Enpr-EMA newsletter 2015

Dear friends and colleagues,

We want to briefly inform you about the activities of Enpr-EMA throughout the year 2014 and plans for 2015.

Enpr-EMA expanded with the addition of 4 new networks that joined as Enpr-EMA registered category 3 networks:

- EPLTN (European Paediatric Liver Transplantation Network);
- PEDDCReN (Paediatric European Digestive Disease Clinical Research Network);
- ESPNIC (European Society of Paediatric Neonatal Intensive Care) Medicine Research Network;
- ECAPN (European Network Child & Adolescent Psychopharmacology Network).

Enpr-EMA has currently 45 registered networks:

- 19 networks (42%) were recognised as Enpr-EMA Category 1 (full Enpr-EMA members);
- 3 networks (7%) were recognised as Category 2;
- 19 networks (42%) were recognised as Category 3;
- 4 networks (9%) were recognised as Category 4.

Information of each individual Enpr-EMA registered network that has submitted their data in a self-assessment report can be found in the fully searchable Enpr-EMA Network Database published on the Enpr-EMA website. This is the central resource for researchers and study sponsors seeking to identify research networks for paediatric clinical trials in Europe. Centres can be identified through networks.

The next Enpr-EMA workshop will be on 28th May at the EMA in London. On the next day there will be a meeting of the network members followed by a meeting of the Coordinating Group.

Working groups

Several ad-hoc working groups (WG) set-up in 2013 developed pragmatic responses to some of the most urgent needs that can be implemented within 6 – 12 months. The focus was on stating what networks can do, or what networks need to do, rather than developing comprehensive guidance. There is already good practice in many of these areas so that EnprEMA needs to focus on disseminating good practice rather than developing new solutions.

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WG "Dialogue and interaction with Ethics Committees":

After analyzing existing problems from a global perspective and a national/local perspective, 7 short-term – and 5 long-term recommendations, i.e. specified deliverables, suggestions for what could be done, were proposed. The group identified variation in requirements for consent / assent as the greatest challenge in achieving ethical approval across EU member states and developed as a practical deliverable a table that shows requirements regarding consent of children in the various member states, including legislative surroundings of the informed consent requirements for pediatric clinical trials. The work done will be submitted to a scientific journal and then also published on the Enpr-EMA website.

WGs "How to establish communication between Enpr-EMA, networks and industry" and "Sharing good practices within Enpr-EMA and with industry" decided to merge.

A survey questionnaire to collect good practice examples was sent to network members and pharmaceutical companies. A total of 89 responses were received: (19 network responses and 70 industry responses). A final report on the outcomes of the survey with examples of good practice as well as a list of ideal services that networks can provide was drafted and will be submitted to a scientific journal and then also published on the Enpr-EMA website.

WG "Framework for networks to interact with industry and regulators when implementation/conduct of clinical trials agreed in PIPs is no longer possible".

The WG identified 2 key priority needs and summarised them in a draft report:

- Both industry and regulators need a "community of experts" for consultation on scientific expertise, feasibility and ethical considerations. Any such "community of experts" need to ensure complete clarity of potential conflicts of interest, in a format that is unambiguous, easy to provide and view. Academic networks themselves need explicit transparency regarding funding sources for their organisation and for any collaborative work or trials conducted under the name of that organisation.
- Multi-stakeholder meetings were identified as an efficient process to benefit patients/public as well as industry, networks and regulators when there are uncertainties about paediatric drug development. The meetings need a clear remit and terms of reference to find solutions. Industry consultation with Enpr-EMA networks should be enhanced when PIP feasibility issues occur.

WG "Neonatology":

To follow-up on a neonatal scientific workshop at FDA, attended by the working group chair, and on preliminary discussions at the annual meeting of the European Academy of Paediatrics, a meeting is being organised at the EMA early 2015 to discuss the way forward to collaborate with FP7 networks/learned societies and PDCO Working Group on neonatology about drug development in neonates and to consider a strong network for the delivery of neonatal trials.

Joint Enpr-EMA/ENCePP Working group "Paediatric Pharmacovigilance":

The WG actively contributed to the drafting of the revised paediatric pharmacovigilance guideline and started discussion with ENCePP colleagues on the types of post-authorisation safety studies (PASS) in preparation of the next planned action point, i.e. to define criteria to conduct PASS studies in children

and create a short questionnaire to ENCePP/Enpr-EMA networks to identify which networks would be able to conduct such studies.

Joint working group "Network funding, sustainability and FP7 Projects":

A manuscript describing the business model of 5 successful networks (case studies) has been drafted. It outlines the needs for funding a successful networks and how resources and time are managed by successful networks. It will be submitted to a scientific journal and then also published on the Enpr-EMA website.

