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EMA/COMP/179739/2014
Human Medicines Research and Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 8-9 April 2014 meeting

Chair – Bruno Sepodes, Vice-Chair – Lesley Greene

Note on access to documents

Some documents mentioned in the agenda/minutes cannot be released at present following a request for access to documents under Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted 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 / opinion

- Treatment of biliary tree cancer - EMA/OD/199/13
- Treatment of dystrophic epidermolysis bullosa - EMA/OD/201/13
- Treatment of follicular lymphoma - EMA/OD/200/13
- Treatment of gastro-entero-pancreatic neuroendocrine tumours - EMA/OD/196/13
- Treatment of inherited retinal disease caused by lecithin: retinol acyltransferase (*LRAT*) or retinal pigment epithelium protein 65 (*RPE65*) mutations - EMA/OD/197/13

2.2. For discussion / preparation for an opinion

- Prevention of scarring in glaucoma filtration surgical procedure - EMA/OD/016/14
- Treatment of amyotrophic lateral sclerosis - EMA/OD/007/14
- Treatment of choroideremia - EMA/OD/015/14
- Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma - EMA/OD/195/13
- Treatment of cystic fibrosis - EMA/OD/002/14
- Treatment of cystic fibrosis - EMA/OD/013/14
- Treatment of glioma - EMA/OD/001/14
- Treatment of glioma - EMA/OD/003/14
- Treatment of Glucose Transporter Type-1 Deficiency Syndrome - EMA/OD/011/14
- Treatment of invasive aspergillosis - EMA/OD/009/14
- Treatment of ischaemic central retinal vein occlusion - EMA/OD/008/14
- Treatment of mucopolysaccharidosis IIIA - EMA/OD/006/14
- Treatment of mucormycosis - EMA/OD/010/14
- Treatment of non-infectious uveitis - EMA/OD/014/14
- Treatment of Stargardt's disease - EMA/OD/005/14
- Treatment of Usher syndrome - EMA/OD/004/14

2.3. Evaluation on-going

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

2.4. Validation on-going

Validation is on-going for 27 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of acute lymphoblastic leukaemia
- Treatment of ovarian cancer
- Treatment of primary myelofibrosis

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 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)

5.1.2 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)

5.1.3 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)

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

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

5.2.1 Sorafenib tosylate; Bayer HealthCare AG:

a) treatment of follicular thyroid cancer (EU/3/13/1199)

b) treatment of papillary thyroid cancer (EU/3/13/1200)

5.2.2 Pasireotide for treatment of Cushing's disease; Novartis Europharm Limited (EU/3/09/671)

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

5.3. On-going procedures

5.3.1 Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin for treatment of follicular lymphoma; Biovest Europe Ltd (EU/3/06/394)

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

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

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

5.3.6 Human heterologous liver cells (for infusion); Cytonet GmbH&Co KG

a) treatment of carbamoyl-phosphate synthase-1 deficiency (EU/3/10/821)

b) treatment of ornithine-transcarbamylase deficiency (EU/3/07/470)

c) treatment of citrullinaemia type 1 (EU/3/10/818)

d) treatment of hyperargininaemia (EU/3/10/819)

e) treatment of argininosuccinic aciduria (EU/3/10/820)

5.3.7 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 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)

5.3.9 Tolvaptan for treatment of autosomal dominant polycystic kidney disease; Otsuka Pharmaceutical Europe Ltd (EU/3/13/1175)

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

5.3.11 Ketoconazole for treatment of Cushing's syndrome; Laboratoire HRA (EU/3/12/965)

5.3.12 Levofloxacin hemihydrate for treatment of cystic fibrosis; Aptalis Pharma SAS (EU/3/08/566)

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

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

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

5.3.16 [Nle4, D-Phe7]-alfa-melanocyte stimulating hormone for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (EU/3/08/541)

5.3.17 L-Asparaginase for treatment of acute lymphoblastic leukaemia; medac Gesellschaft fuer klinische Spezialpraeparate mbH (EU/3/04/258)

5.3.18 (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)

5.3.19 Chimeric monoclonal antibody against GD2 for treatment of neuroblastoma; United Therapeutics Europe Ltd (EU/3/11/879)

6. Any other business

6.1 Committees Secretariat Service

6.2 4th presentation on the EMA move to 30 Churchill Place

6.3 Adaptive licencing