2 May 2013
EMA/COMP/274020/2013
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 14-15 May 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

- For diagnosis of neuroendocrine tumours - EMA/OD/001/13
- For treatment of (localized) neuroendocrine tumours - EMA/OD/002/13
- For treatment of Alagille syndrome - EMA/OD/007/13
- For treatment of primary biliary cirrhosis - EMA/OD/010/13
- For treatment of primary sclerosing cholangitis - EMA/OD/008/13
- For treatment of progressive familial intrahepatic cholestasis - EMA/OD/009/13
- For treatment of Stargardt's disease - EMA/OD/004/13

2.2. For discussion / preparation for an opinion

- For acute lymphoblastic leukaemia - EMA/OD/024/13
- For prevention of graft-versus-host disease - EMA/OD/026/13
- For treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy - EMA/OD/022/13
- For treatment of acute liver failure - EMA/OD/032/13
- For treatment of amyotrophic lateral sclerosis - EMA/OD/011/13
- For treatment of amyotrophic lateral sclerosis - EMA/OD/023/13
- For treatment of cystic fibrosis - EMA/OD/017/13
- For treatment of Hunter syndrome - EMA/OD/021/13
- For treatment of lymphoblastic lymphoma - EMA/OD/027/13
- For treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome) - EMA/OD/035/13
- For treatment of narcolepsy - EMA/OD/029/13
- For treatment of pachyonychia congenital - EMA/OD/028/13
- For treatment of pulmonary alveolar proteinosis - EMA/OD/106/12
- For treatment of renal cell carcinoma - EMA/OD/030/13

- For treatment of renal cell carcinoma in patients with remaining primary tumour or presence of at least one metastasis - EMA/OD/172/12
- For treatment of retinitis pigmentosa - EMA/OD/025/13
- For treatment of retinitis pigmentosa - EMA/OD/031/13
- For treatment of retinitis pigmentosa - EMA/OD/067/13
- For treatment of soft tissue sarcoma - EMA/OD/041/13

2.3. COMP opinions adopted via written procedure following previous meeting

- **Maribavir** for treatment of cytomegalovirus disease, ViroPharma SPRL - EMA/OD/013/13

2.4. Evaluation on-going

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

2.5. Validation on-going

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

3. Requests for protocol assistance

- Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
- Treatment of neovascular glaucoma

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 Revlimid (3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) for treatment of myelodysplastic syndromes; Celgene Europe Limited – UK (EU/3/04/192)

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

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

5.2.2 Pomalidomide Celgene (Pomalidomide) for treatment of multiple myeloma; Celgene Europe Ltd. (EU/3/09/672)

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 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)

5.3.3 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)

5.3.4 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)

5.3.5 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 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)

5.3.9 GPLSCD01 (substance to be reviewed) (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)

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

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

5.3.12 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)

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

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

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

5.3.16 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)

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

5.3.18 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)

5.3.19 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 Report on fee reductions for orphan medicine

6.2 European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP)