16 June 2010
EMA/PDCO/360361/2010

Meeting highlights from the Paediatric Committee, 09-11 June 2010

Opinions on paediatric investigation plans

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

- **Vedolizumab (MLN0002),** from Takeda Global Research & Development Centre (Europe) Ltd, in the therapeutic area of gastroenterology-hepatology;
- **Bosentan monohydrate**, from Actelion Registration Ltd, in the therapeutic area of cardiovascular diseases;
- **Synthetic erythropoietin (EPO) receptor agonist**, from Takeda Global Research & Development Centre (Europe) Ltd, in the therapeutic area of haematology-hemostaseology;
- **Coagulation Factor IX (recombinant)**, from Inspiration Biopharmaceuticals EU, Ltd., in the therapeutic area of haematology-hemostaseology;
- **Recombinant human monoclonal antibody to human interleukin-17A of the IgG1/kappa-class**, from Novartis Europharm Limited, in the therapeutic area of immunology-rheumatology-transplantation;
- **(3R,4R)-4-methyl-3-(methyl-1H-pyrrolo[2,3-d]pyrimidin- 4-ylamino)-β-oxo-1-piperidinepropanenitrile, 2-hydroxy- 1,2,3-propanetricarboxylate (1:1) (CP-690,550)**, from Pfizer Limited, in the therapeutic area of immunology-rheumatology-transplantation.

The PDCO adopted an opinion on the **refusal** of a PIP, including a waiver and deferral, for **4-0-(ß-D-galactopiranosyl)-D-xylopyranose**, from Lactest SL, in the therapeutic area of diagnostic / gastroenterology-hepatology; the PDCO subsequently granted on its own motion a product-specific waiver for this medicine for all subsets of the paediatric population in the specified condition, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The PDCO adopted an opinion on the **refusal** of a PIP, including a waiver and deferral for **Omalizumab**, from Novartis Europharm Limited, in the therapeutic area of pneumology – allergology; the PDCO subsequently granted on its own motion a product-specific waiver for this medicine for all subsets of the paediatric population in the specified condition, on the grounds that the specific

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medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The PDCO adopted an opinion on the **refusal** of a PIP, including a waiver and deferral, for **lidocaine, tetracaine**, from ZARS Pharma, in the therapeutic area of anaesthesiology; the PDCO subsequently granted on its own motion a product-specific waiver for this medicine for all subsets of the paediatric population in the specified condition, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

A 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. These data have to be submitted to the European Medicines Agency, or national competent authorities, as part of an application for a marketing authorisation for a new medicine, or for one covered by a patent. In some cases, a PIP may include a waiver of the studies in one or more paediatric subsets, or a deferral.

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:

- **Perindopril tert-butylamine, Amlodipine besilate**, from Gedeon Richter Plc., in the therapeutic area of cardiovascular diseases;
- **Ramipril, amlodipine**, from EGIS Pharmaceuticals Plc., in the therapeutic area of cardiovascular diseases;
- **Dexamethasone**, from Allergan Pharmaceuticals Ireland, in the therapeutic area of ophthalmology;
- **Tafamidis meglumine**, from FoldRx Pharmaceuticals, Ltd., in the therapeutic area of neurology;
- **Levonorgestrel, ethinylestradiol**, from Teva Pharma B.V., in the therapeutic area of endocrinology-gynaecology-fertility-metabolism.

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

- **Human normal immunoglobulin**, from Bio Products Laboratory, in the therapeutic area of immunology-rheumatology-transplantation / haematology-hemostaseology.

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 1 application was withdrawn during the late stages of the evaluation (30 days or less before opinion).

Interaction with external experts

The PDCO has regular interactions with experts, with a view to bringing state-of-the-art knowledge to the PDCO scientific discussions. One expert in Biostatistics was invited to the June meeting.

Other issues

The PDCO thanked Yvonne Looney (Ireland) for her work, as she has resigned from the Committee.

The PDCO welcomed the new alternate from Ireland, Dr Brian Aylward.

The next meeting of the PDCO will be held on 14-16 July 2010.

– 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/htms/human/paediatrics/decisions.htm
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 'Medicines for children' section of the Agency's website.
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

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Annex of the June 2010 PDCO meeting report

	2008 (January to December)	2009 (January to December)	2010 (January to current month)	Cumulative total (2007 to 2010)
Total number of validated PIP/waiver applications	271	273	228	857[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	186	191	199	615 *(72%)*
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])*	75	72	27	219 *(25%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	10	10	2	23 *(3%)*
PIPs and full waiver indications covered by these applications	395	395	268	1229

Number of Paediatric Committee (PDCO) opinions	2008	2009	2010	Cumulative total
Positive on full waiver	48	67	24	149
Positive on PIP, including potential deferral	81	122	46	251
Negative opinions adopted	4	13	5	22
Positive opinions adopted on modification of a PIP	8	51	58	117
Negative opinions adopted on modification of a PIP	0	0	4	4
Positive opinions on compliance with a PIP	5	8	4	17
Negative opinions on compliance check with a PIP	0	1	0	1

[1] Of which 189 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	2008 (%)	2009 (%)	2009 (%)
Neurology	6	4	4
Uro-nephrology	3	5	1
Gastroenterology-hepatology	3	2	1
Pneumology-allergology	6	6	50
Infectious diseases	8	9	3
Cardiovascular diseases	14	9	9
Diagnostics	1	1	1
Endocrinology-gynaecology-fertility-metabolism	15	16	6
Neonatology-paediatric intensive care	1	2	0
Immunology-rheumatology-transplantation	6	6	4
Psychiatry	3	3	2
Pain	3	6	2
Haematology-haemostaseology	5	6	2
Otorhinolaryngology	1	1	1
Oncology	12	11	6
Dermatology	3	6	2
Vaccines	6	4	2
Ophthalmology	2	2	3
Anaesthesiology	1	1	1
Nutrition	1	0	0