EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Paediatric Committee (PDCO)

PDCO monthly report of opinions on paediatric investigation plans

11-13 January 2012

Opinions on paediatric investigation plans

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

- Eliglustat (tartrate), from Genzyme Europe B.V., in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13, from Baxter Innovations GmbH, in the therapeutic area of haematology-hemostaseology;
- 2-Iminobiotin, from Neurophyxia B.V., in the therapeutic area of neonatology - paediatric intensive care;
- N-[3-[3-cyclopropyl-5-[(2-fluoro-4-iodophenyl)amino]- 6,8-dimethyl-2,4,7-trioxo-3,4,6,7-tetrahydropyrido[4,3-D]pyrimidin-1(2H)-yl]phenyl]acetamide, dimethylsulfoxide solvate, from GlaxoSmithKline Trading Service Limited, in the therapeutic area of oncology;
- Elacytarabine, from Clavis Pharma ASA, in the therapeutic area of oncology;
- Clostridium Botulinum neurotoxin type A (150 kD), free of complexing proteins, from Merz Pharmaceuticals GmbH, in the therapeutic area of ophthalmology / dermatology / neurology;
- Fibrinogen concentrate / thrombin preparation / aprotinin / calcium chloride, from Kedrion S.p.A., in the therapeutic area of other (surgical settings).

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.

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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 PIP adopted in 11 November 2011 for semuloparin sodium, from Sanofi-aventis Recherche & Développement, in the therapeutic area of haematology-hemostaseology, the PDCO adopted a revised positive opinion.

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:

- Diltiazem (hydrochloride), from S.L.A. Pharma (UK) Limited, in the therapeutic area of gastroenterology-hepatology;
- Recombinant Porcine Factor VIII, B-Domain Deleted, from Inspiration Biopharmaceuticals EU, Ltd., in the therapeutic area of 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 5 applications were withdrawn during the late stages of the evaluation (30 days or less before opinion).

The PDCO also noted that an opinion adopted during the December PDCO meeting for bortezomib, from Janssen-Cilag International NV, in the therapeutic area of oncology, has been withdrawn before the decision was adopted by the Agency.

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. One expert was invited to the February meeting with a clinical expertise in paediatric radiology and nuclear medicine, the PDCO discussed the paediatric

development of two radiopharmaceutical products for the diagnostics of myocardial perfusion disturbances.

Other issues

Update of the priority list for studies into off-patent paediatric medicinal products

The PDCO adopted the revision of the priority list for studies into off-patent paediatric medicinal products in advance of the next call from the European Community through the EU's Seventh Framework Programme.

The updated list comprises a number of medicines relating to various therapeutic areas. This will be a reference for applicants for funding from the European Community (7th Framework Programme).

The European Commission, represented by Dr Alexandru-Sorin Costescu (DG RTD), updated the PDCO on the Seventh Framework Programme.

Organisational matters

The PDCO thanked Lida Kalantzi for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 08-10 February 2012.

– 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 January 2012 PDCO meeting report

	2010 (January to December)	2011 (January to December)	2012 (January to current month)	Cumulative total (2007 to 2012)
Total number of validated PIP/waiver applications	326	187	17	1161[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	280	153	15	865 *(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	2	270 *(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	17	1601

Number of Paediatric Committee (PDCO) opinions	2010	2011	2012	Cumulative total
Positive on full waiver	52	45	2	223
Positive on PIP, including potential deferral	201	107	7	520
Negative opinions adopted	7	3	0	27
Positive opinions adopted on modification of a PIP	103	153	11	326
Negative opinions adopted on modification of a PIP	4	2	0	6
Positive opinions on compliance with a PIP	9	9	0	31
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 274 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	0
Uro-nephrology	2	4	0
Gastroenterology-hepatology	1	10	1
Pneumology-allergology	41	10	4
Infectious diseases	4	15	2
Cardiovascular diseases	8	21	2
Diagnostics	1	5	0
Endocrinology-gynaecology-fertility-metabolism	6	28	3
Neonatology-paediatric intensive care	0	0	0
Immunology-rheumatology-transplantation	5	13	2
Psychiatry	1	9	0
Pain	1	2	0
Haematology-haemostaseology	4	18	1
Otorhinolaryngology	3	2	1
Oncology	9	19	2
Dermatology	1	10	1
Vaccines	2	12	0
Ophthalmology	4	8	0
Anaesthesiology	2	1	0
Nutrition	0	0	0
Other		7	2

** One PIP can cover several therapeutic areas*