Medicinal products for paediatric use

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The Commission presents its General Report on experience acquired as a result of the application of Regulation (EC) No 1901/2006 on medicinal products for paediatric use five years after the Regulation's entry into force. The report concludes that despite more than five years of experience, the true impact of the Regulation on the health of children will only become apparent over time as experience is accumulated in the longer term. There are encouraging signs though.

- Better and safer research: before the Paediatric Regulation entered into force, many pharmaceutical companies considered the adult population their key market. Research into the potential use of an adult product in the paediatric population was often side-lined or not considered at all. With the obligations introduced by the Regulation, forcing companies to screen every new (adult) product for its potential paediatric use, the situation has been turned around.

By the end of 2012, the European Medicines Agency had agreed 600 paediatric investigation plans. Of these, 453 were for medicines that were not yet authorised in the EU, while the remainder related to new indications for patent-protected products or paediatric use marketing authorisations.

- More medicines available for children: over 12 years (from 1995 to 2006), 108 of all 317 indications of 262 centrally authorised medicines included the paediatric population. Since the Paediatric Regulation entered into force, 31 out of 152 new medicines have been authorised for paediatric use, 10 of which met the conditions of Article 7. This is no more than a 'snapshot' of the effects of the Regulation as this figure is likely to increase in the future, as a considerable number of the new, already authorised, medicines are subject to an investigation plan where completion was deferred to avoid delays in the authorisation of the adult product. It follows that in the years to come many more of those 152 new medicines are expected to be authorised for paediatric use.

- Increased information on medicines used by children: since 2008, more than 18 000 study reports on roughly 2200 medicinal products have been submitted, revealing the large amount of existing paediatric information available at company level.

Some **lessons** have been learnt in the last five years. Their impact on the overall performance of the Regulation has to be closely monitored. They include:

- Better access to treatment, since 2008, more than 600 paediatric investigation plans have been approved. However, only a minority of them has been completed to date; the vast majority are still on-going. This is due to the **long development cycles of medicinal products**, often lasting more than a decade and the near-systematic deferral of paediatric studies. The high number of deferrals may not have been initially expected, but are currently a reality, as for most of the medicinal products that have been authorised so far, the R&D programme started before the entering into force of the Regulation. Consequently, the paediatric requirements could not be taken into account from the beginning of the product development.

Criticism has been voiced that the Regulation **will fail to ensure a breakthrough** in areas of particular paediatric need, such as paediatric oncology. This argument is related to the fact that the starting point for the majority of paediatric investigation plans is an ongoing R&D programme for a medicinal product for adults. An intrinsic consequence of this approach is that these products primarily target adult conditions.

Moreover, the Regulation grants **waivers from its obligations** where the disease or condition for which the specific medicinal product is intended occurs only in adult populations. This legislative approach creates friction in the case of diseases that are specific and exclusive to children.

- PUMA: the Paediatric Regulation introduced a **new type of marketing authorisation** - the Paediatric Use Marketing Authorisation (PUMA). As an incentive to carry out research into the potential paediatric use of off-patent medicinal products that have been authorised for adults, this marketing authorisation offers 8 years of data and 10 years of market exclusivity to any new off-patent product developed exclusively for use in the paediatric population. To date, only one PUMA has been granted, with a few more projects currently in the pipeline. The EMA will in future accept paediatric investigation plans for a PUMA that cover only certain age groups and not the entire paediatric population. This may offset some of the reservations that currently hamper better endorsement of the PUMA concept.