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

WG 3&5 Steering Committtee members:

Martine Dehlinger-Kremer
Susan Tansey (Acting Chair)
Pirkko Lepola (Co-Chair)
Jenny Preston
Colin Hayward
Mark Sorrentino
Heidrun Hildebrand
Andrea Wassmuth
(Mark Turner)

Other members WG 3&5:

Pamela Dicks
Stefanie Breitenstein
Cristina Serén Trasorras

Enpr-EMA WG 3&5

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

Task 2.;

Network consultation recommendation; 12/2015 -> 04/2017

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
- Final draft 04/2017; version 10 -> FINAL AT THIS POINT

Background for Task 2

- Knowledge from the Task 1 – the Industry perspective & expectations to the networks (more detailed services)
- Need to have more detailed search engine for Enpr-EMA – to add more detailed network service information in addition to the existing membership criteria in the Enpr-EMA database of member networks
- Currently, the list of services (Network Consultation Recommendation, v. 10.0) and the Enpr-EMA Database of member networks are not connected
- The list of services (Network Consultation Recommendation, v. 10.0) has not yet been published on Enpr-EMA website

Version 10.0

NOT YET PUBLISHED ON Enpr-EMA WEBSITE

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 by visiting 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.

This recommendation is suitable to all organizations and companies including those operating outside Europe if applicable according to local regulations and company rules.

Paediatric research network expertise is available for the entire drug development cycle, therefore this recommendation covers the whole drug development process – from scientific idea to clinical studies of the PIPs and safety follow-up after Marketing Authorization.

Version 10.0

The 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**
- **optimising the PIP development focusing on key studies and developing a long term strategy**
- **making an efficient clinical trial conduction**
- **saving time and costs**

