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Procedure Management and Committees Support Division

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

22-24 June 2016

Opinions on paediatric investigation plans

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

- Cabotegravir, EMEA-001418-PIP02-15, from ViiV Healthcare UK Limited, for the prevention of human immunodeficiency virus (HIV-1) infection;
- Eculizumab, EMEA-000876-PIP06-15, from Alexion Europe SAS, for the prevention of graft rejection following solid organ transplantation;
- andexanet alfa, EMEA-001902-PIP01-15, from Portola Pharma UK Limited, for the prevention of factor Xa inhibitor associated haemorrhage and treatment of factor Xa inhibitor associated haemorrhage;
- 3-[[5-chloro-1-[3-(methylsulfonyl)propyl]-1H-indol-2-yl]methyl]-1-(2,2,2-trifluoroethyl)-1,3-dihydro-2H-imidazo[4,5-c]pyridine-2-one, EMEA-001838-PIP01-15, from Janssen-Cilag International NV, for the treatment of lower respiratory tract disease caused by human respiratory syncytial virus (RSV);
- Semaglutide, EMEA-001441-PIP02-15, from Novo Nordisk, for the treatment of type 2 diabetes mellitus;
- Pembrolizumab, EMEA-001474-PIP02-16, from Merck Sharp & Dohme (Europe), Inc, for the treatment of Hodgkin lymphoma;
- Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human ARSA cDNA sequence, EMEA-001765-PIP02-15, from GlaxoSmithKline Trading Services Limited, for the treatment of metachromatic leukodystrophy (MLD);
- Risankizumab, EMEA-001776-PIP01-15, from Boehringer Ingelheim International GmbH, for the treatment of psoriasis;
- Quizartinib, EMEA-001821-PIP01-15, from Daiichi Sankyo Europe GmbH, for the treatment of acute myeloid leukaemia.

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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:

- Hydrochlorothiazide / Amlodipine / Ramipril, EMEA-001942-PIP01-16, from Adamed Sp. z o.o., for the treatment of hypertension;
- Lusutrombopag, EMEA-001905-PIP01-15, from Shionogi Limited, for the treatment of thrombocytopenia secondary to liver disease;
- Rosuvastatin / Amlodipine, EMEA-001935-PIP01-16, from Adamed Sp. z o.o., for the treatment of cardiovascular diseases.

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:

- Brentuximab vedotin, EMEA-000980-PIP01-10-M04, from Takeda Pharma A/S, for the treatment of Hodgkin lymphoma and treatment of anaplastic large cell lymphoma;
- rilpivirine (as hydrochloride), EMEA-000317-PIP01-08-M09, from Janssen-Cilag International NV, for the treatment of human immunodeficiency virus (HIV-1) infection;
- elobixibat, EMEA-001484-PIP01-13-M01, from Elobix AB, for the treatment of chronic constipation;
- ivacaftor / lumacaftor, EMEA-001582-PIP01-13-M04, from Vertex Pharmaceuticals (Europe) Limited, for the treatment of cystic fibrosis;
- Benralizumab, EMEA-001214-PIP01-11-M05, from AstraZeneca AB, for the treatment of asthma;
- lurasidone hydrochloride, EMEA-001230-PIP01-11-M02, from Sunovion Pharmaceuticals Ltd., for the treatment of schizophrenia;
- Anidulafungin, EMEA-000469-PIP01-08-M06, from Pfizer Limited, for the treatment of invasive candidiasis;
- (3-((4-Benzoyl-1-piperazinyl)(oxo)acetyl)-4-methoxy-7-(3-methyl-1H-1,2,4-triazol-1-yl)-1H-pyrrolo[2,3-c]pyridin-1-yl)methyl dihydrogen phosphate, 2-amino-2-(hydroxymethyl)-1,3-propanediol (1:1), EMEA-001687-PIP01-14-M01, from Bristol-Myers Squibb International Corporation, for the treatment of human immunodeficiency virus (HIV-1) infection;
- budesonide, EMEA-001087-PIP02-12-M02, from Vectura Limited, for the treatment of asthma;

- Tenofovir alafenamide (as fumarate), EMEA-001584-PIP01-13-M01, from Gilead Sciences International Ltd., for the treatment of chronic viral hepatitis B;
- Adalimumab, EMEA-000366-PIP05-12-M02, from AbbVie Limited, for the treatment of non-infectious uveitis;
- Turoctocog alfa, EMEA-000428-PIP01-08-M03, from Novo Nordisk A/S, for the treatment of hereditary factor VIII deficiency;
- Chimeric anti-disialoganglioside (GD2) monoclonal antibody, EMEA-001285-PIP01-12-M02, from United Therapeutics Europe Limited, for the treatment of neuroblastoma;
- Pneumococcal polysaccharide serotype 6B conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 1 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 18C conjugated to tetanus toxoid / Pneumococcal polysaccharide serotype 5 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 19F conjugated to diphtheria toxoid / Pneumococcal polysaccharide serotype 14 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 9V conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 4 conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 23F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein / Pneumococcal polysaccharide serotype 7F conjugated to protein D (derived from non-typeable Haemophilus influenzae) carrier protein, EMEA-000673-PIP01-09-M09, from GlaxoSmithKline Biologicals S.A., for the prevention of diseases caused by streptococcus pneumoniae and prevention of acute otitis media caused by non-typeable Haemophilus influenzae;
- Dupilumab, EMEA-001501-PIP01-13-M03, from Regeneron Pharmaceuticals, Inc, for the treatment of atopic dermatitis;
- Recombinant human beta-glucuronidase, EMEA-001540-PIP01-13-M01, from Ultragenyx UK Limited, for the treatment of Mucopolysaccharidosis type VII;
- Solithromycin, EMEA-001581-PIP01-13-M03, from Triskel EU Services, Ltd, from ACE Pharmaceuticals BV, for the treatment of bacterial pneumonia, treatment of tularaemia and treatment of anthrax;
- Levamisole (hydrochloride), EMEA-001885-PIP01-15-M01, for the treatment of glomerulonephritis and nephrotic syndrome.

For vemurafenib, EMEA-000978-PIP01-10-M01, from Roche Registration Limited, for the treatment of melanoma the PDCO adopted a positive opinion and granted a product-specific waiver, on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

Opinion on compliance check

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

- Aripiprazole, EMEA-C-000235-PIP02-10-M02, from Otsuka Pharmaceutical Europe Ltd., for the treatment of bipolar affective disorder and treatment of schizophrenia.

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 8 applications were withdrawn during the late stages of the evaluation (30 days or less before completion of the procedure).

New meeting dates adopted

PDCO meeting dates for 2016-2018 were adopted during the June 2016 plenary meeting. From November 2016, PDCO plenary meetings will be extended to include Tuesday afternoons.
The new dates are published on the EMA website at:
http://www.ema.europa.eu/docs/en_GB/document_library/Other/2015/03/WC500184395.pdf

Other matters

The PDCO thanked Romaldas Maciulaitis and Rugile Pilviniene for their work at the end of their mandates as member and, respectively, alternate for Lithuania.

The next meeting of the PDCO will be held on 20-22 July 2016.

– 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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