EPTRI
European Paediatric Translational Research Infrastructure

THE CHILDREN'S MEMORIAL HEALTH INSTITUTE

EPTRI General Assembly and Scientific Meeting

Warsaw, 28-29 July, 2026

Perspectives on Enpr-EMA Strategic Plans 2026-2028

Ricardo Fernandes

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Enpr EMA NETWORK

EPTRI Scientific Meeting | 28–29 July 2026 | Warsaw, Poland

Disclaimer

Roles and affiliations

- **Current EnprEMA (Co-)Chair**
- **Lead, STAND4Kids (Pediatric Trial Network), Portugal**
- **Chief Medical Officer, conect4children-Stichting**
- **Associate Professor, Clinical Pharmacology, Faculty of Medicine, University of Lisbon**
- **Pediatrician**

The views expressed in this presentation are my personal views and may not be understood or quoted as being made on behalf of or reflecting the position of the European Medicines Agency or one of its committees or other bodies.

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Network perspectives at a critical juncture for R&I

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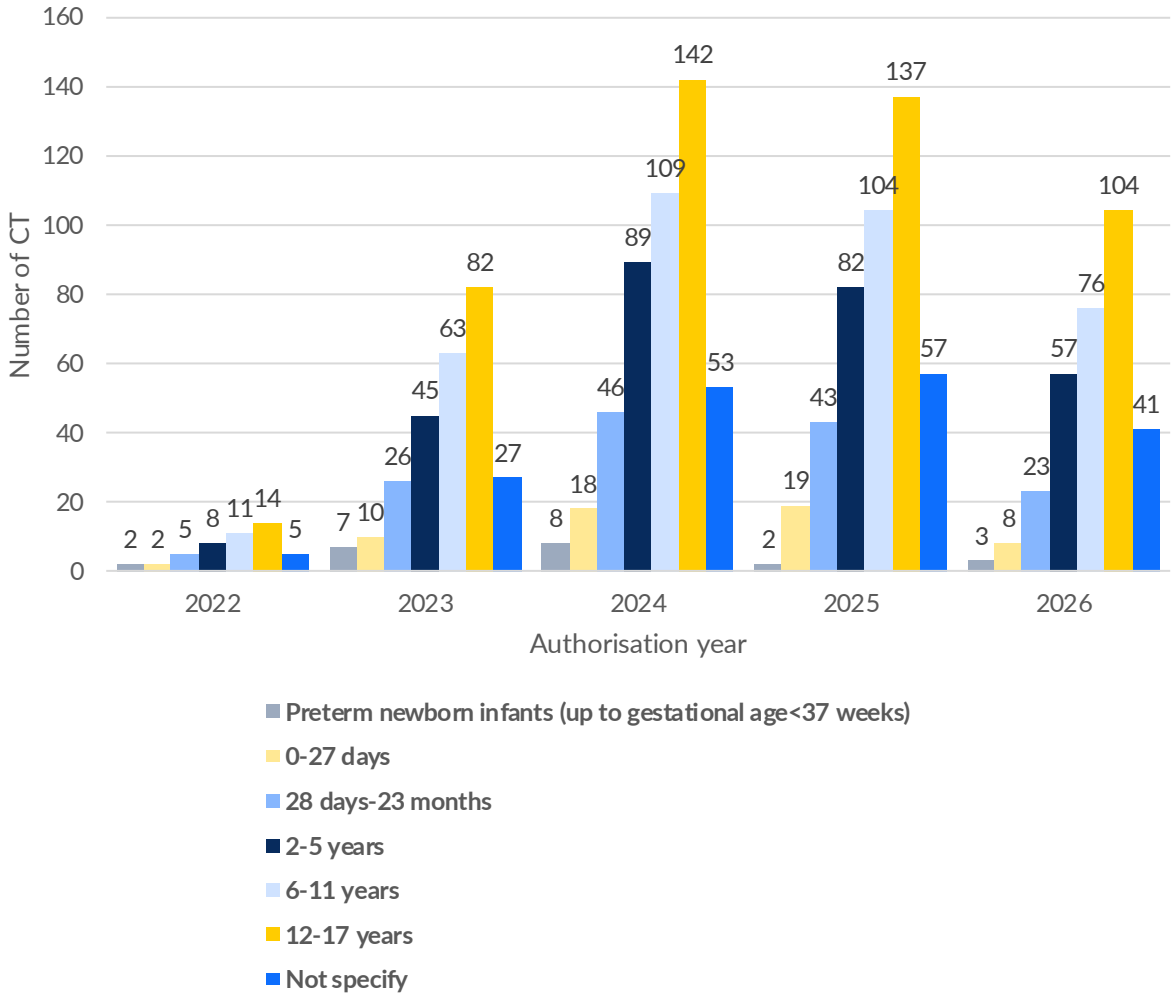
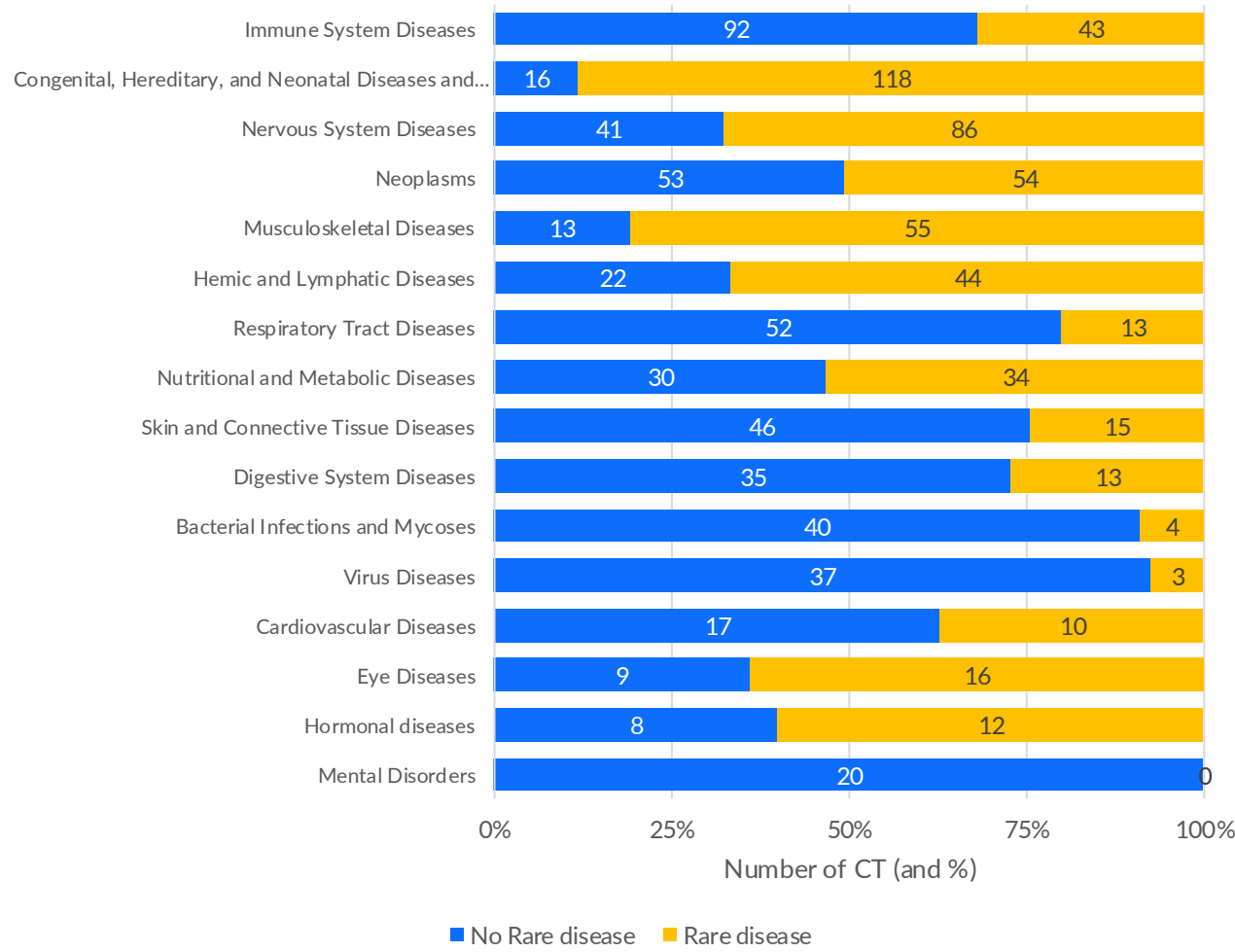
Network perspectives at a critical juncture for R&I

