Consultation response

'EXPERIENCE ACQUIRED' AND 'LESSONS LEARNT' SUBMITTED FOR PUBLIC CONSULTATION

Deadline for Public Consultation: 28 November 2012

This is the response from the Neonatal and Paediatric Pharmacists Group (NPPG)

NPPG is a stakeholder organisation

NPPG was formed in 1994, with the aim to improve the care of neonates, infants and children by advancing the personal development of pharmacists and the provision of quality pharmacy services in relation to practice, research and audit, education and training, communication and advice. Membership is open to any pharmacist, pharmacy technician or corporate body with a pharmaceutical interest in paediatric or neonatal pharmacy.

The group has links with various paediatric organisations. The Medicines Committee is a joint committee of the Royal College of Paediatrics and Child Health (RCPCH) and the NPPG. The Medicines Committee has highlighted paediatrics at Government level and as part of that framework the first national paediatric formulary (Medicines for Children) was produced. That venture joined forces with the BNF and in 2005 the first BNF for Children (BNFC) was distributed to all prescribers in the UK. The BNFC is now published annually and NPPG has input to the review and updating of the information contained. NPPG also works closely with several Department of Health groups, as well as NICE and the Health Commission.

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?

NPPG agrees that the Paediatric Regulation has been instrumental in initiating the development of medicines in children. One reason for this is that it is mandatory - unlike the USA where the FDA issued Guidance to the Pharmaceutical Industry. This is an important distinction. In the EU there is a commitment from both sides that allows agreement to occur and for suggestions to be made on how to take individual issues forward.

We consider that the Paediatric Regulation has allowed groups of individuals from across the EU to work together. In many instances however there is a lack of information on the particular disease states. There are examples of conditions where a consensus has been developed by groups of experts – e.g. Sepsis. This demonstrates the large amount of work being undertaken behind the scenes. Another major impact is that once a product is authorised it has impact on all 27 countries. Without the Paediatric Regulation all these issues would have taken much longer to achieve.

Consultation item No 2: Do you agree with the above assessment?
Overall - we consider that the Paediatric Regulation has been a major step forward however it may still be too early in the process to see fully what could be achieved. We agree that it is still too early to see major benefits from the regulation, however it is essential that a framework is in place to ensure that the pharmaceutical companies follow through their PIPs in a timely manner and therefore provide the information and formulations required.

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?

We agree that the uptake of the PUMA concept has been a disappointment. This seems likely to be due to the lack of market incentive and insufficient financial reward.

We are pleased to see some progress with the approval of Buccolam however the lack of uptake for the PUMA concept is disappointing. In our opinion the process needs to be made as simple as possible whilst maintaining safety. It should be more widely advertised to smaller companies such as Specials companies etc. Feedback from the company who has successfully been through the process should be sought regarding their thoughts on this. Perhaps a top 10 key desirable products should be put together and put out to tender to stimulate activity in this field?

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 are unable to comment on this

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

We agree that all new medicines should be screened for potential use in children but accept that the starting point for development is driven by conditions and usage in the adult population. It is inevitable that product development follows adult medicine research programmes but at least the paediatric population will benefit to a degree. We do not however need for example, multiple treatments for type 2 diabetes for children as seems to be happening – one or two would suffice.

Further means of stimulating drug development of medicines for paediatric conditions where there are gaps in current availability is still needed and a means of focussing on these is required.

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

Yes

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 – this is proving to be successful and we are pleased that the information being generated is available across the whole of the EU however we feel that this needs to be better publicised.

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?

It may be too early to judge this and could be dependent on individual healthcare professionals. However we feel that there needs to be a drive to raise awareness and engage healthcare professionals to encourage them to take this new information on board and use it in day to day practice. Generally healthcare professionals are receptive to new information if it is evidence based and if it relates to the clinical usage of a drug in the practice they are involved with. However there needs to be an efficient means of ensuring this information reaches prescribers and pharmacists. In the UK the BNFC would seem the ideal vehicle for this.

We also consider that it is important to have the time for education and training and then to develop interest and support for research and development in this important area. We do note that many healthcare professionals are busy undertaking basic services and that it is often difficult to find the time for research and development. Properly funded research and training to support this should be a priority.

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?

As mentioned under our response to Question 5 - it is important that duplication of trials for ‘’me-too’’ type drugs should be avoided.

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

We do see the need for the PIP to be submitted early in the development of the medicine in order that this information is taken into account during later development should it continue for that medicine.

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 – this has been a significant achievement bringing Paediatric issues higher up the agenda and has lead to other indirect outcomes. For example - the establishment of comprehensive Educational training programmes for healthcare professionals e.g. Global Research in Paediatrics (GRIP). However there is now a need to concentrate on identifying priorities for paediatric drug/formulation development and developing methods to stimulate these priorities to be met.

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?

We have been pleased to see the development of newly licensed products for children although we had expected that it would be easier for companies to use the data they already had and for them to obtain a licence.

We also consider that it is important that suitable age appropriate formulations are developed for children and this is a particular issue we wish to see being taken forward in the future. We are disappointed to see that in some cases the products being marketed are less than ideal. In some instances this is because they have a limited shelf life and are not ready to use. In other cases the formulations have multiple excipients. Examples we have noticed are sildenafil (Revatio 10 mg/ml powder for oral suspension) and losartan (COZAAR 2.5 mg/ml powder and solvent for oral suspension).

It is also disappointing to see that many of the new products which have been developed are expensive and this may be an issue in their uptake.

We are also disappointed in the lack of research monies which we had expected to be available to take forward some of the issues covered by the Regulation.

Overall - we feel that we have not seen the real impact of this legislation yet. This is all still new for those involved and we are all still learning. The time frame for development of medicines is such that it will take another 5-10 years to see the full picture. We look forward to further progress in this important area.