28 October 2013
EMA/COMP/632148/2013
Human Medicines Research and Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 5-6 November 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 2nd discussion / an opinion

- Prevention of necrotizing enterocolitis - EMA/OD/112/13
- Treatment of Alagille syndrome - EMA/OD/120/13
- 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 hypoparathyroidism - EMA/OD/102/13
- Treatment of non-small cell lung carcinoma that is anaplastic lymphoma kinase-positive - EMA/OD/113/13
- 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

2.2. For discussion / preparation for an opinion

- Prevention of arteriovenous access failure in haemodialysis patients - EMA/OD/139/13
- Prevention of graft- versus-host disease - EMA/OD/146/13
- Prevention of neovascular glaucoma - EMA/OD/130/13
- Treatment of acute lymphoblastic leukaemia - EMA/OD/143/13
- Treatment of acute myeloid leukaemia - EMA/OD/141/13
- Treatment of acute myeloid leukaemia - EMA/OD/147/13
- Treatment of adenovirus infection in allogeneic haematopoietic stem cell transplant recipients - EMA/OD/135/13
- Treatment of ameloblastoma - EMA/OD/110/13
- Treatment of aneurysmal subarachnoid haemorrhage - EMA/OD/131/13

- Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma - EMA/OD/109/13
- Treatment of cystic fibrosis - EMA/OD/095/13
- Treatment of dengue fever - EMA/OD/142/13
- Treatment of Dravet syndrome - EMA/OD/140/13
- Treatment of epidermolysis bullosa - EMA/OD/145/13
- Treatment of haemophilia A - EMA/OD/144/13
- Treatment of hepatitis delta virus infection - EMA/OD/132/13
- Treatment of malignant mesothelioma - EMA/OD/138/13
- Treatment of plasma cell myeloma - EMA/OD/125/13
- Treatment of primary sclerosing cholangitis - EMA/OD/136/13
- Treatment of progesterone receptor negative endometrial cancer in combination with progestin therapy - EMA/OD/097/13
- Treatment of type 1 diabetes mellitus patients with residual beta-cell function - EMA/OD/075/13
- Treatment of type 1 diabetes mellitus patients with residual beta-cell function - EMA/OD/128/13

2.3. Evaluation on-going

Evaluation is on-going for 13 applications which will be discussed at the November meeting.

2.4. Validation on-going

Validation is on-going for 32 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of graft-versus-host disease
- Treatment of Fabry disease
- Treatment of chronic iron overload requiring chelation therapy

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

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

5.2.1 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)

5.2.2 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)

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

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

5.2.5 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)

5.2.6 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. 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 Cerdelga ((1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt) for treatment of Gaucher disease; Genzyme Europe BV (EU/3/07/514)

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

5.3.5 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)

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

5.3.7 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)

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

5.3.9 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)

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

5.3.11 Olaparib AstraZeneca AB (Olaparib) for treatment of ovarian cancer; AstraZeneca AB (EU/3/07/501)

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

5.3.13 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)

5.3.14 Sylvant (Chimeric-anti-interleukin-6 monoclonal antibody) for treatment of Castleman's disease; Janssen-Cilag International N.V.; (EU/3/07/508)

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

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

5.3.17 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)

6. Procedural aspects

6.1 The European Network of Centres for Pharmacoepidemiology & Pharmacovigilance (ENCePP)

6.2 Inter-Committee Scientific Advisory Group (IC-SAG) for Oncology

6.3 Creation of the inter-active PDCO/COMP working group on conditions in rare diseases

6.4 EMA policy on fee reductions for designated orphan medicinal products

7. Any other business

7.1 Proposal for a publication strategy (including book on rare diseases)

7.2 Grounds of major contribution to patient care

7.3 Similarity group

7.4 Public consultation on the *EC Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another* ENTR/6283/00 Rev 3.