Enpr-EMA WG 3&5

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

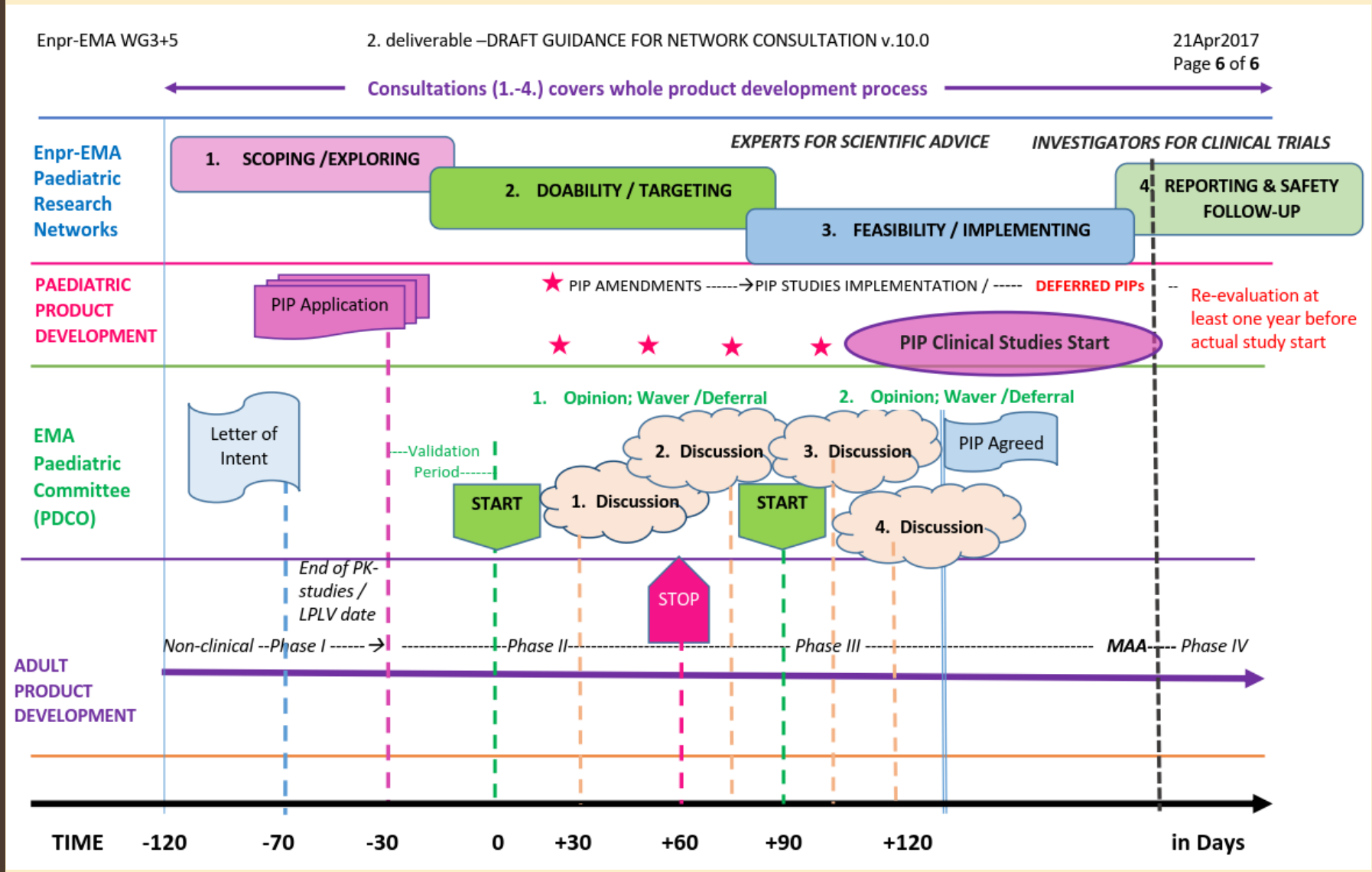
- 3rd contact to selected network

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

Version 10.0 – Consultation diagram



Enpr-EMA WG 3&5

Task 3.; *– original plan (2017)*:

- Pilot period - test phase - for using these Enpr-EMA consultation services FOR FREE
- ➢ Selection of interested networks (e.g. 5-6) -> *SURVEY*
- ➢ *Selection of interested companies for pilot phase (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

Enpr-EMA WG 3&5

- Selection of interested networks (e.g. 5-6) ?
- Questionnaire prepared and sent out to Enpr-EMA Category 1 (N=21) networks 3rd April 2018
- Responses requested by 4th May 2018
- Extended deadline to 14th May 2018
- ➢ 7 responses (N=8–1 excluded) received and could be analysed

similar activities was known to be planned by C4C EU-project – details were not known by the beginning of the year 2018!

- *Resulted –––> very low number of responses*
- Invitation letter to industry prepared *(ready to use – if it will be used…)*

Enpr-EMA WP3+5 DRAFT Pilot Phase Service Request model for selected research networks

Description of the survey answerer	
Name of the Enpr-EMA member network	
Country	
Contact person for enquiries / service requests	

Description of the network	
Type of your network	☐ network of sites ☐ network of sites and research infrastructure ☐ national network of researchers ☐ international network of researchers ☐ national disease specific network ☐ international disease specific network
Population covered	☐ preter m newborn ☐ term newborn ☐ infants (1 month to < 24 months) ☐ children (2y to < 12y) ☐ adolescents (12y to < 18y)
Specialities / conditions covered	☐ all ☐ multispecialty ☐ single specialty if multispecialty or single specialty, select all the specialties / conditions that apply / are covered by the network:

3 Questions

PIP / product specific expert advice	*Tick off (X) for service option and for fees*		
1. Consultation: SCOPING / EXPLORING - 1st contact to selected network **TIME POINT: Very early on drug development process and before preliminary PIPs**	**Service available for this expert advice by the Network / Network Experts**	**Service available without service fees (for free)**	**Service available with service fees or under Consultation Agreement**
1. Identification of condition or mechanism of action with potential for paediatric use/confirmation of unmet therapeutic need	☑	☐	☑
2. Identification of knowledge gaps & plans to fill those knowledge gaps including pre-clinical studies required	☑	☐	☑
3. Designing global clinical development plan a) Target population and age categories b) Primary & secondary endpoints / outcome selection c) Use of modelling & simulation and other tools including PK and PD modelling	☑	☐	☑
4. Concepts for PIP / PSP (U.S.) studies and plans for other jurisdictions	☑	☐	☑
5. Feasibility of studies The availability and the number of possible trial subjects according to the prevalence and health care status and practices (including off-label use) in each country to lead to more realistic recruitment targets and timelines. Also important to take into account the number of other products in development for the same condition.	☑	☐	☑

