European Medicines Agency

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PRESS RELEASE

Meeting highlights from the Paediatric Committee, 11-13 November 2009

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

- **Benzamide, 4-[4-[[2-(4-chlorophenyl)-5,5-dimethyl-1-cyclohexen-1-yl]methyl]-1-piperazinyl]-N-[[4-[[(1R)-3-(4-morpholinyl)-1-[(phenylthio)methyl] propyl]amino]-3-[(trifluoromethyl)sulfonyl]phenyl] sulfonyl]-hydrochloride(1:2) (ABT-263)**, from Abbott Laboratories, in the therapeutic area of oncology;
- **Bevacizumab**, from Roche Registration Ltd, in the therapeutic area of oncology;
- **Belimumab**, from Glaxo Group Limited, in the therapeutic area of immunology–rheumatology-transplantation;
- **Recombinant human monoclonal antibody to the p40 subunit of human interleukin-12 and human interleukin-23 of the IgG1-class (ABT-874)**, from Abbott Laboratories Ltd, in the therapeutic area of dermatology;
- **Velaglucerase alfa**, from Shire Pharmaceuticals Ireland Limited, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- **Fampridine**, from Acorda Therapeutics, Inc., in the therapeutic area of neurology;
- **Aliskiren**, from Novartis Europharm Ltd., in the therapeutic area of cardiovascular diseases.
- **Ferumoxytol**, from AMAG Pharmaceuticals, Inc., in the therapeutic area of haematology-haemostaseology.

- The PDCO adopted an opinion on the **refusal** of a PIP and a positive opinion for a product-specific waiver for one paediatric subset on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients for **Allogeneic ex vivo expanded umbilical cord blood cells**, from Teva Pharma GmbH, in the therapeutic area of haematology-haemostaseology / oncology. The PDCO subsequently granted on its own motion a product-specific waiver for this medicine for all other subsets of the paediatric population in the specified conditions, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients;
- The PDCO adopted an opinion on the **refusal** of a PIP, including waiver and deferral, for **fentanyl citrate**, from Nycomed Danmark ApS, in the therapeutic area of pain.

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 (EMEA), 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.

* Name of the company for Ferumoxytol has been corrected

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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:

- **Paracetamol / ibuprofen**, from Vale Pharmaceuticals Limited, in the therapeutic area of pain;
- **Tramadol hydrochloride / paracetamol**, from Labopharm Europe Limited, in the therapeutic area of pain;
- **Amlodipine besilate / bisoprolol fumarate**, from EGIS Pharmaceuticals PLC, in the therapeutic area of cardiovascular diseases;
- **Nitric oxide**, from INO Therapeutics AB, in the therapeutic area of cardiovascular diseases.

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 **Atorvastatin calcium** from Pfizer Limited, in the therapeutic area of metabolism.

A compliance check is performed to verify that all the measures agreed in a PIP and reflected in the EMEA 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 EMEA 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 **5** 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. Four experts were invited to the November meeting:

— Two clinical experts in child and adolescent psychiatry, the PDCO discussed the development of medicinal products for the treatment of reading disorder;
— One expert in paediatric gastroenterology, the PDCO discussed development of medicinal products for children with inflammatory bowel disease; and
— One expert in haematology and oncology, the PDCO discussed development of medicines for children with haematological malignancies.

Other issues
The PDCO thanked **Margarita Guizova,** the Bulgarian delegate, for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 9-11 December 2009.

– END –

Notes:

1. PDCO opinions on PIPs and waivers are transformed into EMEA decisions within the timeframe laid down by the Paediatric Regulation (Regulation (EC) No 1901/2006, as amended). The decisions can be found on the EMEA website at: http://www.emea.europa.eu/htms/human/paediatrics/decisions.htm
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 EEA, have to provide either the results of studies in children conducted in accordance with an approved PIP, or an EMEA decision on a waiver or on a deferral.
3. More information about the PDCO and the Paediatric Regulation is available in the 'Medicines for children' section of the EMEA website.
4. This meeting report, together with other information on the work of the EMEA, can be found on the EMEA website: http://www.emea.europa.eu

Enquiries only to: paediatrics@emea.europa.eu

Annex of the November 2009 PDCO meeting report

	2007 (August to December)	**2008** (January to December)	**2009** (January to current month)	**Cumulative total**
Total number of validated PIP/waiver applications	**85**	**271**	**258**	**614**[1]
Applications submitted for a product not yet authorised (Article 7[2])	39	186	179	404 (66%)
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])	45	75	69	189 (31%)
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation (Article 30[2])	1	10	10	21 (3%)
PIPs and full waiver indications covered by these applications	**202**	**395**	**349**	**946**

Number of Paediatric Committee (PDCO) opinions	**2007**	**2008**	**2009**	**Cumulative total**
Positive on full waiver	10	48	62	120
Positive on PIP, including potential deferral	2	81	118	201
Negative opinions adopted	0	4	13	17
Positive opinions adopted on modification of a PIP	0	8	43	51
Positive opinions on compliance with a PIP	0	5	8	13
Negative opinions on compliance check with a PIP	0	0	1	1

[1] Of which 153 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	**2007** (%)	**2008** (%)	**2009** (%)
Neurology	12	6	4
Uro-nephrology	-	3	5
Gastroenterology-hepatology	9	3	5
Pneumology-allergology	8	6	6
Infectious diseases	12	8	8
Cardiovascular diseases	12	14	8
Diagnostics	-	1	1
Endocrinology-gynaecology-fertility-metabolism	19	15	16
Neonatology-paediatric intensive care	-	1	2
Immunology-rheumatology-transplantation	5	6	6
Psychiatry	5	3	3
Pain	1	3	6
Haematology-haemostaseology	1	5	5
Otorhinolaryngology	-	1	1
Oncology	11	12	11
Dermatology	1	3	6
Vaccines	2	6	4
Ophthalmology	1	2	2
Anaesthesiology	-	1	1
Nutrition	1	1	0