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Human Medicines Division

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

23-26 June 2020

## Opinions on paediatric investigation plans

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

- Fasinumab, EMEA-002059-PIP02-19, from Regeneron Ireland D.A.C., for the treatment of chronic musculoskeletal pain and treatment of chronic non-musculoskeletal pain;
- Cilofexor, EMEA-002554-PIP02-19, from Gilead Sciences International Ltd., for the treatment of primary sclerosing cholangitis;
- Guselkumab, EMEA-001523-PIP05-19, from Janssen-Cilag International N.V., for the treatment of Crohn's Disease;
- Resamirigene bilparvovec, EMEA-002571-PIP01-19, from Audentes Therapeutics, Inc., for the treatment of X-linked myotubular myopathy;
- Soticlestat, EMEA-002572-PIP02-19, from Takeda Pharma A/S, for the treatment of Dravet syndrome and treatment of Lennox-Gastaut syndrome;
- (R)-1-(1-acryloylpiperidin-3-yl)-4-amino-3-(4-phenoxyphenyl)-1H-imidazo[4,5-c]pyridin-2(3H)-one, EMEA-002566-PIP01-19, from Genzyme Europe B.V., for the treatment of multiple sclerosis;
- Darunavir / Ritonavir, EMEA-002537-PIP02-19, from PharOS - Pharmaceutical Oriented Services Ltd, for the treatment of human immunodeficiency virus (HIV-1) infection;
- Marstacimab, EMEA-002285-PIP02-19, from Pfizer Europe MA EEIG, for the treatment of congenital haemophilia A and treatment of congenital haemophilia B;
- Lonapegsomatropin, EMEA-002692-PIP01-19, from Ascendis Pharma Endocrinology Division A/S, for the treatment of growth hormone deficiency;
- Rilzabrutinib, EMEA-002438-PIP02-19, from Principia Biopharma, Inc., for the treatment of immune thrombocytopenia;

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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:

- Sasanlimab, EMEA-002777-PIP01-20, from Pfizer Europe MA EEIG, for the treatment of ureter and bladder carcinoma;
- Ombitasvir / paritaprevir / ritonavir, EMEA-001440-PIP01-13-M03, from AbbVie Ltd, for the treatment of chronic hepatitis C;
- Oportuzumab monatox, EMEA-002797-PIP01-20, from Sesen Bio, Inc, for the treatment of urothelial carcinoma;
- Reslizumab, EMEA-001202-PIP02-13-M04, from Teva Pharmaceuticals Europe, for the treatment of asthma;
- Sacubitril/valsartan, EMEA-000316-PIP03-20, from Novartis Europharm Ltd., for the prevention of heart failure;
- Pravastatin sodium / ezetimibe, EMEA-002805-PIP01-20, from Laboratoires SMB S.A., for the treatment of hypercholesterolaemia and prevention of cardiovascular events;
- Nivolumab, EMEA-001407-PIP03-20, from Bristol-Myers Squibb Pharma EEIG, for the treatment of all conditions in the category of malignant neoplasms (except central nervous system neoplasms, haematopoietic and lymphoid tissue neoplasms other than Hodgkin lymphoma);
- Dupilumab, EMEA-001501-PIP05-20, from Regeneron Ireland DAC, for the treatment of Bullous Pemphigoid;
- Half-life extended bispecific T-cell engager (BiTE) antibody construct that binds to prostate-specific membrane antigen and cluster of differentiation 3, with a single chain fragment crystallizable moiety, EMEA-002655-PIP02-20, from Amgen Europe BV, for the treatment of prostate cancer;
- Elafibranor, EMEA-001857-PIP02-20, from Genfit SA, for the treatment of primary biliary cholangitis;
- Benralizumab, EMEA-001214-PIP06-20, from AstraZeneca AB, for the treatment of bullous pemphigoid;
- Balixafortide, EMEA-002718-PIP02-20, from Polyphor Deutschland GmbH, for the treatment of breast cancer;
- Dasabuvir (sodium monohydrate), EMEA-001439-PIP01-13-M03, from AbbVie Ltd, for the treatment of chronic hepatitis C;
- Rimegepant, EMEA-002812-PIP01-20, from Biohaven Pharmaceuticals, Inc., for the treatment of prevention of migraine headaches.