All Survey Categories were derived from the following existing data elements:

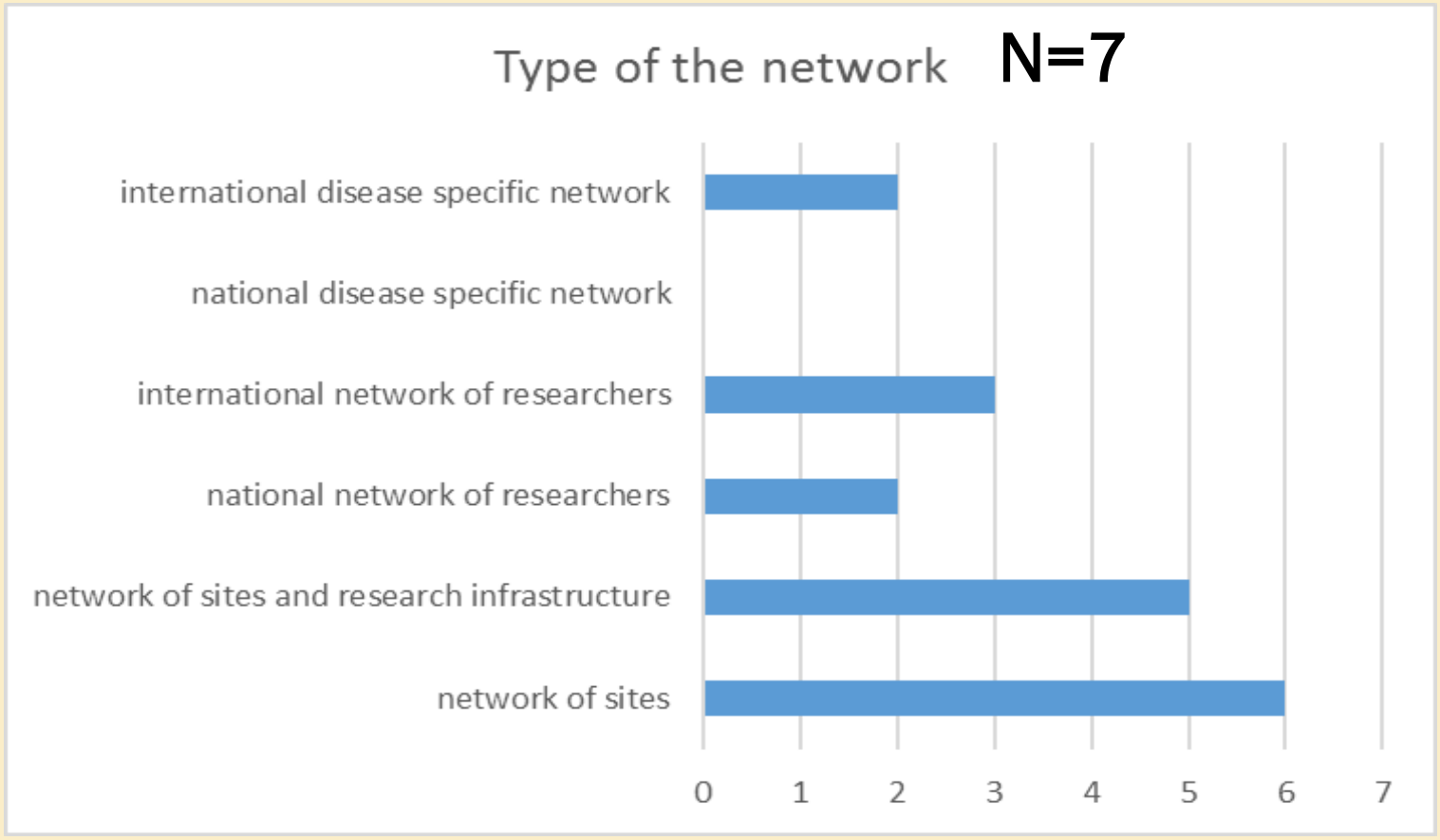
a) Network consultation recommendation (2015-2017) – *Task 2.*
b) Expertise required by the Industry; Publication (2013-2014) – *Task 1.*

