EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

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

28-30 April 2020

## Opinions on paediatric investigation plans

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

- Lenvatinib, EMEA-001119-PIP03-19, from Eisai GmbH, for the treatment of all conditions included in the category of malignant neoplasms except haematopoietic and lymphoid tissue neoplasms, papillary thyroid cancer, follicular thyroid cancer and osteosarcoma;
- Gepotidacin, EMEA-002443-PIP02-18, from GlaxoSmithKline Trading Services Limited, for the treatment of uncomplicated urogenital gonorrhea;
- Autologous haptenised and irradiated cells derived from glioma, EMEA-002661-PIP01-19, from ERC Belgium, for the treatment of glioma;
- Allogeneic haptenised and irradiated cells derived from glioma, EMEA-002662-PIP01-19, from ERC Belgium, for the treatment of glioma;
- Allogeneic haptenised and irradiated cell lysates derived from glioma, EMEA-002663-PIP01-19, from ERC Belgium, for the treatment of glioma;
- Autologous haptenised and irradiated cell lysates derived from glioma, EMEA-002664-PIP01-19, from ERC Belgium, for the treatment of glioma;
- Sodium alginate oligosaccharide, EMEA-002321-PIP01-17, from AlgiPharma AS, for the treatment of cystic fibrosis;
- Atropine (sulphate), EMEA-002545-PIP01-19, from Fondazione Per La Ricerca Farmacologica Gianni Benzi Onlus, for the treatment of myopia;
- Dexamethasone (sodium phosphate) encapsulated in human autologous erthrocytes, EMEA-001957-PIP02-19, from EryDel S.p.A, for the treatment of ataxia telangiectasia;
- Gepotidacin, EMEA-002443-PIP01-18, from GlaxoSmithKline Trading Services Limited, for the treatment of uncomplicated urinary tract infections;

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- Bis-choline tetrathiomolybdate (ALXN1840), EMEA-002232-PIP02-19, from Alexion Europe S.A.S., for the treatment of Wilson Disease;
- Fidanocogene elaparvovec, EMEA-002362-PIP02-19, from Pfizer Europe MA EEIG, for the treatment of congenital factor IX deficiency (haemophilia B).
- Adeno-associated viral vector serotype 8 containing the human RPGR gene (AAV8-RPGR), EMEA-002601-PIP01-19, from Nightstar Europa Limited, for the treatment of X-linked retinitis pigmentosa

## 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:

- Ezetimibe / Atorvastatin, EMEA-002649-PIP02-20, from ELPEN Pharmaceutical Co. Inc., for the prevention of cardiovascular events;
- Adenovirus encoding vascular endothelial growth factor C (AdAptVEGF-C), EMEA-002748-PIP01-20, from Herantis Pharma Plc, for the treatment of secondary lymphoedema associated with the treatment of breast cancer;
- Monalizumab, EMEA-002751-PIP01-19, from AstraZeneca AB, for the treatment of head and neck epithelial malignant neoplasms;
- Chloroprocaine (hydrochloride), EMEA-000639-PIP06-20, from Sintetica GmbH, for ocular surface anaesthesia;
- Adeno-associated virus serotype 2 (AAV2) encoding human aromatic L-amino acid decarboxylase (hAADC), EMEA-002753-PIP01-19, from Neurocrine Therapeutics, Ltd., for the treatment of Parkinson's Disease;
- Ribociclib (succinate), EMEA-002765-PIP01-19, from Novartis Europharm Limited, for the treatment of breast cancer;
- Betahistine (dihydrochloride), EMEA-002652-PIP01-19, from Auris Medical Ltd, for the treatment of peripheral vertigo;
- IMG-2789, EMEA-002752-PIP01-19, from Imago Biosciences BV, for the treatment of myeloproliferative neoplasms.

The PDCO adopted one opinion on the **refusal** of a request for waiver for:

- Bilastine, EMEA-000347-PIP05-20, from FAES FARMA S.A., for the treatment of acute type I hypersensitivity reactions.

