28 April 2011
EMA/PDCO/300057/2011

Monthly report

Paediatric Committee (PDCO)

18-20 April 2011

Opinions on paediatric investigation plans

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

- Octocog alfa (recombinant coagulation factor VIII), from Bayer Schering Pharma AG, in the therapeutic area of haematology-hemostaseology;
- Human normal immunoglobulin, from Bio Products Laboratory, in the therapeutic area of immunology-rheumatology-transplantation / haematology-hemostaseology;
- Treosulfan, from medac Gesellschaft für klinische Spezialpräparate mbH, in the therapeutic area of immunology-rheumatology-transplantation / oncology;
- 1-[2-(2,4-dimethyl-phenylsulfanyl)phenyl]piperazine (Lu AA21004), from H. Lundbeck A/S, in the therapeutic area of psychiatry;
- Cobicistat, from Gilead Sciences International Limited, in the therapeutic area of infectious diseases;
- Elvitegravir/cobicistat/emtricitabine/tenofovir disoproxil (as fumarate), from Gilead Sciences International Limited, in the therapeutic area of infectious diseases;
- Perflubutane, from Granzer Regulatory Consulting & Services, in the therapeutic area of diagnostic / cardiovascular diseases;
- Ipilimumab, from Bristol-Myers Squibb International Corporation, in the therapeutic area of oncology;
- Ombrabulin, from Sanofi-Aventis recherche & developpement, in the therapeutic area of oncology;
- (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt (FBS0701), from FerroKin BioSciences, Ltd, in the therapeutic area of haematology-hemostaseology;

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- Recombinant fusion protein consisting of Human Coagulation Factor IX attached to the Fc domain of Human IgG1 (rFIXFc), from Biogen Idec Ltd., in the therapeutic area of haematology-hemostaseology;
- Autologous oral mucosal epithelial cells, from CellSeed Europe S.A.R.L., in the therapeutic area of ophthalmology;
- Edoxaban tosylate, from Daiichi Sankyo Development Limited, in the therapeutic area of cardiovascular diseases.

Adoption of an opinion following re-examination

The PDCO adopted one opinion after re-examination, on a request for modification of an agreed PIP.

A re-examination of the opinion can be requested by the applicant within 30 days following receipt of the opinion of the PDCO. The grounds for the re-examination should be based only on the original information and scientific data provided in the application that were previously available to the PDCO and on which the initial opinion was based. This may include new analysis of the same data or minor protocol amendments to a previously proposed study. Significant changes to the previous plan cannot be part of the re-examination process.

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:

- Canagliflozin/Metformin, from Johnson & Johnson Pharmaceutical Research & Development (J&J PRD), in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Gallium [68 Ga] Chloride / Germanium [68 Ge] Chloride, from Eckert & Ziegler Radiopharma GmbH, in the therapeutic area of other (Radiopharmaceuticals);
- Rivastigmine , from Novartis Europharm Ltd., in the therapeutic area of Neurology.

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 four 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. Three experts were invited to the April meeting, to discuss the potential needs, utility and safety of a new antipsychotic for the treatment of adolescent schizophrenia, and to discuss the potential needs and utility of a new immunoglobulin product for the treatment of paediatric patients with chronic inflammatory demyelinating polyneuropathy.

PDCO interactions

The Chair of the CHMP's Blood Products Working Party (BPWP) attended the meeting of the PDCO in order to enhance the scientific collaboration between the two groups.

Other issues

The PDCO thanked Alexandra Soldatou, former alternate from Greece, for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 18-20 May 2011.

Procedural Announcement

Requirement reminder for all Marketing Authorisation Holders that have been granted a deferral in their agreed Paediatric investigational Plan

The Agency would like to remind the Marketing Authorisation Holders of the obligation to fulfil the requirement of Article 34(4) of Regulation (EC) No 1901/2006 ("Paediatric Regulation"), which states that an **Annual report on deferred measures** should be submitted, for authorised medicinal products if a deferral was granted for the completion of an agreed Paediatric Investigation Plan.

Annual reports should be submitted according to the guidance available on the EMA's website and using the template for annual report on a deferral.

– 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 April 2011 PDCO meeting report

	2009 (January to December)	2010 (January to December)	2011 (January to current month)	Cumulative total (2007 to 2010)
Total number of validated PIP/waiver applications	273	326	66	1022[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	191	280	54	750 *(73%)*
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	11	246 *(24%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	10	4	1	26 *(3%)*
PIPs and full waiver indications covered by these applications	395	403	80	1444

Number of Paediatric Committee (PDCO) opinions	2009	2010	2011	Cumulative total
Positive on full waiver	67	52	17	193
Positive on PIP, including potential deferral	122	201	41	447
Negative opinions adopted	13	7	1	25
Positive opinions adopted on modification of a PIP	51	103	42	204
Negative opinions adopted on modification of a PIP	0	4	0	4
Positive opinions on compliance with a PIP	8	9	0	22
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 240 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	4
Uro-nephrology	5	2	0
Gastroenterology-hepatology	2	1	3
Pneumology-allergology	6	41	3
Infectious diseases	9	4	7
Cardiovascular diseases	9	8	10
Diagnostics	1	1	1
Endocrinology-gynaecology-fertility-metabolism	16	6	11
Neonatology-paediatric intensive care	2	0	2
Immunology-rheumatology-transplantation	6	5	5
Psychiatry	3	1	3
Pain	6	1	0
Haematology-haemostaseology	6	4	7
Otorhinolaryngology	1	3	0
Oncology	11	9	4
Dermatology	6	1	2
Vaccines	4	2	5
Ophthalmology	2	4	3
Anaesthesiology	1	2	0
Nutrition	0	0	0
Other			2

** One PIP can cover several therapeutic areas*