Challenges in the EU paediatric trial landscape

Clinical trial applications authorised for paediatric population by age of clinical trials participants

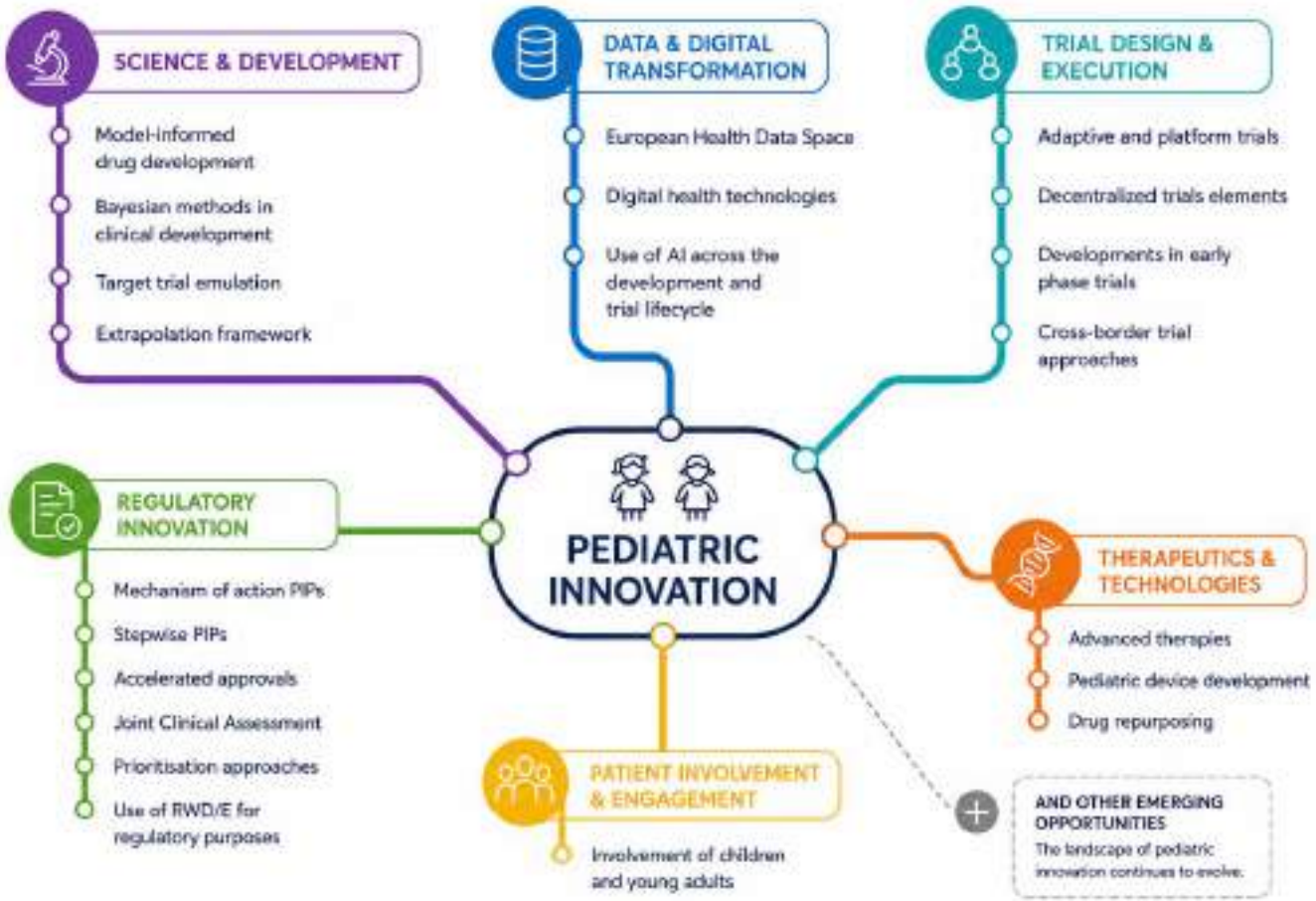


Clinical trial applications authorised by therapeutic area and investigating a rare disease in paediatric population



Preliminary data provided by the EMA; 2026 – YTD.

Opportunities in the road to pediatric innovation



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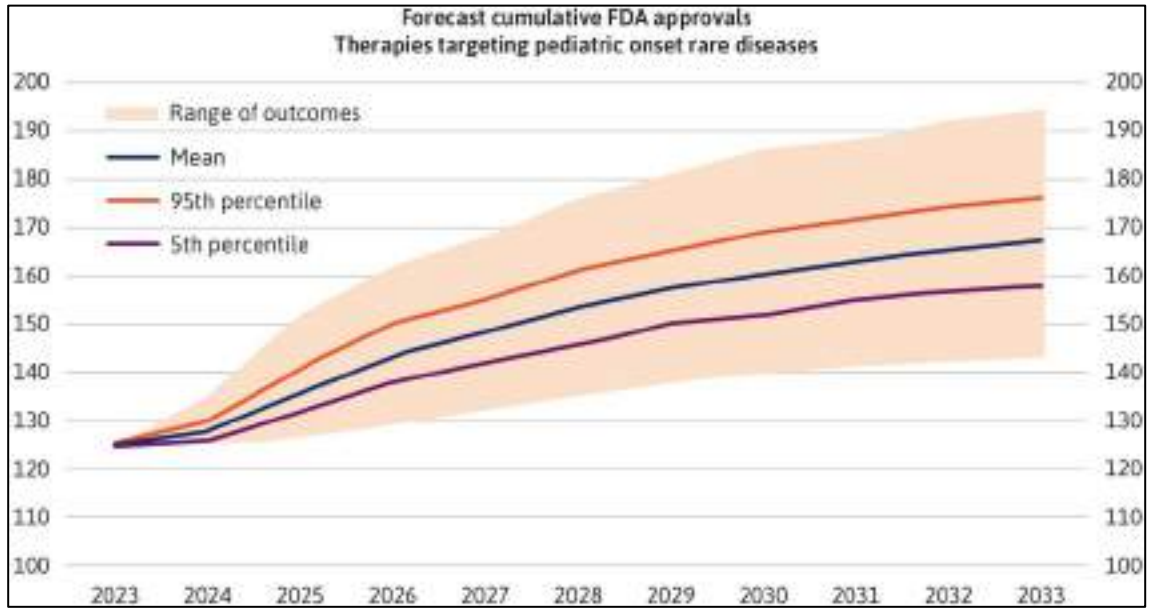
Pediatric-onset rare disease therapy pipeline yields hope for some and gaps for many: 10-year projection of approvals, treated patients, and list price revenues

Colin M. Young, PhD; Sharon E. Phelps, PhD, MPH; Annie Kennedy, BS; Jamie Sullivan, MPH; Bailie McGowan, MPH; Mark R. Trusheim, MS