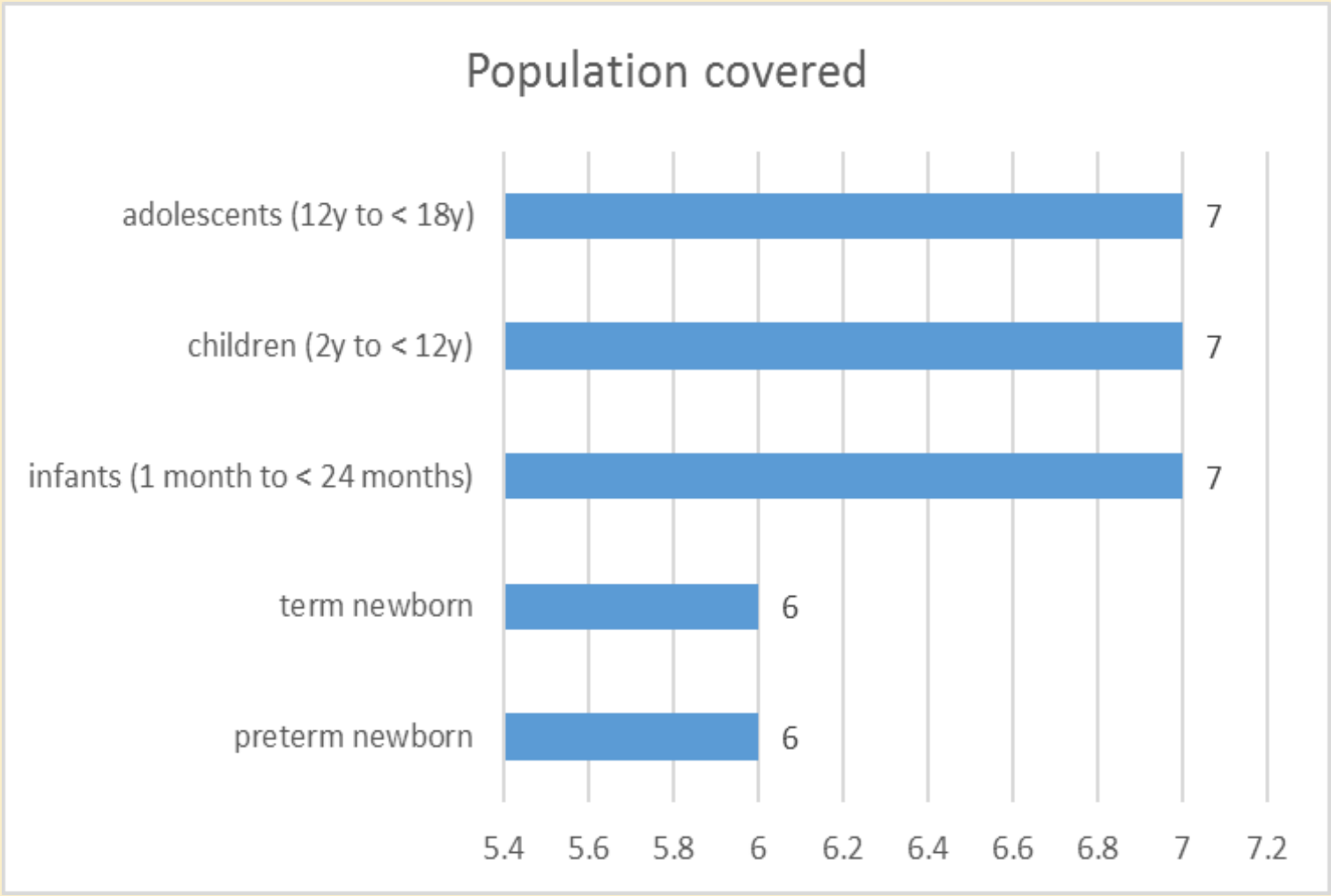
a) 4 categories:
1. Scoping – 22 elements
2. Doability – 9 elements
3. Feasibility – 13 elements
4. Reporting – 3 elements

