12 February 2010
EMA/PDCO/97062/2010

Meeting highlights from the Paediatric Committee, 17-19 February 2010

Opinions on paediatric investigation plans

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

- **Voriconazole**, from Pfizer Limited, in the therapeutic area of Infectious diseases;
- **Recombinant Coagulation Factor VIII (N8)**, from Novo Nordisk A/S, in the therapeutic area of Haematology-haemostaseology;
- **LysB29(Nε-hexadecandioyl-γ-Glu) des(B30) human insulin**, from Novo Nordisk A/S, in the therapeutic area of Endocrinology-gynaecology-fertility-metabolism;
- **LysB29(Nε-hexadecandioyl-γ-Glu) des(B30) human insulin / insulin aspart**, from Novo Nordisk A/S, in the therapeutic area of Endocrinology-gynaecology-fertility-metabolism;
- **Anidulafungin**, from Pfizer Limited, in the therapeutic area of Infectious diseases;
- **Lisdexamfetamine dimesylate**, from Shire Pharmaceutical Contracts Ltd, in the therapeutic area of Psychiatry;
- **Decitabine**, from Janssen-Cilag International NV, in the therapeutic area of Oncology;
- **Semuloparin sodium**, from Sanofi-aventis Recherche & Développement, in the therapeutic area of Haematology-haemostaseology;
- **(2R, 3aR, 10Z, 11aS, 12aR, 14aR)-N-(cyclopropylsulfonyl)-2-[[2-(4-isopropyl-1, 3-thiazol-2-yl)-7-methoxy-8-methyl-4-quinolinyl]oxy]-5-methyl-4, 14-dioxo-2, 3, 3a, 4, 5, 6, 7, 8, 9, 11a, 12, 13, 14, 14a-tetradecahydrocyclopenta [c] cyclopropa [g] [1, 6] diazacyclo-tetradecine-12a (1H)-carboxamide**, from Tibotec BVBA, in the therapeutic area of Infectious diseases;
- **Plant cell expressed recombinant human glucocerebrosidase**, from Protalix B.V., in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- **Ciclosporin**, from Novagali Pharma S.A., in the therapeutic area of Ophthalmology.

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The PDCO adopted an opinion on the **refusal** of a PIP and deferral, for **polihexanide**, from B.Braun Melsungen AG, in the therapeutic area of Other (Wound care).

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:

- **Budesonide / salmeterol xinafoate**, from Laboratoires SMB s.a., in the therapeutic area of Pneumology-allergology;
- **Tartaric acid**, from Pneumoflex E.U. Limited, in the therapeutic area of Uro-nephrology.

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 6 applications, of which 4 were request of modification to agreed PIPs, were withdrawn during the late stages of the evaluation (30 days or less before opinion).

The PDCO noted that a request for modification of an agreed PIP was withdrawn before the EMEA decision.

New meeting dates adopted

The PDCO meeting dates for 2012 and 2013 were adopted during the February meeting. These dates are important for applicants in planning the submission of applications for PIPs, requests for waivers, requests for modification of an agreed PIP, and requests for compliance checks. The dates will be published on the Agency's website at: http://www.ema.europa.eu/htms/human/paediatrics/pdco.htm

Other issues

The PDCO welcomed the new alternates from Poland and Romania, Dr Jolanta Witkowska-Ożogowska and Dr Roxana Mustata, who have been nominated by The Office for Registration of Medicinal Products, Medical Devices and Biocidal Products in Poland and by the CHMP respectively.

The next meeting of the PDCO will be held on 17-19 March 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 EEA, 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

Enquiries only to: paediatrics@ema.europa.eu

Annex of the February 2009 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	54	683[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	186	191	43	459 *(67%)*
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	10	202 *(30%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	10	10	1	22 *(3%)*
PIPs and full waiver indications covered by these applications	395	395	64	1025

Number of Paediatric Committee (PDCO) opinions	2008	2009	2010	Cumulative total (2007-2010)
Positive on full waiver	48	67	4	129
Positive on PIP, including potential deferral	81	122	16	221
Negative opinions adopted	4	13	3	20
Positive opinions adopted on modification of a PIP	8	51	23	82
Positive opinions on compliance with a PIP	5	8	1	14
Negative opinions on compliance check with a PIP	0	1	0	1

[1] Of which 166 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 (%)	2010 (%)
Neurology	6	4	5
Uro-nephrology	3	5	0
Gastroenterology-hepatology	3	2	2
Pneumology-allergology	6	6	30
Infectious diseases	8	9	7
Cardiovascular diseases	14	9	8
Diagnostics	1	1	0
Endocrinology-gynaecology-fertility-metabolism	15	16	7
Neonatology-paediatric intensive care	1	2	0
Immunology-rheumatology-transplantation	6	6	7
Psychiatry	3	3	3
Pain	3	6	2
Haematology-haemostaseology	5	6	5
Otorhinolaryngology	1	1	2
Oncology	12	11	13
Dermatology	3	6	0
Vaccines	6	4	2
Ophthalmology	2	2	5
Anaesthesiology	1	1	2
Nutrition	1	0	0