EPTRI

European Paediatric Translational Research Infrastructure

# Enpr-EMA and European networks of paediatric research

Pirkko Lepola, Chair Enpr-EMA

Helsinki University Hospital, Department of Children and Adolescents, Digital and Innovation Services, Helsinki, Finland

EPTRI Stakeholders Roundtable – Virtual meeting - July 9th, 2020

This project has received funding from the European Union's Horizon 2020 research and innovation programme under Grant Agreement No 777554

# Disclaimer

The views expressed in the following slides are those of Pirkko Lepola and should not be attributed directly to Enpr-EMA (European Network of Paediatric Research at the European Medicines Agency) or other presented initiatives.

Some of the information have been provided by courtesy of Enpr-EMA, EMA. Information of current initiatives are from public domains or public or from provided presentations, incl. info as it is.

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# EU Paediatric Regulation (2007)

27.12.2006 EN Official Journal of the European Union L 378/1

I

(Acts whose publication is obligatory)

*Ref. USA (1997): Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA).*

REGULATION (EC) No 1901/2006 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 12 December 2006

on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004

(Text with EEA relevance)

**75%**

**Pharma industry represents the majority of paediatric clinical trials**

THE EUROPEAN PARLIAMENT AND THE COUNCIL OF THE EUROPEAN UNION, (4)

Having regard to the Treaty establishing the European Community, and in particular Article thereof,

Having regard to the proposal from the Commission,

Having regard to the Opinion of the European Economic and Social Committee (1),

Having consulted the Committee of the Regions,

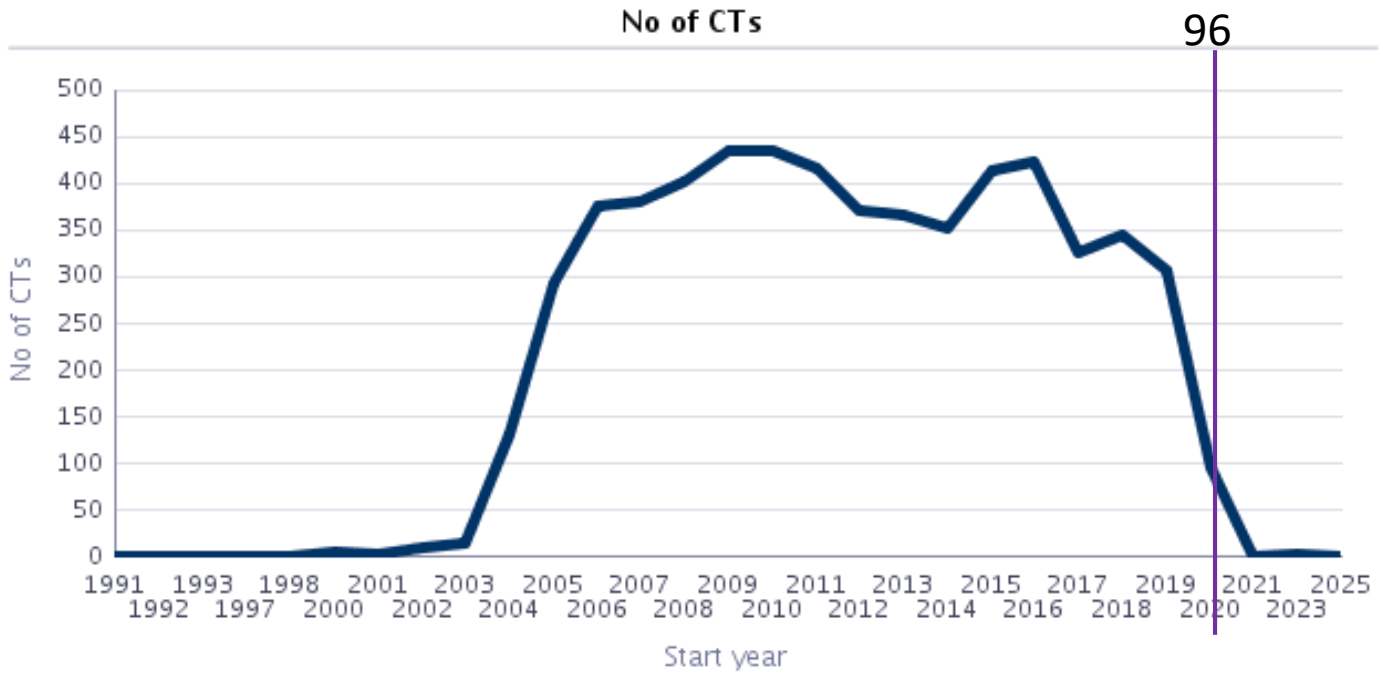
This Regulation aims to facilitate the development and accessibility of medicinal products for use in the paediatric population, to ensure that medicinal products used to treat the paediatric population are subject to ethical research of high quality and are appropriately authorised for use in the paediatric population, and to improve the information available on the use of medicinal products in the various paediatric populations. These objectives should be achieved without subjecting the paediatric population to unnecessary clinical trials and without delaying the authorisation of medicinal products for other age populations.

