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

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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

New task 2.;

Network consultation recommendation; 12/2015 ->

Deliverable 2: Consultation recommendation document + diagram to be placed on Enpr-EMA website for the sponsors and CROs;

- Distributed for consultations; 06/2016 – 08/2016
  -> 12 expert opinions; from industry, CRO and academia
- Final draft 01/2017; version 8.
- Diagram needs to be still amended / corrected

Draft text; version 8.:

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Enpr-EMA WG 3+ 5: Network consultation recommendation

This is a recommendation for pharmaceutical or biotechnology companies and contract research organisations, who are planning paediatric studies / Paediatric Investigation Plans (PIP) in the EU. This recommendation is to assist companies in taking advantage of the scientific and logistical expertise in paediatric clinical research, available from the paediatric clinical research networks of the Enpr-EMA (European network of paediatric research at the European Medicines Agency).

To contact these paediatric research networks of the Enpr-EMA, send an e-mail to: enprema@ema.europa.eu . You may check the current paediatric research networks availability, and the services offered by these networks from the Enpr-EMA database: http://enprema.ema.europa.eu/enprema/. This includes updated list of available networks, including information of the capabilities & services provided by each network, and the web-link to the network´s websites for further contacts.

Draft model v. 8.0:

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Recommendation includes four different time points with detailed consultation issues at each point.

These right time consultations can offer key benefits for companies by:

- meeting the patients' needs, with a targeted and evidence-based feasibility
- enhancing the product development process by helping to create a relevant drug development plan
- efficient clinical trial conduction
- optimising the PIP development focusing on key studies and developing a long term strategy
- saving time and costs

Draft text:

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1. Consultation: SCOPING / EXPLORING

– 1st contact to selected network

TIME POINT: Very early on drug development process and before preliminary PIPs

2. Consultation: DOABILITY / TARGETING

- 2nd contact to selected network

TIME POINT: Confirmation to scoping - before PIP submission

3. Consultation: FEASIBILITY / IMPLEMENTING

- 3rd contact to selected network

TIME POINT: after agreed PIP / adoption of opinion or requested PIP modification

4. Consultation: REPORTING & SAFETY FOLLOW-UP

– 4th contact after implementation of PIP studies and adult Marketing Authorization

NOTE:
Under each time point, is the explanation of all identified information and related sevices

Will have integrated / linked text under the main titles

Draft text:

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EXAMPLE OF TIME POINT 1. Scoping / Exploring text

This is the recommended initial option especially for rare diseases and conditions. At this point, it is possible to discuss with the sponsor the following information:

- Identification of agent with condition or mechanism with potential for paediatric use/confirmation of unmet therapeutic need
- Identification of knowledge gaps & plans to fill those knowledge gaps including pre-clinical studies required
- Designing global clinical development plan
  - Target population and age categories
  - Primary & secondary endpoints / outcome selection
  - Use of modelling & simulation and other tools including PK and PD modelling
- Concepts for PIP / PSP (U.S.) studies and plans for other jurisdictions
- Developing PIP / PSP (U.S.) studies and plans for other jurisdictions
- **Feasibility of studies** The availability and the number of possible trial subjects according to the prevalence and health care status and practices in each country to lead to more realistic recruitment targets and timelines.
- Risk-benefit analysis
- Study design and methodology, as well as relevant ancillary studies
- Natural history of the disease in children/ current standard of care & response to standard of care therapy
- Need for long-term follow-up
- Early information on the similarity of drug disposition (ADME)
- Genetics & pathophysiology & similarity of disease between adults and children