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Research infrastructures supporting paediatrics

A diverse status quo

Types of infrastructures

- ESFRI Research Infrastructures: ECRIN, EATRIS, BBMRI…
- Specialty networks: ECRAID, ECFS-CTN, TREAT-NMD…
- European Reference Networks: ERKNet, ERN PaedCan,…
- Paediatric research networks: EPTRI, conect4children-Stichting and National Hubs, TEDDY, ITCC, PENTA, ESCAPE, ECHO,…

Infrastructures differ in:

- Scope
- Perspective & funding (public/private)
- Thematic, lifecycle and population focus
- Country and site footprint
- Governance
- Roles
- Services
- Access
- Flexibility and readiness to different users

Different levels of suitability for purpose in supporting the lifecycle of paediatric medicines development

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EnprEMA: network of networks

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

London, 10 December 2008
Doc. Ref. EMEA/643421/2008

ANNOUNCEMENT

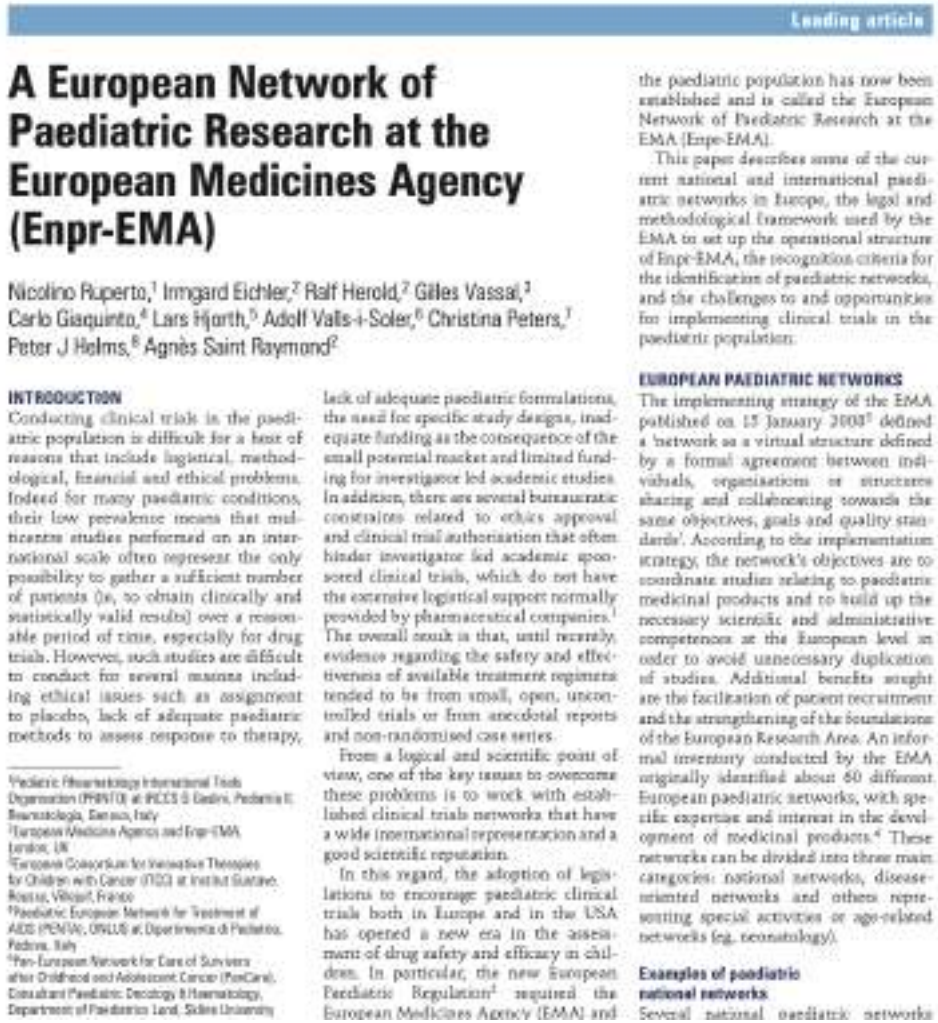
FIRST EMEA WORKSHOP ON EUROPEAN PAEDIATRIC NETWORK

to be held at the EMEA
on Monday, 16 February 2009

The EMEA will convene a one-day workshop on the European paediatric network. One of the objectives of the Paediatric Regulation (EC) No 1901/2006, as amended, is to foster high quality ethical research on medicinal products to be used in children. This should be achieved through efficient inter-network and stakeholder collaboration. To achieve this objective, the EMEA is tasked with developing a European paediatric network of existing national and European networks, investigators and centers with specific expertise in the performance of studies in the paediatric population.