# After 10 years – progress report

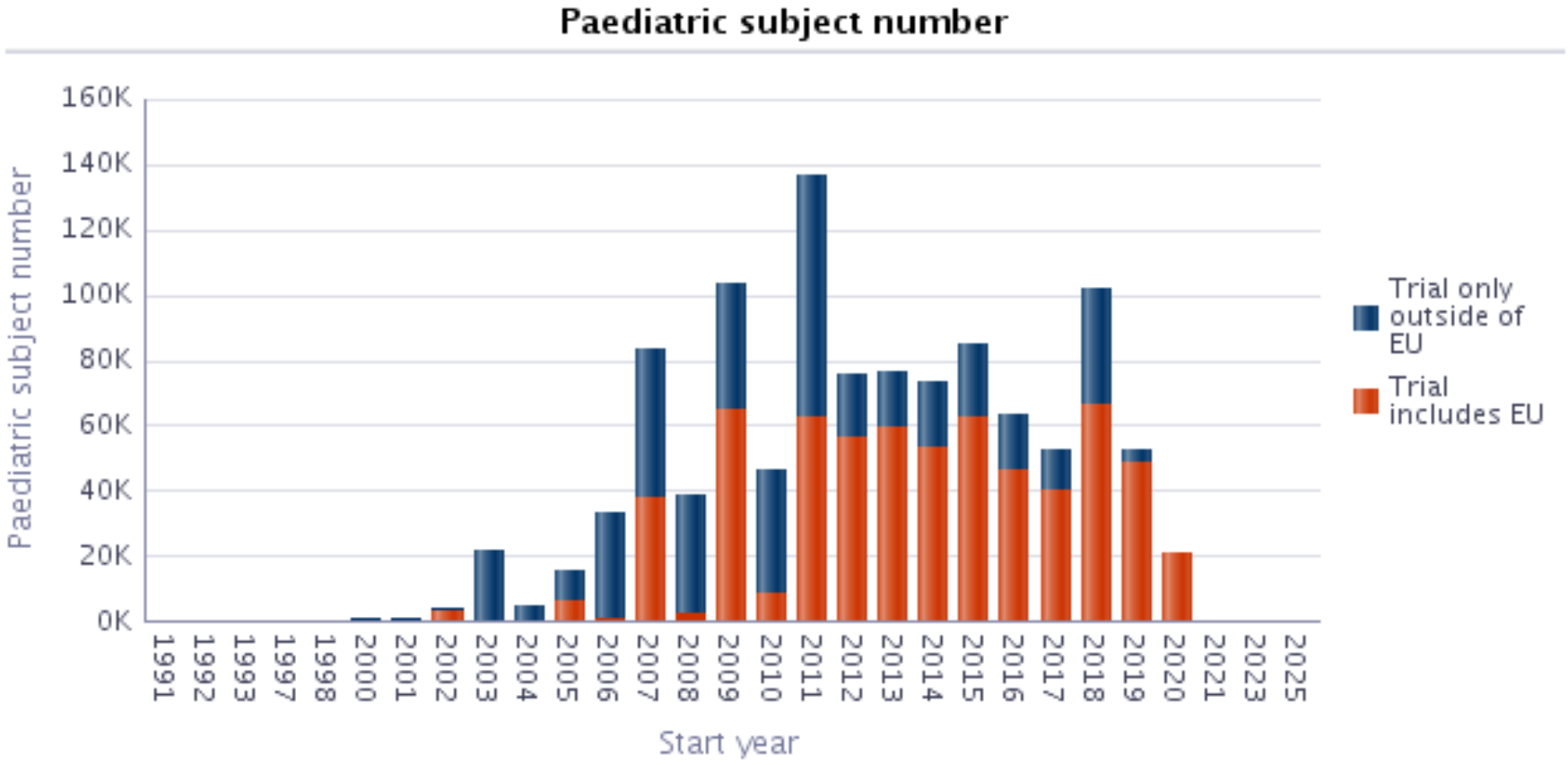


# 2020 May EMA / EudraCT data

- PIPs agreed (decisions): > 1200 (cumulative)
- Decisions on PIP modifications: > 1990
- Full waivers granted: > 800



# 2020 May EudraCT data



**Legal basis:**
Paediatric Regulation,
**Article 44**

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# Enpr-EMA

## European Network of Paediatric Research at the European Medicines Agency (2011)

**Network of research networks, investigators and centres with recognised expertise in performing clinical trials in the paediatric population**

**Mission statement**

Enpr-EMA will facilitate studies in order to increase availability of medicinal products authorised for use in the paediatric population.

# EMA – Enpr-EMA web-pages

Path to the website: ema.europa.eu -> partners & networks -> networks -> Enpr-EMA

**Contacts: enprema@ema.europa.eu**

# Enpr-EMA Key operations

- To facilitate dialogue of networks with Paediatric Committee (PDCO) and National Competent Authorities
- To link together existing networks
- To provide expertise and access to infrastructure for industry to conduct studies in children
- To define consistent and transparent quality standards
- To harmonise clinical trial procedures
- To define strategies for resolving major challenges
- To communicate with external stakeholders

# Enpr-EMA stakeholders

- Pharmaceutical Industry
- Medical device industry
- CRO's
- Patients, parents and patient organisations
- National Competent Authorities
- Ethics Committees
- Hospital pharmacists
- Research Nurses
- Research Networks & learned societies

# What Enpr-EMA DO

- Shares best practices and expertise with other centres/networks
- Provides guidance and connection between stakeholders
- Facilitates communication with PDCO
- Facilitates communication between various groups
- Updates with regulatory news & communication to members Collates mutual responses to Public CA consultations (EU /US)
- Supports Ad hoc Working Groups (currently 6 active ones)
- Orgnizes ad hoc meetings (TCs) for WGs if needed
