



PRESS RELEASE

Meeting highlights from the Paediatric Committee, 9-11 April 2008

Positive opinions on paediatric investigation plans adopted

The European Medicines Agency's (EMA) Paediatric Committee (PDCO) adopted positive opinions on paediatric investigation plans for the following medicines:

- Albumin interferon alfa-2b from Novartis Europharm Ltd, in the therapeutic area of hepatology;
- Clopidogrel hydrogen sulfate, from Sanofi Pharma and from Bristol-Myers Squibb Pharma EEIG, in the therapeutic area of cardiovascular diseases;
- N-Acetyl-L-Cysteine, L-Alanine, L-Alanyl-L-Glutamine, L-Arginine hydrochloride, Glycine, Glycyl-L-Tyrosine, L-Histidine, L-Isoleucine, L-Leucine, L-Lysine acetate, L-Methionine, L-Phenylalanine, L-Proline, L-Serine, Taurine, L-Threonine, L-Tryptophan, L-Valine, from Fresenius Kabi Deutschland GmbH, in the therapeutic area of nutrition;
- Recombinant human monoclonal antibody to human IL-1beta, from Novartis Europharm Limited, in the therapeutic area of immunology;
- Dalbavancin, from Pfizer Limited, in the therapeutic area of infectious diseases;
- Adenovirus-mediated Herpes simplex virus-thymidine kinase gene, from Ark Therapeutics Ltd, in the therapeutic area of oncology.

A paediatric investigation plan (PIP) sets out a programme for the development of a medicine in the paediatric population. The PIP aims to generate the necessary quality, safety and efficacy data through studies to support the authorisation of the medicine for use in children of all ages. In some cases, a PIP may include a waiver to study one or more age groups of children.

Opinions on product-specific waivers

The PDCO adopted a product-specific waiver for arzoxifene, from Eli Lilly and Company Ltd, in the therapeutic area of oncology, recommending that the obligation to submit data obtained through clinical studies with children be waived in all subsets of the paediatric population.

Waivers can be issued if there is evidence showing that the medicinal product concerned is likely to be ineffective or unsafe in the paediatric population, or that the disease or condition targeted occurs only in adult populations, or that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

Decisions adopted

Following the adoption of opinions by the PDCO, the EMA adopted decisions on PIPs for paliperidone and doripenem monohydrate. In addition the Agency adopted decisions on product-specific waivers in all subsets of the paediatric population for flosfuridine tidoxil and for naproxcinod. These decisions will be published shortly on the EMA website at:

<http://www.emea.europa.eu/htms/human/paediatrics/decisions.htm>

Class waivers

The PDCO adopted an opinion on a list of class waivers for conditions that do not affect children and for which the requirement to submit a PIP can therefore be waived. This new opinion complements a first decision adopted by EMEA on 3 December 2007 on the list of class waivers, which was released for public consultation.

The PDCO followed a step-wise approach for the discussion and adoption of condition waivers. Once the list of class waivers was endorsed, the PDCO considered in a second step each individual new condition proposed to be included in the list of waivers during the public consultation phase and adopted a further list of waivers.

The Agency will take a final decision on the PDCO opinion on the list of class waivers within 10 days. Once approved, the list of waivers will be regularly updated in light of the advance of knowledge and science in the paediatric field.

Finalisation of the update of the priority list of off-patent medicines

The PDCO finalised the update of the priority list for studies into off-patent medicines (not covered by a patent in Europe) in advance of the next call from the European Commission in July 2008 for funding through the EU's Seventh Framework Programme. The priority list for studies into off-patent medicines helps to ensure that funds are directed into research of off-patent medicines, for which there is a high need in the paediatric population. Ultimately, the aim is that more of these medicines will be submitted to the EMEA for a paediatric-use marketing authorisation.

The updated list comprises approximately 30 medicines relating to the therapeutic areas of gastroenterology, oncology, immunology, infectious diseases, neonatology, neuropaediatrics, child and adolescent psychiatry, metabolism/endocrinology, pain/anaesthesiology, cardiovascular system, nephrology/urology and rheumatology.

The list has been set up from a public health perspective. In a first step the conditions with higher therapeutic needs were identified, mostly based on the severity of the disease, the paediatric groups affected (with special priority for the neonatal population), the non-availability of treatment alternatives, and the high prevalence of the disease in the paediatric population. In a second step published therapeutic reviews of medicines used in children were analysed to identify off-label products of therapeutic interest.

The list will be released for public consultation on the 'Medicines for children' section of the EMEA website shortly. The consultation will be open until 26 May 2008.

Six projects are currently being funded by the Seventh Framework Programme, following the first call for proposals.

The PDCO continued its interaction with experts in the area of paediatric anaesthesiology and puberty with a view to bringing state-of-the-art knowledge to the PDCO's scientific discussion.

The next meeting of the PDCO will be held on 6-8 May 2008.

-- ENDS --

Notes:

1. PDCO opinions on PIPs and waivers are transformed into EMEA decisions within the timeframe laid down by the Paediatric Regulation ([Regulation \(EC\) No 1901/2006](#), as amended).
2. The current priority list for studies into off-patent medicinal products can be found at: <http://www.emea.europa.eu/pdfs/human/paediatrics/19797207en.pdf>. The list was adopted on 27 April 2007 by the Paediatric Working Party, – the former EMEA temporary expert working party on paediatric medicines.

3. More information about the EU's Seventh Framework Programme can be found at:
http://cordis.europa.eu/fp7/home_en.html
4. More information about the PDCO and the Paediatric Regulation is available in the 'Medicines for children' section of the EMEA website:
<http://www.emea.europa.eu/htms/human/paediatrics/introduction.htm>
5. This press release, together with other information on the work of the EMEA, can be found on the EMEA website: <http://www.emea.europa.eu>

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OVERVIEW OF PAEDIATRIC INVESTIGATION PLAN/WAIVER APPLICATIONS

	2007 (August to December)	2008 (January- April)	Cumulative Total
Total number of validated PIP / waiver applications	85	88¹	173²
Applications submitted for a product not yet authorised (<i>Article 7</i>) ³	39	71	110 (64%)
Applications submitted for a product already authorised still under patent in view of a submission of a variation/extension for a new indication, pharmaceutical form or route of administration (<i>Article 8</i>)	45	13	58 (33%)
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation (<i>Article 30</i>)	1	4	5 (3%)
PIPs and full waiver indications covered by these applications	202	139	341

Number of Paediatric Committee (PDCO) opinions	2007	2008	Total
On full waiver	10	5	15
On PIPs including potential deferral	2	16	18

¹ figures including 10 April 2008 start of procedure; the figure does not include products which are currently under validation.

² of which 39 are requests for full waiver.

³ Applications submitted in accordance with Regulation (EC) No 1901/2006, as amended.

Areas covered by PIPs/waiver applications:	2007 (%)	2008 %
Neurology	12	4
Uro-nephrology	-	7
Gastroenterology-hepatology	9	1
Pneumology-allergology	8	4
Infectious diseases	12	5
Cardiovascular diseases	12	9
Diagnostics	-	3
Endocrinology-gynaecology-fertility-metabolism	19	20
Neonatology-paediatric intensive care	-	-
Immunology-rheumatology-transplantation	5	6
Psychiatry	5	2
Pain	1	1
Haematology-haemostaseology	1	7
Otorhinolaryngology	-	-
Oncology	11	17
Dermatology	1	1
Vaccines	2	9
Ophthalmology	1	1
Anaesthesiology	-	2
Nutrition	1	1