Minutes of the 2025 annual internal meeting of the members and Coordinating Group of the European network of paediatric research at the EMA (EnprEMA)

Date: Tuesday, 19 November 2025

Location: EMA, Amsterdam and online

The 2025 annual meeting of EnprEMA member networks took place on 19 November at the European Medicines Agency (EMA) headquarters in Amsterdam, with virtual participation available for remote attendees. The agenda featured updates from EnprEMA working groups, internal activities, strategic objectives, EnprEMA and discussions on innovation and modernisation strategies to advance paediatric clinical research. A key highlight of the event was the election of a new Chair of the EnprEMA Coordinating Group.

Chairpersons: Pirkko Lepola, Gunter Egger

Update from EnprEMA working groups:

Working group on paediatric clinical trial site quality criteria

Ricardo Fernandes presented the progress of the EnprEMA working group on quality criteria for paediatric clinical trial sites.

In 2022, EnprEMA and connect4children (c4c) held a workshop to define quality requirements for paediatric clinical trial sites. Based on its outcomes, the working group established a shared definition of "quality of paediatric sites", reviewed existing guidance and standards, and produced a report outlining key criteria for paediatric sites.

The report underwent several revisions including a public consultation with EnprEMA networks. The comments received ranged from minor editorial adjustments to significant conceptual recommendations. Following incorporation of the feedback and alignment with existing guidance, such as ICH E6 (R3), the group finalised the document to prepare it for publication and dissemination. In addition, a guidance document will be published on the EnprEMA website.

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Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-quality-criteria-paediatric-clinical-trial-sites-enpr-ema-initiative-r-fernandez-p-skovby_en.pdf

Working group on cross-border clinical trials

Begonya Nafria presented the progress of the EnprEMA working group addressing cross-border access to paediatric clinical trials. The group aims to analyse barriers to international patient participation and examine ethical concerns arising from exclusion criteria based on language requirements or country of residence.

Analysis of multiple data sources - including study protocols from public registries such as ClinicalTrials.gov and surveys of trial sites, patients, and sponsors - revealed that language criteria are frequently applied and that many sites lack dedicated support for the potential inclusion of international participants. Conversely, survey results highlighted patients' willingness to adopt decentralised or digital technologies to reduce travel burdens, as well as the need for standardised processes and early planning for translation and validation of trial materials.

The working group is preparing several manuscripts and will develop recommendations to facilitate the inclusion of paediatric patients in international clinical trials, with a draft expected for public consultation in 2026.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-paediatric-cross-border-clinical-trials-enpr-ema-working-group-b-nafria_en.pdf

Working group on paediatric research nurses

Pamela Dicks presented on behalf of the working group on paediatric research nurses, which has undertaken an analysis of the role of research nurses across the European Union. This work has primarily focused on career pathways, professional development opportunities, employment conditions, and the structural factors contributing to persistent vacancies. This initiative aims to advance and strengthen the role of paediatric research nurses across the European Union.

The results of two surveys that were distributed to research nurses and research nurse managers revealed consistent issues across countries, including contract instability, insufficient salary levels, challenges in retaining experienced staff, limited formal recognition of the profession, unclear role definitions, and restricted opportunities for career progression.

The findings also emphasise the need for a dedicated European research nurse network to facilitate the exchange of best practices, advocate for improved working conditions, and support the development of standardised job descriptions aimed at elevating the professional standing of paediatric research nurses.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-enpr-ema-paediatric-research-nurse-working-group-p-dicks_en.pdf

Working group on patient and public involvement (PPI) and Young Person's Advisory Groups (YPAG)

Segolene Gaillard presented the working group's strategy for Patient and Public Involvement (PPI), which focuses on mapping current PPI capacities in paediatrics, identifying training and resource

requirements, and establishing a structured framework to support the systematic engagement of children, young people, and parents throughout the research process.

The initiative began with the development of a questionnaire to assess existing PPI structures and activities. The survey gathers information on the characteristics, roles, and needs of both individual contributors and organised PPI groups in paediatrics, with the aim of capturing insights related to both disease specific and broader PPI activities.

