

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: _____ Gilead Sciences International Ltd _____

Transparency Register ID number (for organisations): _____

Country: _____ United Kingdom _____

E-mail address: _____ IntlRARegIntel@gilead.com _____

Received contributions may be published on the Commission's website, with the identity of the contributor. Please state your preference:

- My contribution may be published under the name indicated; I declare that none of it is subject to copyright restrictions that prevent publication
- My contribution may be published but should be kept anonymous; I declare that none of it is subject to copyright restrictions that prevent publication
- I do not agree that my contribution will be published at all

Please indicate whether you are replying as:

- A citizen
- A business
- A non-governmental organisation (NGO)
- An industry association
- A patient group
- A healthcare professional organisation
- Academia or a research or educational institute
- A public authority
- Other (please specify)

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?

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?

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 Have existing treatments been replaced by new licensed treatments?

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?

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?

2.6. The orphan reward

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

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?

The legal procedure framing agreement of the Paediatric Investigation Plan (PIP) is very limiting and forces re-submission of the whole PIP if an agreement with the PDCO cannot be reached at Day 120. A suggestion would be the possibility to allow multiple rounds of Requests for Modifications, similar to Requests for Supplementary Information for variations.

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?

As a general comment, it could be noted that oncology has been highlighted as an area where the 'mechanism of action' principle has been applied to ensure paediatric development occurs. However, it is very difficult to predict the condition PDCO may make a 'recommendation' to develop. The choice of classification system and HLT/PT appears arbitrary. Consequently, it is very difficult for an applicant to plan for or predict a PDCO recommendation without jeopardizing the whole Paediatric Investigation Plan.

2.9. Deferrals

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

There is a general agreement that the deferral system works well and allows a reasonable balance between patient safety, PDCO's desire for expedited paediatric research, and overall drug development timelines.

2.10. Voluntary paediatric investigation plans

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

It could be considered a positive step for the EMA to look to other countries' regulatory systems, e.g. US FDA, to provide a voluntary scheme that enables applicants to gain meaningful rewards such as Rare Pediatric Diseases Priority Review Voucher, which is transferrable.

2.11. Biosimilars

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

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?

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?

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?

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?

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?

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?