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: ASSOCIATION LISA FOREVER

Transparency Register ID number (for organisations): **368026425968-07**

Country: FRANCE_____

E-mail address: lisaforeverassociation@yahoo.fr

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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
- ○ A cademia or a research or educational institute
- ○ A public authority

☒ Other (please specify) Association a but non lucratif

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?

Oui nous sommes totalement d'accord pour que le developpement de nouveaux medicaments pour enfants soit soutenus par la legislation afin de simplifier le processus de validation des nouvelles drogues pour les enfants et leur offrir une chance supplémentaire de guerir.

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?

Croire que le cancer de l'enfant et le cancer de l'adulte peuvent etre soignés avec les memes medicaments est une mauvaise analyse. Les cancers chez l'enfant sont spécifiques et les medicaments administrés à l'adulte ne sont pas forcément adaptés pour les enfants.

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 Have existing treatments been replaced by new licensed treatments?

A notre connaissance, specifiquement les médulloblastomes, les traitements n'ont pas forcement evolués. Nous savons que les fonds sont alloués particulièrement au cancer de l'enfant qui n'ont à ce jour aucune chance d'etre soigné comme par exemple le gliome du tronc cerebral infiltrant.

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?

Pas de commentaires

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?

Il est clair que les entreprises ne developpent pas de medicaments pour l'enfant car ceci n'est pas assez rentable d'un point de vue economique. Meme si cela parait abherent, qu'il soit necessaire de recompenser une entreprise pour la motiver à participer à la guérison des enfants. Si cependant, nous pouvons les inciter à developer de nouveaux medicaments, pourquoi pas metre en place une reduction d'impots ou de leurs charges par le gouvernement.

2.6. The orphan reward

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

Pas de commentaires

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?

Pas de commentaires

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?

Pas de commentaires

2.9. Deferrals

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

Pas de commentaires

2.10. Voluntary paediatric investigation plans

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

Il est regrettable que la vie de nos enfants dependent de la rentabilité économique des entreprises pharmaceutiques

2.11. Biosimilars

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

Pas de commentaires

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?

Pas de commentaires

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?

Pas de commentaires

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?

Il est un peu premature de savoir si la reglementation pédiatrique a déjà un effet sur les traitements pour les enfants.

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?

Il est important de former également les fonctions support (medecin de ville, education nationale etc) afin que le diagnostic ou la prise en charge soient completes.