The questionnaire will be finalised by January 2026 and will remain open until April 2026. Subsequent data analysis is scheduled for the following months, with results to be presented at the next coordinating group meeting.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-patient-public-involvement-ppi-working-group-s-gaillard_en.pdf

Discussion on the need for a Working Group on clinical trials in emergency settings.

The topic was postponed for the next Coordinating Group meeting, to discuss it in the context of other priority areas.

ACT EU: Collaboration with EnprEMA

Laura Pioppo and Monique Al delivered an in-depth presentation on the ACT EU initiative, outlining its main objectives. A proposal was presented to organise a joint workshop to define concrete actions to support the dialogue between regulators, sponsors and patients' representatives on clinical trials in paediatric population, which stemmed from earlier discussions between the leadership teams of both EnprEMA and ACT EU. This proposal builds upon the outcomes of the recently conducted workshop on the Assessment of Clinical Trials Applications involving Paediatric Patients that was held on 14 and 15 of July 2025, which aims to strengthen regulatory coherence and foster enhanced collaboration among stakeholders engaged in paediatric research.

The ACT EU programme is a collaborative initiative led by the European Commission, the Heads of Medicines Agencies, and the European Medicines Agency. It is designed to modernise and reinforce the clinical trial environment across Europe by promoting clinical trials that are more efficient, scientifically robust, and operationally streamlined. The overarching ambition of the programme is to ensure that Europe remains a competitive and attractive region for clinical research, particularly through initiatives that support the conduct of better, faster, and more innovative trials.

The outcomes of the assessors' and ethics committees' workshop held in July were examined in detail. The discussions underscored several areas requiring improved regulatory alignment, including divergent interpretations of existing frameworks, inconsistencies between Paediatric Investigation Plan assessments and clinical trial application evaluations, and persistent challenges related to inclusivity within paediatric studies. Participants also highlighted the importance of developing coherent extrapolation methodologies and recognised the need for earlier and more meaningful involvement of patients and families in the planning and evaluation of paediatric clinical research.

Drawing on these findings, a series of proposed actions was presented to support improved decision making and strengthen available guidance in the field. These actions include the development of a structured decision-making framework for paediatric clinical trials, the revision of the recommendation

paper on ethical considerations in clinical trials involving minors, and the preparation of sponsor guidance and targeted training materials.

The forthcoming ACT EU/EnprEMA workshop will bring together regulators, industry representatives, patient organisations, and other key partners to refine these initiatives and support their implementation across the paediatric research community.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-act-eu-collaboration-enpr-ema-l-pioppo-m-al_en.pdf

The future of Neonatology Research

Mark Turner presented key findings and recommendations from The Lancet Child & Adolescent Health Commission on the future of neonatology, emphasising persistent global barriers to neonatal research and the need for coordinated international action. He highlighted structural obstacles, methodological gaps, and the importance of establishing a unified global framework to support high-impact neonatal research.

The identified barriers to neonatal research included excessive administrative burden, inconsistent regulatory interpretation, limited availability of neonatal expertise, privacy issues preventing data collection and sharing and inadequate support for industry engagement. To address these issues, the Commission recommends strengthened communication between regulatory authorities and ethics committees, more flexible consent procedures, and a greater focus on clinically meaningful patient outcomes.

To advance global collaboration, the Lancet Commission proposes the creation of a mission-oriented global alliance and an international neonatal research consortium. These entities would coordinate priority research areas, advocate for sustainable funding, and ensure integration of patient and family perspectives in strategic planning.

Although engagement efforts are ongoing, challenges remain in mobilising adequate resources and embedding neonatal expertise within existing organisational structures. Continued collaboration and targeted investment will be essential to strengthen neonatal research capacity worldwide.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-lancet-child-adolescent-health-commission-future-neonatology-m-turner_en.pdf

Election of the new chair of EnprEMA's Coordinating Group

The EnprEMA Coordinating Group conducted an election to appoint its new Chair. Following brief presentations in which the candidates outlined their experience and strategic priorities for the network, Ricardo Fernandes was elected by majority vote.

Upon election, Ricardo Fernandes expressed appreciation for the group's confidence, acknowledged the contributions of the outgoing Chair and fellow candidates, and reaffirmed his commitment to advancing the network's objectives.

