



15 November 2013  
EMA/562919/2013  
Human Medicines Development and Evaluation

## Inventory of paediatric therapeutic needs

### Neurology

Agreed by PDCO	October 2013
Adopted by PDCO for release for consultation	9-11 October 2013
Start of public consultation	9 May 2014
End of consultation (deadline for comments)	9 July 2014
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](mailto: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 neurology

Product	Needs
<b>Multiple Sclerosis</b>	
Interferon Beta-1	For treatment of relapsing multiple sclerosis: <ul style="list-style-type: none"> <li>PK, efficacy and safety in children aged 10-16 years</li> </ul>
<b>Migraine</b>	
Propranolol	For prevention of migraine: <ul style="list-style-type: none"> <li>Data on PK, efficacy and safety in children from 6 years of age.</li> </ul>
Topiramate	For prevention of migraine: <ul style="list-style-type: none"> <li>Data on PK, efficacy and safety in children from 6 years of age.</li> </ul>
Triptans (in general)  (Please note that for Rizatriptan a waiver has been agreed for children below 6 years of age and a PIP and deferral has been agreed for children over 6 years)	For treatment of headache phase of migraine attacks: <ul style="list-style-type: none"> <li>Data on PK, efficacy and safety in children from 6 years.</li> </ul>

Product	Needs
<b>Epilepsy</b>	
Carbamazepine	For treatment of partial onset seizures: <ul style="list-style-type: none"> <li>• Age appropriate slow release formulation.</li> </ul>
Clobazam	For short-term treatment of seizures and epileptic syndromes: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety in children below 6 years of age</li> <li>• Data on long-term safety and efficacy in all paediatric age groups</li> </ul>
Clonazepam	For short-term treatment of seizures and treatment of epileptic syndromes: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety in children below 3 months</li> <li>• Data on long-term safety in all paediatric age groups</li> <li>• Age appropriate formulation</li> </ul>
Felbamate	For treatment of epilepsy syndromes and refractory epilepsies: <ul style="list-style-type: none"> <li>• Data on PK in children aged below 4 years</li> <li>• Data on long term safety</li> </ul>
Lamotrigine	For treatment of partial and general seizures and Lennox-Gastaut syndrome:  Data on PK, efficacy and safety in children below 2 years of age
Lidocaine	For treatment of neonatal seizures: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety for intravenous formulation.</li> </ul>
Lorazepam	For treatment of status epilepticus: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety</li> </ul> Age appropriate formulation
Midazolam	PIP agreed for treatment of epileptic seizures.
Oxcarbazepine	For treatment of partial onset seizures: <ul style="list-style-type: none"> <li>• Long term safety</li> </ul> Age appropriate slow release formulation

Product	Needs
Phenobarbitone	For treatment of neonatal seizures: <ul style="list-style-type: none"> <li>• Long term safety data</li> <li>• Age appropriate liquid and IV formulation</li> </ul>
Sultiam	For treatment of epilepsy syndromes: <ul style="list-style-type: none"> <li>• Data on PK, safety and efficacy</li> <li>• Age appropriate formulation</li> </ul>
Thiopental	For treatment of status epilepticus: <ul style="list-style-type: none"> <li>• Data on efficacy and safety</li> </ul>
Topiramate	For treatment of epilepsy syndromes: <ul style="list-style-type: none"> <li>• Data on efficacy (data only available for partial-onset seizures and Lennox-Gastaut syndrome)</li> <li>• Age-appropriate formulation.</li> </ul> For treatment of neonatal seizures: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety for intravenous formulation.</li> </ul>
Valproate	For high-dose treatment of generalised epilepsy and partial onset seizures: <ul style="list-style-type: none"> <li>• Data on PK and safety</li> <li>• Data on PK, safety and efficacy in infants below 2 months of age</li> </ul> For treatment of status epilepticus: <ul style="list-style-type: none"> <li>• Data on efficacy and safety of intravenous formulation</li> </ul>
Vigabatrin	Treatment of infantile spasms (West-syndrome): <ul style="list-style-type: none"> <li>• Age appropriate formulation</li> </ul>
Zonisamide	For treatment of generalised epilepsy: <ul style="list-style-type: none"> <li>• Data on PK, safety and efficacy</li> <li>• Age appropriate formulation</li> </ul>

Product	Needs
<b>Other</b>	
Baclofen	For treatment of muscular spasticity of cerebral origin: <ul style="list-style-type: none"> <li>• Data on PK, safety and efficacy in children below 4 years of age</li> </ul>
Botulium toxin A	For treatment of muscular spasticity of cerebral origin: <ul style="list-style-type: none"> <li>• Data on safety and efficacy in children below 12 years of age</li> </ul>
Carbidopa	For treatment of dyskinesia: <ul style="list-style-type: none"> <li>• Data on PK, safety and efficacy</li> </ul>
Levodopa	For treatment of dyskinesia: <ul style="list-style-type: none"> <li>• Data on PK, safety and efficacy</li> </ul>
Tetrabenazine	For treatment of dystonia, movement disorders, extrapyramidal dyskinesia: <ul style="list-style-type: none"> <li>• Data on PK, efficacy and safety</li> <li>• Age appropriate intravenous formulation</li> </ul>