WORKING GROUP 3&5:

PUBLIC-PRIVATE PARTNERSHIP

Susan Tansey / Pirkko Lepola
Chair / Co-Chair WG 3&5

Enpr-EMA WG 3&5

Role:

- Facilitate communication between industry and networks
- Gather examples of good practice from Networks and industry working with Enpr-EMA networks
- Develop proposals to disseminate examples of good practice to Enpr-EMA networks and industry

Enpr-EMA WG 3&5

Current WG 3&5 members:

Martine Dehlinger-Kremer
Stefanie Breitenstein
Susan Tansey (Acting Chair)
Pirkko Lepola (Co-Chair)
Jenny Preston
Pamela Dicks

New industry collaborators for WG 3&5:
Dr. Colin Hayward, Chief Medical Officer at Premier Research
Dr. Enrico Bosone - Director Patient Access Policy, EMEA Celgene
Dr. Chris Walker - Regulatory Affairs Executive Director Amgen

Enpr-EMA WG 3&5

Completed task 1.;
Network & Industry survey; 12/2013 - 02/2014

Deliverable 1: Publication: ***'Pharmaceutical Industry and Pediatric Clinical Trials networks in Europe- how do they communicate?' ,*** *Applied Clinical Trials, Jan 08 2016.*

Pirkko Lepola, Susan Tansey, Pamela Dicks, Jennifer Preston, Martine Dehlinger-Kremer

Acknowledgements to Enpr-EMA original WG for help & review:
Dr. Christina Peters (EBMT, Austria), Dr. Richard Trompeter (GOSH, IPTA, UK), Mrs Lynda Wight (TOPRA, UK), Dr. Klaus Hartman (Biomedpark, Germany), Dr. Stefanie Breitenstein (Bayer Pharma, Germany).

Enpr-EMA WG 3&5

Publication: ***'Pharmaceutical Industry and Pediatric Clinical Trials networks in Europe- how do they communicate?' ,*** *Applied Clinical Trials, Jan 08 2016.*

Methods & results:

Industry Survey: A web survey of 13 questions was sent via e-mail to over 600 industry contacts between 13Dec2013 and 14Feb2014.

Network survey: A web survey of 11 questions, was sent via e-mail to all Enpr-EMA Category 1-2 member networks (N=20) between the 13Dec2013 and 14Feb2014.

- **70 industry responses**; including large Pharmaceutical companies, Biotech companies, Consulting companies and Contract Research Organizations. 71% were from a company with a pediatric council. The majority (66%) were already aware of Enpr-EMA, although only 46% had worked (either personally or their company) with Enpr-EMA or another CT network. The majority of the respondents (90%) said they had experience of phase I-IV CTs, while 23% and 34% had experience in epidemiological- and registry based studies, respectively.

- **16 CT network responses**; Of all networks, the distribution was between disease specific (44%), age specific (25%) and national (31%) networks. The majority (63%) had less than 0.5 Full Time Equivalent (FTE) working with a role as industry liaison. The most common services provided by the networks, were site identification (80%), protocol development (74%) and feasibility assessments (67%). Some networks also provided support for the design of research protocols, and reviewing the content of patient information (53%), and some were able to support trial set up (40%).

- **RESULT:** Suggestions for better network visibility and minimum set of capabilities required for CT networks to provide better services for the pharma industry.

Enpr-EMA WG 3&5

Publication: 'Pharmaceutical Industry and Pediatric Clinical Trials networks in Europe- how do they communicate?' , Applied Clinical Trials, Jan 08 2016.

Task 1; proposals for good practice (1) :

- List of ideal network capabilities and services (identified benefits by the companies)

List of ideal network capabilities and services (identified benefits by the companies)
Scientific Expertise
• Review of PK/PD study protocol • Provide guidance and consultation on study protocol design / scientific advice; develop and implement a complex protocol • Identification of KOLs, Pediatric MD consultants for help with planning and design of studies • Provide Expert opinion / comment on ethical consideration • Have readiness to propose new sub-studies or supplement trials • Have large pool of experts who have experience of pediatric clinical trials / protocol development • Provide good quality data
Feasibility Assessment (local / national)
• Provide a rapid opinion about the suitability of the study in "the Country" sites • Provide a rapid feasibility of the procedures / study protocol / conduction the study • Identify a potential target number of eligible patients / Access to patients • Identify of suitable research sites / access to clinical sites and assessing those sites • Obtain clinician/researcher perspectives: benefit/risk balance of treatments, study procedures, trial methodology
Patient Involvement / Investigators Involvement
• Identify of potential issues e.g.; difficulties in the recruitment • Help to develop recruitment materials • Engage input from families and children o Review and give feedback of patient information sheets and other applicable study documents o Provide guidance on preparing the best information for children o Obtain patient perspectives: needs /concerns, acceptable benefit/risk balance of treatments, study procedures, etc. • Welcome and support researchers looking to set up pediatric studies
Legal advice / Administration & Management
• Review of set up contracts and costs • Review of set up cost sharing of investigator fees and other (e.g. laboratory) costs • Provide centralized administrative process and communications; negotiations, contracts, Ethics Committee contact, site management • Manage the study by the network • Give assistance with Regulatory Authorities interactions • Linking EU and US activities • Provide single CDA, single trial agreement, single Informed Consent for Ethics Committees
Training
• Assist with R&D (methodology, pharmacology, medical care standards, ethics etc.) and training
Communication
• Share information between the clinics and sponsor • Provide better and more uniform communication with participating sites • Provide network comments and feedback for the protocols
Resources
• Enough supporting staff (including a designated industry person) and compensation possibilities • Enough funding to support services
Timing
• Provide services in good time; contract negotiations, protocol development, information about the process, feasibility assessment