- Facilitates access to SMEs for collaboration
- Organizes Annual Workshop for all stakeholders
- Organizes regular CG meetings (3 / year)
- Provides regular Newsletters to stakeholders
- Provides secretariat and support for meetings
- Provides access information on EC framework programmes

# What Enpr-EMA does NOT do

- fund studies
- act as a CRO and manage studies
- decide on research priorities

  As this is the responsibility of;

  - the Member States
  - the Commission through the Community programmes
  - each individual network

# Membership recognition criteria

- Networks to be recognised by quality of paediatric research
- 6 recognition criteria and quality standards for self-assessment
  - Research experience and ability
  - Efficiency requirements
  - Scientific competencies and capacity to provide expert advice
  - Quality management
  - Training and educational capacity to build competences
  - Involvement of patients, parents or their organisations
- Each criterion composed of several sub items
- Set of minimum criteria to be fulfilled
- Self-assessment to updated annually

# Breakdown of networks by type and category

| National | Oncology/ Haematologic Malignancies | Diabetes/ Endocrinology/ metabolic disorders/ Gynaecology | Gastroenterology / Hepatology | Allergology/ Immunology/ Rheumatology | Stem Cell /Organ Transplantation/ Haematology/Haemostaseology | Respiratory diseases /Cystic Fibrosis |
|---|---|---|---|---|---|---|
| DCRI | ITCC | | | PRINTO | EBMT | ECFS-CTN |
| NIHR-MCRN | | | | | | |
| ScotCRN | | | | | | |
| FinPedMed | Newclastle-CLLG | | PEDDCReN | | | SPACE |
| PEDMED-NL | IBFMSG | | EPLTN | JSWG of PRES | | |
| MICYRN | | | | | | |
| CICPed | CLG- of EORTC | | | | | |
| RIPPS | CEPOETA | | | | | |
| Okids | | | | | | |
| RECLIP | | | | | | |
| NETSTAP | | | | | | |
| IPCRN | | | | | | |
| MCRN-Hungary | | | | | | |
| SwissPedNet | | | | | | |
| Red SAMID | | | | | | |
| NCCHD-Japan | | | | | | |
| NorPedMed | | | | | | |
| C4c | | | | | | |
| Pedstart | | | | | | |
| Stand4Kids | | | | | | |

