Minutes - Enpr-EMA Coordinating Group & networks meeting

Date: 12 June 2024; 15:30-17:00 CET; via Webex

Chairpersons: Pirkko Lepola / Gunter Egger

Invitees: Coordinating Group, Network members and observers.

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Update on legislative progress for new pharmaceutical legislation:

Pirkko Lepola gave an update on the main aspects of the revision of the pharmaceutical legislation related to paediatric medicine development. The legislation has completed the revision of the EU Parliament, which adopted its position on 10 April 2024, and is currently under review by the European Council. The new EU Parliament, following the recent EU elections, will continue to follow up on the legislation.

Key changes in the proposal:

The main changes in the proposal include:

- Structural changes in the European Medicines Agency (EMA) and its committees.
- New approaches regarding the regulation of paediatric medicines, such as adaptative models for Paediatric Investigations Plans (sPIP), strategies for medicine development based on the mechanism of action, shortened timelines for deferrals, implementation of a temporary waiver, increased transparency on PIPs, the inclusion of nonprofit applications to enhance repurposing of medicines, and the continuation of rewards.

The proposal also suggests broadening the remit of Enpr-EMA, potentially including a role in prioritisation and involvement in larger multistakeholder discussions.

Enpr-EMA Working Group update:

Paediatric clinical trial site quality criteria:

The conduct of paediatric clinical trials involves additional complexities compared to adult trials, increasing the demand for clinical trial site quality and performance. To adress this, two subgroups of the Enpr-EMA working group, representing all stakeholders, have collaborated to define the

quality criteria for paediatric clinical trial sites, and identify such quality standards applicable for all trial sites. The joint findings were presented during the meeting by Ricardo Fernandes and Pernille Skovby.

Pernille Skovby presented the working group's activities, which have involved several steps: literature search and indexing; development of categories based on literature, experience and previous work done by c4c and the Enpr-EMA working group on international collaboration on paediatric site requirements; followed by adjustments to consider paediatric specificities.

The categories developed include:

- Staff experience and requirements,
- Documentation,
- Infrastructure,
- Cycle times,
- Patient engagement.

Project outcomes:

A core definition of 'paediatric site' has been developed, demonstrating that common areas and categories for performing clinical trials can be identified across countries and specialties. The project aims to provide a roadmap for exemplary practices in paediatric clinical trials, establishing a core set of site readiness practices applicable across different geographies and specialties. This standardisation could streamline site assessment and selection for high-quality paediatric clinical trials.

Next steps:

The results report has received input from the Good Clinical Practice Inspectors Working Group (GCP IWG) and it will be submitted for input from Enpr-EMA members in the summer. The final document will be presented at the Enpr-EMA Annual Meeting in October 2024 and published on the Enpr-EMA website. Further publications and dissemination strategies will be defined at a later stage.

Cross-border clinical trials:

The working group aims to facilitate cross-border access for paediatric patients to clinical trials conducted in a language other than the patient's mother tongue, and in a country different from their country of residence in Europe.

Current activities:

The group is currently collecting data to identify cases where language or the country of residence has been used as eligibility criteria in paediatric clinical trials. The following data sources are being used:

- Survey of clinical sites in Europe: Collecting information about good practices and instances of discrimination based on language as an exclusion criterion.
- Survey of parents and young patients: Exploring their experiences and preferences,

including views on decentralised elements in clinical trials.

- Clinicaltrials.gov analysis: Analysing protocols of active paediatric studies in the USA to identify eligibility criteria related to mother tongue and country of residence of the patient.
- Questionnaire and expert interviews: Gathering insights from industry representatives and academic experts.
- Case study: Conducting an in-depth analysis of a specific instance.

Begonya Nafria presented the data collected so far, including the protocols identified on clinicaltrials.gov and results from the site and parent surveys. These efforts have already identified 9 cases of discrimination based on language or country of residence.

Next steps:

The group will continue data collection and analysis with the goal of developing consensus guidance following a consultation process with all relevant stakeholders.

International collaboration:

The working group has developed two manuscripts focused on the clinical trial authorisation process and the ethics review process for paediatric clinical trials across the six jurisdictions represented in the group. The aim is to support the conduct of global paediatric clinical trials.