This European paediatric network itself is not intended to perform trials, or to fund studies or research. One of the goals is to link together and co-ordinate existing networks and to establish a platform for communication and exchange of information between networks inside and outside the EU. (A detailed list of the short term and long term goals of the network can be found in and downloaded from "The Network of Paediatric Networks at the EMEA: Implementing Strategy" http://www.emea.europa.eu/pdfs/human/paediatrics/54352307en.pdf)
While there is no formal obligation for networks to participate in the European paediatric network, their active contribution is strongly encouraged for the benefit of the paediatric population. Advantages to joining the network include

- Be visible as a potential site for externally sponsored clinical trials
- Be consulted for expert opinion when paediatric investigation plans in their field of expertise are discussed and developed
- Share the skills and expertise of other national and European networks
- Shape and influence future development in paediatric research



Leading article

A European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA)

Nicolino Ruperto,[1] Irmgard Eichler,[2] Ralf Herold,[2] Gilles Vassal,[3] Carlo Giaquinto,[4] Lars Hjorth,[5] Adolf Valls-i-Soler,[6] Christina Peters,[7] Peter J Helms,[8] Agnès Saint Raymond[2]

INTRODUCTION

Conducting clinical trials in the paediatric population is difficult for a host of reasons that include logistical, methodological, financial and ethical problems. Indeed for many paediatric conditions, their low prevalence means that multicentre studies performed on an international scale often represent the only possibility to gather a sufficient number of patients (ie, to obtain clinically and statistically valid results) over a reasonable period of time, especially for drug trials. However, such studies are difficult to conduct for several reasons including ethical issues such as assignment to placebo, lack of adequate paediatric methods to assess response to therapy, lack of adequate paediatric formulations, the need for specific study designs, inadequate funding as the consequence of the small potential market and limited funding for investigator led academic studies. In addition, there are several bureaucratic constraints related to ethics approval and clinical trial authorisation that often hinder investigator led academic sponsored clinical trials, which do not have the extensive logistical support normally provided by pharmaceutical companies.[1] The overall result is that, until recently, evidence regarding the safety and effectiveness of available treatment regimens tended to be from small, open, uncontrolled trials or from anecdotal reports and non-randomised case series.

From a logical and scientific point of view, one of the key issues to overcome these problems is to work with established clinical trials networks that have a wide international representation and a good scientific reputation.

In this regard, the adoption of legislations to encourage paediatric clinical trials both in Europe and in the USA has opened a new era in the assessment of drug safety and efficacy in children. In particular, the new European Paediatric Regulation[2] required the European Medicines Agency (EMA) and the paediatric population has now been established and is called the European Network of Paediatric Research at the EMA (Enpr-EMA).

This paper describes some of the current national and international paediatric networks in Europe, the legal and methodological framework used by the EMA to set up the operational structure of Enpr-EMA, the recognition criteria for the identification of paediatric networks, and the challenges to and opportunities for implementing clinical trials in the paediatric population.

EUROPEAN PAEDIATRIC NETWORKS

The implementing strategy of the EMA published on 15 January 2008[3] defined a 'network as a virtual structure defined by a formal agreement between individuals, organisations or structures sharing and collaborating towards the same objectives, goals and quality standards'. According to the implementation strategy, the network's objectives are to coordinate studies relating to paediatric medicinal products and to build up the necessary scientific and administrative competences at the European level in order to avoid unnecessary duplication of studies. Additional benefits might are the facilitation of patient recruitment and the strengthening of the foundations of the European Research Area. An informal inventory conducted by the EMA originally identified about 60 different European paediatric networks, with specific expertise and interest in the development of medicinal products.[4] These networks can be divided into three main categories: national networks, disease-oriented networks and others representing special activities or age-related networks (eg, neonatology).

Examples of paediatric national networks

Several national paediatric networks

[1]Pediatric Rheumatology International Trials Organization (PRINTO) at IRCCS G Gaslini, Pediatria II, Reumatologia, Genova, Italy
[2]European Medicines Agency and Enpr-EMA, London, UK
[3]European Consortium for Innovative Therapies for Children with Cancer (ITCC) at Institut Gustave Roussy, Villejuif, France
[4]Paediatric European Network for Treatment of AIDS (PENTA), ONLUS at, Dipartimento di Pediatria, Padova, Italy
[5]Pan-European Network for Care of Survivors after Childhood and Adolescent Cancer (PanCare), Consultant Paediatric Oncology & Haematology, Department of Paediatrics Lund, Skåne University

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European network of paediatric research at EMA (Enpr-EMA)

(contact: enprema@ema.europa.eu)

- Provides **platform for dialogue** on paediatric medical research to foster paediatric clinical trials and development of medicines for children
- Sets up **multistakeholder Working Groups** to enhance trial design & practices
- Has more than **50 paediatric research networks as members** globally **-> Join as a new member!***
- Brings together **patients, academics, health care professionals, medicine developers, regulators** as stakeholders
- Is an **expert network** to the EMA Paediatric Committee (**PDCO**)
- Publishes **guidance, articles, best practice recommendations**
- Organises **annual open workshop** at EMA/Amsterdam for all stakeholders