50 networks total

20 Category I networks = full membership

**Category 1:** Networks fulfilling all minimum criteria for membership of Enpr-EMA.
**Category 2:** Networks potentially fulfilling all minimum criteria – but needing to clarify some issues before becoming a member of Enpr-EMA.
**Category 3:** Networks currently not yet fulfilling minimum criteria.
**Category 4:** Networks not performing clinical trials; e.g. methodology, infrastructure,

**Unable to fill self-assessment report**

**SPECIAL ACTIVITIES / AGE GROUPS**

| Psychiatry/ Neurology | Infectious diseases/ Vaccinology | Intensive Care/Pain/ Anaesthesiology/ Surgery | European neonatal network | European paediatric pharmacists | special activities (Phv, long term follow up, community paediatricians) | Expertise in clinical trial methodology |
|---|---|---|---|---|---|---|
| EUNETHYDIS | PENTA-ID | | GNN | | FIMP-MCRN | TEDDY |
| ECAPN | UKPVG | ESPNIC | INFANT | | Futurenest CR | EAPRASnet |
| | RITIP | | Neo-circulation | | | PedCRIN |
| | ReSViNet | | ESDPPP | | | EYPAG net |
| | | | | | | TREAT-NMD |

# Enpr-EMA Coordinating Group

- **Acting as a operational center of Enpr-EMA**
  - **forum for communication, contribute to the short and long-term strategy of the network**, discuss and solve operational and scientific issues for the network, and **report to the Paediatric Committee, which acts as the scientific committee of Enpr-EMA.**
- **Consists active participants from category 1 networks (18) + 2 PDCO members** *(only EU/EEA networks shall become full members with speaking and voting rights)* + Special activity / age group networks and organisations with additional expertise and observers
- The Enpr-EMA Secretariat will provide the secretariat
- The CG is co-chaired by the Chair elected from among the members and the EMA
- Representatives of the European Commission may attend as observers
- Industry can be invited as observer  to attend CG meetings

