



30 April 2015
EMA/PDCO/52553/2015 Corr.1
Human Medicines Research and Development Support

Inventory of paediatric therapeutic needs

Endocrinology

Adopted by PDCO for release for consultation	20 March 2015
Start of public consultation	8 May 2015
End of consultation (deadline for comments)	6 July 2015 ¹
Adoption by PDCO for final release	

Comments should be provided using this [template](#). The completed comments form should be sent to paediatrics@ema.europa.eu

Objective of the list

Based on Article 43 of the European Union [Paediatric Regulation](#) the Paediatric Committee at the European Medicines Agency (PDCO) is working to establish an inventory to identify the needs in the different therapeutic areas where there should be research and development of medicinal products for children. The inventory is based on the results of a [survey](#) of all paediatric uses of medicines in Europe and on the existing list of paediatric needs established by the former Paediatric Working Party; it will be published progressively by therapeutic area. Further information can be found on the [EMA website](#).

Disclaimer

The lists should not be viewed as a prescription tool nor as recommendations for treatment.

The authorisation status of the medicinal products as well as on available formulation(s) was taken into account. However, this information is limited and not available for all European Member States. Users of this list are advised to check the authorisation status of the medicinal products of interest.

The methodology used to establish the list was based as much as possible on existing evidence. It is acknowledged that identification of needs for research into medicinal products for paediatric use is partly based on subjective criteria and may change over time and according to region. This may also be the case should further information of which the PDCO is not aware become available (e.g. on

¹ Deadline for comments extended to 6 July 2015



pharmacokinetics, safety and efficacy, submission of Paediatric Investigation Plans on listed products, etc.).

Notes

For the designation of the products International Non-proprietary Names (INN) are used whenever possible. Products are listed in alphabetical order within the product classes, not in order of priority.

If not stated otherwise, the needs concern all paediatric age-groups.

For information on products with an agreed Paediatric Investigation Plan (PIP) please consult the [EMA website](#).

Product	Needs
Insulines	
Inhaled human insulin	Data on PK, PD, efficacy and safety in children and adolescents < 18 years. Data on long-term safety.
Oral antidiabetics	
Acarbose	Data on PK, PD, efficacy and safety in children >7 years in diabetes type I and other potential indications (dumping syndrome and postprandial hypoglycemia, Impaired Glucose Tolerance). Data on PK, PD, efficacy and safety in children <2 years in dumping syndrome.
Repaglinide	Data on PK, PD, safety and efficacy for all age groups above 2 years of age.
Desmopressin	New strengths for oral use in neonates and children.
Corticosteroids	
Fludrocortisone	Need for doses for neonates and children <2 years of age. Data on long term safety.
Sex hormones	
Testosterone	Data on PK, efficacy and safety for the gel formulation.
Testosterone undecanoate	Data on PK, safety and efficacy for all age groups.

Product	Needs
	Need for different strengths.
Dihydrotestosterone	Data on PK, PD, safety and efficacy for all age groups.
Ethinyl estradiol	Data on PK, PD, safety and efficacy. Need for 2, 5 and 10 mcg tablets and age appropriate formulations.
Estrogens	Data on PK, PD, safety and efficacy in children > 10 years of age.
Conjugated estrogens	Data on PK, PD and safety in neonates for cream formulation.
Lipid lowering drugs	
Cholestyramine	Data on safety and efficacy in children < 6 years of age. Palatable age-appropriate formulation.
Other endocrine drugs	
Glucagon	Need for continuous subcutaneous insuline infusion pump presentation for administration in neonates and children.