European Medicines Agency

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Meeting highlights from the Paediatric Committee, 24-26 June 2009

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

- **Human coagulation Factor VIII/von Willebrand Factor complex concentrate**, from CSL Behring, in the therapeutic area of Haematology-Hemostaseology;
- **Midazolam hydrochloride**, from Auralis Limited, in the therapeutic area of Neurology;
- **Recombinant human monoclonal antibody to human interleukin-17A of the IgG1/kappa-class**, from Novartis Europharm Ltd, in the therapeutic area of Dermatology;
- **Human immunoglobulin**, from Orfagen, in the therapeutic area of Dermatology;
- **A/Viet Nam/1194/2004 (H5N1) virus surface inactivated antigen**, from Novartis Vaccines and Diagnostics S.r.l., in the therapeutic area of Vaccines.

The PDCO adopted an opinion on the **refusal** of a PIP, for **live bacterium bacteroides thetaiotaomicron**, from GT Biologics, in the therapeutic area of gastroenterology-hepatology.

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.

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:

- **5-Aminolevulinic acid, hydrochloride**, from Biofrontera Bioscience GmbH, in the therapeutic area of Dermatology;
- **Desvenlafaxine succinate monohydrate**, from Laboratorios Almirall S.A., in the therapeutic area of Psychiatry;
- **Patupilone**, from Novartis Europharm Ltd, in the therapeutic area of Oncology;
- **Dirucotide acetate**, from Eli Lilly and Company Limited, in the therapeutic area of Neurology;
- **Testosterone**, from Procter & Gamble Pharmaceuticals UK Ltd, in the therapeutic area of Endocrinology-Gynaecology-Fertility-Metabolism;
- **Mifepristone / misoprostol**, from Sun Pharmaceutical Industries Europe B.V., in the therapeutic area of Endocrinology-Gynaecology-Fertility-Metabolism.

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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 7 applications were withdrawn during the late stages of the evaluation (30 days or less before opinion), and 1 application was withdrawn during the re-examination phase.

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. One expert was invited to the June meeting. With a clinical expert in paediatric pulmonary arterial hypertension, the PDCO discussed current medical practices and possible study design in persistent pulmonary hypertension of the newborns.

Cooperation with FDA

At the June meeting, the PDCO welcomed a representative from the Office of Paediatric Therapeutics of the US Food and Drug Administration (FDA), who attended within the framework of the 'Principles of interaction between EMEA and FDA paediatric 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 objective of the cooperation between the EMEA and FDA in the field of paediatric medicines is to facilitate the framework for global paediatric development plans, compatible for both agencies, with the aim of avoiding exposing children to unnecessary trials.

Informal meeting

On 23 June 2009, the PDCO held an informal meeting at EMEA to review the work done and the processes put in place during its third year. The PDCO discussed improvements in the functioning of the Committee, 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 adopted, together with the CHMP, the Guideline on investigation of medicinal products in the term and pre-term neonate. A high number of comments received during the public consultation phase had been considered. The guideline addresses makes specific recommendations for the neonate subset of the paediatric population, who has high unmet therapeutic needs. The guideline should contribute to scientifically sound and feasible developments of medicines for preterm and term neonates, taking into consideration the vulnerability of this subset.

The next meeting of the PDCO will be held on 22-24 July 2009.

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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

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Annex of the 24-26 June 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**	**128**	**484**[1]
Applications submitted for a product not yet authorised *(Article 7*[2]*)*	39	186	84	309 *(64%)*
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	37	157 *(32%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30*[2]*)*	1	10	7	18 *(4%)*
PIPs and full waiver indications covered by these applications	**202**	**395**	**179**	**776**

Number of Paediatric Committee (PDCO) opinions	**2007**	**2008**	**2009**	**Cumulative total**
Positive on full waiver	10	48	41	99
Positive on PIP, including potential deferral	2	81	78	161
Negative opinions adopted	0	4	9	13
Positive opinions adopted on modification of a PIP	0	8	15	23
Positive opinions on compliance with a PIP	0	5	3	8

[1] Of which 114 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	2
Uro-nephrology	-	3	5
Gastroenterology-hepatology	9	3	2
Pneumology-allergology	8	6	5
Infectious diseases	12	8	8
Cardiovascular diseases	12	14	8
Diagnostics	-	1	1
Endocrinology-gynaecology-fertility-metabolism	19	15	22
Neonatology-paediatric intensive care	-	1	1
Immunology-rheumatology-transplantation	5	6	8
Psychiatry	5	3	2
Pain	1	3	5
Haematology-haemostaseology	1	5	5
Otorhinolaryngology	-	1	0
Oncology	11	12	14
Dermatology	1	3	5
Vaccines	2	6	4
Ophthalmology	1	2	3
Anaesthesiology	-	1	0
Nutrition	1	1	0