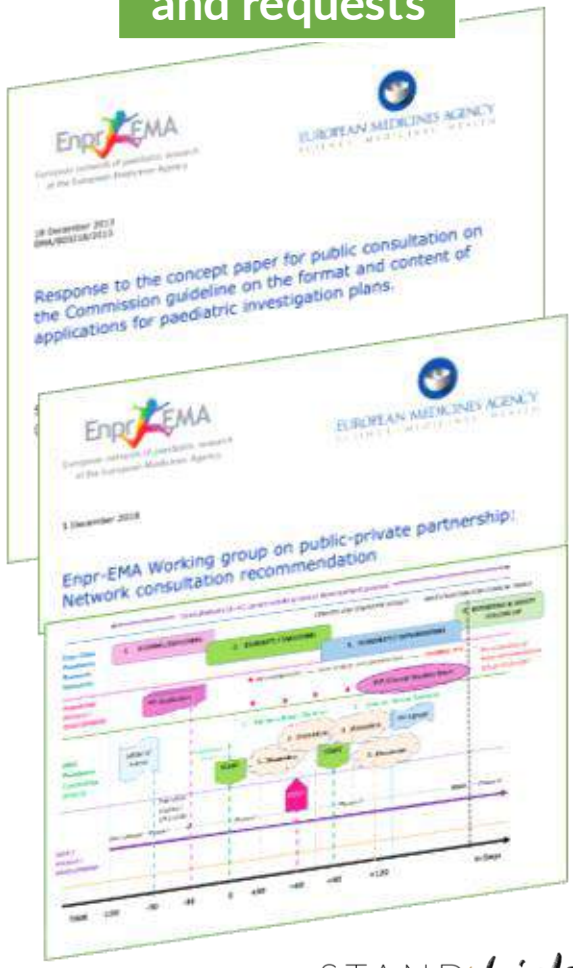
- **Website path: EMA https://www.ema.europa.eu/en/homepage -> Partners & networks -> Networks**
- **-> European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA)** https://www.ema.europa.eu/en/partners-networks/networks/european-network-paediatric-research-european-medicines-agency-enpr-ema
- **Database of member networks:** http://enprema.ema.europa.eu/enprema/search.php
- ***Membership form:** http://enprema.ema.europa.eu/enprema/images/Self_Assessment_PDF_Form_v5.pdf

Examples of EnprEMA contributions

Workshops

Working Groups and recommendations

Consultations and requests



Community building

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EnprEMA today

EnprEMA plenary

- **58 paediatric research networks are members**
- Expert network of the PDCO
- Platform for dialogue to build up competences and **to foster paediatric clinical trials**
- Shares expertise and best practices, publishes recommendations
- Enhances inter-network and stakeholder collaboration

PDCO

- Supports the development of EnprEMA
- **2 Members in Coordinating Group**

EnprEMA Coordinating Group (CG)

Operational centre / governance body of Enpr-EMA

EnprEMA Working Groups

- Cross-border access to CTs
- Patient- & Public involvement
- International cooperation
- Trial site standards
- Nurses

EMA

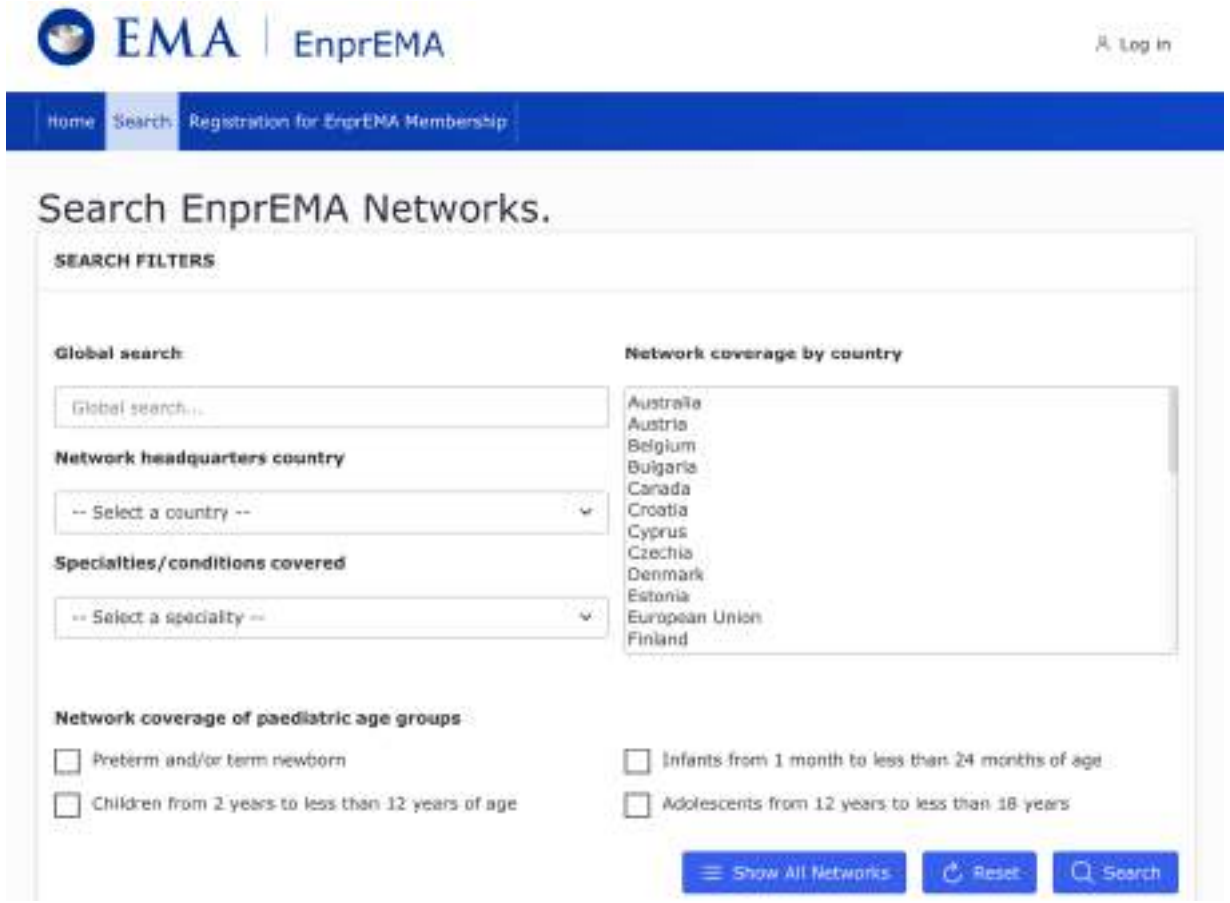
- Provides the secretariat support
- Hosts EnprEMA meetings etc.
- EMA appoints **co-chair of CG**

