European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Response to the concept paper for public consultation on the Commission guideline on the format and content of applications for paediatric investigation plans.

Submitted by The European Network of Paediatric research at the EMA (Enpr-EMA)

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The European Network of Paediatric Research at the EMA (Enpr-EMA) proposes to add a few short sentence encouraging applicants to consult members of Enpr-EMA, either national or specialty networks, when developing a Paediatric Investigating Plan (PIP).

This could be added in section 2.5.4. Paediatric clinical studies:

The European Network for Paediatric Research at the European Medicines Agency (EnprEMA) provides a point of contact for a number of specialty and multi-specialty networks. The involvement of clinical research networks enhances the development of PIPs in a number of ways; the involvement of networks therefore should always be considered when preparing the Paediatric Investigation Plan. Areas of expertise that can be offered by Enpr-EMA members will be found at the Enpr-EMA database.

Early involvement of paediatric clinical research networks during preparations of PIPs may be beneficial in several aspects as networks can help with:

1. Natural history studies
    - Identification of existing databases
    - Locate study sites and investigators to conduct natural history studies
2. Validated feasibility studies
    - Access to clinicians who can provide data about patient throughput or develop bespoke feasibility assessments
3. Patient and public involvement
    - What are the important needs?
    - What are important outcomes?
    - What are acceptable trial procedures / visit schedules etc.?
4. Discussion with professionals
    - Clarifying scientific questions

The potential benefits of these relationships are:

- Indications tailored to clinical need and available populations
- Drug development plans and trial protocols tailored to feasibility assessments
- Increased engagement with clinicians: quicker recruitment if recognised experts or key opinion leaders in the field have contributed to study design
- Increased engagement with children, young people and families resulting in increased adherence to protocols
- Validated data for regulators about feasibility and family views, potentially allowing for smoother evaluations of PIPs
- Clinical Research Networks can also contribute to parallel discussions about drug development and health technology assessments.