Enpr-EMA WG 3&5

Publication: 'Pharmaceutical Industry and Pediatric Clinical Trials networks in Europe- how do they communicate?' , Applied Clinical Trials, Jan 08 2016.

Task 1; proposals for good practice (2) : Recommendations for all parties involved in the survey (networks, industry and Enpr-EMA)

Recommendations to industry
• Have a named person or committee within your organization that has a responsibility for pediatrics to facilitate direct lines of communication between Pharma, CRO's and networks
• Consult with experts and develop own pediatric expertise to ensure high quality and feasible protocols
• Consult with relevant networks as early in the protocol development process as possible
o Including consulting with patient groups or patient organisations to review the impact of a protocol on children and families in order to reduce the burden on participants wherever possible and result in a protocol that is more acceptable and feasible.
o Set realistic timeframes for feasibilities to ensure quality responses
• Provide feedback to PIs who have not been successful in site selection.
Recommendations to networks
• Have clear guidance on network websites on the services provided, in which specialties and age groups
• Have a dedicated industry contact if possible
• Promote the involvement of patients and families as early in the protocol development process as possible
• Set realistic timescales regarding time to set up and initiate trials
• Set realistic targets regarding recruitment
• Develop the network towards an "ideal network" i.e. having the minimal set of capabilities
Recommendations to Enpr-EMA:
• Provide a searchable database that outlines each network, contact details, specialties, age groups etc. (Now been achieved)
• Support networks and industry partners to develop and improve collaboration, e.g. by annual Enpr-EMA workshops
• Support networks and industry partners to involve patients and families as early in the protocol development process as possible. (This could be achieved by setting up an European wide virtual young persons´/family forum, hosted by Enpr-EMA)
• Support to create and gain the "minimal set of capabilities" for the member networks

Enpr-EMA WG 3&5

Task 2; planned activities in 2015 - 2016;

1. Develop a model to put on the EMA website showing how industry can engage with EnprEMA/networks and how this could benefit them.

1. Draft an advertisement that could be used to publicise this model by End March 2016

   - Draft (v. 3.0)
   - Circulate to 1) industry representatives (new collaborators) and 2) for Enpr-EMA CG
   - Feedback
   - Finalization

Enpr-EMA WG 3&5

Draft text:

