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EMA/COMP/792332/2012
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 8-9 January 2013 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 beta-thalassemia intermedia and major, OD/138/12.
- 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 myelodysplastic syndromes, OD/139/12.

2.2. For discussion / for opinion

- Prevention of graft rejection following solid organ transplant, OD/165/12.
- Treatment of achromatopsia caused by mutations in the CNGB3 gene, OD/109/12.
- Treatment of acute myeloid leukemia, OD/167/12.
- Treatment of amyloid light chain AL amyloidosis, OD/129/12.
- Treatment of autosomal dominant polycystic kidney disease, OD/163/12.
- Treatment of chronic non-infectious uveitis, OD/161/12.
- Treatment of Duchenne muscular dystrophy, OD/164/12.
- Treatment of emphysema secondary to congenital alpha-1 antitrypsin deficiency, OD/166/12.
- Treatment of glioma, OD/157/12.
- Treatment of hepatocellular carcinoma, OD/159/12.
- Treatment of Niemann-Pick's disease, type C, OD/160/12.
- Treatment of sickle cell disease, OD/162/12.
- Treatment of systemic sclerosis, OD/156/12.
- Treatment of X-linked juvenile retinoschisis (XLRS), OD/108/12.

2.3. Evaluation on-going

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

2.4. Validation on-going

Validation is on-going for twenty two applications for orphan designation.

3. Requests for protocol assistance

- Treatment of pouchitis.
- Treatment of acute myeloid leukaemia.

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

5.2. 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 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).

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

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 Cometriq [Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt] for treatment of medullary thyroid carcinoma; TMC Pharma Services Ltd (OD/088/08, EU/3/08/610).

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

5.3.5 Delamanid ((R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phoxymethyl}-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 Exjade (4-(3,5-bis(hydroxy-phenyl)-1,2,4) triazol-1-yl) benzoic acid) for treatment of chronic iron overload requiring chelation therapy; Novartis Europharm Limited (OD/061/01, EU/3/02/092) Type II variation – new indication: treatment of infrequently transfused beta-thalassemia major patients.

5.3.7 Folcepri (N-[4-[[2-amino-3,4-dihydro-4-oxo-6-pteridiny]methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine to be used with folic acid) for diagnosis of positive folate receptor status in ovarian cancer, Endocyte Europe, B.V. (OD/055/12, EU/3/12/1043)

5.3.8 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).

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

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

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

5.3.12 Neocepri (Folic acid to be used with N-[4-[[2-amino-3,4-dihydro-4-oxo-6-pteridiny]methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine) for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (OD/056/12, EU/3/12/1044)

5.3.13 Opsumit (Macitentan) for treatment of pulmonary arterial hypertension; Actelion Registration Ltd. (OD/023/11, EU/3/11/909).

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

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

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

5.3.17 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).

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

5.3.19 Translarna (3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid) for treatment of Duchenne muscular dystrophy; PTC Therapeutics Ltd (OD/106/04, EU/3/05/278).

5.3.20 Vynfinit (Vincaläuboblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[[(2-amino-3,4-dihydro-4-oxo-6-pteridiny]methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine) for treatment of ovarian cancer; Endocyte Europe, B.V. (OD/094/11, EU/3/12/959).

5.3.21 Winfuran (-)-17(cyclopropylmethyl)-1,14 β-dihydroxy-4,5 alpha-epoxy-6β-[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).

5.3.22 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)

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 COMP informal meeting to be held on 28 February - 1 March 2013 in Dublin.

7.3 COMP Work Programme 2013-2015.