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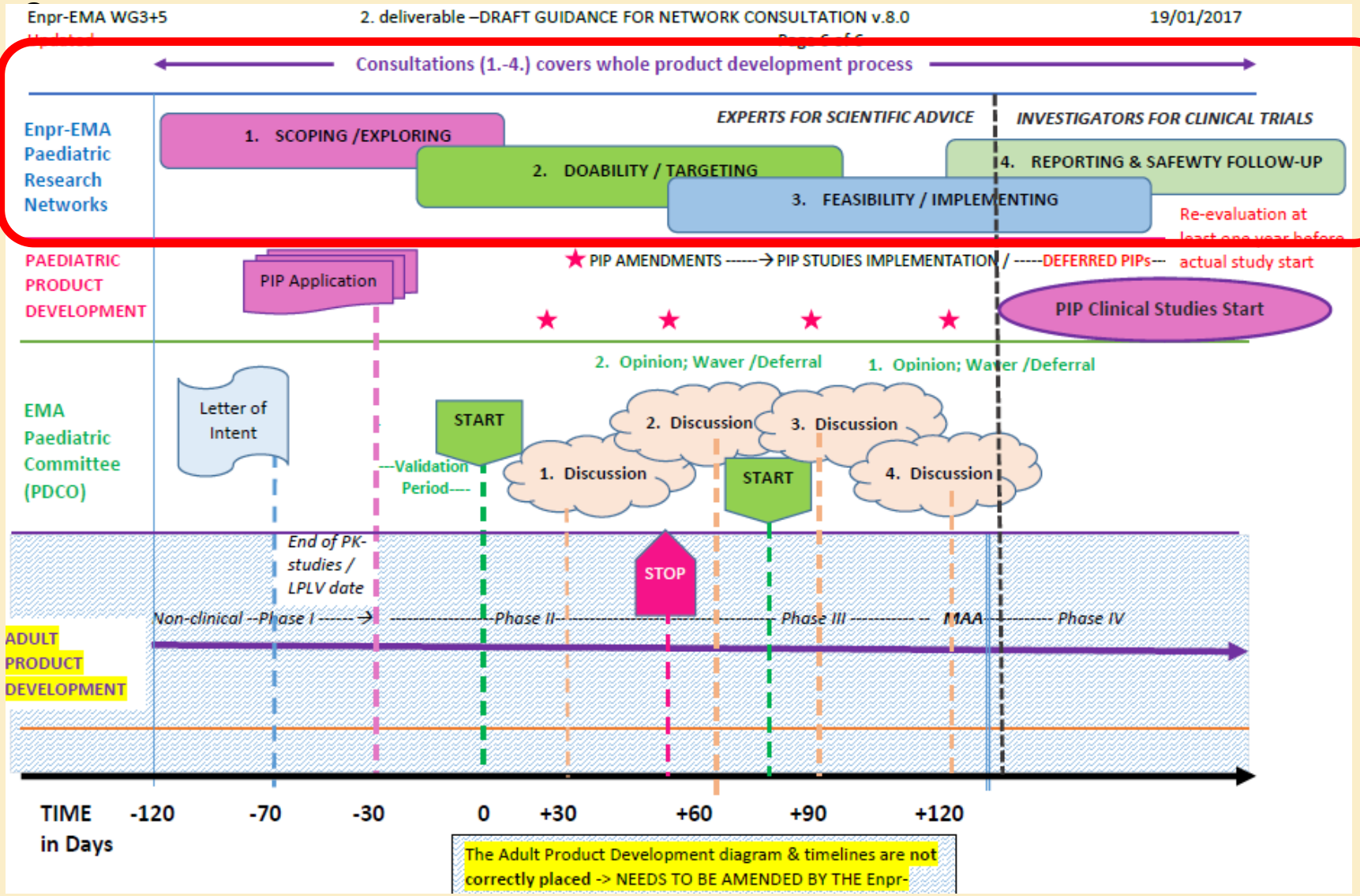
Draft diagram – version



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New task 2.; Network consultation recommendation

Deliverable 2: Consultation recommendation document + diagram

Next steps; proposal for Enpr-EMA CG:

- **Pilot period - test phase - for using these services FOR FREE**
- **Selection of interested companies for pilot phase (e.g. 5-6)**
- **Selection of interested networks (e.g. 5-6)**
- **Max. 1-2 cases / company to be served**
- **After pilot phase; survey to these companies; evaluation and analysis of these services – did they bring any value?-> PoC**
- **Publication of the survey results; collected experience**
- **Decision of the continuation; with OR without the fees**

