Minutes - Enpr-EMA Coordinating Group meeting

Date: 4 March 2024; 15:30-17:00 CET; via Webex

Chairpersons: Pirkko Lepola / Gunter Egger

Invitees: Coordinating Group members and observers

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Adoption of agenda:

The agenda was adopted without changes.

Introduction of new members and observers of the Coordinating Group (CG):

POLPEDNET (Polish Paediatric Clinical Trials Network), a network founded in 2017 covering all paediatric specialties, counting with 9 major centres in Poland, and representing a c4c national hub, was warmly welcomed as category 2 member of Enpr-EMA.

Thomasz Grybek, representative of EURORDIS and a patient representative in PDCO, was warmly welcomed as new observer of the CG.

A conflict of interest was declared by Marek Migdal as head of POLPEDNET and member of Enpr-EMA CG, thus he did not participate in endorsement activities.

Enpr-EMA membership criteria and revision of the self-assessment form:

It was highlighted that the original self-assessment form and membership criteria served their purpose very well during the initial years of the establishment of Enpr-EMA, but that the present revision of the self-assessment form was necessary to optimise the information collected in relation to a changed research landscape and to reduce bureaucracy, while keeping an adequate amount of data of each of the networks to be provided publicly via the Enpr-EMA database and ensuring a level of quality standards for Enpr-EMA members.

In addition, the revision was considered necessary to delete or clarify those sections which have been unclear, and to delete sections which required references that had not been provided by most of the networks for many years (or never), and which were no longer deemed necessary.

The proposed revised form was shared with the CG members in advance of the meeting for their review and comments, which were discussed during the meeting.

The proposed changes concerned the information collected regarding the network details,

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requirements for provision of supporting evidence, information on the governance structure, access to expert groups, quality management and the involvement of public and patient organisations.

In general, the requirement to be able to involve patient organisations in the setting up of clinical trials was perceived as being difficult to fulfil for some of the networks, mostly for industry-sponsored trials. However, considering the growing relevance of the involvement of patients in the setting up of paediatric clinical trials, it was agreed that the information on the access to these groups should be available to describe the network characteristics. It was agreed that a question in this reqard should be answered by all networks applying for Enpr-EMA membership.

It was concluded that an investigation into the current abilities of member network to involve patient organisations could be part of the remit of the new working group on patient and public involvement (PPI).

Overall, the members were in favour of the proposed changes to the self-assessment form.

EMA will implement the revised self-assessment form together with an update of the Enpr-EMA database, in consultation with EMA's IT department. This work will probably not be completed prior to 2025.

Of note: The scope of the current review of Enpr-EMA membership criteria and self-assessment form was limited to the membership of research networks. Discussions on inclusion of other stakeholder groups, as well as of Enpr-EMA's processes and governing structure in light of the proposed new European pharmaceutical legislation will be planned in due course.

Enpr-EMA Workplan for 2024/25:

Enpr-EMA's workplan for the remainder of 2024 and 2025 was discussed. The 4 active working groups (WG), the WG on international collaboration, the WG of research nurses, the WG on cross-border access to paediatric clinical trials and the WG on paediatric clinical trial site quality requirements will continue their activities. Current activities of the working groups include publications on the requirements for clinical trial applications and ethics reviews in different jurisdictions, an analysis of the data collected regarding the role and current situation of paediatric clinical study nurses, work on guidance to facilitate cross-border clinical trials and the drafting of recommendations on paediatric clinical trial site standards.

At the Enpr-EMA annual meeting conducted in October 2023, new topics of interest for future projects were identified, including the need for raising awareness and mapping of existing young person advisory groups (YPAGs), repurposing of medicines and the use of real-world data in the paediatric environment, clinical trials in special circumstances, data sharing, the inclusion of adolescents in adult trials and training needs for the members.

An update on the latest activities regarding these topics was shared with the members:

- Repurposing of medicines, use of real-world evidence: Members planning to engage in a specific project for repurposing of a medicine for a paediatric indication were informed of the possibility to discuss the necessary regulatory steps, including general principles on the use of real world data, with EMA during an academia briefing meeting organised by EMA's Regulatory Science and Academia Task Force (academia@ema.europa.eu).
- Patient and public involvement: Segolene Gaillard provided an overview of the activities that have been carried out since the Enpr-EMA Annual Meeting on the topic of 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. Thus, there is a need to standardise processes and requirements across Europe. The objective of mapping initiatives and existing groups was agreed, involving the creation of a database with the identified groups and their characteristics in terms of activities and type of expertise, and to evaluate the needs for these groups concerning training, resources and guidelines on methods and processes, to develop an appropriate framework for PPI activities in the EU.

