EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Paediatric Committee (PDCO)

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

13-15 August 2014

Opinions on paediatric investigation plans

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

- Potassium citrate monohydrated / potassium hydrogen carbonate (ADV7103), from Advicenne Pharma, for the treatment of cystinuria;
- Brodalumab, from Amgen Europe B.V., for the treatment of psoriasis;
- Expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue (Cx601), from TiGenix, S.A.U., for the treatment of anal fistula;
- Nanobody directed towards the fusion protein of human respiratory syncytial virus (ALX-0171), from Ablynx NV, for the treatment of lower respiratory tract disease caused by human respiratory syncytial virus (RSV)
- D-Argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416), from Amgen Europe B.V., for the treatment of hyperparathyroidism;
- Solithromycin, from Triskel EU Services, Ltd, for the treatment of bacterial pneumonia and treatment of gonococcal infection;
- Recombinant soluble fusion protein with a modified form of the extracellular domain of human activin receptor IIB linked to the human IgG1 Fc domain (ACE-536), from Acceleron Pharma, Inc., for the treatment of myelodysplastic syndromes and treatment of beta-thalassaemia;
- Ibuprofen (sodium dihydrate), from Proveca Limited, for the treatment of pain and treatment of fever;
- Eltrombopag (olamine), from GlaxoSmithKline Trading Services Limited, for the treatment of aplastic anaemia;
- Potassium citrate monohydrated / Potassium hydrogen carbonate, from Advicenne Pharma, for the treatment of renal tubular acidosis.

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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 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:

- Edoxaban (tosylate), from Daiichi Sankyo Development Limited, for the prevention of arterial thromboembolism, treatment of venous thromboembolism and prevention of venous thromboembolism;
- Migalastat (hydrochloride), from Amicus Therapeutics UK Ltd, for the treatment of Fabry disease;
- Tenofovir (disoproxil fumarate), from Gilead Sciences International Limited, for the treatment of human immunodeficiency virus (HIV) disease resulting in other conditions and treatment of chronic viral hepatitis B;
- Dabigatran etexilate, from Boehringer Ingelheim International GmbH, for the prevention of thromboembolic events and treatment of thromboembolic events;
- Amikacin (sulfate), from Insmed Incorporated, for the treatment of Pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients and treatment of nontuberculous mycobacterial (NTM) lung infection;
- Brentuximab vedotin, from Takeda Pharma A/S, for the treatment of Hodgkin lymphoma and treatment of anaplastic large cell lymphoma;
- Mepolizumab, from GSK Trading Services Limited, for the treatment of asthma;
- Secukinumab, from Novartis Europharm Limited, for the treatment of chronic idiopathic arthritis (including rheumatoid arthritis, spondyloarthritis, psoriatic arthritis and juvenile idiopathic arthritis);
- Tedizolid (phosphate), from Cubist Pharmaceuticals (UK) Limited, for the treatment of complicated skin and soft tissue infections;
- 7-[4-(4-Benzo[b]thiophen-4-ylpiperazin-1-yl)butoxy]quinolin-2(1H)-one (OPC-34712), from Otsuka Europe Development and Commercialisation Ltd, Zweigniederlassung Frankfurt am Main, for the treatment of schizophrenia;
- Alogliptin, from Takeda Development Centre Europe Ltd, for the treatment of type 2 diabetes mellitus;
- Metformin (hydrochloride), from EffRx Pharmaceuticals SA, for the treatment of polycystic ovary syndrome;
- Sirukumab, from Janssen-Cilag International NV, for the treatment of chronic idiopathic arthritis (including rheumatoid arthritis, ankylosing spondylarthritis, psoriatic arthritis and juvenile idiopathic arthritis).

The PDCO adopted 1 opinion on the **refusal** of modifications to an agreed PIP for:

- Sunitinib, from Pfizer Limited, for the treatment of gastro-intestinal stromal tumour.

Hydroxyzine hydrochloride

The PDCO members adopted their response to the PRAC List of Questions on the use of hydroxyzine hydrochloride containing products in the paediatric population.

Withdrawals

The PDCO noted that 7 applications were withdrawn during the late stages of the evaluation (30 days or less before completion of the procedure).

Other matters

The PDCO welcomed Riccardo Riccardi, Antje Christin Neubert, Jan Taminiau in their new role as members, nominated to represent healthcare professionals.

The PDCO welcomed Maria Grazia Valsecchi and Doina Plesca in their new role as alternates, nominated to represent healthcare professionals.

The PDCO welcomed Günther Auerswald in his new role as member, nominated to represent patients' organisations.

The PDCO welcomed Paola Baiardi and Kerry Leeson-Beevers in their new role as alternates, nominated to represent patients' organisations.

The PDCO thanked Anthony Nunn, Adriana Ceci and Jean-Pierre Aboulker for their work at the end of their mandate.

The PDCO thanked Alexandra Compagnucci for her work at the end of her mandate.

The PDCO thanked Mathias Keller, Gerard Nguyen for their work at the end of their mandate.

The PDCO thanked Gerlind Bode for her work at the end of her mandate.

The next meeting of the PDCO will be held on 10-12 September 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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