PCPD/12/01 — Public Consultation on paediatric report – Responses from the Austrian Medicines and Medical Devices Agency

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?

Yes, we agree. While some MAHs (mainly from outside the EU) still seem not to be aware of implications of this regulation, for most MAHs the paediatric development has become a part of drug development. Companies often come earlier to the NCA to discuss development plans and also to find regulatory approval of their plans.

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

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

We partly agree. It cannot currently be expected that most products under the obligation to fulfill a PIP are ready for submitting a marketing authorization yet. This is also due to the fact, that the paediatric part of the development is often deferred due to scientific and medical reasons. However, the need to wait for a decade seems too pessimistic. Key performance indicators (e.g. numbers of children included in trials) in addition to MAs should be considered and followed in addition to marketing authorizations. Any improvement of information on use of medicinal products in children will be valid, including information of what NOT to use in children, i.e. also if not resulting in MA improved knowledge could well have been acomplished.

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?

The acceptance of the PUMA concept is indeed rather limited. On the one hand it appears that MAHs have limited personal resources with regard to paediatric development often occupied by the obligatory PIP tasks. This point might improve in the future when MAHs get more experience with PIP procedures. Another point seems to be the legal uncertainty with regard to the concept of market and data exclusivity. While a MAH might formally have exclusivity, this would not in practice prevent physicians from using products with the same active ingredient but lower costs based on the available scientific evidence. Reimbursement rules in several member states even force them to follow this approach for financial reasons.

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.

We agree.

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?

The Paediatric Regulation is not designed to solve all problems with regard to paediatric medicines (especially enforcing development). However, this cannot be interpreted as missing the point, but rather that - especially for old & approved drug indications – the generation of data on use in children cannot be solved if restricted to this regulation. For new drugs and indications on the other hand the regulation has the potential of being a very effective tool in addressing the problem.

6. The burden/reward ratio —A balanced approach?

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

N/A

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?

Yes we agree. However, as MAHs have little interest in updating the SmPC the crucial point is that the implementation of the outcome of Article 45/46 is actively driven by the competent authorities. As it is not compulsory for the MAH to implement the information, this has shown to be rather resource and time consuming for the competent authorities.
On the other hand it will be very important to prioritise the active substances for the future to make the work-sharing procedures not only a very labour-intensive exercise but also a useful tool that makes the most urgently needed information available for the public as soon as possible.

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 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?

We agree partly. While implementation of some of the measures to solve this problem will be dependent on national legislations, also harmonization of approaches on an EU level is deemed needed. This could for example include coordination of such issues via the Enpr-EMA paediatric network at EMA. Hence efforts to solve this problem should be addressed on both national and EU level.

9. Clinical trials with children: no specific problems detected

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

10. Unnecessary efforts? Non-completed paediatric investigation plans

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

We do not agree to this point that in case of discontinued adult development plans an already agreed PIP has to be considered as an unnecessary effort. First, any drug development bears the risk to be discontinued for several possible reasons. In all these cases any prior efforts could be considered "unnecessary", therefore this should not be used as an argument specific against the requirement of early submission of a PIP.
In addition, it should not be disregarded, that information collected could still be of value for other similar development programs. Especially in the still rather new area of paediatric development the exercise of submitting a PIP can very often be considered as an extensive and useful learning exercise to a MAH company that will make subsequent similar exercises more efficient in term of how to design the development appropriately, how to approach the regulators, how to involve investigators and so one.
We have also received feedback from MAHs, that this early involvement of regulators (not a regulatory requirement in development of new products before – with the advantage of being free of charge) was perceived by some as a valuable input on the development plan as a whole as in the end both paediatric and adult development are integral components of the program.

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?

Yes we agree. The Paediatric Regulation raised awareness for the paediatric needs as well as knowledge how to address these scientifically. Undoubtedly the requirement of submitting PIPs has largely improved involvement of experts in this field (on the regulator, industry and investigator side).
With the regard to the role of Enpr-EMA this currently still has to be considered limited. The awareness about this network and how and for what purposes it could be of value seems limited in the community (also compare comment to item 8).

12. ANY OTHER ISSUE?

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

None