Stakeholder engagement

- **Patients, academics, health care professionals, medicine developers, regulators are important stakeholders** (in addition to networks)
- Annual meeting (F2F) for all stakeholders
- Involved in Working Groups
- Involved in Coordinating Group

EnprEMA members & stakeholders

- Networks (multi-specialty/single specialty) are members of EnprEMA
  - ”accreditation” via membership criteria (Cat 1-3)
  - increased visibility via publication in Enpr-EMA database
- Patients, academia, industry, individual centres and investigators are not members but important stakeholders
- Coordinating Group (CG) (EnprEMA’s governing body) involves all stakeholders
  - oversees EnprEMA activities
  - sets short- and long-term strategy



EnprEMA at a critical juncture for R&I

Yayoi Kusama
INFINITY MIRRORED ROOM

New legislation through a transformation lens

December 2025
Political agreement on new pharmaceutical legislation

2026
Adopted acts of the new pharmaceutical legislation enter into force

2026-2028
Transition phase
-EU Member States update their national laws to reflect the new rules
-European Commission adopts implementing and delegated acts to help implement the new rules
-EMA and national competent authorities develop implementation guidance and adapt their procedures and IT systems

2028
New pharmaceutical legislation becomes applicable

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Some overarching NPL transformation themes

Adaptation (adaptive procedures, Mechanism of Action)

Acceleration (timelines)

Innovation (nonprofit drug repurposing, regulatory sandboxes)

Support (changes in commitees, EnprEMA)

KEY ELEMENTS OF THE EU PHARMACEUTICAL REFORM

December 2025
#HealthUnion

Access to **safe, effective medicines** for all EU citizens

- **Greater and earlier availability** of medicines to patients in all Member States.
- **Day-1 access to generic and biosimilar medicines**, with improved tools to allow them to enter the market as soon as protection periods end.
- **Access to new medicines** where there are currently no treatments, with incentives to support the development of medicines in much needed areas, including rare diseases and for children.

Tens of millions more EU patients will have access to new medicines, thanks to incentives to develop medicines for unmet medical needs and provisions for Member States to require companies to supply their markets.

A **simpler, more competitive environment** for EU industry

- **Shorter authorisation procedures**, with a more streamlined structure in the European Medicines Agency (EMA), to increase efficiency and cut cost and red tape for companies.
- **Faster assessments**, without compromising safety standards, so that medicines can reach the market more quickly. EMA evaluations will be reduced from 210 days to 180 days.
- **Digital solutions**, such as online submissions and electronic leaflets, to provide patients with information in their own languages.

Simplification measures, including removal of duplicative procedures, will save public authorities and businesses 300 million a year.

The **right conditions for innovation**, to deliver for patients

- **More adaptive regulatory framework**, to support innovative solutions that can bring new medicines from laboratory to market faster.
- **Groundbreaking approaches such as regulatory sandboxes** to test new approaches and accelerate the development of innovative therapies.
- **Internationally competitive incentives** to ensure Europe remains an attractive hub for investment and innovation.
- **Simplified manufacturing of innovative medicines** by allowing decentralised sites to manufacture them at the patient's bedside.
- **Support for novel antibiotics**, to combat antimicrobial resistance, through new voucher scheme and potential guaranteed revenue scheme for companies investing in their development.

Stronger **safeguards against medicine shortages**

- **Stronger supply chain obligations on companies** to prevent shortages.
- **EU framework to monitor and tackle shortages** of medicines, with a stronger coordination role for EMA in case of critical shortages.
- **An EU-wide list of critical medicines**, and the evaluation of supply chain vulnerabilities, to give clearer overview of potential weaknesses to be addressed.

More **EU-level support** and coordination

- **Early scientific support** from the EMA for developers of promising medicines – particularly SMEs and non-profit organisations.
- **Strengthened cooperation** among regulatory authorities, joint inspections and EMA support for more efficient supervision systems.

An extra year of regulatory protection for companies that deliver functioning innovative therapies against multi-drug resistant infections will make the EU's system one of the most generous in the world.

Leveraging networks towards impact

What do we want to achieve?