Next steps:

The manuscripts are close to completion and are scheduled for submission for publication in October.

Research staff, nurses:

The group was formed to strengthen the role of paediatric research nurses in clinical trials across Europe. To explore the current landscape, a survey was distributed to paediatric research nurses and paediatric research nurse managers. The survey aimed to understand employment conditions, identify career and development opportunities, assess training needs, and address recruitment as well as staff retention issues. The survey was translated into French, Italian, and Spanish and was distributed among Enpr-EMA members and c4c.

Preliminary findings:

Pernille Skovby told about the main findings. As of now, 158 responses from 12 countries have been received. Preliminary conclusions indicate a desire among research nurses in Europe to form a community to enhance peer-to-peer communication, training, and support.

Next steps:

It is planned to present the results and conclusions at the Enpr-EMA Annual Meeting in October.

Patient and Public Involvement (PPI) and Young Person's Advisory Groups (YPAG)

PPI activities and capabilities in paediatric clinical trials in Europe are not very well known.

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Consequently, there is a need to develop and strengthen PPI capabilities in paediatrics in Europe and to standardise the process to ensure meaningful involvement of paediatric patients and parents.

Formation of a new working group:

To address this need, a new Enpr-EMA working group on patient and public involvement is being created. The proposed objectives of this group are:

- Mapping existing PPI capabilities in paediatrics,
- Evaluating the needs of identified groups in terms of training and resources,
- Developing an appropriate framework to involve children, young people and parents.

Call for volunteers:

A call for volunteers to join the group has been made. Interested members should send their expression of interest to Enpr-EMA secretariat (enprema@ema.europa.eu) by the end of June.

The first meeting of the group will take place in July 2024, to define the objectives, working plan, tasks and potential outcomes of the group.

Review/prioritisation of topics for annual meeting on 1st/2nd October 2024:

There was a general discussion about the topics to be presented in the upcoming Enpr-EMA Annual Meeting. Throughout the year, several topics were identified. These were presented to confirm and prioritise the most relevant ones for the members.

The discussion (supported by voting via SLIDO) indicated high interest in the following topics:

- Mechanism of action concept for paediatric drug development,
- New pharmaceutical legislation and impact on paediatric medicines,
- Health Technology Assessment and the potential for patient involvement in decision-making,
- Real World Data for regulatory purposes,
- Clinical Trial Regulation (CTR) and Clinical Trial Information System (CTIS), particularly to share experiences from networks on the implementation of the CTR for paediatric clinical trials and low interventional clinical trials.,
- Stepwise Paediatric Investigation Plan (PIP).

Additionally, members expressed interest in paediatric medical devices and the COMBINE project (regulatory assessment of combination trials with medicines and medical devices).

Next steps:

Following these discussions and SLIDO voting results a draft agenda for the Enpr-EMA Annual Meeting will be prepared and shared with the Coordinating Group for finalisation.

A.O.B:

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Members were informed about the following upcoming meetings:

- **1st & 2nd October 2024: Annual Enpr-EMA Meeting**
  - Location: EMA, Amsterdam
  - 1st October: Closed meeting for Enpr-EMA networks
  - 2nd October: Open workshop for all interested stakeholders
  - Further information regarding registration will be shared shortly.
- **22nd & 23rd October 2024: Annual EFGCP "Better Medicines for Children" Conference**
  - Location: Brussels
  - For more information and registration, please click here.

Announcements:

- ACT EU Multistakeholder Platform Advisory Group (MSP AG) has been established with Maria Lamas (Executive Director of the Spanish Medicines Agency) as the Regulatory Co-chair and Denis Lacombe (CEO at EORTC) as the Stakeholder Co-chair. The first MSP AG meeting will be on 4 July. This meeting will not be broadcast, but agendas and minutes will be published on the ACT EU webpage.
- Two new ACT EU Advice Pilots to improve clinical trials in Europe have been launched. More information can be found in this news announcement
- Public consultation on the Declaration of Helsinki on Medical Research involving Human Subjects, open for comments until 24 June 2024. Information on the consultation and how to submit feedback can be found here.