Furthermore, as a result of the discussion of the Enpr-EMA self-assessment form, category 1 networks and their status of access to patient and parent groups will be investigated.

For these purposes a new WG will be created, chaired by Segolene Gaillard. A call for volunteers to participate in this working group will be made among the members.

- Training needs: Members were informed that a collaboration with ACT EU has been established to ensure that paediatric requirements are covered within their activities (e.g. events, training offerings).
- It was agreed that the topics related to clinical trials in special circumstances, data sharing, and the inclusion of adolescents in adult trials would not be priority actions of Enpr-EMA for the period 2024/25.
- In addition, a member of the CG suggested that medical devices for the paediatric population were a topic worthwhile for Enpr-EMA to engage in. However, in the lack of concrete proposals for objectives in this regard, the discussion on the potential inclusion of medical devices in a future working group of Enpr-EMA was postponed. However, due to the existence of a new project called "COMBINE" on the regulation of medicinal products and devices, it was deemed necessary to build upon the outcomes of this group before starting Enpr-EMA activities on this topic. (https://health.ec.europa.eu/medical-devices-topics-interest/combined-studies_en)
- Lastly, access to medicines, and health technology assessment were raised as topics of interest for paediatrics. It was highlighted that in preparation of the implementation of the new HTA Regulation many efforts are being made to facilitate equal access to medicines across Europe.

It was concluded that the main activities of Enpr-EMA for 2024 and 2025 will be focused on Patient and Public Involvement in paediatric clinical trials with the creation of a new working group, the finalisation of the ongoing activities of the existing working groups and in addition, following up on the results of the ongoing work on combining medicines and medical devices assessment, and the upcoming HTA activities.

Raising awareness about Enpr-EMA.

Aiming to increase the awareness of and to publicise Enpr-EMA across all stakeholders and countries, some measures were discussed to promote Enpr-EMA activities, the benefits for the members and for paediatric patients, along with the information on the Enpr-EMA member networks and database.

The following ideas were raised by the members:

- Increase communication using the already established communication channels, targeting

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all European paediatric disease groups, societies, and pharmaceutical companies.

- Increase the presence in social media and networks.
- Report on achievements via newsletters.
- Publications, presentations at conferences and promoting events.

It was pointed out that the increase in communication efforts should be made by EMA and Enpr-EMA members via their applicable communication channels and web pages.

In addition, a promotional slide informing audiences about Enpr-EMA, its main objectives and contact details has been created and will be published on the Enpr-EMA website. Enpr-EMA members are encouraged to use it (e.g. display it at the end of their presentations) at any applicable conference or symposium.

A.O.B:

Members were informed about the following upcoming meetings:

- The **next coordinating group and networks meeting** will take place on the 12th of June 2024 via Webex.
- The next **Enpr-EMA annual meeting and workshop** will take place on the 1st and 2nd of October 2024 at the premises of the European Medicines Agency and online via Webex.
- The 5th Nordic Conference on Paediatric and Orphan Medicines will take place on the 14th and 15th of May 2024 in Helsinki.

An update was given on ACT EU activities related to the paediatric environment:

- A multistakeholder platform has been created with paediatric representation within the advisory group, that will permit a closer collaboration between Enpr-EMA and ACT EU and the creation of joint events. A full list of the members can be found here: https://accelerating-clinical-trials.europa.eu/our-work/multi-stakeholder-platform/msp-advisory-group-members_en
- The ACT EU methodology workshop took place in November 2023, where several Enpr-EMA members participated. The methodology guidance report, as an outcome of the workshop, is available here: https://accelerating-clinical-trials.europa.eu/document/download/451d68ff-a998-43cf-a590-45b781c28ff1_en?filename=ACT%20EU%20multi-stakeholder%20workshop%20on%20methodology%20guidance%20%E2%80%93%20report.pdf