European Medicines Agency
Press office

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PRESS RELEASE
Meeting highlights from the Paediatric Committee, 29-31 July 2008

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

- **Dabigatran etexilate,** from Boehringer Ingelheim International GmbH, in the therapeutic area of haematology and haemostaseology;
- **Thrombin alfa** (recombinant), from Bayer HealthCare AG, in the therapeutic area of haematology and haemostaseology;
- **Pramipexole dihydrochloride monohydrate,** from Boehringer Ingelheim International GmbH, in the therapeutic area of neurology;
- **Retigabine** from Valeant Pharmaceuticals Ltd, in the therapeutic area of neurology.

The PDCO adopted negative opinions for PIPs for **ezetimibe and simvastatin**, from Merck Sharp & Dohme, and for **nicotinic acid** (in an extended release form), **simvastatin** and **laropiprant,** from Merck Sharp & Dohme (Europe) Limited, both in the therapeutic area of endocrinology and metabolism. The PDCO adopted subsequently on its own motion positive opinions on full waivers for these medicines in all subsets of the paediatric population, on the grounds that these products do not represent a significant therapeutic benefit over existing treatments for the paediatric population.

A paediatric investigation plan (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. In some cases, a PIP may include a waiver to study one or more age groups of children, or a deferral when it is appropriate to conduct studies in adults prior to initiating studies in the paediatric population, or when studies in the paediatric population would take longer to conduct than studies in adults.

Opinions on product-specific waivers
The PDCO adopted an opinion for a product-specific waiver, recommending that the obligation to submit data obtained through clinical studies with children be waived in all subsets of the paediatric population, for **bortezomib,** from Janssen-Cilag International, in the therapeutic area of oncology.

Waivers can be issued if there is evidence showing that the medicinal product 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 specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The PDCO discussed the applicability of class waivers for products intended to treat conditions included in the list of class waivers. Companies developing a product for a condition included in the list of class waivers can request confirmation of whether the scope of the EMEA decision on a class

* The number of positive opinions on full waivers and positive opinions on PIPs including potential deferrals has been corrected in the annex.

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waiver for a condition is applicable to their product. Upon review by the PDCO, companies will receive an outcome letter confirming whether or not their product is considered to fall under the scope of the EMEA Decision on class waivers.

Compliance check with PIPs

The PDCO endorsed a document which describes the EMEA compliance check with an agreed PIP, which is a prerequisite for the validation of new marketing authorisation applications and applications for extensions or variations of indications. In addition to the Paediatric Committee, the EMEA has liaised with the EMEA's Committee for Medicinal Products for Human Use (CHMP) and the Co-ordination Group for Mutual Recognition & Decentralised Procedures for Human Medicines for the drafting of this document.

As of 26 July 2008, pharmaceutical companies who submit an application for a marketing authorisation for a medicine 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. This will apply from 26 January 2009 for medicines that are already authorised and for which a company is submitting an application for an extension of indication.

At the request of the Committee for Medicinal Products for Human Use, a competent authority or the applicant, the PDCO will assess and formulate an opinion on the compliance of the application for marketing authorisation with the agreed paediatric investigation plan concerned, i.e. assess whether all measures agreed in a PIP have been carried out in accordance with the EMEA decision on the PIP. Compliance is one of several prerequisites for obtaining the rewards and incentives provided for by the Paediatric Regulation.

Cooperation with US-FDA

The PDCO welcomed the attendance at the meeting of a representative of the US Food and Drug Administration (FDA) in the framework of the Principles of Interaction between EMEA and FDA Pediatric Therapeutics. According to the terms of these principles, EMEA staff may attend the FDA's Pediatric Implementation Team meetings and FDA staff may attend the EMEA's Paediatric Committee meetings to enable regulators from either agency to observe operational activities, and to optimise mechanisms and timing of information exchanges.

The objectives of the cooperation between the EMEA and FDA in the field of paediatric medicines are to facilitate the framework for global paediatric development plans, compatible for both agencies, with the aim of avoiding exposing children to unnecessary trials.

The next meeting of the PDCO will be held on 27-29 August 2008.

-- ENDS --

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: http://www.emea.europa.eu/htms/human/paediatrics/decisions.htm
2. The Principles of Interaction between EMEA and FDA Pediatric Therapeutics can be found on the EMEA website: http://www.emea.europa.eu/pdfs/general/direct/pr/interactions.pdf
3. The document on procedural advice for validation of new marketing authorisation applications for extensions/variations and compliance check with an agreed PIP is published in the Medicines for children section of the EMEA website.
4. More information about the PDCO and the Paediatric Regulation is available in the 'Medicines for children' section of the EMEA website.
5. This press release, together with other information on the work of the EMEA, can be found on the EMEA website: http://www.emea.europa.eu

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OVERVIEW OF PAEDIATRIC INVESTIGATION PLAN/WAIVER APPLICATIONS

	2007 (August to December)	**2008** (January-end July)	**Cumulative Total**
Total number of validated PIP / waiver applications	**85**	**174**[1]	**259**[2]
Applications submitted for a product not yet authorised *(Article 7)*[3]	39	122	161 *(62%)*
Applications submitted for a product already authorised still under patent in view of a submission of a variation/extension for a new indication, pharmaceutical form or route of administration *(Article 8)*	45	47	92 (*36%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30)*	1	5	6 *(2%)*
PIPs and full waiver indications covered by these applications	**202**	**259**	**461**

Number of Paediatric Committee (PDCO) opinions	**2007**	**2008**	**Total**
Positive on full waiver	10	25	35
Positive on PIPs including potential deferral	2	43	45
Negative Opinions adopted	0	1	1
Positive Opinions adopted on Modification of the PIP	0	1	1
Positive opinion on Compliance with PIP	0	1	1

[1] Figures including 31 July 2008 start of procedure; the figure does not include products which are currently under validation
[2] Of which 57 are requests for full waiver
[3] Applications submitted in accordance with Regulation (EC) No 1901/2006, as amended.

Areas covered by PIPs/waiver applications:	**2007 %**	**2008 %**
Neurology	12	3
Uro-nephrology	-	4
Gastroenterology-hepatology	9	3
Pneumology-allergology	8	4
Infectious diseases	12	7
Cardiovascular diseases	12	12
Diagnostics	-	2
Endocrinology-gynaecology-fertility-metabolism	19	19
Neonatology-paediatric intensive care	-	-
Immunology-rheumatology-transplantation	5	5
Psychiatry	5	4
Pain	1	3
Haematology-haemostaseology	1	6
Otorhinolaryngology	-	-
Oncology	11	14
Dermatology	1	2
Vaccines	2	8
Ophthalmology	1	2
Anaesthesiology	-	1
Nutrition	1	1