30 April 2015
EMA/PDCO/43828/2015 Corr.1
Human Medicines Research and Development Support

# Inventory of paediatric therapeutic needs

## Gastroenterology

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|---|---|
| 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

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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.

Products with an agreed Paediatric Investigation Plan (PIP) are not included in the list - for information on these products please consult the EMA website.

| ***Product*** | ***Needs*** |
|---|---|
| **Antiemetics** | |
| Granisetron | Data on PK, efficacy and safety in infants and neonates in chemotherapy-induced and post-operative nausea<br>Data on efficacy and safety in cyclical vomiting syndrome |
| Ondansetron | Data on efficacy and safety in cyclical vomiting syndrome<br>Data on safety in acute gastroenteritis vomiting in children from 6 months to less than 12 years of age<br>Appropriate strength rectal formulation |
| **Laxatives** | |
| Bisacodyl | Data on long-term efficacy and safety<br>Age-appropriate oral formulation |
| **Bile acid preparations** | |
| Ursodeoxycholic acid | Data on efficacy and safety in cholestatic conditions<br>Data on efficacy and safety in autoimmune hepatitis<br>Data on efficacy and safety in hepatobiliary disorder associated with cystic fibrosis |
| **Motility drugs** | |
| Erythromycin | Data on efficacy and safety in gastro-intestinal stasis |
| **Biological agents – immunomodulators** | |

| ***Product*** | ***Needs*** |
|---|---|
| Rituximab | Data on PK, efficacy and safety in liver transplantation |
| **Disease modifying and immunosuppressive agents** | |
| Azathioprine | Data on efficacy and safety in Crohn's disease (alone or in combination with a biological), and in Ulcerative Colitis<br>Age-appropriate oral formulation |
| Cyclophosphamide | Data on PK, efficacy and safety in severe autoimmune enterocolitis<br>Age-appropriate formulation |
| Cyclosporin | Data on efficacy and safety in Ulcerative Colitis<br>Age-appropriate oral formulation |
| Methotrexate | Data on efficacy and safety in Crohn's disease (alone or in combination with a biological), and in Ulcerative Colitis<br>Age-appropriate oral formulation |
| Mycophenolate mofetil | Data on PK, efficacy and safety in small bowel transplantation and liver transplantation<br>Data on PK, efficacy and safety in autoimmune and inflammatory bowel diseases and in autoimmune hepatitis |
| Sirolimus | Data on PK, efficacy and safety in Ulcerative Colitis and Crohn's Disease<br>Data on PK, efficacy and safety in small bowel allograft<br>Data on PK, efficacy and safety in neonatal colitis |
| Tacrolimus | Data on PK, efficacy and safety in Ulcerative Colitis and Crohn's Disease<br>Data on PK, efficacy and safety in small bowel allograft<br>Data on PK, efficacy and safety in neonatal colitis |
| **External pancreatic insufficiency treatments** | |
| Pancreatic enzymes from cells of porcine origin | Age-appropriate formulation for newborns and infants |
| **Other drugs for peptic ulcer and gastro-oesophageal reflux disease** | |
| Alginic acid | Data on efficacy and safety in gastroesophageal reflux disease |

| ***Product*** | ***Needs*** |
|---|---|
| | Age-appropriate formulation |
| **Proton pump inhibitors** | |
| e.g. esomeprazole, lansoprazole, omeprazole, pantoprazole, rabeprazole[2] | Data on PK, efficacy and safety in prophylactic use (ulcer prevention during intensive care treatment, treatment with corticosteroids) including parenteral use and gastric use (i.e. via feeding tube). |

[2] PIP agreed for rabeprazole for the "Treatment of *Helicobacter pylori* in patients with peptic ulcer disease", "Treatment of gastric ulcer", "Treatment of duodenal ulcer", "Treatment of Zollinger-Ellison syndrome" and "Treatment of gastro-oesophageal reflux disease"