# Enpr-EMA links (2020)

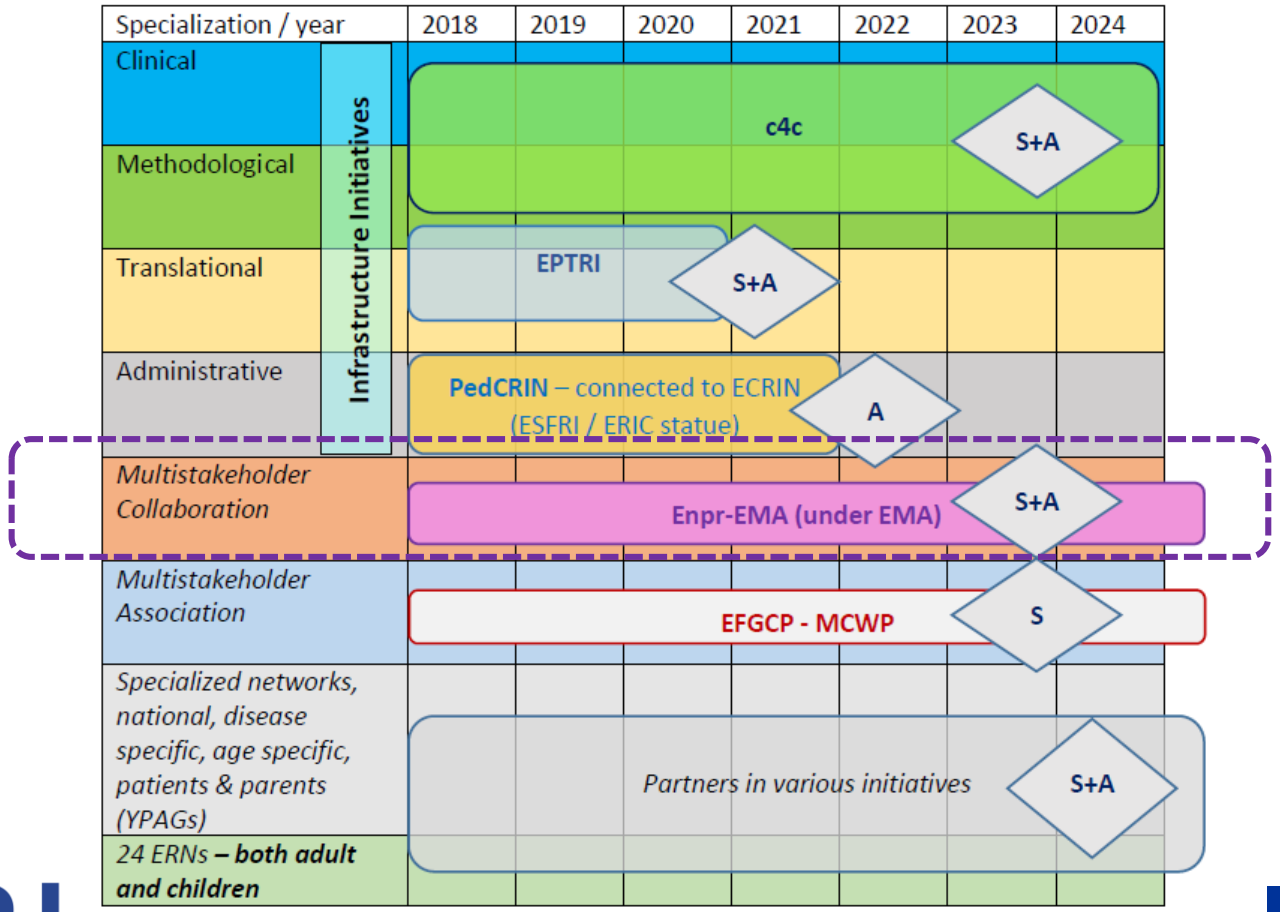
| | |
|---|---|
| **Paed specialty & national networks (EU and Global)** | Members of Enpr-EMA and non-EU as observers |
| **PDCO/EMA** | Members of CG, secretariat |
| **c4c (conect4children)** | Several Enpr-EMA members, and EMA as an affiliated partner |
| **EC (European Commission)** | Linked via secretariat (and CG observers) |
| **EAP (European Academy of Paediatrics)** | Member of CG |
| **Patients & Parents - eYPAGnet** | Member of CG and PDCO patient rep. |
| **ECRIN – PedCRIN (European Clinical Research Infrastructure Network, paediatric extension project)** | Several members of the Enpr-EMA in PedCRIN project under ECRIN |
| **ERNs (European Reference Networks)** | Member of CG as observer |
| **Industry/CROs** | Members of CG as industry observes |
| **NCAs (within and beyond Europe)** | Connected via EMA Secretariat and Enpr-EMA International WG |
| **HTAs (Health Technology Assessment Agencies)** | Connection via EMA Secretariat |
| **EUREC – European network of Ethics Committees** | Connected via Enpr-EMA Ethics WG |
| **MRCT (Multiregional Clinical Trial Initiative)** | Several Enpr-EMA members in collaboration |
| ***EPTRI (European Paediatric Translational Research Infrastructure)*** | *Current collaborative Representative is member of CG via TEDDY network* |

