Public Consultation on European Commission General Report on Experience Acquired as a Result of the Application of the Paediatric Regulation (Article 50(2) of Regulation (EC) No 1901/2006) 'Experience Acquired' and 'Lessons Learnt'

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Comments from Therakind Limited (a SME)

Date: 27 November 2012

1. A CHANGE OF CULTURE: NOWADAYS PAEDIATRIC DEVELOPMENT IS AN INTEGRAL PART OF PRODUCT DEVELOPMENT

Consultation item No 1: Do you agree that the Paediatric Regulation has paved the way for paediatric development, making it an integral part of the overall product development of medicines in the European Union?

Comment:
Yes

2. HAS THE REGULATION DELIVERED IN TERMS OF OUTPUT? TOO EARLY TO JUDGE.

Consultation item No 2: Do you agree with the above assessment?

Comment:
Currently it is too early to assess whether this regulation has delivered in terms of a reduction in the off-label use of medicinal products. The off-label use of drugs is still prevalent across the EU.

3. THE PUMA CONCEPT: A DISAPPOINTMENT

Consultation item No 3: Do you share this view? Could you give specific reasons for the disappointing uptake of the PUMA concept? Is it likely that PUMA will become more attractive in the coming years?

Comment:
There are several reasons for the disappointing uptake in the PUMA. Potentially the rewards may not cover the cost and risk of development. Additionally the protection of the actual data has yet to be proven. It is noteworthy that, despite considerable EU funding, not one of the Seventh Framework Programme (FP7) funded projects for development of off-patent paediatric medicinal products have resulted in grant of a PUMA to date. The one PUMA granted thus far was funded privately. Perhaps the change to have a SME as a major project partner in this funding will improve this situation in the future. It may be even more effective if SMEs have to be the main partner in the FP7 project consortium.
One concern with the PUMA process is that the economic benefits have yet to be proven. There appears to be no formal (legal) process by which off-label/unlicensed use becomes redundant when a PUMA is granted. Although this is implied, in reality this is not the case; prescribers still have the choice to prescribe either authorized products off-label or unlicensed products manufactured specifically.

4. WAITING QUEUES? NO EVIDENCE OF DELAYS IN ADULT APPLICATIONS

Consultation item No 4: Do you agree that, generally speaking, the paediatric obligations have no impact on timelines in adult development, as there is no evidence for delays in marketing authorisation applications for reasons of compliance with the paediatric obligation? If you feel that there is an impact, practical examples would be appreciated.

Comment:
No comment

5. MISSING THE POINT? PAEDIATRIC DEVELOPMENT IS DEPENDENT ON ADULT DEVELOPMENT, NOT PAEDIATRIC NEEDS

Consultation item No 5: Do you have any comments on the above?

Comment:
No comment.

6. THE BURDEN/REWARD RATIO – A BALANCED APPROACH?

Consultation item No 6: Do you agree with the above?

Comment:
No comment.

7. ARTICLES 45/46: THE HIDDEN GEM OF THE PAEDIATRIC REGULATION

Consultation item No 7: Do you agree that Articles 45/46 have proved to be an efficient and successful tool for gathering and compiling existing paediatric data and making it available to the competent authorities and subsequently, via databases, to the interested public?

Comment:
It is not clear whether these data could be used to support a PIP for a PUMA if the authorised product Summary of Product Characteristics (SmPC) has not been updated. It is suggested that updating of the product SmPC should be mandatory where sufficient data are available, to avoid off-label and potentially incorrect dosing in children.

8. LOST IN INFORMATION: HEALTHCARE PROFESSIONALS NOT AS RECEPTIVE AS EXPECTED.

Consultation item No 8: Do you agree that healthcare professionals may not always be as receptive to the new scientific information on the use of particular products in children as might be expected? Do you agree that this problem has to be addressed primarily at national level? How could healthcare professionals be more interested and engage in paediatric clinical research?

Comment:
Historically as there were relatively few products with a paediatric license there was little alternative to using an off-label product. We have found that not only are some healthcare professionals not interested in paediatric clinical research they are not even aware that the drugs they are prescribing or dispensing are not licensed for paediatric use nor whether an approved alternative is available. This should definitely be addressed at a national level but also at a local level. Once a drug is licensed for paediatric use regulatory authorities should notify prescribers and the bodies which control the hospital or family doctor formularies that the approved drug should be used rather than an existing unlicensed drug unless clinically justified.

9. CLINICAL TRIALS WITH CHILDREN: NO SPECIFIC PROBLEMS DETECTED

Consultation item No 9: Do you have any comments on development in clinical trials with children following the adoption of the Regulation and in view of the above description?

Comment:
No comment.

10. UNNECESSARY EFFORTS? NON-COMPLETED PAEDIATRIC INVESTIGATION PLANS

Consultation item No 10: Do you have any comments on this point?

Comment:
No comment

11. SOPHISTICATED FRAMEWORK OF EXPERTISE ACHIEVED

Consultation item No 11: Do you agree that the Paediatric Regulation has contributed substantially to the establishment of a comprehensive framework of paediatric expertise in the European Union?

Comment:
We agree that the regulation has generally facilitated this process.

12. ANY OTHER ISSUES?

Consultation item No 12: Overall, does the implementation of the Regulation reflect your initial understanding/expectations of this piece of the legislation? If not, please precise your views. Are there any obvious gaps with an impact on paediatric public health needs?

Comment:
The following gaps have been identified for which options have been suggested:

If products which are discontinued during the adult development phase could be obtained by third parties to assess their suitability for paediatric use, then there could be an increase in paediatric specific products coming through development where the third party progresses the further development.

If clinical trials have been completed in accordance with a PIP and the applicant decides not to submit a marketing authorisation application, or deferred studies are not completed in the appropriate timeframe after a marketing authorisation has been approved, the applicant should be obliged to allow a third party which has declared its intention to continue to develop the medicinal product in question for paediatric use, to use the pharmaceutical, nonclinical and clinical documentation generated or contained in the file of the medicinal product on the basis of Article 10c of Directive 2001/83/EC, as appropriate.