Enpr-EMA WG 3+ 5: Network consultation process & guideline

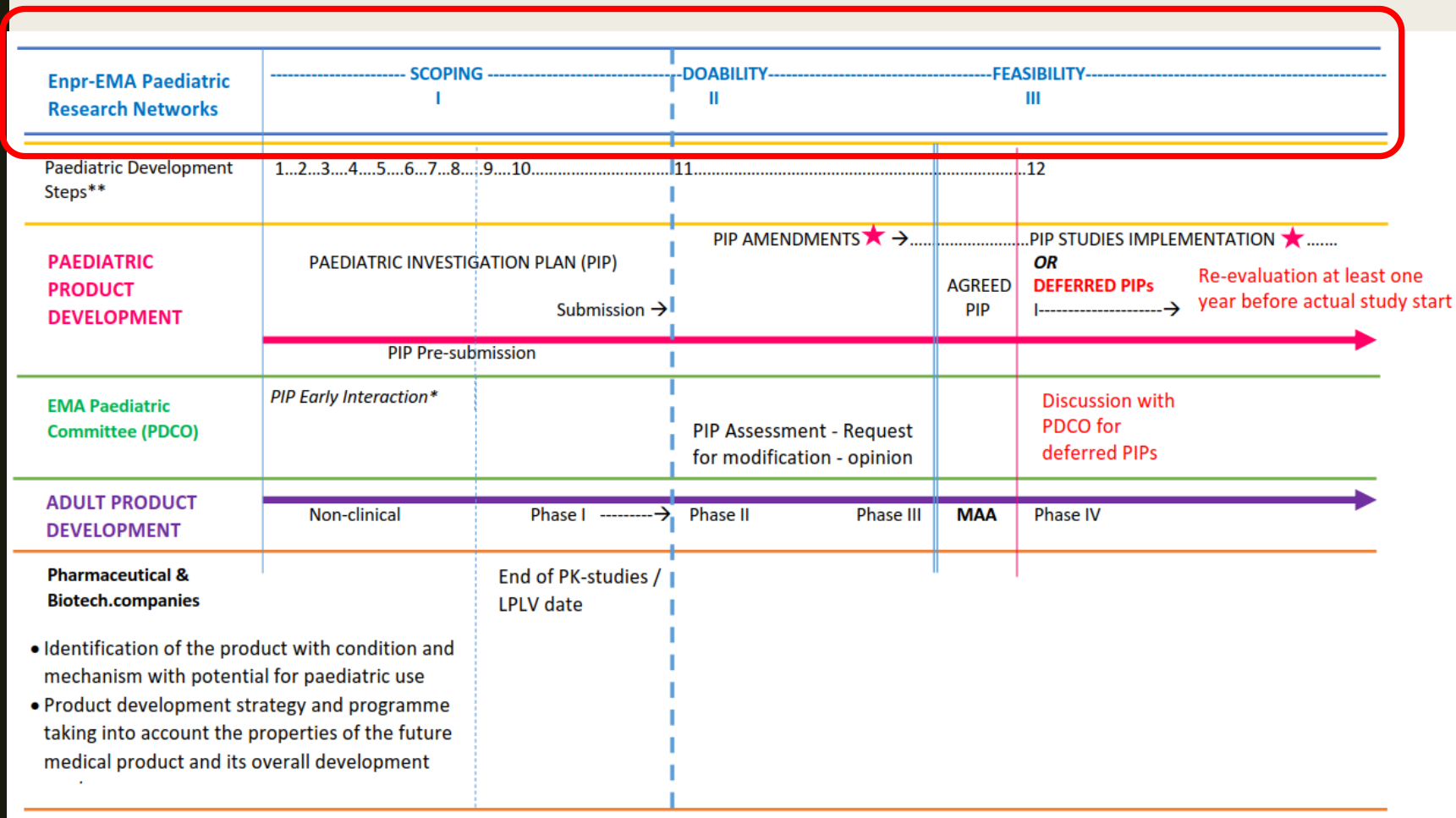
This is a guideline for pharmaceutical or biotechnology companies and contract research organisations who are planning paediatric studies/PIPs in the EU, to help assist them in taking advantage of the scientific and logistical expertise in paediatric clinical research that is available from the paediatric clinical research networks that are linked to EnprEMA. Trials conducted outside EU area are not fully covered by the services of the Enpr-EMA network. Please check the current network availability from the Enpr-EMA database: http://enprema.ema.europa.eu/enprema/ .

Key benefits for companies:

Concept proof for PIP trials, quick site & patient identification, rapid feasibility process

Enpr-EMA WG 3&5

Draft model v. 2.0:



**1. Identification of agent with condition or mechanism with potential for paediatric use, 2. Identification of therapeutic need for the agent
3. Scoping out existing information (pre-clinical, adult, children) of relevance to the therapeutic need, 4. Reviewing the approach to take (extrapolation vs complete programme), 5.Identification of knowledge gaps, 6. Designing plans to fill knowledge gaps, 7. Designing global development plan, 8. Concepts for PIP / PSP and plans for other jurisdictions, 9. Developing PIP / PSP and plans for other jurisdictions, 10. Seeking regulatory advice, 11. PDCO process, 12. Doing the PIP studies (&amendments).

Draft text:

Enpr-EMA WG 3&5

Recommended timing for the network consultations:

1st Consultation: SCOPING – 1st contact before PIP submission

- send email to enprema@ema.europa.eu

Time point: AS EARLY AS POSSIBLE BEFORE PIP PLANS

This is best suitable option especially for rare diseases and conditions. The contact should be made as early as possible before PIP plans to discuss the following information:

- **Review of preliminary PIP plans**
- **Exploratory advice; concept proof – YES / NO- product/trial applicability to paediatric population**
- **Supporting information about the suitable population and availability in Europe by country**
- **Details of specific challenges; in recruiting & set-up times and with targeted patient population**

Will have integrated / linked text under the main titles

Enpr-EMA WG 3&5

Draft text:

2nd Consultation: DOABILITY - 2nd contact before PIP submission

- a weblink to Enpr-EMA network database

Time point: BEFORE VALIDATION / PIP SUBMISSION

This second contact should be made as early as possible before PIP submission to discuss the following information:

- **Adult and paediatric indications**
- **PK & PD modelling**
- **Extrapolation of efficacy from adult population**
- **Target population including age categories**
- **Inclusion criteria relevancy**
- **Formulation and dosing issues**
- **Protocol relevancy to practice**
- **Ethical issues**
- **Practical trial procedures**
- **Preliminary site identification**
- **Patient and family involvement**

Will have integrated / linked text under the main titles

Enpr-EMA WG 3&5

Draft text:

3rd Consultation: FEASIBILITY – 3rd contact after agreed PIP

- a weblink to Enpr-EMA network database

Time point: AFTER AGREED PIP / ADOPTION ON OPINION or REQUESTED MODIFICATION

This contact should be made as soon as possible the agreed PIP by EMA PDCO in order to implement latest changes in the clinical practice and in the occurrence of competing trials and to discuss/confirm the following information:

- **Target population**
- **The protocol relevancy to practice**
- **Compliance with timelines**
- **Trial sites identification**
- **Ethical issues**
- **Practical procedures**
- **Patient and family involvement**

Will have integrated / linked text under the main titles