22 January 2015
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Paediatric Committee (PDCO)

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

14-16 January 2015

Opinions on paediatric investigation plans

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

- Coagulation Factor VIIa (Recombinant), from LFB SA, for the treatment of congenital coagulation disorders and treatment of acquired haemophilia;
- Olesoxime, from TROPHOS SA, for the treatment of spinal muscular atrophy;
- (1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) phosphorodiamidate [TH-302], from Merck KGaA, for the treatment of soft tissue sarcoma and treatment of Ewing sarcoma;
- Eravacycline, from Tetraphase Pharmaceuticals, Inc., for the treatment of complicated intra-abdominal infection and treatment of urinary tract infection;
- Emtricitabine / propan-2-yl N-[(S)-({[(2R)-1-(6-amino-9H-purin- 9-yl)propan-2-yl]-oxy}methyl)(phenoxy) phosphoryl]-Lalaninate, (2E)-but-2-enedioate (2:1), from Gilead Sciences International Ltd., for the treatment of human immunodeficiency virus (HIV-1) infection;
- Efinaconazole, from PharmaSwiss Česká republika s.r.o., for the treatment of onychomycosis.

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:

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- Hydromorphone (hydrochloride) / naloxone (hydrochloride), from Develco Pharma GmbH, for the treatment of pain and treatment of opioid-induced constipation;
- Oxycodone (hydrochloride) / naloxone (hydrochloride), from Develco Pharma GmbH, for the treatment of pain and treatment of opioid-induced constipation;
- Perindopril / Amlodipine, from Adamed Sp. z o.o., for the treatment of hypertension;
- Candesartan / amlodipine, from Adamed Sp. z o.o., for the treatment of hypertension.

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:

- Sieved freeze-dried allergen extract of Dermatophagoides pteronyssinus / Sieved freeze-dried allergen extract of Dermatophagoides farinae, from Stallergenes, for the treatment of allergic rhinitis and treatment of asthma;
- Insulin degludec / insulin aspart, from Novo Nordisk A/S, for the treatment of type I diabetes mellitus and treatment of type II diabetes mellitus;
- Diphtheria toxoid / Tetanus toxoid / Bordetella pertussis antigen: Pertussis toxoid / Bordetella pertussis antigen: Filamentous Haemagglutinin / Bordetella pertussis antigen: Pertactin / Inactivated poliovirus: type 1 (Mahoney strain) / Inactivated poliovirus: type 2 (MEF-1 strain) / Inactivated poliovirus: type 3 (Saukett strain), from GlaxoSmithKline Biologicals S.A., for the prevention of infectious diseases caused by Corynebacterium diphtheriae / Clostridium tetani / Bordetella pertussis / Poliovirus types 1, 2 and 3;
- Amikacin (sulfate), from Insmed Incorporated, for the treatment of *Pseudomonas aeruginosa* lung infection/colonisation in cystic fibrosis patients and treatment of nontuberculous mycobacterial lung infection;
- Riociguat, from Bayer Pharma AG, for the treatment of pulmonary hypertension;
- Lixisenatide, from Sanofi-Aventis R&D, for the treatment of type 2 diabetes mellitus;
- Recombinant human N-acetylgalactosamine-6-sulfatase (BMN110), from BioMarin Europe Limited, for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome);
- Deferasirox, from Novartis Europharm Limited, for the treatment of chronic iron overload requiring chelation therapy;
- Pegylated human interferon beta-1a, from Biogen Idec Ltd., for the treatment of multiple sclerosis;
- Albiglutide, from GlaxoSmithKline Trading Services Limited, for the treatment of type 2 diabetes mellitus;
- Human fibrinogen / human thrombin, from ProFibrix BV, for the treatment of haemorrhage resulting from a surgical procedure;

- Allantoin, from Scioderm, Inc., for the treatment of epidermolysis bullosa.

Opinion on compliance check

The PDCO adopted a positive opinion on a (full) compliance check for Human normal immunoglobulin, from Baxter Innovations GmbH, for the treatment of primary immunodeficiency (PID).

A compliance check is performed to verify that all the measures agreed in a PIP and reflected in the Agency's decision have been conducted in accordance with the decision, including the agreed timelines. Full compliance with all studies/measured contained in the PIP 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 Agency's 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 6 applications were withdrawn during the late stages of the evaluation (30 days or less before completion of the procedure).

Other matters

The PDCO thanked Kolbeinn Gudmundsson for his work at the end of his mandate as alternate for Iceland.

The next meeting of the PDCO will be held on 11-13 February 2015.

– 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

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