Improving the implementation of the Paediatric Medicine Regulation – recent changes made by the PDCO

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- I am employed by a regulatory agency, and have nothing to disclose.
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(Acts whose publication is obligatory)


of 12 December 2006


This Regulation aims to facilitate the development and accessibility of medicinal products for use in the paediatric population, to ensure that medicinal products used to treat the paediatric population are subject to ethical research of high quality and are appropriately authorised for use in the paediatric population, and to improve the information available on the use of medicinal products in the various paediatric populations. These objectives should be achieved without subjecting the paediatric population to unnecessary clinical trials and without delaying the authorisation of medicinal products for other age populations.
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on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive

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Topics

- Life cycle approach
- Committee interaction
- Waiver
Research
- Intensive basic research for new molecules,
in-vitro tests, animal studies

Pre-clinical development
- In-vitro tests, animal studies on efficacy
- Trials to test on potential risks

Clinical Phase 1
- Trials on tolerability
- First in Human trials

Clinical Phase 2
- PK trials for confirmation
- First trials on efficacy (PD) in patients

Clinical Phase 3
- Final confirmation of efficacy in large trials
- Building up the safety database on adverse drug reaction

Marketing Authorisation
- Assessment of clinical trial development
- Seeking positive opinion (e.g. EMA)

Only few reach the target

Basic Research
- high impact
- scientific publication

PIP submission

PIP assessment
and
Life cycle

MA & Public Health interest

Time span of app. 13.5 years to MAA

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Life cycle approach of paediatric medicinal product development

Pre-authorisation
- Phase 1

Approval
- Phase 2
- Phase 3

Post-marketing
- Phase 4

Clinical trial program dominated by adult development plan

Adult pre-/ clinical trial program

Early dialog
- PIP opinion including waiver & deferred clinical trials

Not deferred Study

Studies deferred

PASS/ PAES
Pilot of early dialogue project with applicants started in Q3 - 2015
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**EMA**
Scientific and regulatory support

**PDCO and the power of AC/DC**
- **Assessing**
  - PIPs
- **Contributing**
  - with expertise
  - guidelines
- **Deciding**
  - PIP opinion
- **Cooperating**
  - ENPREMA network
  - CHMP/SAWP
  - PRAC (WG)
  - COMP (WG)
  - CAT (case by case)

**Industry**
- PIP application
- Discussing paediatric issues
- Medicinal product development
- Early dialog

**Public health**
- Input from children into CT
- Education (GRIP)
- Communicating awareness
CHMP/SAWP - PDCO interaction

- Pre-EU Regulation (PEG)
- Expert comments (PDCO)
- Organised SA process contribution
- Contribution and SAWP reflection
- Discussion and PDCO endorsement

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In accordance with the Paediatric Regulation (Regulation (EC) No 1901/2006), the PDCO adopted a review of the class waiver list on 23 July 2015. The review included all previously granted class waivers, most of which referred to specific diseases.
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Condition versus Indication

Condition

How to identify the condition of potential paediatric interest, starting from the adult indication(s)?

Indication

Indication(s) targeting MAA Coming mainly from adults

Need of a systematic and consistent approach to provide a framework and some degree of predictability for applicants and PDCO

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EMA Review of class waiver list (23 July 2015)

• Previously, a class waiver covered any medicine for treatment of 46 listed conditions / diseases that occur only in adults

• Rigorous scientific review of the list, based on experience and recent data on medicines and diseases, incl. data from paediatric studies

• Outcome: Class waiver list limited to 20 classes of medicines for 15 conditions, and to all medicines for 11 adult conditions

• Consequence: Pharmaceutical companies now have to engage with the PDCO, either to agree a PIP or a product specific waiver

• However: Legal reasons for waiver are unchanged – PDCO has to grant waiver if disease occurs only in adults
Ways forward

- **Innovative clinical trial methodology** (modeling/simulation => Extrapolation)
- **Increase public awareness regards** added value of authorised products and age appropriate formulation
- Inform patients of opportunities for **participation in clinical trials**
- Advocate **public funding** for specific children’s health research
- Assure that authorised & adapted paediatric medicines **become and remain available**

By all optimism, we need to be realistic!
The way ahead to get equal opportunities for paediatric medicines is long, but we can get there.
Child health

Research & development

Together we can be strong – Thanks for listening