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PRESS RELEASE
Meeting highlights from the Paediatric Committee, 12-14 November 2008

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

- **Fingolimod hydrochloride**, from Novartis Europharm Limited, in the therapeutic area of neurology;
- **Treprostinil**, from United Therapeutics Europe Ltd, in the therapeutic area of cardiovascular diseases;
- **Telaprevir**, from Tibotec bvba, in the therapeutic area of infectious diseases;
- **Cyclo {{(E,Z)-(2S,3R,4R)-3-hydroxy-4-methyl-2-(methylamino)nona-6,8-dienoyl}-L-2-aminobutyryl-N-methyl-glycyl-N-methyl-L-leucyl-L-valyl-N-methyl-L-leucyl-L-alanyl-D-alanyl-N-methyl-L-leucyl-N-methyl-L-leucyl-N-methyl-L-valyl},** from Lux Bioscience GmbH, in the therapeutic area of ophthalmology.

The PDCO adopted an opinion on the refusal of a PIP and deferral for **travoprost / brinzolamide**, from Alcon Laboratories (UK) Ltd, in the therapeutic area of ophthalmology. The PDCO granted, subsequently, a product-specific waiver for this medicine for all subsets of the paediatric population in the specified condition, on the grounds that this medicine does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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 EMEA as part of an application for a marketing authorisation for new medicines or products covered by a patent. 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.

Adoption of a revised opinion
The PDCO adopted revised opinions for the following products:

- Following the re-examination of the positive opinion on a PIP adopted in August 2008 for **liraglutide**, from Novo Nordisk A/S, in the therapeutic area of endocrinology, metabolism, fertility and gynaecology, the PDCO adopted a revised positive opinion.
- Following the re-examination of the negative opinion for a full waiver adopted in September 2008 for **perflubutane**, from Nycomed Danmark ApS, in the therapeutic area of cardiovascular diseases, the PDCO maintained its opinion and adopted a negative opinion for a waiver in all subsets of the paediatric population on the basis that the condition does not meet the grounds for a waiver.
- Following the re-examination of the positive opinion on a PIP adopted in September 2008 for **skimmed cow's milk powder**, from DBV Technologies, in the therapeutic area of diagnostic & other, the PDCO agreed the PIP and granted a waiver for one or more subsets of the

paediatric population on the grounds that the specific medicine is likely to be ineffective or unsafe in part or all of the paediatric population.

- Following the re-examination of the positive opinion on a PIP adopted in August 2008 for **vicriviroc maleate**, from Schering-Plough Europe, in the therapeutic area of infectious diseases, the PDCO agreed the PIP and granted a deferral.
- Following the re-examination of the positive opinion on a PIP adopted in August 2008 for **diphtheria, tetanus, acellular pertussis, haemophilus B, and poliomyelitis vaccine**, from Sanofi Pasteur MSD SNC, in the therapeutic area of vaccines, the PDCO agreed the PIP and granted a waiver for one or more subsets of the paediatric population on the grounds that the specific medicine does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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 which were previously available to the PDCO and on which the initial opinion is 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:

- **Eprotirome**, from KaroBio AB, in the therapeutic area of endocrinology, metabolism;
- **Pirfenidone,** from InterMune Inc., in the therapeutic area of pneumology.

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

Non-clinical working group

A working group for questions on non-clinical development, with particular focus on juvenile animal studies, has been set up with experts who will discuss the non-clinical part of selected PIPs. The first meeting was held in November and will be followed by regular monthly teleconferences. The working group will report to the PDCO.

The PDCO regularly interacts and cooperates with experts in specific scientific or technical fields, to keep abreast of recent developments and be able to apply state-of-the art knowledge in its scientific assessments.

The next meeting of the PDCO will be held on 10-12 December 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 at: http://www.emea.europa.eu/htms/human/paediatrics/decisions.htm
2. 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, also for medicines that are already authorised, and for which a company is submitting an application for an extension of indication, or for a new route of administration, or for a new pharmaceutical form.

3. More information about the PDCO and the Paediatric Regulation is available in the 'Medicines for children' section of the EMEA website.
4. 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 to November)	**Cumulative Total**
Total number of validated PIP / waiver applications	**85**	**242**[1]	**327**[2]
Applications submitted for a product not yet authorised *(Article 7*[3]*)*	39	167	206 *(63%)*
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*[3]*)*	45	67	112 (*34%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30*[3]*)*	1	8	9 *(3%)*
PIPs and full waiver indications covered by these applications	**202**	**358**	**560**

Number of Paediatric Committee (PDCO) opinions	**2007**	**2008**	**Cumulative Total**
Positive on full waiver	10	40	50
Positive on PIPs including potential deferral	2	74	76
Negative opinions adopted	0	2	2
Positive opinions adopted on modification of the PIP	0	5	5
Positive opinion on compliance with PIP	0	4	4

[1] Figures include procedures that started on 13 November 2008.
[2] Of which 75 are requests for full waiver.
[3] Applications submitted in accordance with the cited article of Regulation (EC) No 1901/2006, as amended.

Areas covered by PIPs/waiver applications	2007 %	2008 %
Neurology	12	5
Uro-nephrology	-	3
Gastroenterology-hepatology	9	3
Pneumology-allergology	8	5
Infectious diseases	12	8
Cardiovascular diseases	12	13
Diagnostics	-	1
Endocrinology-gynaecology-fertility-metabolism	19	17
Neonatology-paediatric intensive care	-	1
Immunology-rheumatology-transplantation	5	6
Psychiatry	5	3
Pain	1	3
Haematology-haemostaseology	1	5
Otorhinolaryngology	-	-
Oncology	11	13
Dermatology	1	3
Vaccines	2	7
Ophthalmology	1	2
Anaesthesiology	-	1
Nutrition	1	1