7 January 2014
EMA/COMP/741437/2013
Human Medicines Research & Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 7-9 January 2014 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

- Diagnosis of gastro-entero-pancreatic neuroendocrine tumours - EMA/OD/152/13
- Prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection - EMA/OD/134/13
- Prevention of delayed graft function after renal transplantation - EMA/OD/154/13
- Treatment for acute myeloid leukaemia - EMA/OD/160/13
- Treatment of acute myeloid leukaemia - EMA/OD/150/13
- Treatment of cystic fibrosis - EMA/OD/156/13
- Treatment of cystic fibrosis - EMA/OD/159/13
- Treatment of epidermolysis bullosa - EMA/OD/149/13
- Treatment of recent-onset Type 1 Diabetes with residual beta cell function - EMA/OD/157/13

2.2. For discussion / preparation for an opinion

- Diagnosis of gastro-entero-pancreatic neuroendocrine tumours (GEP NETs) - EMA/OD/173/13
- Prevention of antibody-mediated rejection after solid organ transplantation - EMA/OD/176/13
- Prevention of bronchopulmonary dysplasia - EMA/OD/161/13
- Prevention of bronchopulmonary dysplasia in premature neonates of less than 32 weeks of gestational age - EMA/OD/172/13
- Prevention of graft rejection following solid organ transplantation - EMA/OD/168/13
- Treatment of adrenoleukodystrophy - EMA/OD/170/13
- Treatment of amyotrophic lateral sclerosis - EMA/OD/178/13
- Treatment of cystic fibrosis - EMA/OD/166/13
- Treatment of Duchenne muscular dystrophy - EMA/OD/162/13
- Treatment of familial amyloid polyneuropathy - EMA/OD/098/13
- Treatment of familial chylomicronemia syndrome - EMA/OD/180/13

- Treatment of Farber disease - EMA/OD/167/13
- Treatment of glioma - EMA/OD/174/13
- Treatment of glycogen storage disease type II (Pompe's disease) - EMA/OD/148/13
- Treatment of optic neuritis - EMA/OD/175/13
- Treatment of pancreatic cancer - EMA/OD/164/13
- Treatment of Polycythaemia vera - EMA/OD/169/13
- Treatment of Rett syndrome - EMA/OD/163/13
- Treatment of small cell lung cancer - EMA/OD/094/13
- Treatment of systemic sclerosis - EMA/OD/179/13

2.3. Appeal procedure

2.3.1 5-Chloro-N2-[2-isopropoxy-5-methyl-4-(4-piperidinyl)phenyl]-N4-[2-(isopropylsulfonyl)phenyl]-2,4-pyrimidinediamine for treatment of non-small cell lung carcinoma (NSCLC) that is anaplastic lymphoma kinase (ALK)-positive, Novartis Europharm Limited - EMA/OD/113/13

2.4. Evaluation on-going

Evaluation is on-going for 15 applications which will be discussed at the February meeting.

2.5. Validation on-going

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

3. Requests for protocol assistance

- Treatment of chronic lymphocytic leukaemia
- Treatment of follicular lymphoma
- Treatment of spinal cord injury
- Treatment of tuberculosis
- Treatment of acute lymphoblastic 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

5.1.1 Deltyba (previously 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)

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

5.1.3 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)

5.1.4 Sirturo [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)

5.1.5 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)

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

5.2.1 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)

5.2.2 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)

5.2.3 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)

5.2.4 Nexavar (Sorafenib tosylate), Bayer HealthCare AG, (Type II variation):

- treatment of follicular thyroid cancer (EU/3/13/1199)
- treatment of papillary thyroid cancer (EU/3/13/1200)

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

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

5.2.8 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. On-going procedures

5.3.1 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)

5.3.2 Corluxin (Mifepristone) for treatment of hypercortisolism (Cushing's syndrome) of endogenous origin; FGK Representative Service GmbH (EU/3/11/925)

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

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

5.3.5 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)

5.3.6 Imbruvica (1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one) for treatment of mantle cell lymphoma; Janssen-Cilag International N.V. (EU/3/13/1115)

5.3.7 Ketoconazole AID-SCFM for treatment of Cushing's syndrome; Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare (EU/3/12/1031)

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

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

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

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

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

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

6. Procedural aspects

6.1 Proposals for the COMP involvement in validation of public summaries of the COMP opinions on review of orphan designation

7. Any other business

7.1 Similarity group

- Similarity – orphan medicines EMA/84728/2013

7.2 Revision of 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.

7.3 European Conference for Rare Diseases (ECRD) to be held on 8-10 May 2014 in Berlin

7.4 Presentation on the EMA move to 30 Churchill Place.