16 February 2012
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Paediatric Committee (PDCO)

PDCO monthly report of opinions on paediatric investigation plans

08-10 February 2012

Opinions on paediatric investigation plans

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

- Rubidium (82Rb) chloride, from Advanced Accelerator Applications, in the therapeutic area of diagnostics;
- Rubidium (82Rb) chloride, from Jubilant DraxImage Inc, in the therapeutic area of diagnostics;
- ALX-0081, anti-von Willebrand Factor Nanobody, from Ablynx NV, in the therapeutic area of haematology-hemostaseology;
- Ustekinumab, from Janssen-Cilag International NV, in the therapeutic area of immunology-rheumatology-transplantation;
- Hepatitis B (rDNA) surface antigen (adjuvanted), from Dynavax International BV, in the therapeutic area of vaccines;
- Modified Vaccinia Ankara - Bavarian Nordic virus (smallpox), from Bavarian Nordic A/S, in the therapeutic area of vaccines;
- Nitisinone, from Swedish Orphan Biovitrum International AB, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Anakinra, from Swedish Orphan Biovitrum AB, in the therapeutic area of immunology-rheumatology-transplantation.

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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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:

- Deoxycholic acid, from Intendis GmbH, in the therapeutic area of dermatology.

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 2 applications were withdrawn during the late stages of the evaluation (30 days or less before opinion).

The PDCO welcomed the alternate from Norway, Dr Ine Blankenberg Skottheim, who has been nominated by the Norwegian Medicines Agency.

The next meeting of the PDCO will be held on 07-09 March 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 February PDCO meeting report

	2010 (January to December)	2011 (January to current month)	2012 (January to December)	Cumulative total (2007 to 2011)
Total number of validated PIP/waiver applications	326	187	28	1172[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	280	153	23	873 *(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	5	273 *(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	28	1612

Number of Paediatric Committee (PDCO) opinions	2010	2011	2012	Cumulative total
Positive on full waiver	52	45	3	224
Positive on PIP, including potential deferral	201	107	15	528
Negative opinions adopted	7	3	0	27
Positive opinions adopted on modification of a PIP	103	153	21	336
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 279 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	1
Uro-nephrology	2	4	0
Gastroenterology-hepatology	1	10	1
Pneumology-allergology	41	10	4
Infectious diseases	4	15	2
Cardiovascular diseases	8	21	5
Diagnostics	1	5	0
Endocrinology-gynaecology-fertility-metabolism	6	28	5
Neonatology-paediatric intensive care	0	0	0
Immunology-rheumatology-transplantation	5	13	4
Psychiatry	1	9	0
Pain	1	2	0
Haematology-haemostaseology	4	18	1
Otorhinolaryngology	3	2	1
Oncology	9	19	3
Dermatology	1	10	3
Vaccines	2	12	1
Ophthalmology	4	8	0
Anaesthesiology	2	1	0
Nutrition	0	0	0
Other		7	2

* *One PIP can cover several therapeutic areas*