Minutes - Enpr-EMA Coordinating Group & networks meeting

Date: 6 June 2023; 15:00-16:30 CEST; 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):

Mariska Mulder, new observer representative of EUCOPE (European Confederation of Pharmaceutical Entrepreneurs), was warmly welcomed.

Accelerating Clinical Trials in the EU (ACT EU):

Support for academic sponsors to set up multi-national clinical trials:

As part of priority action 2, ACT EU will implement activities to support academic sponsors in setting up multi-national clinical trials.

An initial analysis identified need for support in the areas of coordination, network connection, data sharing, funding, training as well as regulatory and legal support.

Enpr-EMA members were welcomed to provide further input to this analysis and report on the hurdles experienced in the conduct of multi-national academic clinical trials. Also, feedback on which initiatives are already available to facilitate these trials and what actions could ACT-EU implement to support the conduct of such trials were expressed to be welcome. All Enpr-EMA members were asked to send feedback or suggestions to: acteu@ema.europa.eu or to academia@ema.europa.eu

Moreover, as part of priority action 3, ACT EU will develop a multi-stakeholder platform to promote dialogue amongst the stakeholders of clinical trials. Interested members were invited to take part in a kick-off meeting related to the setting up of this multi-stakeholder platform.

During the meeting, several opportunities for collaboration between initiatives performed by Enpr-

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EMA members and ACT-EU were highlighted:

1. Conect4children (c4c) has worked with sites and academic sponsors to aid the design, setting up and conduct of paediatric clinical trials.

2. The European Clinical Research Infrastructure Network's (ECRIN) facilitates the set up and conduct of multinational, mainly academic clinical trials, in Europe. Interactions between ECRIN and other platforms such as ACCELERATE have already occurred.

3. The European Rare Disease Research Coordination and Support Action consortium (ERICA), a project of the European Reference Networks (ERN), aims to facilitate access to high quality cross-border healthcare and promote and foster cooperation on rare disease healthcare between member states. A questionnaire has been developed to identify hurdles for conducting clinical trials in paediatric rare diseases. The results of the questionnaire were expected by the end of June.

It was stressed that paediatric specificities need to be addressed in ACT EU's deliverables and that those initiatives already ongoing in the paediatric area, as the ones listed above, need to be taken into account.

Recommendations regarding decentralised elements in clinical trials:

As part of priority action 8, ACT EU has brought stakeholders together to implement recommendations for the conduct of decentralised clinical trials, where new methodologies can improve trial accessibility and flexibility for the patients, by various ways, such as via home health visits, electronic informed consent and direct shipment of medicinal products to patients.

As a result, a recommendation paper on decentralised elements in clinical trials has been published including an Annex with national provisions that will be updated on a regular basis.

Representatives from Enpr-EMA and PDCO had participated in the drafting group and made comments to the draft prior to publication. It was thus assured that the decentralised clinical trials (DCT) recommendations also apply to the paediatric population and a need for specific paediatric recommendations in relation to DCT was thus not identified in this first version of the paper. PDCO and Enpr-EMA will continue to be involved in ongoing discussions to further improve the DCT paper and ensure incorporation of specific paediatric recommendations if found relevant.

Related information on the work of ACT EU on decentralised clinical trials can be found at the following links:

ACT-EU Multi-Stakeholder meeting on decentralised clinical trials (video recording and documents):

- ACT EU multi-stakeholder meeting on decentralised clinical trials | European Medicines Agency (europa.eu)

ACT-EU recommendation paper:

- Recommendation paper on decentralised elements in clinical trials (europa.eu)

Publication in The Lancet:

- Decentralised elements in clinical trials: recommendations from the European Medicines Regulatory Network - The Lancet
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Enpr-EMA Working group update:

WG International collaboration

Clinical Trial authorisation and ethics review across jurisdictions:

Aiming to support the conduct of global paediatric clinical trials, the group has been working on describing the CTA and EC processes for obtaining regulatory approval for the conduct of paediatric

clinical trials across the six jurisdictions, including Regulatory Authority authorisation and Institutional Review Board (IRB) /Ethics Committee (EC) review. Each topic is covered in a separate manuscript. Both manuscripts are close to completion, and submission to "Therapeutic Innovation & Regulatory Science" journal.

