EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 August 2019
EMEA/PDCO/481535/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

Written procedure 20-23 August 2019

Opinions on paediatric investigation plans

The PDCO adopted an opinion(s) on the **refusal** of a PIP, including a deferral for:

- Ecopipam (hydrochloride), EMEA-002564-PIP01-19, from Emalex Biosciences, Inc., for the treatment of Tourette syndrome.

For this medicine the PDCO granted a product-specific waiver on its own motion for all subsets of the paediatric population in the specified condition, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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:

- Autologous human T cells genetically modified ex-vivo with a lentiviral vector encoding a chimeric antigen receptor (CAR) for B-cell maturation antigen (BCMA), EMEA-002585-PIP01-19, from Janssen-Cilag International N.V., for the treatment of multiple myeloma;
- Aprocitentan, EMEA-001978-PIP02-19, from Idorsia Pharmaceuticals Deutschland GmbH, for the treatment of hypertension;
- Timolol (as timolol maleate) / bimatoprost, EMEA-002583-PIP01-19, from Laboratoires Théa, for the treatment of glaucoma and treatment of ocular hypertension;

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- 2-(3-(1-carboxy-5-[(6-[^{18}F]fluoropyridine-3-carbonyl)-amino]-pentyl)-ureido)-pentanedioic acid, EMEA-002608-PIP01-19, from Curium Netherlands BV, for the diagnosis of prostate cancer;
- Human fibrinogen, EMEA-001931-PIP02-19, from Biotest AG, for the treatment of acquired fibrinogen deficiency;
- 7-(2-Methoxy-3,5-dimethylpyridin-4-yl)-1-[(3S)-tetrahydrofuran-3-yl]-1,5-dihydro-4H-pyrazolo[4,3-c]quinolin-4-one maleate, EMEA-002424-PIP02-19, from Eisai Ltd., for the treatment of dementia with lewy bodies;

Opinion on compliance check

The PDCO adopted positive opinions on (full) compliance check for:

- Dabigatran etexilate mesilate, EMEA-C-000081-PIP01-07-M11, from Boehringer Ingelheim International GmbH, for the treatment of thromboembolic events;
- Caplacizumab, EMEA-C-001157-PIP01-11-M02, from ABLYNX NV, for the treatment of thrombotic thrombocytopenic purpura;

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/measures 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 1 application was withdrawn during the late stages of the evaluation (30 days or less before completion of the procedure).

Other matters

The next meeting of the PDCO will be held on 17-20 September 2019.

– 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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