20 July 2011
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**Monthly report**

# Paediatric Committee (PDCO)

15-17 June 2011

## Opinions on paediatric investigation plans

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

- Gadobutrol, from Bayer Schering Pharma AG, in the therapeutic area of diagnostics;

The PDCO adopted an opinion on the **refusal** of a PIP, including deferral, for rubidium Rb-82 Chloride, from DRAXIMAGE, a division of DRAXIS Specialty Pharmaceuticals Inc., in the therapeutic area of diagnostics;

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:

- Valsartan / amlodipine besilate, from Gedeon Richter Plc., in the therapeutic area of cardiovascular diseases;
- Amlodipine besylate / perindopril erbumine, from KBM Pharma OÜ, in the therapeutic area of cardiovascular diseases;
- Florbetaben, from Bayer Schering Pharma AG, in the therapeutic area of diagnostic;

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- Vitamin D3 / strontium ranelate, from Les Laboratoires Servier, in the therapeutic area of Immunology-rheumatology-transplantation;
- Teriparatide, from Eli Lilly & Company Limited, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Recombinant salmon calcitonin, from Novartis Europharm Ltd, in the therapeutic area of endocrinology-gynaecology;
- Aflibercept, from Bayer Schering Pharma AG, in the therapeutic area of ophthalmology.

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.

## Opinion on compliance check

The PDCO adopted a positive opinion on compliance check for azelastine hydrochloride / fluticasone propionate, from MEDA Pharma GmbH & Co. KG, in the therapeutic area of pneumology-allergology.

A compliance check is performed to verify that all the measures agreed in a PIP and reflected in the Agency's decision have been conducted in accordance with the decision, including the agreed timelines. Compliance is one of several prerequisites for obtaining the rewards and incentives provided for in Articles 36 to 38 of the Paediatric Regulation.

Before the submission of a request for a compliance check, applicants are encouraged to consult the Agency's Procedural advice for validation of a new marketing authorisation application or extension/variation application and compliance check with an agreed PIP.

## Withdrawals

The PDCO noted that 3 applications, of which one was a request for a modification to an agreed PIP, 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 June meeting with clinical expertise in paediatric nuclear imaging and Gaucher disease. The PDCO discussed the potential needs, utility and safety of a radionuclide generator agent, and the possibility of novel and cooperative study designs for the treatment of Gaucher disease.

## Informal meeting

On 31 May-1 June 2011, the PDCO held an informal meeting Budapest to review the work done and the processes put in place during its fith year. The PDCO discussed improvements in the functioning of

the PDCO, in particular timelines, summary reports, interactions with experts, learned societies and industry, and priorities in the implementation of the Paediatric Regulation.

**Other issues**

The PDCO thanked Ilze Barene (Latvia) for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 13-15 July 2011.

**– 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

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# Annex of the June PDCO meeting report

| | 2009 (January to December) | 2010 (January to December) | 2011 (January to current month) | Cumulative total (2007 to 2011) |
|---|---|---|---|---|
| Total number of validated PIP/waiver applications | 273 | 326 | 99 | 1056[1] |
| Applications submitted for a product not yet authorised *(Article 7[2])* | 191 | 280 | 78 | 775 *(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 | 20 | 255 *(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 | 117 | 1481 |

| Number of Paediatric Committee (PDCO) opinions | 2009 | 2010 | 2011 | Cumulative total |
|---|---|---|---|---|
| Positive on full waiver | 67 | 52 | 28 | 204 |
| Positive on PIP, including potential deferral | 122 | 201 | 56 | 462 |
| Negative opinions adopted | 13 | 7 | 2 | 26 |
| Positive opinions adopted on modification of a PIP | 51 | 103 | **66** | 228 |
| Negative opinions adopted on modification of a PIP | 0 | 4 | 0 | 4 |
| Positive opinions on compliance with a PIP | 8 | 9 | 2 | 24 |
| 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 250 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 | 6 |
| Uro-nephrology | 5 | 2 | 1 |
| Gastroenterology-hepatology | 2 | 1 | 6 |
| Pneumology-allergology | 6 | 41 | 3 |
| Infectious diseases | 9 | 4 | 8 |
| Cardiovascular diseases | 9 | 8 | 13 |
| Diagnostics | 1 | 1 | 3 |
| Endocrinology-gynaecology-fertility-metabolism | 16 | 6 | 17 |
| Neonatology-paediatric intensive care | 2 | 0 | 3 |
| Immunology-rheumatology-transplantation | 6 | 5 | 7 |
| Psychiatry | 3 | 1 | 4 |
| Pain | 6 | 1 | 0 |
| Haematology-haemostaseology | 6 | 4 | 10 |
| Otorhinolaryngology | 1 | 3 | 0 |
| Oncology | 11 | 9 | 9 |
| Dermatology | 6 | 1 | 2 |
| Vaccines | 4 | 2 | 6 |
| Ophthalmology | 2 | 4 | 4 |
| Anaesthesiology | 1 | 2 | 0 |
| Nutrition | 0 | 0 | 0 |
| Other | | | 4 |

** One PIP can cover several therapeutic areas*