# Paediatric Research Landscape by focus groups

**European Paediatric Specific Research Infrastructure initiatives & networks & associations**

**S= Sponsored trials**

**A= Academic trials**



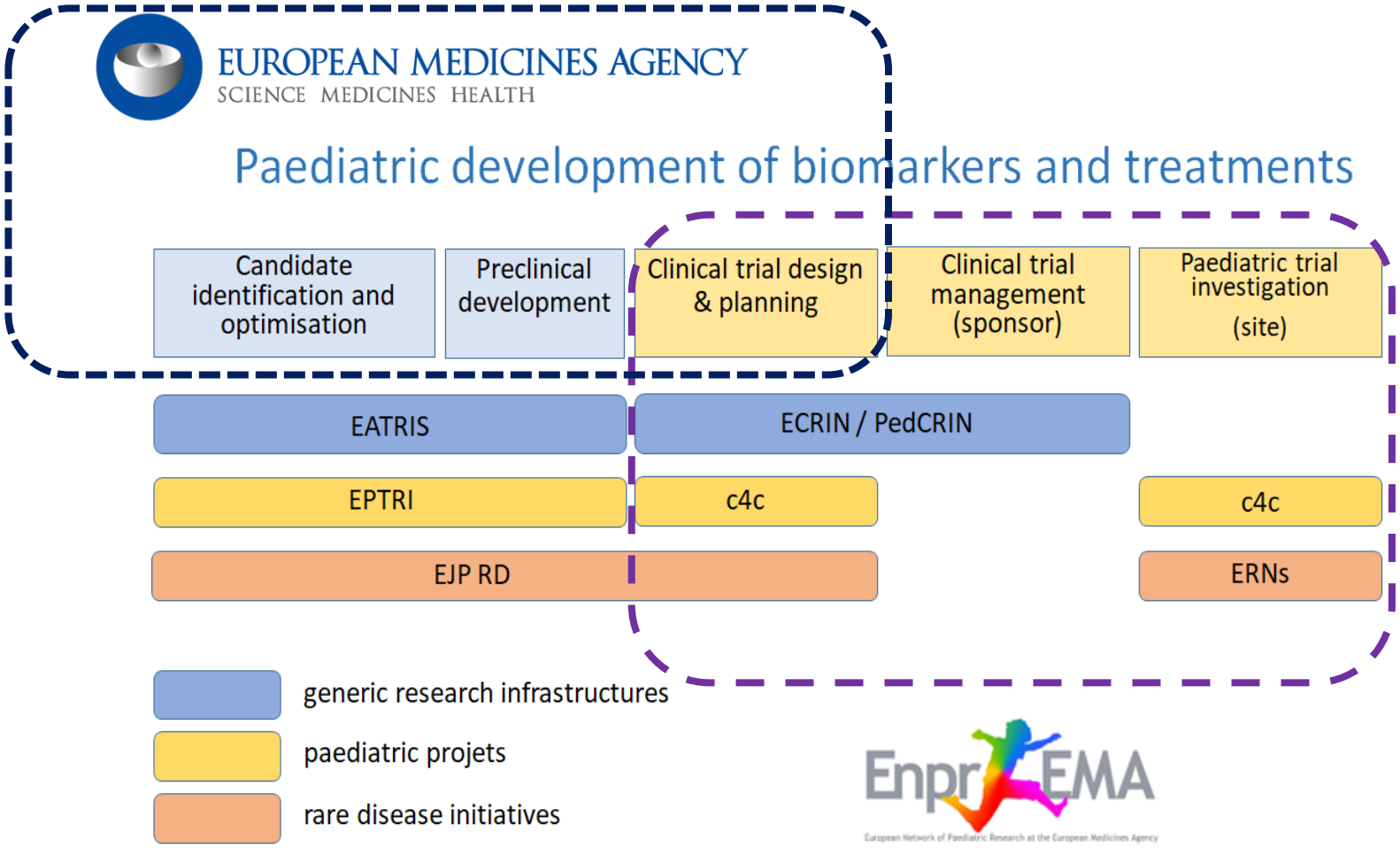



EPTRI
European Paediatric Translational Research Infrastructure

# Paediatric Research Landscape by activities

| | Industry sponsored trials | Non-industry trials (academic) | Development of methods, biomarkers etc. | Design of trials | Delivery of trials (conduction) | Other features |
|---|---|---|---|---|---|---|
| **c4c** | ✓ | ✓ | ✗ | ✓ | ✓ | Funded – all diseases, all trial phases, medicines, devices, biologicals – only pediatric trials |
| **PedCRIN** | ✗ | ✓ | ✗ | ✗ | (✓) Administrative work | Cross-border trial management only – ECRIN model based on country memberships |
| **EJP-RD** | ✓ | ✓ | ✓ | ✓ | ✓ | Rare diseases – adult and pediatric population |
| **EPTRI** | ✓ | ✓ | ✓ | ✗ | ✗ | Preparation of clinical trials, advice in paediatric drug development |
| **Enpr-EMA at EMA** | ✓ | ✓ | ✗ | indirectly | indirectly | Regulatory Authority – EU-level coordination of collaborative supportive work – No trials - Not funded |
| **eYPAGnet** | ✓ | ✓ | ✗ | indirectly | indirectly | Young people – healthy and patients; provides valuable advice, opinions and views for trials |

# Paediatric research ecosystem



# Thank You!