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:

- Monovalent, recombinant, replication-incompetent human adenovirus serotype 26-vectored vaccine encoding the pre-fusion conformation-stabilised F protein derived from the RSV A2 strain, EMEA-002172-PIP02-17-M01, from Janssen-Cilag International NV, for the prevention of lower respiratory tract disease caused by respiratory syncytial virus (RSV);
- Apixaban, EMEA-000183-PIP02-12-M03, from Bristol-Myers Squibb / Pfizer EEIG, for the treatment of venous thromboembolism;
- Apixaban, EMEA-000183-PIP01-08-M08, from Bristol-Myers Squibb / Pfizer EEIG, for the prevention of venous thromboembolism and arterial thromboembolism;
- Elvitegravir / cobicistat / emtricitabine / tenofovir alafenamide, EMEA-001460-PIP01-13-M04, from Gilead Sciences International Ltd., for the treatment of human immunodeficiency virus (HIV-1) infection;
- Darvadstrocel, EMEA-001561-PIP01-13-M01, from Takeda Pharma A/S, for the treatment of perianal fistula;
- Outer Membrane Vesicles (OMV) from N. meningitidis Strain NZ 98/254 / recombinant Neisseria meningitis group B Protein 936-741 / meningococcal group W-135 oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / meningococcal group A oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / meningococcal group C oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein / recombinant Neisseria meningitis group B Protein 287-953 / recombinant Neisseria meningitis group B Protein 961c / meningococcal group Y oligosaccharides conjugated to Corynebacterium diphtheriae CRM197 protein (MenABCWY), EMEA-001260-PIP01-11-M01, from GSK Vaccines s.r.l., for the prevention of meningococcal meningitis;
- Ticagrelor, EMEA-000480-PIP01-08-M13, from AstraZeneca AB, for the prevention of thromboembolic events;
- Erenumab, EMEA-001664-PIP02-15-M04, from Novartis Europharm Limited, for the prevention of migraine headaches;
- Maralixibat chloride (LUM001), EMEA-001475-PIP03-17-M02, from Mirum Pharmaceuticals, for the treatment of progressive familial intrahepatic cholestasis;
- Human fibrinogen concentrate (BT524), EMEA-001931-PIP01-16-M02, from Biotest AG, for the treatment of congenital fibrinogen deficiency;
- Eptinezumab, EMEA-002243-PIP01-17-M01, from H. Lundbeck A/S, for the prevention of migraine headaches;
- Lucerastat, EMEA-002095-PIP01-16-M01, from Idorsia Pharmaceuticals Deutschland GmbH, for the treatment of Fabry disease;
- Ligelizumab, EMEA-001811-PIP02-15-M03, from Novartis Europharm Limited, for the treatment of chronic spontaneous urticaria;
- Satralizumab, EMEA-001625-PIP01-14-M05, from Roche Registration GmbH, for the treatment of neuromyelitis optica;
- Tofacitinib, EMEA-000576-PIP01-09-M12, from Pfizer Limited, for the treatment of chronic idiopathic

arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis);

- Upadacitinib, EMEA-001741-PIP04-17-M01, from Abbvie Ltd, for the treatment of atopic dermatitis;
- Autologous CD34+ cells transduced with lentiviral vector encoding the human beta-globin gene, EMEA-001933-PIP01-16-M01, from Orchard Therapeutics (Europe) Ltd, for the treatment of beta-thalassemia

## Opinion on compliance check

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

- Adalimumab, EMEA-C-000366-PIP02-09-M06, from AbbVie Limited, for the treatment of ulcerative colitis;
- Lipegfilgrastim, EMEA-C-001019-PIP01-10-M05, from UAB "Sicor Biotech", for the prevention of chemotherapy-induced febrile neutropenia and treatment of chemotherapy-induced neutropenia;
- Zoledronic acid, EMEA-C-000057-PIP01-07-M07, from Novartis Europharm Limited, for the treatment of osteoporosis and treatment of Paget's disease of the bone;

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

## New meeting dates adopted

PDCO updated meeting dates for 2020-2021 were adopted during the April meeting. These dates are important for applicants in planning the submission of applications for PIPs, requests for waivers, requests for modification of an agreed PIP, and requests for compliance checks. The dates are published on the Agency's website at:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/document_listing/document_listing_000293.jsp&murl=menus/regulations/regulations.jsp&mid=WC0b01ac0580025b91&jsenabled=true

## Other matters

The PDCO thanked Pia Annunen for her work as she has resigned from the Committee.

The next meeting of the PDCO will be held on 26th-29th May 2020.

**– 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: https://www.ema.europa.eu/en/medicines/ema_group_types/ema_pip

3. More information about the PDCO and the Paediatric Regulation is available in the Regulatory section of the Agency's website:

   https://www.ema.europa.eu/en/committees/paediatric-committee-pdco

   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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