The group also reflect positively on the mandate of Pirkko Lepola, conveying sincere appreciation for her dedication, steady leadership and the significant contributions made under her guidance. Her commitment and professionalism were warmly acknowledged, and gratitude was extended for the strong foundation she leaves for the work ahead.

Paediatric Clinical Research: Overcoming challenges and hurdles to foster innovation with EnprEMA

Angeliki Siapkara, Dominik Karres, Sabine Scherer, and Maria Sheean presented an overview of recent developments related to mechanism of action–based Paediatric Investigation Plans (PIPs) and their potential implications for regulatory processes, methodological standards, and stakeholder collaboration in paediatric drug development. Under the proposed revised pharmaceutical legislation, paediatric drug development would be less exclusively driven by adult indications and the mechanism of action of a product could also determine the scope of expected paediatric development. Concepts such as the early identification of unmet medical needs, and the systematic assessment of product and disease related mechanisms of action already play an important role in PIP development and are not new but may gain increased relevance under the revised framework.

Drawing on experience from paediatric oncology, where mechanism of action–based requirements have already been applied by the U.S. Food and Drug Administration under the RACE act, as well as through selected voluntary European submissions, the presenters highlighted the need for robust non-clinical evidence packages, stepwise development strategies, and multidisciplinary scientific evaluation. These experiences demonstrate both the potential advantages and the practical challenges associated with adopting mechanism of action assessments at an early stage of paediatric development. In this context a draft Concept Paper on proof-of-concept data supporting the development of anti-cancer products in paediatric patients is expected to be released for public consultation in the first quarter of 2026. EMA intends to organise a workshop in September 2026 to address the comments received during the public consultation.

The Paediatric Committee (PDCO) has identified several general key issues, including the definition of the applicable PIP condition, the need to revise the class waiver list, the limitations of available paediatric data, and the constraints of extrapolating strategies at very early development stages when adult data are not yet available.

The presenters emphasised the central role that research networks are expected to play e.g. by developing and reviewing methodological guidance, preparing structured case studies to support regulatory decision making, assisting with prioritisation, and facilitating coordinated stakeholder engagement. Such activities will be crucial to ensuring that paediatric development programmes remain feasible, scientifically robust, and responsive to unmet clinical needs.

Presentation:

https://www.ema.europa.eu/en/documents/presentation/presentation-paediatric-clinical-research-overcoming-challenges-hurdles-foster-innovation-enpr-ema-siapkara_en.pdf

https://www.ema.europa.eu/en/documents/presentation/presentation-non-oncological-moa-based-pip-initial-deliberations-pdco-working-group-discussions-s-scherer_en.pdf-0

https://www.ema.europa.eu/en/documents/presentation/presentation-mechanism-action-based-pips-paediatric-oncology-m-sheean_en.pdf

Strategic aims for 2025-2027

The EnprEMA Coordinating Group was renewed in April 2025 for a new mandate extending through 2027. As the governing body of the network, the group plays a central role in shaping its short- and long-term strategic direction. During the meeting, Pirkko Lepola led a discussion focused on defining the group's key priorities for the upcoming year, informed by reflections on the current European clinical research landscape, noting the decline in the number of clinical trials conducted in Europe,

including paediatric studies. This downward trend was identified as a pressing issue requiring coordinated action to revitalise trial activity, foster methodological innovation, and promote a more sustainable environment for paediatric drug development.

In the context of strategic alignment, the group considered how EnprEMA can contribute to key European Union priorities within the evolving life sciences framework. Several areas for meaningful impact were identified, including the strengthening of research ecosystems, support for the development and assessment of advanced therapies, enhanced use of high quality real world data, and deeper engagement with patients, families, and the wider public. These priorities were recognised as essential for improving the effectiveness, relevance, and inclusiveness of paediatric clinical research across Europe.

