22 November 2012
EMA/COMP/751952/2012
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 5-6 December 2012 meeting

Chair – Bruno Sepodes, Vice-Chair – Lesley Greene

Note on access to documents

The procedures discussed by the COMP are on-going and therefore are considered confidential. Additional details on these procedures will be disclosed in the COMP meeting reports (after the COMP opinion is adopted). Documents mentioned in the agenda cannot be released at present as they are currently in draft format or are classified as confidential. They will become public when adopted in their final form or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

- Adoption of the draft Agenda.
- Adoption of the draft Minutes from the previous meeting.
- Declaration of conflicts of interest.

2. Applications for orphan medicinal product designation

2.1. For opinion

- Treatment of complex regional pain syndrome, OD/125/12.
- Treatment of familial adenomatous polyposis, OD/130/12.
- Treatment of growth hormone deficiency, OD/133/12.

2.2. For discussion / for opinion

- Treatment of 5q spinal muscular atrophy, OD/140/12.
- Treatment of achondroplasia, OD/149/12.
- Treatment of beta-thalassemia intermedia and major, OD/138/12.
- Treatment of beta-thalassemia intermedia and major, OD/146/12.
- Treatment of follicular lymphoma, OD/158/12.
- Treatment of glioma, OD/148/12.
- Treatment of haemophilia A, OD/152/12.
- Treatment of haemophilia B, OD/151/12.
- Treatment of high altitude pulmonary oedema, OD/144/12.
- Treatment of inoperable chronic thromboembolic pulmonary hypertension, OD/154/12.
- Treatment of moderate and severe traumatic brain injury, OD/141/12.
- Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome), OD/150/12.
- Treatment of myelodysplastic syndromes, OD/139/12.
- Treatment of ovarian cancer, OD/147/12.
- Treatment of pancreatic cancer, OD/145/12.
- Treatment of retinitis pigmentosa, OD/159/11.
- Treatment of systemic sclerosis, OD/153/12.
- Treatment of Wilson's disease, OD/142/12.

2.3. Evaluation on-going

Twelve applications for orphan designation will not be discussed as evaluation is on-going.

2.4. Validation on-going

Validation is on-going for seventeen applications for orphan designation.

3. Requests for protocol assistance

- Treatment of cystic fibrosis.
- Treatment of pancreatic cancer.

4. Overview of applications

- Update on applications for orphan medicinal product designation submitted/expected.
- Update on orphan applications for marketing authorisation.

5. Review of orphan designation for orphan medicinal products for marketing authorisation

5.1. Orphan designated products for which CHMP opinions have been adopted

5.1.1 Istodax ((E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia-5,8,20,23- tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone) for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated); Celgene Europe Limited (OD/056/05, EU/3/05/328).

5.2. Orphan designated products for discussion prior to adoption of CHMP opinion

5.2.1 Bosulif (Bosutinib) for treatment of chronic myeloid leukaemia; Pfizer Limited (OD/160/09, EU/3/10/762).

5.2.2 Jenzyl ((1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate); Merck Sharp & Dohme Limited

- treatment of soft tissue sarcoma (OD/050/05, EU/3/05/312);
- treatment of primary malignant bone tumours (OD/055/05, EU/3/05/321).

5.3. On-going procedures

5.3.1 Bedaquiline ((1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethano) for treatment of tuberculosis; Janssen-Cilag International N.V. (OD/024/05, EU/3/05/314).

5.3.2 Cholic Acid FGK for treatment of inborn errors of primary bile acid synthesis responsive to treatment with cholic acid; FGK Representative Service GmbH (OD/080/09, EU/3/09/683).

5.3.3 Cysteamine bitartrate [Cysteamine bitartrate (gastroresistant)] for treatment of cystinosis; Raptor Pharmaceuticals Europe B.V. (OD/034/10, EU/3/10/778).

5.3.4 Defitelio (Defibrotide); Gentium S.p.A.

- prevention of hepatic veno-occlusive disease (OD/025/04, EU/3/04/211);
- treatment of hepatic veno-occlusive disease (OD/026/04, EU/3/04/212).

5.3.5 Delamanid ((R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole) for treatment of tuberculosis; Otsuka Novel Products GmbH (OD/094/07, EU/3/07/524).

5.3.6 Iclusig (benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]); ARIAD Pharma Ltd

- treatment of acute lymphoblastic leukaemia (OD/122/09, EU/3/09/715);
- treatment of chronic myeloid leukaemia (OD/121/09, EU/3/09/716).

5.3.7 Kinaction (Masitinib mesilate) for treatment of pancreatic cancer; AB Science (OD/063/09, EU/3/09/684).

5.3.8 Loulla (Mercaptopurine) for treatment of acute lymphatic leukaemia, Only For Children Pharmaceuticals (OD/065/07, EU/3/07/496).

5.3.9 PAS-GR (Para-aminosalicylic acid) for treatment of tuberculosis; Lucane Pharma SA (OD/072/10, EU/3/10/826).

5.3.10 Pheburane (Sodium phenylbutyrate) for treatment of carbamoyl-phosphate synthase-1 deficiency; Lucane Pharma SA (OD/098/11, EU/3/12/951).

5.3.11 Pomalidomide Celgene (Pomalidomide) for treatment of multiple myeloma, Celgene Europe Ltd. (OD/053/09, EU/3/09/672).

5.3.12 Raxone (previously SAN Idebenone; Idebenone) for treatment of Leber's hereditary optic neuropathy; Santhera Pharmaceuticals (Deutschland) GmbH (OD/076/06, EU/3/07/434).

5.3.13 Revlimid (3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) for treatment of myelodysplastic syndromes; Celgene Europe Limited – UK (OD/083/03, EU/3/04/192).

5.3.14 Scenesse ([Nle4, D-Phe7]-alfa-melanocyte stimulating hormone, Afamelanotide) for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (OD/108/07, EU/3/08/541).

5.3.15 Masican N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole for treatment of malignant gastrointestinal stromal tumours; AB Science (OD/061/04, EU/3/04/251).

5.3.16 Winfuran (-)-17(cyclopropylmethyl)-1,14 ß-dihydroxy-4,5 alpha-epoxy-6ß-[N-methyl-trans-3-(3-furyl) acrylamido] morphinan hydrochloride for treatment of uremic pruritus; Toray International U.K. Limited (OD/020/02, EU/3/02/115).

5.3.17 Vantobra, tobramycin (inhalation use) for treatment of *Pseudomonas Aeruginosa* lung infection in cystic fibrosis; PARI Pharma GmbH (OD/094/08, EU/3/09/613).

6. Procedural aspects

6.1 Appointment of the COMP representatives to the EMA Scientific Advice Working Party (SAWP) to be held at the January 2013 meeting

http://www.ema.europa.eu/ema/index.jsp?curl=pages/contacts/CHMP/people_listing_000022.jsp&mid=WC0b01ac0580028d94

7. Any other business

7.1 COMP Informal Meeting held on 22-23 November 2012 in Rome.

7.2 Managing Meeting Documents (MMD).