23 February 2011
EMA/PDCO/126704/2011
Human Medicines Development and evaluation

Monthly report

Paediatric Committee (PDCO)

16-18 February 2011

Opinions on paediatric investigation plans

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

- Recombinant human N-acetylgalactosamine-6-sulfatase (BMN110), from BioMarin Europe Limited, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Dimethyl fumarate (BG00012), from Biogen Idec Ltd., in the therapeutic area of neurology;
- Abatacept, from Bristol-Myers Squibb Pharma EEIG, in the therapeutic area of immunology-rheumatology-transplantation;
- Ciclosporin, from APT Pharmaceuticals Limited, in the therapeutic area of immunology-rheumatology-transplantation;
- Bevacizumab, from Roche Registration Ltd, in the therapeutic area of oncology;
- (Propane-1-sulfonic acid {3-[5-(4-chlorophenyl)-1H-pyrrolo[2,3-b]pyridine-3-carbonyl]-2,4-difluoro-phenyl}-amide), from F. Hoffmann La Roche Ltd, in the therapeutic area of oncology;
- Imatinib mesilate, from Novartis Europharm Limited, in the therapeutic area of cardiovascular diseases;
- Valganciclovir hydrochloride, from Roche Registration Ltd, in the therapeutic area of infectious diseases;
- Human Cell Line recombinant human Factor VIII (human-cl rhFVIII), from Octapharma Pharmazeutika Produktionsges.m.b.H, in the therapeutic area of haematology-hemostaseology;
- Rotavirus type G3, rotavirus type G1, rotavirus type G2, rotavirus type P1A[8], rotavirus type G4, from Sanofi Pasteur MSD, in the therapeutic area of vaccines;

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)2 7418 8613
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

- Lubiprostone, from Sucampo Pharma Europe L, in the therapeutic area of gastroenterology-hepatology;
- Fibrinogen (human plasma-derived), from LFB Biotechnologie, in the therapeutic area of Haematology-haemostaseology;
- Azelastine hydrochloride / Fluticasone propionate, from MEDA Pharma GmbH & Co. KG, in the therapeutic area of Pneumology-Allergology;

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, 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:

- Human Normal Immunoglobulin, from LFB Biotechnologies, in the therapeutic area of immunology-rheumatology-transplantation;
- Perindopril tert-butylamine, Amlodipine besilate, from Gedeon Richter Plc., in the therapeutic area of cardiovascular diseases;
- Flutemetamol, from GE Healthcare Limited, in the therapeutic area of diagnostic;
- Rivastigmine, from Novartis Europharm Ltd., in the therapeutic area of neurology;
- Iloperidone, from Vanda Pharmaceuticals Limited, in the therapeutic area of psychiatry.

The PDCO adopted one opinions on the **refusal** of a request for waiver for:

- Aciclovir, from BioAlliance Pharma, in the therapeutic area of infectious 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.

Withdrawals

The PDCO noted that one application was 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. Two experts were invited to the February meeting with a clinical expertise in neurology; the PDCO discussed the potential needs in and characteristics of autoimmune polyneuritic diseases.

PDCO interactions

Representatives of the International Pediatric Multiple Sclerosis Study Group (IPMSSG) approached PDCO to open a dialogue on PIPs in paediatric MS. The main issues that the industry faces are the scarcity of paediatric patients, increasing competition due to several active compounds being developed, and the need for adequate study design and endpoints. The Committee agreed that a workshop targeting these issues would be very beneficial.

A representative of the Royal College of Paediatrics and Child Health (RCPCH) presented to the PDCO the guide. 'Not Just a Phase', which provides information to ensure the safe, meaningful and ethical participation of children and young people within the delivery of child health services and, practically demonstrates how collaboration between Health Care Professionals and young people can contribute towards creating a culture of participation.

Other issues

The PDCO welcomed the new member from Belgium, Professor Koenraad Norga, who has been nominated by Agence Fédérale des Médicaments et des Produits de Santé.

The Committee thanked Ann Marie Kaukonen for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 16-18 March 2011.

– END –

Notes:

1. PDCO opinions on PIPs and waivers are transformed into Agency's decisions within the timeframe laid down by the Paediatric Regulation (Regulation (EC) No 1901/2006, as amended). The decisions can be found on the Agency's website at: http://www.ema.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 European Union, have to provide either the results of studies in children conducted in accordance with an approved PIP, or an Agency's 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 Agency's website.
4. This meeting report, together with other information on the work of the Agency's, can be found on the Agency's website: http://www.ema.europa.eu

Enquiries only to: paediatrics@ema.europa.eu

Annex of the February PDCO meeting report

	2009 (January to December)	2010 (January to December)	2011 (January to current month)	Cumulative total (2007 to 2010)
Total number of validated PIP/waiver applications	273	326	29	985[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	191	280	24	720 *(73%)*
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])*	72	43	4	239 *(24%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	10	4	1	26 *(3%)*
PIPs and full waiver indications covered by these applications	395	403	36	1400

Number of Paediatric Committee (PDCO) opinions	2009	2010	2011	Cumulative total
Positive on full waiver	67	52	11	187
Positive on PIP, including potential deferral	122	201	21	427
Negative opinions adopted	13	7	1	25
Positive opinions adopted on modification of a PIP	51	103	23	185
Negative opinions adopted on modification of a PIP	0	4	0	4
Positive opinions on compliance with a PIP	8	9	0	22
Negative opinions on compliance check with a PIP	1	0	0	1
Opinions adopted under Art. 14.2	0	2	0	2

[1] Of which 223 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	2009 (%)	2010 (%)	2011 (Number of areas covered)*
Neurology	4	3	2
Uro-nephrology	5	2	0
Gastroenterology-hepatology	2	1	2
Pneumology-allergology	6	41	2
Infectious diseases	9	4	3
Cardiovascular diseases	9	8	4
Diagnostics	1	1	0
Endocrinology-gynaecology-fertility-metabolism	16	6	5
Neonatology-paediatric intensive care	2	0	2
Immunology-rheumatology-transplantation	6	5	2
Psychiatry	3	1	2
Pain	6	1	0
Haematology-haemostaseology	6	4	2
Otorhinolaryngology	1	3	0
Oncology	11	9	2
Dermatology	6	1	1
Vaccines	4	2	1
Ophthalmology	2	4	0
Anaesthesiology	1	2	0
Nutrition	0	0	0
Other			2

** One PIP can cover several therapeutic areas*