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: ____Therakind Ltd_____

Transparency Register ID number (for organisations): _____

Country:___UK_____

E-mail address:___admin@therakind.com_____

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- o My contribution may be published under the name indicated; I declare that none of it is subject to copyright restrictions that prevent publication

Please indicate whether you are replying as:

- o A business

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

- o Small enterprise (under 50 employees)

Please indicate the level at which your organisation is active:

- o EU

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?

We agree that supporting legislation is necessary to support evidence-based paediatric medicines and has led to increased paediatric research and licensed paediatric medicines.
In order for the Paediatric Regulations to become a self-sustaining system, in addition to financial incentives there is a need for additional legislation/action to encourage prescribing of licensed 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?

We agree with the example of the EU legislation on rare diseases that effect is partly dependant on factors that cannot be influenced by legislation.

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 Have existing treatments been replaced by new licensed treatments?

In order to ensure new licensed treatment does replace existing unlicensed treatments, there is a need for better reimbursement systems to ensure licensed medicines are used over unlicensed alternatives.
If a new improved formulation for paediatric patients is developed the fact that this may lead to better compliance is not usually recognised in reimbursement.
The current reimbursement values given to reformulated medicines licensed via PIP to PUMA are not significant in some EU countries (even when modest) thereby reducing the incentive to develop off-patent medicines licensed via PIP to PUMA.

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?

Preparation of PIPs can be burdensome and costly, particularly with respect to the level of detail required for the Background Information section. We propose that, where appropriate, a simpler PIP consisting of limited background information should be acceptable.

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?

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?

2.9. Deferrals

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

2.10. Voluntary paediatric investigation plans

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

The commercial reward for developing off-patent medicines to be licensed via PIP to PUMA is currently often not significant enough to stimulate the interest (to carry out paediatric research) that could be justified due to high development costs and subsequent challenges regarding reimbursement pricing.

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?

The uptake of PUMAs has been low and is disappointing. Possibly the current reimbursement values given to reformulated medicines licensed via PIP to PUMA are not significant in some EU countries (even when modest) thereby reducing the incentive to develop off-patent medicines licensed via PIP to PUMA. Clearly this is outside the legislation but is a factor that could be addressed at a national level.

The failure of previous year's off-patent medicines projects should not be preventing a new round of funding from the Commission. Development of off-patent medicines for paediatric use can be further stimulated by a more efficient delivery of funding from the Commission - we would suggest: more direct funding to SMEs, with reduced regulations on consortium partners and sub-contractors – as this would enable consortiums to develop off patent medicines for paediatric use more efficiently. PUMAs can be successful but there is a need for funding in this area.

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?
Non-reimbursement of national experts could lead to inappropriate 'experts' reviewing the PIPs. This would create a situation where inappropriate 'experts' could potentially make requests for inappropriate studies.

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 positive result of the legislation is a significant increase in paediatric research and the increased awareness around the need for research in the field of paediatrics.

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?