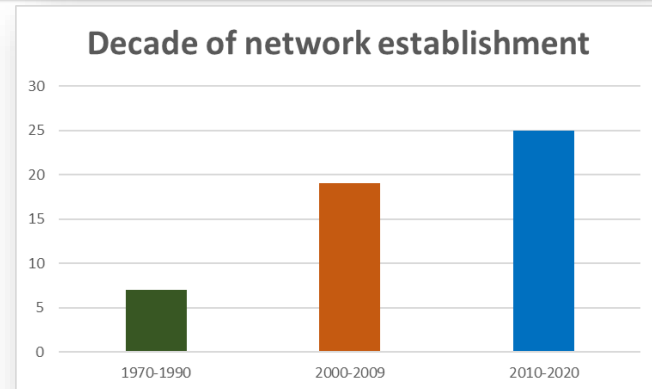


## Category 1: Fulfilling all minimum requirements

*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 paediatric clinical trials but have other expertise**



# Member networks by therapeutic area and expertise 2022

|    | multispecialty national | multi-specialty / multi-national | Oncology / Haematology / Malignancies | Allergy / 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                              | ITCC                                  | PRINTO                              | PENTA-ID                          | ECFS-CTN                      | GNN         | EBMT PDWP                                   | EUNETHYDIDS            | ESPNIC  | AEPC Task Force | PEDDCr eN                     | -  | -                       | TEDDY                     | FP-MCRN                    |
| 2  | PCIC-Belgium            |                                  | Newcastle-CLLG                        | JSWG of PRES                        | UKPVG                             | SPACE                         | INFANT      | EPLTN                                       | 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 | NCCHD-Japan             |                                  |                                       |                                     |                                   |                               |             |   |                        |   |                 |                               |  |                         |                           |                            |

Meaning / benefit of double- or triple...multispecialty memberships and by different criteria for networks and other stakeholders (e.g. sponsors)? Example of c4c

| C4c National Hubs / National Networks   | Double / triple | Enpr-EMA membership   | Other memberships... |
|---|-----------------|---|----------------------|
| 1. Austria       | x               | OKIDS   |                      |
| 2. Belgium       | -               | ECFS-CTN, EORTC CLG, EAPRASnet, PCI Center, Belgium, ESDPPP         |                      |
| 3. Czech         | -               | CEPOETA   |                      |
| 4. Denmark       | -               | -   | NORDICPEDMED         |
| 5. Estonia       | -               | -   |                      |
| 6. Finland       | x               | FINPEDMED   | NORDICPEDMED         |
| 7. France        | x               | RIPPS, PEDSTART, PIPDnet, ITCC                                      |                      |
| 8. Germany       | -               | JSWG of PRES, GNN, MIWG-JIA, EPLTN, NETSTAP e.V.                    |                      |
| 9. Greece        | -               | -   |                      |
| 10. Hungary      | x               | HUNPEDNET, MCRN   |                      |
| 11. Ireland      | -               | INFANT, NCRC  |                      |
| 12. Italy        | x               | FP-MCRN, I-BFM-SG, TEDDY, c4c, ECAPN, PENTA, PRINTO                 |                      |
| 13. Netherlands  | x               | PEDMED-NL, RESvinet, EUNETHYDIS                                     |                      |
| 14. Norway       | x               | NORPEDMED   | NORDICPEDMED         |
| 15. Poland       | (x)             | POLPEDNET   |                      |
| 16. Portugal     | x               | STAND4KIDS  |                      |
| 17. Spain        | x               | RECLIP, RITIP, REDSAMID, eYPAGnet, EBMT PDWP                        |                      |
| 18. Sweden       | -               | -   | NORDICPEDMED         |
| 19. Switzerland  | x               | SWISSPEDNET, ESPNIC, AEPIC  |                      |
| 20. UK          | x               | NIHR-CRN, Newcastle-CCLG, UKPVG, SPACE, PEDDren, TREAT-NMD, ScotCRN |                      |