During the discussion, EU-Innovation Network (EU-IN) highlighted that they are producing a report on clinical trial authorisation for innovative therapies and technologies and that a collaboration with the group could be established.

WG Research staff, nurses

Aiming to strengthen the role of paediatric research nurses in clinical trials across Europe, a questionnaire will be launched to gain a better understanding of the employment conditions, to identify career and development opportunities, training needs and recruitment as well as staff retention issues. The survey has been translated into several languages and adapted to target research nurses and research nurse managers. The distribution of the survey will be performed through the nurses working group, local networks, Enpr-EMA and C4C. The working group was invited to share the final survey with the Enpr-EMA Secretariat for dissemination among the members. Collaborations were established via volunteers for the supporting the processing and analysing of the data.

The next activities of the group will be focused on establishing peer support and training activities.

WG Cross-border clinical trials

The working group has the objective of facilitating the cross-border access of paediatric patients to clinical trials in Europe.

The data collection phase has been divided in 3 phases:

1. **Analysis of clinical trial protocols** in the Clinicatrials.gov data platform, to obtain information about study design and eligibility criteria in terms of mother tongue and country of residence of the patient.

2. **Questionnaires sent to the clinical trial sites** to collect information about good practices and cases of discrimination based in the use of language as exclusion criterion.
3. **Questionnaire sent to patients and patients' organisations** to gather information about specific cases where patients were not able to participate in a clinical study based on their mother tongue and/or country of residence.

The aim of this working group is to publish guidance, after a public consultation, to facilitate the inclusion of paediatric patients in cross-border clinical trials in Europe.

WG Paediatric clinical trial site quality criteria

As a result of the workshop on paediatric clinical trial site quality criteria, co-organised in 2022 by Enpr-EMA and c4c, three action points were identified: definition of quality of paediatric clinical trial sites, identification and mapping of existing quality standards in use, and implementation of the recommendations for quality criteria/standards.

Two sub-working groups were created to elaborate on the action points:

WG 1: working on defining what is a paediatric clinical trial site, what is quality of a paediatric site,

why are paediatric site standards needed and how to identify them across different jurisdictions, paediatric age ranges and sponsors.

WG 2: working on identifying and mapping existing quality criteria/standards for sites. Building upon the knowledge gained with the previous industry and CRO survey on understanding the concept of site quality criteria and its later division into categories.

A short update on the groups' work was provided. It is expected that preliminary results can will be presented at the annual Enpr-EMA meeting in October 2023.

WG Off-label evidence

The group has published an article that outlines the fact that approximately 50% of the medicines used for children in the EU are used off-label and that in many cases there is sufficient evidence data of their use that could permit regulatory approval and inclusion of the paediatric indication in the product information.

The main objective of the article is to advocate for repurposing of paediatric medicines.

The publication can be found at the following link (no free access):

Off-label is not always off-evidence: authorising paediatric indications for old medicines - The Lancet Child & Adolescent Health

New pharmaceutical legislation & Enpr-EMA:

It was mentioned that within the European Commission's proposal for the revision of the EU pharmaceutical legislation, some changes are contemplated for the role of Enpr-EMA, including the widening of its objectives, to cover topics such as unmet medical needs and to expand the members to patients and medicine developers. Related information could be found at the following link:

https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12963-Revision-of-the-EU-general-pharmaceuticals-legislation_en

Next events:

- **ACT EU multi-stakeholder workshop on ICH E6(R3), public consultation** on the 13th and 14th July 2023
  https://www.ema.europa.eu/en/events/act-eu-pa04-multi-stakeholder-workshop-ich-e6-r3-public-consultation

- **Enpr-EMA face-to-face annual meeting** to be held on the 9th and 10th October 2023.