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Human Medicines Development and Evaluation

Draft inventory of paediatric therapeutic needs

Therapeutic area nephro-urology

Agreed by PDCO	July 2013
Adopted by PDCO for release for consultation	19 July 2013
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Agreed by PDCO	
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 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.

The shaded products represent those where a positive decision has been adopted on a Paediatric Investigation Plan (PIP). For further information please consult the [EMA website](#).

Therapeutic area nephro-urology

Please also refer to the published inventory on needs for the cardiovascular therapeutic area and, as soon as published, to immunology and neonatology.

Product	Needs
Diuretics	
Bendroflumethiazide	<ul style="list-style-type: none"> Age-appropriate formulation.
Chlorothiazide	<p>For treatment of hypertension:</p> <ul style="list-style-type: none"> Data on PK/dose, efficacy and safety. <p>For treatment of oedema in bronchopulmonary dysplasia:</p> <ul style="list-style-type: none"> Data on PK/dose, efficacy and safety. Age appropriate formulation.
Hydrochlorothiazide	<p>For treatment of oedema and hypertension:</p> <ul style="list-style-type: none"> Data on PK, efficacy and safety. Age-appropriate formulation. <p>For treatment of tubulopathies:</p> <ul style="list-style-type: none"> Data on PK, efficacy and safety. Age-appropriate formulation.
Metolazone	<p>For treatment of oedema and hypertension:</p> <ul style="list-style-type: none"> Data on PK, efficacy and safety. Age-appropriate formulation.
Amiloride	<p>For treatment of oedema (adjunctive):</p> <ul style="list-style-type: none"> Data on PK, safety and efficacy in children > the age of 3 months. <p>For treatment of congenital tubulopathies:</p> <ul style="list-style-type: none"> Data on PK, efficacy and safety.

Product	Needs
Triamterene	For treatment of oedema (adjunctive): <ul style="list-style-type: none"> • Data on PK, safety and efficacy. For treatment of congenital tubulopathies: <ul style="list-style-type: none"> • Data on PK, efficacy and safety.
Bumetanide	For treatment of oedema: <ul style="list-style-type: none"> • Data on PK, safety and efficacy in children <u>below</u> the age of 12 years.
Spironolactone	For treatment of bronchopulmonary dysplasia (BPD), ascites, oedema in neonates: <ul style="list-style-type: none"> • Data on PK, efficacy and safety. • Age-appropriate formulation.
Butizide	Comments required: <ul style="list-style-type: none"> • Define needs. • Rank in order of priority.
Indapamide	
Clopamide	
Chlortalidone	
Etacrynic acid	
Eplerenon	
Torasemide	

Product	Needs
Angiotensin receptor blockers	
<p>The following 'sartans' are on the list of paediatric needs for nephrology by the former Paediatric Working Party (PEG), further 2 have been added from the survey of the use of medicinal products in children in Europe.</p> <p>Comments required on products used including the condition and age-group and define any needs.</p>	
Angiotensin Receptor Blockers ('Sartans')	<p>For treatment of chronic kidney diseases associated with hypertension and proteinuria:</p> <ul style="list-style-type: none"> • Data on PK, safety and efficacy. • Age-appropriate formulation. <p>For prevention and treatment of diabetic nephropathy:</p> <ul style="list-style-type: none"> • Data on PK, safety and efficacy. • Age-appropriate formulation.
Losartan	PIP agreed for treatment of hypertension and treatment of proteinuria.
Valsartan	PIP agreed for treatment of hypertension.
Irbesartan	<p>Comments required:</p> <ul style="list-style-type: none"> • Define needs (see also 'general needs' for 'sartans' above). • Rank in order of priority.
Candesartan	
Telmisartan	
Eprosartan	
Olmesartan	