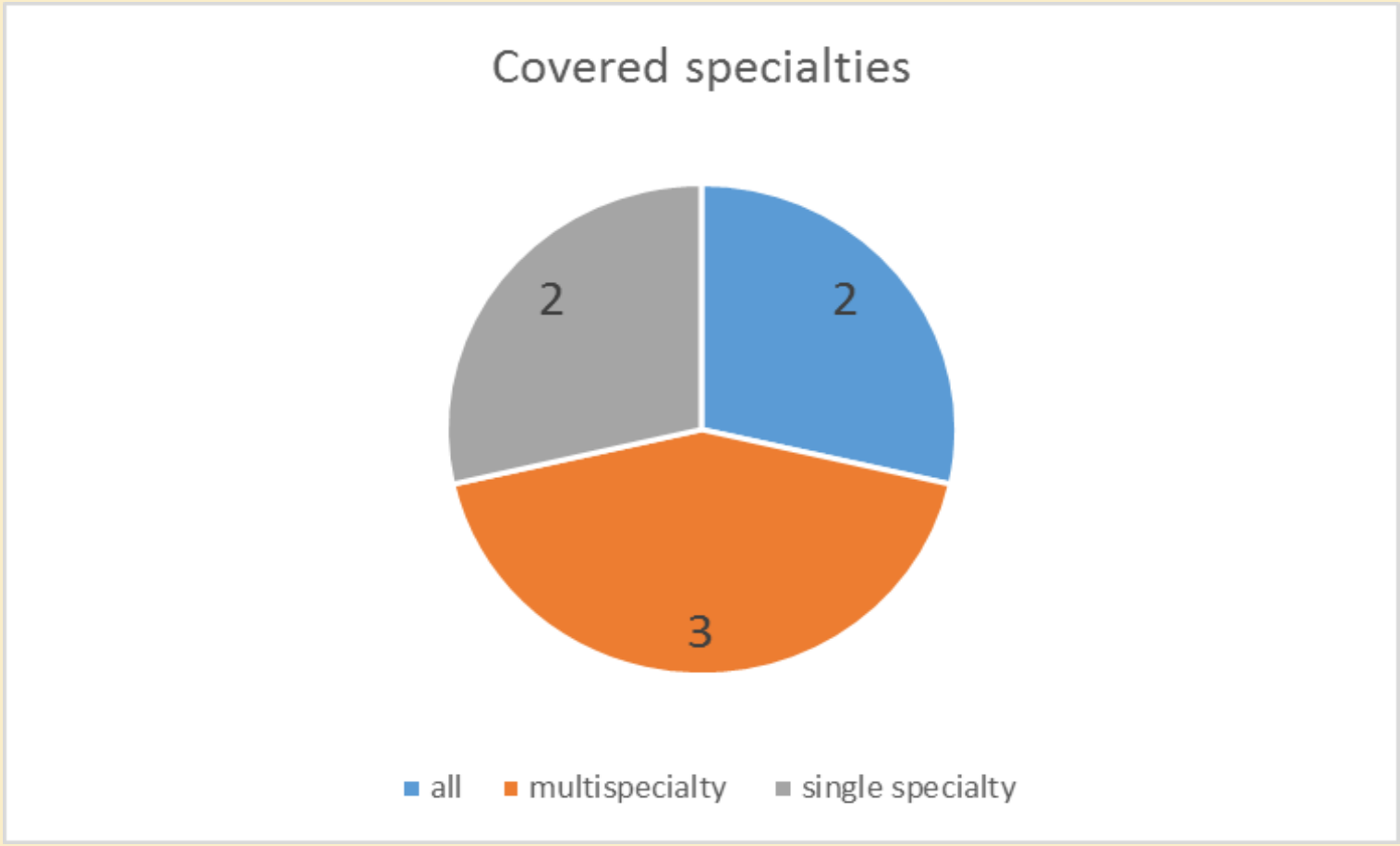
b) 4 categories:
1. Scientific Advice, Communication, Training and Recourses,
2. Legal / Administrative Advice
3. Feasibility Assessment
4. Patient Involvement;

