

The accountable number of authorised medicines Consultation in relation to the Paediatric Report

Ref. PCPM/16 – Paediatric Report

1. PART I - GENERAL INFORMATION ABOUT RESPONDENTS

Your name or name of the organisation/company: Dr. med. Dirk Mentzer

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- A citizen

If you are a business, please indicate the size of your business

- Self-employed
- Micro-enterprise (under 10 employees)
- Small enterprise (under 50 employees)
- Medium-sized enterprise (under 250 employees)
- Large company (250 employees or more)

Please indicate the level at which your organisation is active:

- Local
- National
- Across several countries
- EU
- Global

2. PART II – CONSULTATION ITEMS

(You may choose not to reply to every consultation items)

2.1. More medicines for children

Consultation item No 1: Do you agree that specific legislation supporting the development of paediatric medicines is necessary to guarantee evidence-based paediatric medicines?

Yes, the legislation is supporting the development of paediatric medicines and needed to overcome market forces hindering the development

2.2. Mirroring paediatric needs

Consultation item No 2: Do you have any comments on the above? To what extent and in which therapeutic areas has the Regulation contributed to the availability of important new treatment options?

The research and development of medicinal products is mainly driven by aim to address disease affecting large populations. For these disease the Paediatric regulation has fulfilled it objective to secure the paediatric needs in the respective medicinal product development, but the paediatric specific disease are still neglected.

2.3. Availability of paediatric medicines in the EU

Consultation item No 3: In your experience, has the number of new paediatric medicines available in Member States substantially increased? Have existing treatments been replaced by new licensed treatments?

No Comments

2.4. Reasonable costs

Consultation item No 4: Do you have any comments on the costs for pharmaceutical companies to comply with an agreed paediatric investigation plan?

No Comments

2.5. Functioning reward system

Consultation item No 5: Do you agree that the reward system generally functions well and that early, strategic planning will usually ensure that a company receives a reward?

No Comments.

2.6. The orphan reward

Consultation item No 6: How do you judge the importance of the orphan reward compared to the SPC reward?

No Comments

2.7. Improved implementation

Consultation item No 7: Do you agree that the Regulation's implementation has improved over time and that some early problems have been solved?

No Comments

2.8. Waivers and the ‘mechanism of action’ principle

Consultation item No 8: Do you have any comments on the above? Can you quantify and qualify missed opportunities in specific therapeutic areas in the last ten years?

No Comments

2.9. Deferrals

Consultation item No 9: Do you agree with the above assessment of deferrals?

The deferrals implemented to avoid marketing authorisation of adult indications in case paediatric medicinal product development is expected to delay initial marketing authorisation application. As reflected consultation item 1 and 2 the main driver for medicinal product development is still based on the objective to cover the medical need related to adult disease. Hence, the ground to grant a deferral as described in Art. 20 of the regulation applies and is therefore granted by the PDCO in the vast majority of PIP applications. However, accepting that the medical need to treat adults has priorities over the medical need to treat children will in future not significantly improve the timely authorisation of medicinal products for adults. In this respect the EC should review the Art 20 (2) and propose respective grounds to grant a deferral. This may be feasible by adding ground and scenarios for granting deferrals into the current EC guideline (*Guideline on the format and content of applications for agreement or modification of a paediatric investigation plan...*)

2.10. Voluntary paediatric investigation plans

Consultation item No 10: Do you have any comments on the above?

No comments.

2.11. Biosimilars

Consultation item No 11: Do you have any comments on the above?

No comments.

2.12. PUMA — Paediatric-use marketing authorisation

Consultation item No 12: Do you share the view that the PUMA concept is a disappointment? What is the advantage of maintaining it? Could the development of off-patent medicines for paediatric use be further stimulated?

From the point of view of the paediatrician the PUMA concept is a disappointment, as still a respective number of pharmaceutical companies have no marketing authorisation, which are used in daily praxis.

2.13. Scientifically valid and ethically sound — Clinical trials with children

Consultation item No 13: Do you have any comments on developments in clinical trials with children following the adoption of the Regulation and in view of the above discussion?

No comments.

2.14. The question of financial sustainability

Consultation item No 14: Do you have any views on the above and the fact that the paediatric investigation plan process is currently exempt from the fee system?

It is appreciated, that waving the fees for any regulatory assessment related to a paediatric medicinal product development is a strong incentive to the applicants. However, without knowing the financial impact on the public health costs a definite comment is not possible.

2.15. Positive impact on paediatric research in Europe

Consultation item No 15: How do you judge the effects of the Paediatric Regulation on paediatric research?

The awareness to develop a systematic infrastructure to support high quality paediatric research is increasing. The most recent IMI (*Innovative Medicines Initiative*) is supporting a project to build a EU wide paediatric research network to facilitate paediatric research and feasibility of clinical trials in the paediatric population.

2.16. “Mirror, mirror on the wall” - Emerging trends and the future of paediatric medicines

Consultation item No 16: Are there any emerging trends that may have an impact on the development of paediatric medicines and the relevance of the Paediatric Regulation?

No comments.

2.17. Other issues to be considered

Consultation item No 17: Overall, does the Regulation’s implementation reflect your initial understanding/expectations of this piece of legislation? If not, please explain. Are there any other issues to be considered?

No comments.