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Paediatric Committee (PDCO)

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

12-14 November 2014

## Opinions on paediatric investigation plans

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

- Mepolizumab, from GSK Trading Services Limited, for the treatment of vasculitides;
- Covalently closed DNA plasmids coding for cytomegalovirus phosphoprotein 65 and glycoprotein B genes (ASP0113), from Astellas Pharma Europe B.V., for the prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity;
- Human immunoglobulin G2Lambda monoclonal antibody directed against thymic stromal lymphopoietin (MEDI9929), from MedImmune, Ltd, for the treatment of asthma;
- Clostridium difficile toxin B human monoclonal antibody, from Merck Sharp & Dohme (Europe), Inc., for the treatment of Clostridium difficile infection.

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:

- Ibandronic acid (in combination with calcium (carbonate) / cholecalciferol), from Gedeon Richter Plc., for the combination treatment of osteoporosis;

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- Urofollitropin, from Regiomedica GmbH, for the treatment of female infertility and treatment of hypogonadotrophic hypogonadism;
- Calcium (carbonate) / cholecalciferol (in combination with ibandronic acid), from Gedeon Richter Plc., for the combination treatment of osteoporosis;
- Saxagliptin / dapagliflozin, from AstraZeneca AB, for the treatment of type 2 diabetes mellitus;
- Amlodipine / Ramipril, from Helm Portugal, Lda., for the treatment of hypertension;
- Zoledronic acid (monohydrate) (in combination with calcium (carbonate) / cholecalciferol), from Gedeon Richter Plc., for the prevention of skeletal related events in patients with advanced malignancies involving bone;
- Calcium (carbonate) / cholecalciferol (in combination with zoledronic acid (monohydrate)), from Gedeon Richter Plc., for the prevention of skeletal related events in patients with advanced malignancies involving bone.

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:

- Liraglutide, from Novo Nordisk A/S, for the treatment of type 2 diabetes mellitus;
- Bilastine, from Faes Farma S.A., for the treatment of allergic rhinoconjunctivitis and treatment of urticaria;
- Human normal immunoglobulin, from Kedrion S.p.A., for the treatment of primary immunodeficiency (PID);
- Posaconazole, from Merck Sharp & Dohme (Europe), Inc., for the prevention of invasive fungal infections and treatment of invasive fungal infections;
- Vedolizumab, from Takeda Pharma A/S, for the treatment of Crohn's disease and treatment of ulcerative colitis;
- Nonacog beta pegol, from Novo Nordisk A/S, for the treatment of hereditary factor IX deficiency;
- Drisapersen, from Prosensa Therapeutics B.V., for the treatment of Duchenne muscular dystrophy;
- Pollen from Dactylis glomerata, Lolium perenne, Phleum pratense, Festuca pratensis, Secale cereale, from ALK-Abelló A/S, for the treatment of allergic rhinitis / rhino-conjunctivitis;
- Pollen from Phleum pratense, from ALK-Abelló A/S, for the treatment of allergic rhinitis / rhino-conjunctivitis;
- Deferiprone, from Consorzio per Valutazioni Biologiche e Farmacologiche, for the treatment of chronic iron overload;

- Dabrafenib, from GlaxoSmithKline Trading Service Limited, for the treatment of melanoma and treatment of solid malignant tumours (excluding melanoma);
- Serelaxin, from Novartis Europharm Ltd., for the treatment of acute heart failure;
- Human fibrinogen, from Octapharma Pharmazeutika Produktionsges.m.b.H, for the treatment of congenital fibrinogen deficiency;
- Recombinant single chain coagulation factor VIII, from CSL Behring GmbH, for the treatment of congenital factor VIII deficiency;
- Tolvaptan, from Otsuka Pharmaceutical Europe Ltd., for the treatment of dilutional hyponatraemia and treatment of polycystic kidney disease;
- Lumacaftor / ivacaftor, from Vertex Pharmaceuticals (Europe) Ltd., for the treatment of cystic fibrosis.

## Opinion on compliance check

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

- Eltrombopag, from GlaxoSmithKline Trading Services Limited, for the treatment of idiopathic thrombocytopenia purpura (ITP);
- Insulin detemir, from Novo Nordisk A/S, for the treatment of type I diabetes mellitus and treatment of type II diabetes mellitus;
- Autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA, from GlaxoSmithKline Trading Services Limited, for the treatment of severe combined immunodeficiency due to adenosine deaminase deficiency;
- Glycopyrronium (bromide), from Proveca Limited, for the treatment of sialorrhoea.

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

## Informal meeting

On 15-17 October 2014, the PDCO held a joint informal meeting with the Scientific Advice Working Party in Rome, under the auspice of the Italian Presidency of the Council of the European Union, to review the work done and the processes put in place during the past year. The PDCO discussed improvements in the functioning of the PDCO, in particular interactions with the Scientific Advice Working Party, and aspects relevant to the future European Commission Report which will be prepared ten years after adoption of the Paediatric Regulation.

## Other matters

The PDCO welcomed Brian Aylward in his new role as member, nominated to represent Ireland.

The PDCO thanked Kevin Connolly for his work as he has resigned from the Committee.

The PDCO welcomed Jaroslav Sterba in his new role as member and Peter Szitanyi in his new role as alternate, nominated to represent the Czech Republic.

The PDCO thanked Marina Fertek for her work at the end of her mandate as alternate.

The next meeting of the PDCO will be held on 10-12 December 2014.

## – 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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