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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

24-27 April 2018

Opinions on paediatric investigation plans

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

- Daratumumab, EMEA-002152-PIP01-17, from Janssen-Cilag International N.V., for the treatment of lymphoid malignancies (except mature B-cell neoplasms);
- Isatuximab, EMEA-002205-PIP01-17, from Sanofi-Aventis Recherche & Développement, for the treatment of malignant neoplasms of the haematopoietic and lymphoid tissue;
- Lasmiditan, EMEA-002166-PIP01-17, from Eli Lilly and Company Limited, for the treatment of migraine headaches;
- Setmelanotide, EMEA-002209-PIP01-17, from Rhythm Pharmaceuticals, Inc, for the treatment of appetite and general nutrition disorders.

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

- Somapacitan, EMEA-001469-PIP02-17, from Novo Nordisk A/S, for the treatment of short stature.

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 is likely to be ineffective or unsafe in part or all of the paediatric population and Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

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:

- Andecaliximab, EMEA-002304-PIP01-17, from Gilead Sciences International Ltd, for the treatment of gastric and gastroesophageal junction adenocarcinoma;
- Split influenza virus, inactivated containing antigens equivalent to the A/H1N1-like strain / split influenza virus, inactivated containing antigens equivalent to the A/H3N2-like strain / split influenza virus, inactivated containing antigens equivalent to the B-like strain, EMEA-002353-PIP01-18, from Sanofi Pasteur, for the prevention of influenza infection;
- Trandolapril / verapamil, EMEA-002276-PIP01-17, from Abbott Laboratories, 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.

The PDCO adopted 1 opinion advocating the review of a granted waiver for:

- Delafloxacin, EMEA-001080-PIP01-10, from A.Menarini - Industrie Farmaceutiche Riunite - s.r.l., for the treatment of local infections of skin and subcutaneous tissues.

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:

- Apixaban, EMEA-000183-PIP02-12-M02, from Bristol-Myers Squibb / Pfizer EEIG, for the treatment of venous thromboembolism;
- Apixaban, EMEA-000183-PIP01-08-M06, from Bristol-Myers Squibb / Pfizer EEIG, for the prevention of arterial thromboembolism and prevention of venous thromboembolism;
- Apremilast, EMEA-000715-PIP03-11-M05, from Celgene Europe Limited, for the treatment of psoriasis;
- Baricitinib, EMEA-001220-PIP01-11-M03, from Eli Lilly and Company Limited, for the treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis);
- Betrixaban, EMEA-001834-PIP02-16-M01, from Portola Pharma UK Limited, for the prevention of venous thromboembolism;
- Ceftaroline fosamil, EMEA-000769-PIP01-09-M08, from Pfizer Limited, for the treatment of community-acquired pneumonia and treatment of complicated skin and soft tissue infections;
- Ceftazidime / avibactam, EMEA-001313-PIP01-12-M07, from Pfizer Limited, for the treatment of intra-abdominal infections, treatment of urinary tract infections, treatment of pneumonia and treatment of infections due to aerobic Gram-negative organisms;

- Dasabuvir (sodium monohydrate), EMEA-001439-PIP01-13-M02, from Abbvie Ltd, for the treatment of chronic hepatitis C;
- Dupilumab, EMEA-001501-PIP01-13-M05, from Regeneron Pharmaceuticals, Inc, for the treatment of atopic dermatitis;
- Emapalumab, EMEA-002031-PIP01-16-M01, from Novimmune B.V, for the treatment of haemophagocytic lymphohistiocytosis;
- Empagliflozin, EMEA-000828-PIP04-16-M01, from Boehringer Ingelheim International GmbH, for the treatment of type 1 diabetes mellitus;
- Etelcalcetide, EMEA-001554-PIP01-13-M02, from Amgen Europe B.V., for the treatment of hyperparathyroidism;
- Fenfluramine (hydrochloride), EMEA-001990-PIP01-16-M01, from Zogenix International Ltd, for the treatment of Dravet syndrome;
- Formoterol (fumarate dihydrate) / Beclometasone (dipropionate), EMEA-000548-PIP01-09-M08, from Chiesi Farmaceutici S.p.A., for the treatment of asthma;
- Lamivudine / dolutegravir, EMEA-001940-PIP01-16-M01, from ViiV Healthcare UK Limited, for the treatment of human immunodeficiency virus (HIV-1) infection;
- Lubiprostone, EMEA-000245-PIP01-08-M05, from Sucampo AG, for the treatment of constipation;
- Lurasidone (hydrochloride), EMEA-001230-PIP01-11-M04, from AZIENDE CHIMICHE RIUNITE ANGELINI FRANCESCO - A.C.R.A.F. S.p.A., for the treatment of schizophrenia;
- Ombitasvir/ paritaprevir / ritonavir, EMEA-001440-PIP01-13-M02, from Abbvie Ltd, for the treatment of chronic hepatitis C;
- Peanut flour, EMEA-001734-PIP01-14-M02, from Aimmune Therapeutics Inc, for the treatment of peanut allergy;
- Pibrentasvir / glecaprevir, EMEA-001832-PIP01-15-M01, from AbbVie Ltd, for the treatment of chronic hepatitis C;
- Sofosbuvir, EMEA-001276-PIP01-12-M02, from Gilead Sciences International Ltd., for the treatment of chronic hepatitis C;
- Spheroids of human autologous matrix-associated chondrocytes, EMEA-001264-PIP01-12-M02, from CO.DON AG, for the treatment of cartilage disorders;
- Tenofovir alafenamide / emtricitabine, EMEA-001577-PIP02-14-M03, from Gilead Sciences International Ltd., for the treatment of human immunodeficiency virus (HIV-1) infection;
- Velpatasvir / sofosbuvir, EMEA-001646-PIP01-14-M02, from Gilead Sciences International Ltd., for the treatment of chronic hepatitis C.

Withdrawals

The PDCO noted that 8 applications were 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 29 May 2018 - 01 June 2018.

– 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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