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- **After results & decisions; design of the following;**
  - *Standardized fees for all networks*
  - *Reasonable costs – feasible for the industry*

- **Strongly related to 2 developments**
  - *Fees for services (see diagram* page 12)*
    - Discussed with the PDCO 8Nov2016
  - *IMI2 Call 10 project*
    - WP1-4 – Governance, Innovation, Business Case

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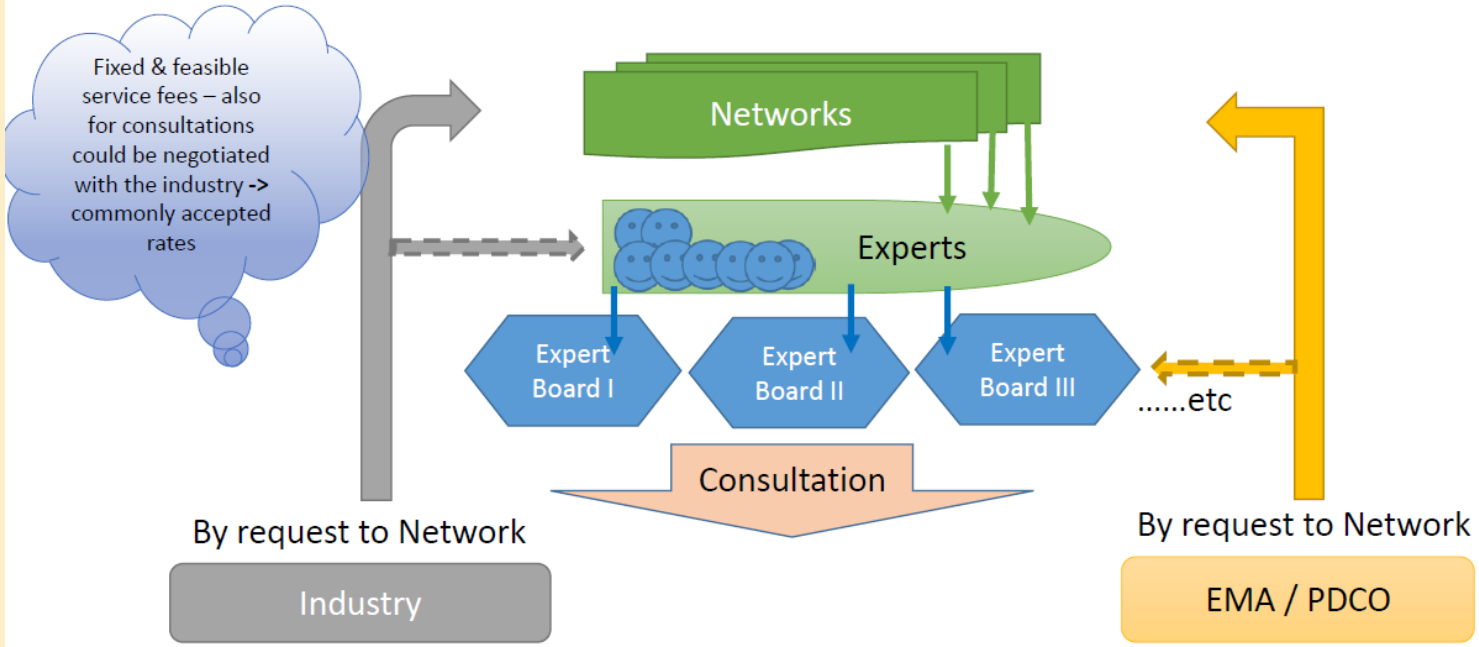
Fees for services *

draft; P.L.08/2016



- With definition of the Expert Board needed (e.g. by subspecialty)
- -> 1-2 individual Experts & opinion
- By Service Fees and Consultation Agreements with individual Experts

Conflict of interest may occur => same experts in both

- With the definition of the Expert Board needed (e.g. by subspecialty)
- -> Consensus decision / opinion of the Expert Board (e.g. Rheumatology Group)
- No Fees -> Scientific Advice
- DoI based