Product	Needs
Ace-inhibitors	
Captopril	<p>For the treatment of hypertension and treatment of heart failure:</p> <ul style="list-style-type: none"> • Data on PK and safety specifically in neonates and infants, re-analysis of the benefit/risk in children based on existing data. • Age-appropriate formulation. <p>For treatment of chronic kidney diseases associated with hypertension and proteinuria:</p> <ul style="list-style-type: none"> • Data on PK, safety and efficacy. • Age-appropriate formulation. <p>For treatment and prevention of diabetic nephropathy:</p> <ul style="list-style-type: none"> • Data on PK, safety and efficacy. • Age-appropriate formulation.
Enalapril	<p>For treatment of proteinuria in nephritis, diabetic nephropathy:</p> <ul style="list-style-type: none"> • Data on PK, safety and efficacy. • Age-appropriate formulation. <p>For treatment of hypertension:</p> <ul style="list-style-type: none"> • Data on PK and safety; re-analysis of the benefit: risk profile in children based on existing data. • Age-appropriate formulation.
Ramipril	<p>For treatment of hypertension and treatment of proteinuria:</p> <ul style="list-style-type: none"> • Re-analysis of the benefit/risk in children based on existing data. • Define lower age limit and investigate where needed. • Age appropriate formulation.
Lisinopril	<p>For treatment of hypertension:</p> <ul style="list-style-type: none"> • Data on PK, efficacy and safety in children below the age of 6 years. • Age-appropriate formulation. <p>For treatment of proteinuria:</p> <ul style="list-style-type: none"> • Data on PK, efficacy and safety. • Age-appropriate formulation.

Product	Needs
Calcium channel blockers	
Amlodipine	For treatment of hypertension: <ul style="list-style-type: none"> • Data on PK/dose, efficacy, safety in children below the age of 6 years. • Age appropriate formulation.
Nicardipine	For treatment of hypertension: <ul style="list-style-type: none"> • Data on PK/dose, efficacy, safety. • Age appropriate formulation.
Nifedipine	For treatment of hypertension: <ul style="list-style-type: none"> • Data on PK/dose, efficacy, safety. • Age appropriate formulation.
Anticholinergic drugs	
oxybutynine	For treatment of neurogenic bladder syndrome: <ul style="list-style-type: none"> • Data on PK, efficacy and safety in children below the age of 5 years. • Data on PK, efficacy and safety for intravesical installation.
trospium	For treatment of idiopathic or neurologic detrusor overactivity: <ul style="list-style-type: none"> • Data on PK, safety, efficacy in children below the age of 12 years. • Age-appropriate formulation.
solifenacin	PIP agreed for treatment of idiopathic overactive bladder syndrome and treatment of neurogenic detrusor overactivity.

Product	Needs
Other	
levamisole	Treatment of steroid sensitive but frequently relapsing NS. PEG: <ul style="list-style-type: none"> • Any formulation. • Data on PK, safety and efficacy in children < 18 years in steroid resistant. • Nephrotic syndrome.
cinacalcet	PIP agreed for treatment of secondary hyperparathyroidism in patients with end-stage renal disease.
pyridoxine	For treatment of hyperoxaluria: <ul style="list-style-type: none"> • Data on PK, safety and efficacy in children below the age of 1 year. • Age-appropriate formulation.
tiopronine	For treatment of cystinuria: <ul style="list-style-type: none"> • Data on PK, safety and efficacy in children below the age of 1 year. • Age-appropriate formulation.
NSAIDS (e.g. indomethacin and others)	For treatment of congenital salt losing tubulopathies nephrogenic diabetes insipidus, and proteinuria in congenital nephrotic syndrome: <ul style="list-style-type: none"> • Data on safety and efficacy.
Aquaretics (AVP receptor antagonists)	
e.g. tolvaptan	For treatment of chronic (>48 hours) dilutional hyponatraemia resistant to fluid restriction, (i.e., euvolemic and hypervolemic hyponatraemia) associated with heart failure, cirrhosis, or SIADH and for treatment of polycystic kidney disease <ul style="list-style-type: none"> • Data on PK/dose, safety and efficacy. • Age-appropriate formulation.