European Medicines Agency

London, 31 July 2009
Doc. Ref..: EMEA/PDCO/449592/2009

Meeting highlights from the Paediatric Committee, 22-24 July 2009

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

- N. meningitidis serogroup A polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup C polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup W polysaccharide conjugated to tetanus toxoid / N. meningitidis serogroup Y polysaccharide conjugated to tetanus toxoid, from GlaxoSmithKline Biologicals s.a, in the therapeutic area of vaccines;
- A derivative of Sodium X-5-Hydroxy-X-6,10-dioxo-3,4,6,9,9a,10-hexahydro-2H-1-oxa-4a,8a-diaza-anthracene-7-carboxylic acid-X-benzylamide, from GlaxoSmithKline Trading Services Ltd., in the therapeutic area of infectious diseases;
- Human normal immunoglobulin, from Kedrion S.p.A., in the therapeutic area of immunology-rheumatology-transplantation;
- Motavizumab, from Abbott Laboratories Limited, in the therapeutic area neonatology - paediatric intensive care;
- 4-[(5R)-6,7-Dihydro-5H-pyrrolo[1,2-c]imidazol-5-yl]-3-flurorbenzonitrile (LCI699), from Novartis Europharm Ltd., in the therapeutic area of cardiovascular diseases;
- Cinacalcet, from Amgen Europe B.V., in the therapeutic area of uro-nephrology;
- Saxagliptin, from Bristol-Myers Squibb/AstraZeneca EEIG, in the therapeutic area of Endocrinology-Gynaecology-Fertility-Metabolism;
- Human normal immunoglobulin, from LFB Biotechnologies, in the therapeutic area of Immunology–rheumatology-transplantation;
- Purified Diphteria Toxoid / Purified Tetanus Toxoid / Purified Pertussis Toxoid (PT) / Purified Filamentous Haemagglutinin (FHA) / Purified Fimbriae Types 2 and 3 (FIM) / Purified Pertactin (PRN) / Inactivated Type 1 Poliovirus (Mahoney) / Inactivated Type 2 Poliovirus (MEF-1) / Inactivated Type 3 Poliovirus (Saukett) / Polyribosylribitol phosphate (PRP) from Haemophilus influenzae type b as PRP-OMPC / Hepatitis B Surface Antigen, recombinant (HBsAg), from Sanofi Pasteur MSD SNC , in the therapeutic area of vaccines;

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.

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 86 13
E-mail: mail@emea.europa.eu http://www.emea.europa.eu

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 / Opium prepared, from Solvay Pharma, in the therapeutic area of pain.
- Ranibizumab from Novartis Europharm Limited, in the therapeutic area of ophthalmology.
- Paracetamol / tramadol, from TEVA Pharma B.V., in the therapeutic area of pain.

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 colesevelam hydrochloride from Genzyme Europe B.V., in the therapeutic area of Endocrinology-gynaecology-fertility-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 one application for a paediatric investigation plan and two applications for modifications 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. Five experts were invited to the July meeting with clinical expertise in paediatric immunology, rheumatology, transplantation and allergology. The PDCO discussed product-related and /or general topics on paediatric medicinal product development.

PDCO interactions
The Chair of the Committee for Advanced Therapies was invited to the meeting of the PDCO to present the activities of the CAT, and in order to enhance the scientific collaboration between the two committees.

The next meeting of the PDCO will be held on 16-18 September 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 July 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**	**152**	**508**[1]
Applications submitted for a product not yet authorised *(Article 7*[2]*)*	39	186	102	327 *(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	42	162 *(32%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30*[2]*)*	1	10	8	19 *(4%)*
PIPs and full waiver indications covered by these applications	**202**	**395**	**207**	**804**

Number of Paediatric Committee (PDCO) opinions	**2007**	**2008**	**2009**	**Cumulative total**
Positive on full waiver	10	48	45	103
Positive on PIP, including potential deferral	2	81	87	170
Negative opinions adopted	0	4	9	13
Positive opinions adopted on modification of a PIP	0	8	22	30
Positive opinions on compliance with a PIP	0	5	4	9
Negative opinions on compliance with a PIP	0	0	1	1

[1] Of which 122 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	3
Pneumology-allergology	8	6	7
Infectious diseases	12	8	9
Cardiovascular diseases	12	14	7
Diagnostics	-	1	1
Endocrinology-gynaecology-fertility-metabolism	19	15	20
Neonatology-paediatric intensive care	-	1	1
Immunology-rheumatology-transplantation	5	6	7
Psychiatry	5	3	2
Pain	1	3	5
Haematology-haemostaseology	1	5	4
Otorhinolaryngology	-	1	1
Oncology	11	12	11
Dermatology	1	3	6
Vaccines	2	6	3
Ophthalmology	1	2	3
Anaesthesiology	-	1	1
Nutrition	1	1	0