- **Informed, sound, fit for purpose PIPs**
- **Well-designed, participant-centered, fit for purpose trials**
- **Completed trials**
- **Completed PIPs**

Which levers do we favor?

- **Goal-driven**
- **Fit for purpose**
- **User-focused**
- **Performance and quality-driven**
- **Predictable**
- **Involving the right stakeholders**
- **Sustainable**

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Principles governing change in networks

Turn opportunities of the new pharma package into action

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EnprEMA mandate starting point…

Reg 1901/2006

Article 44:

1. The Agency shall, with the scientific support of the Paediatric Committee, develop a European network of existing national and European networks, investigators and centres with specific expertise in the performance of studies in the paediatric population.

2. The objectives of the European network shall be, inter alia, to coordinate studies relating to paediatric medicinal products, to build up the necessary scientific and administrative competences at European level, and to avoid unnecessary duplication of studies and testing in the paediatric population.

...and evolution

New Pharmaceutical Legislation (NPL), Proposed Regulation, Article 95:

1. The Agency shall~~, with the scientific support of the Paediatric Committee,~~ develop a European network of ~~existing national and European~~ patient representatives, academics, medicines developers, investigators and centres with ~~specific~~ expertise in the performance of studies in the paediatric population.

2. The objectives of the European network shall be, inter alia, to discuss priorities in the clinical development of medicines for children, in particular in areas of unmet medical need, to coordinate studies relating to paediatric medicinal products, to build up the necessary scientific and administrative competences at European level, and to avoid unnecessary duplication of studies and testing in the paediatric population.

Reflections on future-proofing EnprEMA

RF. Personal views. Generated with ChatGPT

Strategy to action: 2026 and beyond

HOW?

Actively engage, facilitate and contribute

Generate consistent actionable and impactful outputs

- First EnprEMA/ACT EU Workshop (May 26)
- EnprEMA Annual meeting (Nov 26)
- Contribution to relevant EMA events and documents
- Dialogue with stakeholders and decision-makers
- Potential new WGs
- Potential new dissemination and educational events

WHAT?

Accelerate pediatric-centered, aware, relevant and informed drug development research

Focus on critical current and future-facing priorities

- Paediatric trial platforms
- Patient recruitment and retention
- Patient engagement and experience
- Paediatric trial assessment
- Methodologies to support extrapolation and prioritization, including disease modelling, data-driven approaches
- Support to regulatory developments (PIP modalities - MoA, adaptive)
- Infrastructure readiness

- ***Hear, make aware and prepare*** the pediatric community
- ***Engage and inform*** discussions about the future of EnprEMA

Get involved with EnprEMA

Join as a member network

Enpr-EMA welcomes new member networks interested in strengthening collaboration in paediatric medicines research (EnprEma Network Database)

Learn more online

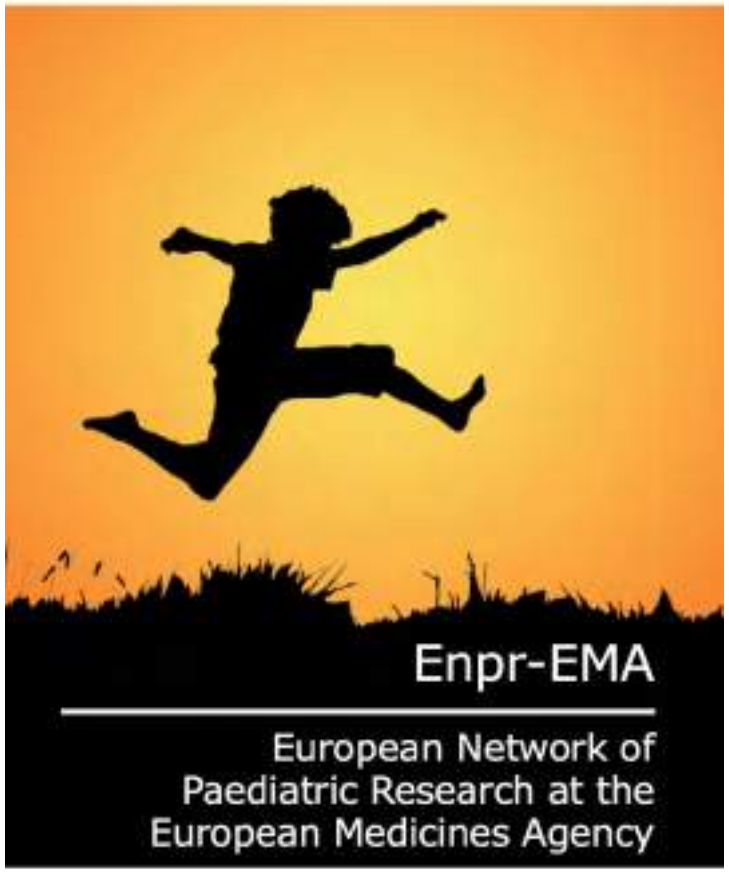
Find information on EnprEMA's current priority initiatives as well as previous outputs on the EnprEMA webpages

Take part in annual meetings and workshops

Stakeholders can also get involved through EnprEMA annual meetings and other workshops that support exchange across stakeholders.

Save the date

5 November 2026: annual EnprEMA stakeholder meeting



Working together towards better medicines for children, step by step

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