- Total - 29 elements

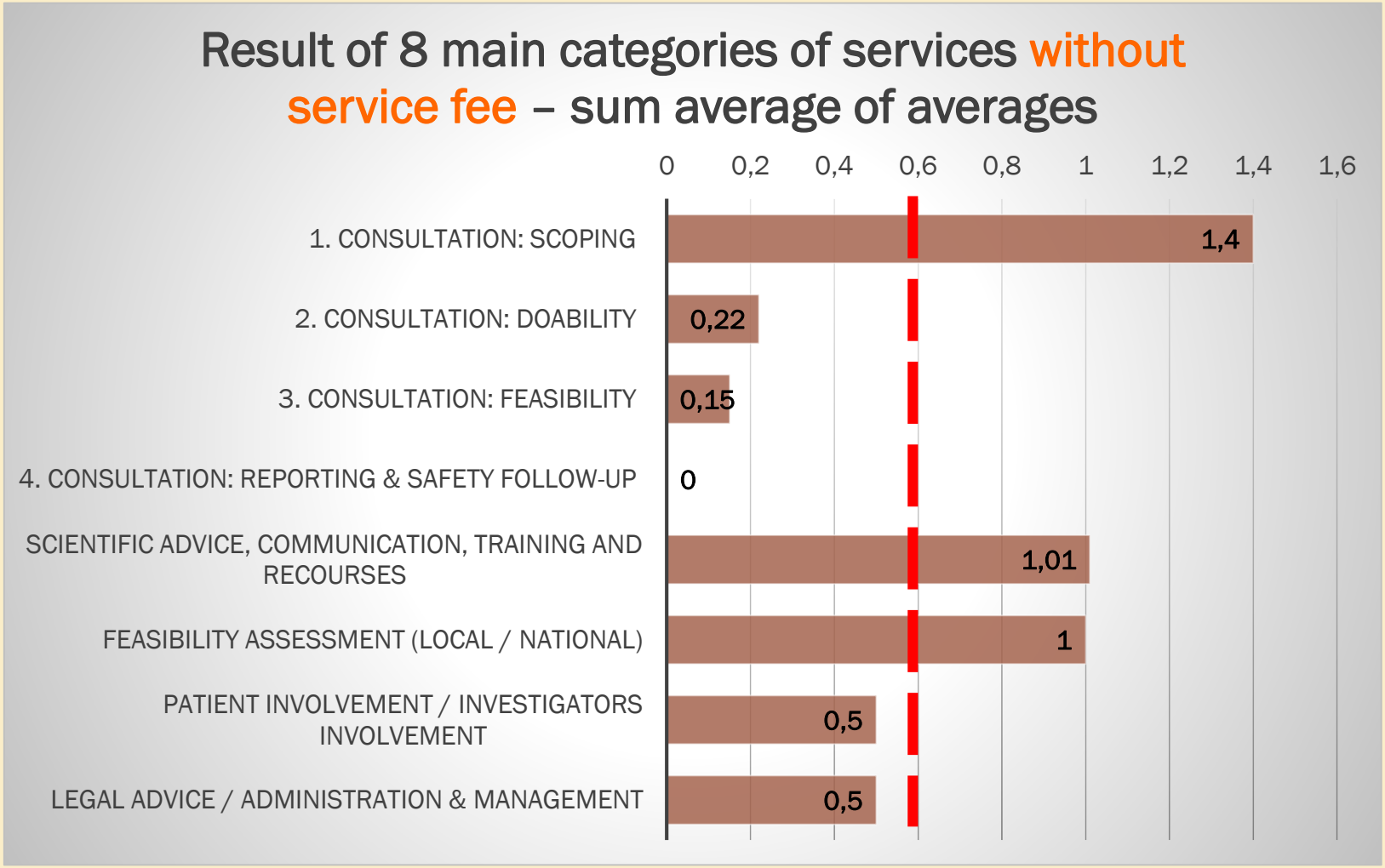
Analysis & Results of the network Questionnaire responses


Type of the network
N=7
international disease specific network
national disease specific network
international network of researchers
national network of researchers
network of sites and research infrastructure
network of sites
0
1
2
3
4
5
6
7


Population covered
adolescents (12y to < 18y)
children (2y to < 12y)
infants (1 month to < 24 months)
term newborn
preterm newborn
7
7
7
6
6
5.4
5.6
5.8
6
6.2
6.4
6.6
6.8
7
7.2


Covered specialties
2
2
3
all
multispecialty
single specialty

Results - Conclusion



Continuation ?

- Result does not encourage to organize pilot period –test phase for networks for free (too few networks operate for free)
- Use of the data (low reliability) need to be dicussed with all stakeholders – usable?
- Use of the consultation recommendation? – not yet connected to Enpr-EMA networks – needs to be discussed
- Further actions under decision of Enpr-EMA