27 September 2013
EMA/COMP/590870/2013
Human Medicines Research and Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 8-10 October 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 of the previous meeting
- Declaration of conflicts of interest

2. Applications for orphan medicinal product designation

2.1. For 2nd discussion / an opinion

- Prevention of graft-versus-host disease - EMA/OD/103/13
- Treatment of acromegaly - EMA/OD/082/13
- Treatment of adult onset still's disease - EMA/OD/099/13
- Treatment of Fabry disease - EMA/OD/100/13
- Treatment of follicular thyroid cancer - EMA/OD/092/13
- Treatment of glioma - EMA/OD/086/13
- Treatment of mucopolysaccharidosis type II (Hunter's syndrome) - EMA/OD/091/13
- Treatment of neurotrophic keratitis - EMA/OD/184/12
- Treatment of papilary thyroid cancer - EMA/OD/093/13

2.2. For discussion / preparation for an opinion

- Prevention of arteriovenous access dysfunction in patients undergoing surgical creation of an arteriovenous access for haemodialysis - EMA/OD/117/13
- Prevention of necrotizing enterocolitis - EMA/OD/112/13
- Treatment of Alagille syndrome - EMA/OD/120/13
- Treatment of diffuse large B-cell Lymphoma - EMA/OD/116/13
- Treatment of eosinophilic esophagitis - EMA/OD/118/13
- Treatment of follicular lymphoma - EMA/OD/111/13
- Treatment of fragile X syndrome - EMA/OD/114/13
- Treatment of glioma - EMA/OD/107/13
- Treatment of graft-versus-host disease - EMA/OD/126/13
- Treatment of hepatocellular carcinoma - EMA/OD/115/13
- Treatment of hypoparathyroidism - EMA/OD/102/13
- Treatment of non-small cell lung carcinoma that is anaplastic lymphoma kinase-positive - EMA/OD/113/13

- Treatment of ovarian cancer - EMA/OD/122/13
- Treatment of primary biliary cirrhosis - EMA/OD/121/13
- Treatment of primary familial intrahepatic cholestasis - EMA/OD/123/13
- Treatment of primary sclerosing cholangitis - EMA/OD/127/13
- Treatment of spinal cord injury - EMA/OD/119/13
- Treatment of Stargardt's disease - EMA/OD/124/13

2.3. Evaluation on-going

Evaluation is on-going for 22 applications which will be discussed at the November meeting.

2.4. Validation on-going

Validation is on-going for 18 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of chronic iron overload requiring chelation therapy
- Treatment of chronic lymphocytic leukaemia
- Treatment of Fabry disease
- Treatment of graft-versus-host disease
- Treatment of hepatocellular carcinoma
- Treatment of malaria
- Treatment of mercury toxicity
- Treatment of ovarian cancer
- Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukemic/disseminated)
- Treatment of Wilson's disease

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

5.2.2 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)

5.2.3 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)

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

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

5.2.6 Sirturo - formerly 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)

5.2.7 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. On-going procedures

5.3.1 Adempas (Methyl 4,6-diamino-2-[1-(2-fluorobenzyl)-1H-pyrazolo[3,4-b]pyridine-3-yl]-5-pyrimidinyl(methyl)carbamate) for treatment of pulmonary arterial hypertension including treatment of chronic thromboembolic pulmonary hypertension; Bayer Pharma AG (EU/3/07/518)

5.3.2 Cyramza (Ramucirumab) for treatment of gastric cancer; Eli Lilly Nederland B.V. (EU/3/12/1004)

5.3.3 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)

5.3.4 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)

5.3.5 Gazyva (Obinutuzumab) for treatment of chronic lymphocytic leukaemia; Roche Registration Limited (EU/3/12/1054)

5.3.6 Holoclar (Ex vivo expanded autologous human corneal epithelium containing stem cells) for treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns; Chiesi Farmaceutici S.p.A. (EU/3/08/579)

5.3.7 Masiviera - formerly Kinaction (Masitinib mesilate) for treatment of pancreatic cancer; AB Science (EU/3/09/684)

5.3.8 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)

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

5.3.10 Scenesse ([Nle4, D-Phe7]-alfa-melanocyte stimulating hormone, Afamelanotide) for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (EU/3/08/54)

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

5.3.12 Vimizim (Recombinant human N-acetylgalactosamine-6-sulfatase) for treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome); BioMarin Europe Ltd (EU/3/09/657)

5.3.13 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)

5.3.14 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)

6. Procedural aspects

6.1 Validation issues for submitted application for treatment of cognitive disease in MPS II patients

7. Any other business

7.1 Reorganisation of the EMA

7.2 First PDCO/COMP workshop on conditions in rare diseases on 9 October 2013