EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 August 2011
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Monthly report

Paediatric Committee (PDCO)

10-12 August 2011

Opinions on paediatric investigation plans

The Paediatric Committee (PDCO) adopted opinions agreeing paediatric investigation plans (PIPs) for the following medicines:

- **Potassium sulphate / magnesium sulphate, heptahydrate / sodium sulphate, anhydrous**, from Ipsen Pharma, in the therapeutic area of gastroenterology-hepatology;
- **Octenidine dihydrochloride / prednicarbate**, from Almirall Hermal GmbH, in the therapeutic area of dermatology;
- **Mepolizumab**, from Glaxo Group Limited, in the therapeutic area of pneumology - allergology;
- **Loxapine**, from Alexza UK Limited, in the therapeutic area of psychiatry;
- **Vatreptacog alfa (activated)**, from Novo Nordisk A/S, in the therapeutic area of haematology-haemostaseology;
- **Deferasirox**, from Novartis Europharm Limited, in the therapeutic area of haematology-haemostaseology;
- **Eltrombopag**, from GlaxoSmithKline Trading Services Limited, in the therapeutic area of haematology-haemostaseology;
- **Colestilan**, from Mitsubishi Pharma Europe Ltd., in the therapeutic area of uro-nephrology.

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Opinions on product-specific waivers

The PDCO adopted positive opinions for product-specific waivers, recommending that the obligation to submit data obtained through clinical studies with children be waived in all subsets of the paediatric population, for the following medicines:

- **Quinidine sulfate / dextromethorphan hydrobromide**, from Avanir Pharmaceuticals, Incorporated, in the therapeutic area of neurology;
- **Vildagliptin**, from Novartis Europharm Limited, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- **Progesterone**, from Teva Pharmaceuticals Europe B.V., in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- **Metformin hydrochloride / dapagliflozin**, from Bristol-Myers Squibb / AstraZeneca EEIG, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism.

The PDCO adopted one opinion on the **refusal** of a request for waiver for:

- **Antithrombin alfa**, from GTC Biotherapeutics UK Limited, in the therapeutic area of haematology-haemostaseology.

Waivers can be issued if there is evidence that the medicine 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 medicine, or the performance of trials, does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

Opinions on modifications to an agreed PIP

The PDCO also adopts, every month, opinions on modifications to an agreed PIP, which can be requested by the applicant when the plan is no longer appropriate or when there are difficulties that render the plan unworkable.

Withdrawals

The PDCO noted that two applications were withdrawn during the late stages of the evaluation (30 days or less before opinion).

Interaction with external experts

The PDCO has regular interactions with academic experts, with a view to bringing state-of-the-art knowledge to the PDCO scientific discussions. Two experts were invited to the August meeting. The PDCO discussed pathophysiology and prevention of major cardiovascular events in patients with chronic kidney disease with a clinical expert in paediatric Nephrology and pathophysiology and treatment of perinatal asphyxia with an expert in neonatal neurology.

Other issues

The PDCO welcomed the new member from the Czech Republic, Professor Jaroslav Sterba, who has been nominated by the State Institute for Drug Control.

The PDCO also welcomed new member representing Health care professionals, Professor Anthony Nunn, and Dr Matthias Keller new member and Dr Gerlind Bode new alternate, representing Patients' organisations, who have been nominated by European Commission

The PDCO thanked Dr Annagrazia Altavilla (Patients' organisations) and Dr Hubert Mottl (Czech Republic) for their work.

The next meeting of the PDCO will be held on 7-9 September 2011.

– END –

Notes:

1. PDCO opinions on PIPs and waivers are transformed into Agency's decisions within the timeframe laid down by the Paediatric Regulation (Regulation (EC) No 1901/2006, as amended). The decisions can be found on the Agency's website at: http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/landing/pip_search.jsp&murl=menus/medicines/medicines.jsp&mid=WC0b01ac058001d129

2. As of 26 January 2009, pharmaceutical companies that submit an application for a marketing authorisation for a medicinal product, or those that submit an application for an extension of indication, a new route of administration, or a new pharmaceutical form of a medicinal product already authorised in the European Union, have to provide either the results of studies in children conducted in accordance with an approved PIP, or an Agency's decision on a waiver or on a deferral.

3. More information about the PDCO and the Paediatric Regulation is available in the Regulatory section of the Agency's website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000023.jsp&murl=menus/regulations/regulations.jsp&mid=WC0b01ac05800240cd

4. This meeting report, together with other information on the work of the Agency's, can be found on the Agency's website: http://www.ema.europa.eu

Enquiries only to: paediatrics@ema.europa.eu

Annex of the August 2011 PDCO meeting report

	2009 (January to December)	2010 (January to December)	2011 (January to current month)	Cumulative total (2007 to 2011)
Total number of validated PIP/waiver applications	273	326	130	1087[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	191	280	103	800 *(74%)*
Applications submitted for a product already authorised and still under patent, in view of a submission of a variation/extension for a new indication, pharmaceutical form or route of administration *(Article 8[2])*	72	43	26	261 *(24%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	10	4	1	26 *(2%)*
PIPs and full waiver indications covered by these applications	395	403	152	1516

Number of Paediatric Committee (PDCO) opinions	2009	2010	2011	Cumulative total
Positive on full waiver	67	52	32	208
Positive on PIP, including potential deferral	122	201	69	475
Negative opinions adopted	13	7	3	27
Positive opinions adopted on modification of a PIP	51	103	97	259
Negative opinions adopted on modification of a PIP	0	4	1	5
Positive opinions on compliance with a PIP	8	9	3	25
Negative opinions on compliance check with a PIP	1	0	0	1
Opinions adopted under Art. 14.2	0	2	0	2

[1] Of which 259 have been requests for a full waiver.
[2] Applications submitted in accordance with the referenced article of Regulation (EC) No 1901/2006, as amended.

Areas covered by PIPs/waiver applications	2009 *(%)*	2010 *(%)*	2011 *(Number of areas covered)**
Neurology	4	3	7
Uro-nephrology	5	2	1
Gastroenterology-hepatology	2	1	6
Pneumology-allergology	6	41	6
Infectious diseases	9	4	10
Cardiovascular diseases	9	8	16
Diagnostics	1	1	4
Endocrinology-gynaecology-fertility-metabolism	16	6	23
Neonatology-paediatric intensive care	2	0	3
Immunology-rheumatology-transplantation	6	5	9
Psychiatry	3	1	6
Pain	6	1	1
Haematology-haemostaseology	6	4	11
Otorhinolaryngology	1	3	0
Oncology	11	9	14
Dermatology	6	1	5
Vaccines	4	2	8
Ophthalmology	2	4	6
Anaesthesiology	1	2	0
Nutrition	0	0	0
Other			4

** One PIP can cover several therapeutic areas*