A summary of lessons learnt from the FP7 programme to fund research into off-patent medicines was published by several Enpr-EMA members (Eur J Pediatr 2014; DOI 10.1007/s00431-014-2398-z)

Following up on discussion at the 2014 annual Enpr-EMA workshop, it was agreed to set up 2 new working groups:

- Best Practices to address issues with EU multi-languages of Young Persons Advisory Groups.
- GCP training across multispecialty and countries.

We thank all working group members for their enthusiasm and efforts.

The report on the 1 year deliverables [1] of the Working Groups is published on the Enpr-EMA website.

Additional Enpr-EMA activities throughout 2014 included:

- Enpr-EMA co-chairs attended a meeting, organised by the American Academy of Pediatrics with financial support from the International Federation of Pharmaceutical Manufacturers' Associations (PharMA), with the objective to discuss the development of a global pediatric clinical trial network. The proposal for such a global pediatric clinical trial network is primarily driven by industry difficulties to deliver the paediatric studies requested by FDA and EMA under the respective paediatric jurisdictions. The proposed structure should ensure single entry portal, single contract with all sites, single national ethics committee approval, sustained infrastructure, performance management in place with regular quality checks of all sites involved, capacity to provide feasibility estimates within short period of time (10 days), to enrol patients as estimated in a timely manner and to conduct and complete the clinical studies fulfilling the high standards required for regulatory trials. The proposed network should span all sub-specialties; all age subsets, all clinical study types (interventional and observational) and trial phases, including PK-PD, phase IV and registries. It should be an independent entity with public-private funding provided by various sources: industry, government, academia and foundations, to provide the sustainable global infrastructure needed to plan, start up and conduct and close out of studies compliant with the necessary regulatory standards. It is planned to start initially in the US and UK and Canada and later to phase out to other European large centres. Enpr-EMA's main role at this stage is to represent European sites and networks to the organisational and operating group to ensure that existing Enpr-EMA national/specialty networks will be considered. We will keep all Enpr-EMA members informed.
- The current activities and status of Enpr-EMA were advertised at the annual conference of the European Academy of Paediatrics (EAPS 2014), where a poster on "*Enpr-EMA: a platform for disseminating good practices about paediatric medicines research across Europe and with international partners*" was displayed during the EAPS poster session.
- An abstract on the results of a 2012 survey on the involvement of young people and families in the Enpr-EMA networks was presented to clinicians who attended the EAPS 2014 in Barcelona [2]

- A plenary discussion, on future approaches to funding of paediatric clinical trials, with representatives from the EC and EFPIA/IMI representatives as well as the Enpr-EMA chair/co-chair and representatives from Enpr-EMA networks was held at the November PDCO meeting. Future activities are to be planned on this topic in 2015.
- The first 3-year term of the mandate for all members representing the Enpr-EMA Coordinating Group expired in November 2014. All members, except two, have been renewed for a second 3-year term. The members who have withdrawn from the Coordinating Group will be replaced in early 2015.
- A framework has been established to allow a representative of EFPIA as well as of EUCOPE (to represent SMEs) to become an observer in the Enpr-EMA coordinating group for selected ad-hoc topics to improve communication and collaboration with industry, which is a main stakeholder of Enpr-EMA but for several reasons has used the resources of Enpr-EMA members only to rather limited extent.
- Encouragement of industry to involve paediatric networks when preparing a PIP has been included into the Q&A section of EMA PIP guidance section of the EMA webpage. (Q31)
- Publication of all meeting minutes of the coordinating group and Enpr-EMA working group meetings on the Enpr-EMA webpage was initiated in 2014 to increase transparency.
- During the Enpr-EMA June 2014 workshop, ICAN Research (International Children's Advisory Network) was created linking up existing European Young Person Advisory Groups with established North American ones into a Communicating International Network for worldwide involvement of young people in research [3].
- Global Alliance for Pediatric Therapeutics: Assent in paediatric patients for participation in clinical trials. Several Enpr-EMA members participated in the survey. The analysis of the survey responses will be published.

We thank you all for your support and activities towards Enpr-EMA.

Mark Turner
Chair

Irmgard Eichler
Co-chair

[1] Minutes of Enpr-EMA Coordinating Group Minutes, 24/10/2014
http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/document_listing/document_listing_000346.jsp&mid=WC0b01ac058050027a#section4

[2] *B.Pelle, P. Helms, J.Drabdwell, J.Preston, M.Turner and I.Eichler. Young people and family involvement in paediatric research networks: outcomes of a survey among Enpr-EMA networks. 5th congress of the European Academy of Paediatric Societies. Primary and General Paediatrics. Child Protection. 19 October 2014.*

[3] ICAN Research: http://www.icanresearch.org/newjersey/