19 August 2015
EMA/PDCO/492280/2015
Procedure Management and Committees Support Division

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

12-14 August 2015

Opinions on paediatric investigation plans

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

- Acotiamide, from Zeria Pharmaceutical Co Ltd, for the treatment of functional dyspepsia;
- Adenovirus associated viral vector serotype 2 containing the human RPE65 gene, from Spark Therapeutics Inc, for the treatment of genetic congenital retinal disorders;
- Peanut flour, from Aimmune Therapeutics, for the treatment of peanut allergy.

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.

Adoption of an opinion following re-examination

The PDCO adopted an opinion for the following product:

- Following the re-examination of the positive opinion on a modification to an agreed PIP with a deferral adopted on 19 June 2015 for Lumacaftor / ivacaftor, from Vertex Pharmaceuticals (Europe) Ltd., for the treatment of cystic fibrosis, the PDCO adopted a revised positive opinion.

A re-examination of the opinion can be requested by the applicant within 30 days following receipt of the opinion of the PDCO. The grounds for the re-examination should be based only on the original information and scientific data provided in the application that were previously available to the PDCO and on which the initial opinion was based. This may include new analysis of the same data or minor protocol amendments to a previously proposed study. Significant changes to the previous plan cannot be part of the re-examination process.

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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:

- Hydromorphone (hydrochloride) / naloxone (hydrochloride), from Mundipharma Research GmbH & Co. KG, for the treatment of pain and treatment of opioid-induced constipation;
- Botulinum Neurotoxin Type A, from CROMA PHARMA GmbH, for the treatment of muscle induced wrinkles;
- Alpha Connexin C-terminal 1 peptide (ACT1), from FirstString Research Inc, for the treatment of venous leg ulcer and treatment of diabetic foot ulcer.

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:

- Azilsartan medoxomil, from Takeda Development Centre Europe Limited, for the treatment of hypertension;
- Linagliptin, from Boehringer Ingelheim International GmbH, for the treatment of type 2 diabetes mellitus;
- Empagliflozin, from Boehringer Ingelheim International GmbH, for the treatment of type 2 diabetes mellitus;
- Canagliflozin, from Janssen-Cilag International NV, for the treatment of type 2 diabetes mellitus;
- Sucroferric oxyhydroxide (mixture of iron (III)-oxyhydroxide, sucrose, starch) (PA21), from Vifor Fresenius Medical Care Renal Pharma, for the treatment of hyperphosphataemia;
- Dopamine, from BrePco Biopharma Limited, for the treatment of vascular hypotensive disorders;
- Odanacatib, from Merck Sharp & Dohme (Europe), Inc., for the treatment of osteoporosis;
- Vonicog alfa, from Baxter Innovations GmbH, for the treatment of von Willebrand Disease;
- Brexpiprazole, from Otsuka Europe Development and Commercialisation Ltd, Zweigniederlassung Frankfurt am Main, for the treatment of schizophrenia;
- Tafluprost, from Santen Oy, for the treatment of glaucoma;
- Azacitidine, from Celgene Europe Ltd, for the treatment of acute myeloid leukaemia and treatment of myelodysplastic syndrome (including juvenile myelomonocytic leukaemia);

- PEGylated recombinant factor VIII, from Baxalta Innovations GmbH, for the treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency);
- Naloxone, from Develco Pharma GmbH, for the treatment of opioid-induced constipation;
- Anti programmed death-ligand 1 (PD-L1) monoclonal antibody (MPDL3280A), from Roche Registration Ltd, for the treatment of all conditions included in the category of malignant neoplasms (except central nervous system tumours, haematopoietic and lymphoid tissue neoplasms).

Withdrawals

The PDCO noted that one application was withdrawn during the late stages of the evaluation (30 days or less before completion of the procedure).

Other matters

The PDCO welcomed the new member, Suzana Mimica Matanović, who has been nominated to represent Croatia.

The PDCO welcomed Marina Dimov Di Gusti in her new role as alternate, who has been nominated to represent Croatia.

The PDCO thanked Bernard Kaić for his work following the end of his mandate as alternate.

The next meeting of the PDCO will be held on 09-11 September 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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