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Questions and answers

EMA/PDCO review of the list of granted Class Waivers

1. What are class waivers?
2. Why has the current class waiver list been revised?
3. How has the list of class waivers been revised?
4. How will this affect the development of medicines in children?
5. When will the revised class waiver list come into effect?

What are class waivers?

Since the Paediatric Regulation came into force on 26 January 2007, all applications for marketing authorisation for new medicines have to submit a paediatric investigation plan (PIP) early in the development of the medicine, describing how the medicine should be studied in children. The EMA's Paediatric Committee (PDCO) assesses and agrees the content of the PIP to ensure that the necessary data are obtained through studies in children, when it is safe to do so, aiming to support the authorisation of the medicine for children. This requirement also applies when a company wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorised and patented.

As it is not always appropriate to develop certain medicines in children, waivers were introduced with the Paediatrics Regulation to avoid unnecessary studies in children. Under the waiver system, companies get an exemption to the requirement to submit a PIP to the Agency where the medicine is likely to be ineffective or unsafe, where the medicine does not represent a significant therapeutic benefit over existing treatments in children or when the medicine is intended to treat a condition that only occurs in adults.

Class waivers are an exemption from the obligation to submit a PIP request, for classes of medicines intended for specific conditions. When the intended use is covered by a class waiver, the developer is not required to submit an application for a medicine to the PDCO, and the PDCO cannot evaluate the potential of the medicine for children.

The list of class waivers is regularly reviewed by the PDCO and is published on the EMA website.

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Why has the current class waiver list been revised?

The PDCO regularly reviews and updates its waivers, as per Article 14 of Regulation (EC) No 1901/2006 (the "Paediatric Regulation"). Previous revisions included the revocation of two class waivers in 2008 and in 2009.

However, the PDCO has now extensively reviewed its current class waiver list taking into account all its regulatory experience and scientific developments since the Paediatric Regulation came into force. Its extensive review, which began in 2011, aimed to assess how well the current class waiver list maintains the balance of supporting development and considering potential benefit of medicines in children, while avoiding to expose children to unnecessary studies.

The PDCO came to the conclusion that the existing class list did not reflect this balance. It noted that most of the class waivers refer only to one disease and thus can be used to avoid considering the potential use of these medicines in children more generally.

It concluded that the current class waiver list resulted in insufficient opportunities for the PDCO to consider the potential benefits of some new medicines for children. For example, between 2012 and 2014, out of 26 new anticancer medicines authorised for use in adults, only 14 had a development plan for use in children.

How has the list of class waivers been revised?

The PDCO reviewed, for all class waivers, the available information on the disease area, the characteristics of the medicines and any available evidence on their possible use in children.

Based on its assessment of the available information, the PDCO made the following revisions to the current class waiver list:

- 8 class waivers for diseases were revoked, because new information available since the class waivers were granted showed that the disease can occur in children;
- 15 class waivers were revised, taking into account the characteristics of the medicines as well as the diseases;
- 9 class waivers were confirmed, and remain unchanged compared with the current list.

The PDCO will continue to revise the class waiver list as more information on medicines and diseases becomes available.

How will this affect the development of medicines in children?

The Paediatric Regulation has been successful in increasing the number of medicines that are researched, developed and authorised for use in children. Between August 2007 and April 2015, EMA and the PDCO have agreed 783 paediatric investigation plans with pharmaceutical companies, to provide data on the efficacy and safety of medicines for diseases of children.

The revision of the current class waiver list is a further step towards supporting the development of medicines in children. Companies developing medicines that are no longer subject to a class waiver under the revised list will need to have a PIP or requests for product-specific waiver for scientific review and agreement by the PDCO. This means that in the future more medicines will be reviewed by the PDCO for potential development for use in children.

Medicine developers are encouraged to engage in early dialogue with EMA, in particular through the recently launched '[early paediatric interaction meetings]', to discuss paediatric needs in early phases of medicine development.

When will the revised class waiver list come into effect?

The PDCO opinion is expected to be transformed into a further decision by the Agency and the revised and revoked class waivers will come into effect in 2018, in accordance with the legal provision in regulation (EC) No. 1901/2006. This means that from 2018, all applications for new medicines or variation of marketing authorisation will be validated against the revised class waiver Decision.