To advance these objectives, members proposed the establishment of new working groups focused on emerging legislative, regulatory, and strategic developments. Suggested areas of focus include facilitating the implementation of the forthcoming pharmaceutical legislation, assessing the EU life sciences strategy from a paediatric perspective, and ensuring timely responses to ongoing regulatory, scientific, and funding initiatives. These efforts aim to strengthen EnprEMA's role to contribute proactively to policy discussions and to strengthen the paediatric research landscape through coordinated, expert-driven action.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-strategic-aims-enpr-ema-2025-2027-p-lepola_en.pdf

New database / IT structure for members

Nuria Barcons provided an overview of the ongoing modernisation of the Enpr-EMA database, highlighting a range of significant enhancements intended to strengthen its functionality, security, and overall user experience.

A key aspect of this modernisation effort is the introduction of a new web-based self-assessment form. The registration process is expected to have reduced manual workload and minimise the risk of errors. The platform will also allow multiple users from the same network to complete the same form collaboratively. Furthermore, users will benefit from faster and more secure system access, supported by improved authentication mechanisms. The new application has been deployed on the 20 January 2026, all networks were kindly asked to update their record in the system by 20 April 2026.

Presentation: https://www.ema.europa.eu/en/documents/presentation/presentation-enpr-ema-modernisation-n-rodriguez-barcons_en.pdf

Enhancing awareness of EnprEMA and feedback on the impact of its work

Members discussed the importance of enhancing awareness and understanding of EnprEMA and its activities across all stakeholder groups and the general public. In particular, the discussion focused on how the network's value, impact, outcomes, and opportunities for collaboration could be communicated more effectively to external audiences.

During the exchange, members emphasised that the level of awareness of EnprEMA varies considerably across therapeutic areas. While certain sectors demonstrate a solid degree of familiarity and engagement with the network, others appear to have limited knowledge of its objectives and available resources. This uneven recognition highlights the need for clearer communication, strengthened branding, and more consistent outreach efforts to ensure that all relevant stakeholders are appropriately informed.

Participants further expressed uncertainty regarding how EnprEMA's outputs, such as recommendation papers and guidance documents, are being utilised by stakeholders, as well as the extent to which these outputs translate into practical impact. The discussion also underscored the importance of reaching children, who are the primary participants of the network's activities, through appropriately adapted and accessible materials. These observations reinforce the need for mechanisms that gather feedback, monitor the use of EnprEMA outputs, and assess their influence on regulatory or developmental practices, as well as explore the possibility of making these outputs age-appropriate.

Enhancing visibility, establishing structured feedback channels, and strengthening engagement strategies were identified as essential steps to reinforce the network's overall effectiveness and broaden its reach.

A.O.B. and wrap-up

The meeting was concluded by the chairs thanking all participants for their contributions. The Annual Internal Meeting was followed by the Annual Meeting of the European network of paediatric research at EMA (EnprEMA) conducted on the 20th of November 2025.

Speakers:

- Al, Monique. CCMO – Central Committee on Research Involving Human Subjects (The Netherlands), CTCG – Clinical Trials Coordination Group, MedEthicsEU
- Barcons, Nuria. European Medicines Agency
- Dicks, Pamela. ScotCRN – Scottish Children's Research Network
- Egger, Gunter. European Medicines Agency, Co-chair of Enpr-EMA
- Fernandes, Ricardo. STAND4Kids – Supporting Paediatric Trials in Portugal
- Gaillard, Segolene. French Paediatric Research Network (Kids France)
- Karres, Dominik. European Medicines Agency
- Lepola, Pirkko. Finnish Paediatric Research Network (FINPEDMED), Chair of Enpr-EMA
- Nafria, Begonya. European Young Persons Advisory Groups Network (eYPAGnet)
- Pioppo, Laura. European Medicines Agency
- Sanchez, Isabel. European Medicines Agency
- Scherer, Sabine. Federal Institute for Drugs and Medical Devices, Germany (BfArM), Paediatric Committee (PDCO)
- Sheean, Maria. European Medicines Agency
- Siapkara, Angeliki. EFPIA – European Federation of Pharmaceutical Industries and Associations, AstraZeneca
- Skovby, Pernille. Danish Paediatric Medicine Research Network (DanPedMed), Paediatric Committee (PDCO)
- Turner, Mark. conect4children stichting, University of Liverpool, United Kingdom