25 January 2013
EMA/COMP/20458/2013
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 5-6 February 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).

Contents

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1. Introduction

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

2. Applications for orphan medicinal product designation

2.1. For opinion

- For prevention of graft rejection following solid organ transplant - EMA/OD/165/12.
- For treatment of autosomal dominant polycystic kidney disease - EMA/OD/163/12.
- For treatment of chronic non-infectious uveitis - EMA/OD/161/12.
- For treatment of emphysema secondary to congenital alpha-1 antitrypsin deficiency - EMA/OD/166/12.
- For treatment of glioma - EMA/OD/157/12.
- For treatment of hepatocellular carcinoma - EMA/OD/159/12.
- For treatment of Niemann-Pick disease - EMA/OD/160/12.
- For treatment of sickle cell disease - EMA/OD/162/12.
- For treatment of X-linked juvenile retinoschisis (XLRS) - EMA/OD/108/12.

2.2. For discussion / preparation for an opinion

- For diagnosis of neuroendocrine tumours - EMA/OD/181/12.
- For treatment of chronic inflammatory demyelinating polyneuropathy - EMA/OD/169/12.
- For treatment of Churg-Strauss syndrome - EMA/OD/174/12.
- For treatment of differentiated thyroid cancer - EMA/OD/173/12.
- For treatment of epidermolysis bullosa - EMA/OD/180/12.
- For treatment of glioma - EMA/OD/170/12.
- For treatment of mantle cell lymphoma - EMA/OD/171/12.
- For treatment of neuroendocrine tumours - EMA/OD/185/12.
- For treatment of neuronal ceroid lipofuscinosis type 2 (NCL2) - EMA/OD/177/12.
- For treatment of non-small-cell lung cancer in patients expressing HLA-A2 - EMA/OD/168/12.
- For treatment of osteonecrosis of the femoral head - EMA/OD/176/12.
- For treatment of pancreatic cancer - EMA/OD/178/12.

- For treatment of *Pseudomonas aeruginosa* infection in cystic fibrosis - EMA/OD/179/12.
- For treatment of Stargardt's disease - EMA/OD/175/12.
- For treatment of systemic sclerosis - EMA/OD/143/12.

2.3. Appeal procedure

- For treatment of complex regional pain syndrome - EMA/OD/125/12.

2.4. Evaluation on-going

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

2.5. Validation on-going

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

3. Requests for protocol assistance

- Treatment of granulomatosis with polyangiitis (Wegener Granulomatosis).

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 Bosulif (Bosutinib) for treatment of chronic myeloid leukaemia; Pfizer Limited (EU/3/10/762).

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

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

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

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. (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 (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 (EU/3/08/610).

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

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

- prevention of hepatic veno-occlusive disease (EU/3/04/211)
- treatment of hepatic veno-occlusive disease (EU/3/04/212).

5.3.6 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 (EU/3/07/524).

5.3.7 Dexamethasone (40 mg tablet) for treatment of multiple myeloma; Laboratoires CTRS (Cell Therapies Research & Services) (EU/3/10/745).

5.3.8 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 (EU/3/02/092). Type II variation - for the treatment of chronic iron overload due to blood transfusions in patients with beta thalassaemia major aged 6 years and older.

5.3.9 Folcepri (N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)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. (EU/3/12/1043).

5.3.10 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 chronic myeloid leukaemia (EU/3/09/716)
- treatment of acute lymphoblastic leukaemia (EU/3/09/715).

5.3.11 Kinaction (Masitinib mesilate) for treatment of pancreatic cancer; AB Science (EU/3/09/684).

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

5.3.13 Neocepri (Folic acid to be used with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)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. (EU/3/12/1044).

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

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

5.3.16 Pomalidomide Celgene (Pomalidomide) for treatment of multiple myeloma; Celgene Europe Ltd. (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 (EU/3/04/192).

5.3.18 Scenesse ([Nle4, D-Phe7]-alfa-melanocyte stimulating hormone, Afamelanotide) for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (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 (EU/3/05/278).

5.3.20 Vynfinit (Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)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. (EU/3/12/959).

5.3.21 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 (EU/3/02/115).

5.3.22 Vantobra, Tobramycin (inhalation use) for treatment of Pseudomonas Aeruginosa lung infection in cystic fibrosis; PARI Pharma GmbH (EU/3/09/613).

5.4. Withdrawn procedures

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

6. Procedural aspects

6.1 Appointment of the 3rd COMP representative to the EMA Scientific Advice Working Party (SAWP)

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.