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Paediatric Committee (PDCO)

PDCO monthly report of opinions on paediatric investigation plans and other activities

05-07 September 2012

Opinions on paediatric investigation plans

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

- Human heterologous liver cells, from Cytonet GmbH&Co. KG, in the therapeutic area of gastroenterology-hepatology;
- Certolizumab pegol, from UCB Pharma SA, in the therapeutic area of immunology-rheumatology-transplantation;
- Ceftobiprole medocaril (sodium), from Basilea Pharmaceutica International Ltd., in the therapeutic area of infectious diseases;
- Dermatophagoides pteronyssinus / dermatophagoides farinae, from ALK-Abelló A/S, in the therapeutic area of pneumology - allergology / oto-rhino-laryngology;
- Bitopertin, from Roche Registration Limited, in the therapeutic area of psychiatry.

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.

Adoption of an opinion following re-examination

The PDCO adopted opinions for the following products:

- Following the re-examination of the positive opinion on a modification to an agreed PIP adopted on 6 July 2012 for oseltamivir phosphate, from Roche Registration Ltd, in the therapeutic area of infectious diseases. The PDCO maintained its opinion.

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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:

- Amlodipine (besylate) / candesartan (cilexetil), from Zentiva k.s., in the therapeutic area of cardiovascular diseases;
- Perindopril (tosilate) / amlodipine (besilate), from Teva Pharma B.V., in the therapeutic area of cardiovascular diseases;
- Atorvastatin / ramipril / acetyl salicylic acid, from Ferrer Internacional, S.A., in the therapeutic area of cardiovascular diseases;
- Folic acid, from Endocyte Europe B.V., in the therapeutic area of diagnostic / oncology;
- Etarfolatide, from Endocyte Europe B.V., in the therapeutic area of diagnostic / oncology;
- Fostamatinib, from AstraZeneca AB, in the therapeutic area of immunology-rheumatology-transplantation;
- Dexketoprofen (trometamol) / tramadol (hydrochloride), from Menarini Ricerche SpA, in the therapeutic area of pain.

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. The PDCO adopted positive opinions, agreeing change(s), for the following products:

- Ticagrelor, from AstraZeneca AB, in the therapeutic area of cardiovascular diseases;
- Dabigatran etexilate, from Boehringer Ingelheim International GmbH, in the therapeutic area of cardiovascular diseases / haematology-haemostaseology;
- Tazarotene, from Orfagen, in the therapeutic area of dermatology;
- Tapentadol (hydrochloride) from Grünenthal GmbH, in the therapeutic area of pain;
- Telbivudine, from Novartis Europharma Limited, in the therapeutic area of gastroenterology-hepatology;

- Dapagliflozin, from Bristol Myers Squibb /AstraZeneca EEIG, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Recombinant human N-acetylgalactosamine-6-sulfatase, from BioMarin Europe Limited, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Peginesatide, from Takeda Global Research & Development Centre (Europe) Ltd, in the therapeutic area of haematology-hemostaseology;
- Cobicistat, from Gilead Sciences International Limited, in the therapeutic area of haematology-hemostaseology;
- Delamanid, from Otsuka Frankfurt Research Institute GmbH, in the therapeutic area of haematology-hemostaseology;
- Bevacizumab, from Roche Registration Ltd., in the therapeutic area of oncology;
- Ciclosporin, from Novagali Pharma S.A.S, in the therapeutic area of ophthalmology;
- Influenza Virus Type A, H1N1 / Influenza Virus Type A, H3N2 / Influenza Virus Type B, Yamagata lineage / Influenza Virus Type B, Victoria lineage, from MedImmune Limited, in the therapeutic area of vaccines.

Withdrawals

The PDCO noted that 3 applications, of which two where requests for modification to an agreed PIP were withdrawn during the late stages of the evaluation (30 days or less before opinion).

Other matters

The PDCO welcomed the new alternate from Slovenia, Dr Tadej Avcin, who has been nominated by the Slovenian Ministry of Health.

The next meeting of the PDCO will be held on 03-05 October 2012.

– END –

Notes:

1. 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.

2. 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

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 September 2012 PDCO meeting report

	2010 (January to December)	2011 (January to December)	2012 (January to current month)	Cumulative total (2007 to present)
Total number of validated PIP/waiver applications	326	187	138	1282[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	280	153	114	964 *(75%)*
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])*	43	33	24	292 *(23%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	4	1	0	26 *(2%)*
PIPs and full waiver indications covered by these applications	403	220	165	1749

Number of Paediatric Committee (PDCO) opinions	2010	2011	2012	Cumulative total (2007 to present)
Positive on full waiver	52	45	31	252
Positive on PIP, including potential deferral	201	107	66	579
Negative opinions adopted	7	3	3	30
Positive opinions adopted on modification of a PIP	103	153	125	440
Negative opinions adopted on modification of a PIP	4	2	1	7
Positive opinions on compliance with a PIP	9	9	2	33
Negative opinions on compliance check with a PIP	0	0	0	1
Opinions adopted under Art. 14.2	2	0	0	2

[1] Of which 321 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	2010 *(%)*	2011 *(Number of areas covered)**	2012 *(Number of areas covered)**
Neurology	3	11	6
Uro-nephrology	2	4	4
Gastroenterology-hepatology	1	10	6
Pneumology-allergology	41	10	7
Infectious diseases	4	15	18
Cardiovascular diseases	8	21	25
Diagnostics	1	5	2
Endocrinology-gynaecology-fertility-metabolism	6	28	20
Neonatology-paediatric intensive care	0	0	2
Immunology-rheumatology-transplantation	5	13	10
Psychiatry	1	9	0
Pain	1	2	8
Haematology-haemostaseology	4	18	7
Otorhinolaryngology	3	2	1
Oncology	9	19	16
Dermatology	1	10	12
Vaccines	2	12	2
Ophthalmology	4	8	4
Anaesthesiology	2	1	2
Nutrition	0	0	0
Other		7	9

** One PIP can cover several therapeutic areas*