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:

- Purified Rabies virus, WISTAR PM/WI 38-1503-3M strain (inactivated), EMEA-002234-PIP01-17-M01, from Sanofi Pasteur, for the prevention of rabies viral infection;
- Ustekinumab, EMEA-000311-PIP04-13-M03, from Janssen-Cilag International NV, for the treatment of Crohn's Disease;
- Beclometasone dipropionate / formoterol fumarate dihydrate / glycopyrronium bromide (CHF 5993), EMEA-001875-PIP02-18-M02, from Chiesi Farmaceutici S.p.A., for the treatment of asthma;
- Perampanel, EMEA-000467-PIP01-08-M14, from Eisai Europe Limited, for the treatment of treatment-resistant epilepsies;
- Efpeglenatide, EMEA-001903-PIP01-15-M01, from Sanofi-aventis recherche et développement, for the treatment of type 2 diabetes mellitus;
- Ladarixin, EMEA-002642-PIP01-19-M01, from Dompé farmaceutici S.p.A, for the treatment of type 1 diabetes mellitus;
- Ceftobiprole medocaril (sodium), EMEA-000205-PIP02-11-M04, from Basilea Pharmaceutica International Ltd., for the treatment of pneumonia;
- Ebola Zaire Vaccine (rVSVΔG-ZEBOV-GP, live), EMEA-001786-PIP01-15-M01, from Merck Sharp & Dohme (Europe), Inc., for the treatment of prevention of Ebola disease;
- Crisaborole, EMEA-002065-PIP01-16-M03, from Pfizer Europe MA EEIG, for the treatment of atopic dermatitis;
- Ustekinumab, EMEA-000311-PIP05-17-M01, from Janssen-Cilag International NV, for the treatment of Ulcerative Colitis;
- Relebactam monohydrate / cilastatin sodium / imipenem monohydrate, EMEA-001809-PIP01-15-M02, from Merck Sharp & Dohme (Europe), Inc., for the treatment of infections caused by gram-negative organisms;
- Belatacept, EMEA-000157-PIP01-07-M05, from Bristol-Myers Squibb Pharma EEIG, for the prevention of rejection of transplanted kidney;
- Glucagon, EMEA-001657-PIP01-14-M01, from Eli Lilly and Company, for the treatment of hypoglycemia;
- Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues, EMEA-001855-PIP01-15-M02, from Genzyme Europe B.V., for the treatment of Haemophilia A and treatment of Haemophilia B;

- Fosnetupitant / palonosetron, EMEA-001198-PIP03-17-M04, from Helsinn Birex Pharmaceuticals Limited, for the prevention of chemotherapy-induced nausea and vomiting;
- Brincidofovir, EMEA-001904-PIP03-18-M01, from Chimerix IRL Limited, for the treatment of smallpox;
- Olenasufligene relduparvovec, EMEA-002122-PIP02-17-M01, from LYSOGENE, for the treatment of mucopolysaccharidosis type IIIA;
- Pitolisant, EMEA-001176-PIP01-11-M05, from Bioprojet PHARMA, for the treatment of narcolepsy;
- Rilpivirine (hydrochloride), EMEA-000317-PIP01-08-M12, from Janssen-Cilag International NV, for the treatment of human immunodeficiency virus (HIV-1) infection;
- Concentrate of proteolytic enzyme in bromelain, EMEA-000142-PIP02-09-M09, from MediWound Germany GmbH, for the treatment of burns;
- Lasmiditan, EMEA-002166-PIP01-17-M04, from Eli Lilly and Company Limited, for the migraine headaches;
- Dermatophagoides pteronyssinus / Dermatophagoides farinae, EMEA-001258-PIP01-11-M06, from ALK-Abelló A/S, for the treatment of treatment of asthma and allergic rhinitis;
- Recombinant human glutamic acid decarboxylase (rhGAD65), EMEA-000609-PIP01-09-M02 from Diamyd Medical AB for the treatment of type 1 diabetes mellitus;

The PDCO adopted opinions on the **refusal** of modifications to an agreed PIP for the following applications:

- Derivative of 4H-pyrazolo[3,4-d]pyrimidin-4-one, EMEA-001742-PIP01-14-M01, from Boehringer Ingelheim International GmbH, for the treatment of schizophrenia

## Opinion on compliance check

No items.

## Withdrawals

The PDCO noted that 4 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 virtual on 21-24 July.

## – 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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