13 November 2012
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Paediatric Committee (PDCO)

PDCO monthly report of opinions on paediatric investigation plans and other activities

07-09 November 2012

Opinions on paediatric investigation plans

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

- Posaconazole, from Merck Sharp & Dohme (Europe), Inc., in the therapeutic area of infectious diseases;
- Faldaprevir, from Boehringer Ingelheim International GmbH, in the therapeutic area of infectious diseases;
- (S)-Isopropyl 2-((S)-(((2R,3R,4R,5R)-5-(2,4-dioxo-3,4-dihydropyrimidin-1(2H)-yl)-4-fluoro-3-hydroxy-4-methyltetrahydrofuran-2-yl)methoxy)(phenoxy)phosphorylamino)-Propanoate (GS-7977), from Gilead Sciences International Ltd., in the therapeutic area of infectious disease;
- Chimeric anti-disialoganglioside (GD2) monoclonal antibody (NSC764038), from United Therapeutics Europe Limited, in the therapeutic area of oncology;
- Spheroids of human autologous matrix-associated chondrocytes, from co.don AG, in the therapeutic area of transplantation;
- Dextran, 3 [(2-aminoethyl)thio]propyl 17-carboxy-10,13,16-tris(carboxymethyl)-8-oxo-4-thia-7,10,13,16-tetraazaheptadec-1-yl 3-[[2-[[1-imino-2-(D-mannopyranosylthio)ethyl]amino]ethyl]thio]propyl ether, from Navidea Biopharmaceuticals Limited, in the therapeutic area of diagnostics;
- Outer Membrane Vesicles (OMV) from N. meningitidis Strain NZ 98/254 / recombinant Neisseria meningitis group B Protein 936-741 / meningococcal group W-135 oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / meningococcal group A oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / meningococcal group C oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / recombinant Neisseria meningitis group B Protein 287-953 / recombinant Neisseria meningitis group B Protein 961c / meningococcal group Y oligosaccharides conjugated to Corynebacterium diphtheriae

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CRM197 protein (MenABCWY), from Novartis Vaccines and Diagnostics S.r.l., in the therapeutic area of vaccines;

- Human normal immunoglobulin (LFB-IgSC), from LFB Biotechnologies, in the therapeutic area of immunology-rheumatology-transplantation.

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:

- Ivermectin, from Galderma R&D, in the therapeutic area of dermatology;
- Expanded autologous bone marrow-derived osteoblastic cells, from Bone Therapeutics S.A., in the therapeutic area of other (musculoskeletal diseases);
- Elagolix, from AbbVie Ltd, in the therapeutic area of endocrinology-gynaecology-fertility-metabolism;
- Skeletal muscle derived cells, from Innovacell Biotechnologie AG, in the therapeutic area of uro-nephrology.

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. The PDCO adopted positive opinions, agreeing change(s), for the following products:

- Prucalopride, from Shire-Movetis NV, in the therapeutic area of gastroenterology-hepatology;
- Cannabidiol / delta-9-tetrahydrocannabinol, from GW Pharma Ltd, in the therapeutic area of neurology;
- N-[6-(cis-2,6-Dimethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy) [1,1'-biphenyl]-3-carboxamide diphosphate, from Novartis Europharm Limited, in the therapeutic area of oncology;
- Ivacaftor, from Vertex Pharmaceuticals Incorporated, in the therapeutic area of other (congenital, hereditary, and neonatal diseases and abnormalities);
- Treprostinil, from United Therapeutics Europe, Ltd., in the therapeutic area of cardiovascular diseases;

- Serelaxin, from Novartis Europharm Ltd., in the therapeutic area of cardiovascular diseases.

Withdrawals

The PDCO noted that 2 applications were withdrawn during the late stages of the evaluation (30 days or less before opinion).

The next meeting of the PDCO will be held on 05-07 December 2012.

– END –

Notes:

1. 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.

2. 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/ema/index.jsp?curl=pages/medicines/landing/pip_search.jsp&murl=menus/medicines/medicines.jsp&mid=WC0b01ac058001d129

3. More information about the PDCO and the Paediatric Regulation is available in the Regulatory section of the Agency's website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000023.jsp&murl=menus/regulations/regulations.jsp&mid=WC0b01ac05800240cd

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 November 2012 PDCO meeting report

	2010 (January to December)	2011 (January to December)	2012 (January to current month)	Cumulative total (2007 to present)
Total number of validated PIP/waiver applications	326	187	166	1310[1]
Applications submitted for a product not yet authorised *(Article 7[2])*	280	153	139	989 *(76%)*
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])*	43	33	26	294 *(22%)*
Applications submitted for an off-patent product developed specifically for children with an age-appropriate formulation *(Article 30[2])*	4	1	1	27 *(2%)*
PIPs and full waiver indications covered by these applications	403	220	205	1789

Number of Paediatric Committee (PDCO) opinions	2010	2011	2012	Cumulative total (2007 to present)
Positive on full waiver	52	45	44	265
Positive on PIP, including potential deferral	201	107	83	596
Negative opinions adopted	7	3	3	30
Positive opinions adopted on modification of a PIP	103	153	148	463
Negative opinions adopted on modification of a PIP	4	2	1	7
Positive opinions on compliance with a PIP	9	9	4	35
Negative opinions on compliance check with a PIP	0	0	0	1
Opinions adopted under Art. 14.2	2	0	0	2

[1] Of which 332 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	2010 *(%)*	2011 *(Number of areas covered)**	2012 *(Number of areas covered)**
Neurology	3	11	11
Uro-nephrology	2	4	4
Gastroenterology-hepatology	1	10	6
Pneumology-allergology	41	10	8
Infectious diseases	4	15	19
Cardiovascular diseases	8	21	33
Diagnostics	1	5	2
Endocrinology-gynaecology-fertility-metabolism	6	28	26
Neonatology-paediatric intensive care	0	0	2
Immunology-rheumatology-transplantation	5	13	13
Psychiatry	1	9	0
Pain	1	2	9
Haematology-haemostaseology	4	18	9
Otorhinolaryngology	3	2	1
Oncology	9	19	17
Dermatology	1	10	13
Vaccines	2	12	2
Ophthalmology	4	8	4
Anaesthesiology	2	1	2
Nutrition	0	0	0
Other		7	13

** One PIP can cover several therapeutic areas*