EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 11-13 November 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 within the framework of Regulation (EC) No 1049/2001 on access to documents because 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

- For prevention of graft-versus-host disease - EMA/OD/163/14
- For treatment of acute myeloid leukaemia - EMA/OD/156/14
- For treatment of familial cerebral cavernous malformations - EMA/OD/161/14
- For treatment of Huntington's disease - EMA/OD/114/14
- For treatment of mantle cell lymphoma - EMA/OD/151/14
- For treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome) - EMA/OD/164/14
- For treatment of myasthenia gravis - EMA/OD/119/14
- For treatment of pancreatic cancer - EMA/OD/143/14
- For treatment of pleural infection - EMA/OD/125/14

2.2. For discussion / preparation for an opinion

- For prevention of bronchopulmonary dysplasia - EMA/OD/183/14
- For treatment of acute myeloid leukaemia - EMA/OD/175/14
- For treatment of acute myeloid leukaemia - EMA/OD/188/14
- For treatment of Aicardi-Goutières syndrome - EMA/OD/205/14
- For treatment of Aicardi-Goutières syndrome - EMA/OD/206/14
- For treatment of amyotrophic lateral sclerosis - EMA/OD/184/14
- For treatment of aspartylglucosaminuria - EMA/OD/172/14
- For treatment of beta-thalassemia intermedia and major - EMA/OD/189/14
- For treatment of B-lymphoblastic leukaemia/lymphoma - EMA/OD/120/14
- For treatment of calciphylaxis - EMA/OD/191/14
- For treatment of cystinosis - EMA/OD/202/14
- For treatment of diffuse large B cell lymphoma - EMA/OD/171/14

- For treatment of epidermolysis bullosa - EMA/OD/197/14
- For treatment of glioma - EMA/OD/176/14
- For treatment of glioma - EMA/OD/181/14
- For treatment of glioma - EMA/OD/200/14
- For treatment of haemolytic uremic syndrome caused by Shiga toxin-producing bacteria - EMA/OD/194/14
- For treatment of hereditary haemorrhagic telangiectasia - EMA/OD/167/14
- For treatment of Huntington's disease - EMA/OD/169/14
- For treatment of hypogonadotropic hypogonadism - EMA/OD/126/14
- For treatment of inborn errors of primary bile acid synthesis - EMA/OD/196/14
- For treatment of interstitial cystitis - EMA/OD/179/14
- For treatment of malignant mesothelioma - EMA/OD/168/14
- For treatment of malignant mesothelioma - EMA/OD/180/14
- For treatment of multiple system atrophy - EMA/OD/193/14
- For treatment of neuroblastoma - EMA/OD/199/14
- For treatment of neurotrophic keratitis - EMA/OD/185/14
- For treatment of non-infectious uveitis - EMA/OD/195/14
- For treatment of ovarian cancer - EMA/OD/157/14
- For treatment of pancreatic cancer - EMA/OD/173/14
- For treatment of pancreatic cancer - EMA/OD/178/14
- For treatment of pancreatic cancer - EMA/OD/187/14
- For treatment of placental insufficiency - EMA/OD/198/14
- For treatment of primary biliary cirrhosis - EMA/OD/158/13
- For treatment of progressive supranuclear palsy - EMA/OD/141/14
- For treatment of Pseudomonas Aeruginosa infections in cystic fibrosis patients - EMA/OD/174/14
- For treatment of Pseudomonas Auriginosa infections in cystic fibrosis patients - EMA/OD/177/14
- For treatment of respiratory distress syndrome in neonates - EMA/OD/182/14
- For treatment of systemic sclerosis - EMA/OD/207/14
- For treatment of the adult T-cell leukemia/lymphoma - EMA/OD/203/14
- For treatment of the adult T-cell leukemia/lymphoma - EMA/OD/204/14
- For treatment of traumatic spinal cord injury - EMA/OD/186/14
- For treatment of WHIM syndrome - EMA/OD/142/14
- For treatment of WHIM syndrome - EMA/OD/190/14

- For treatment of Wilson's disease - EMA/OD/201/14

2.3. Appeal procedure

None.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for 26 applications for orphan designation.

3. Requests for protocol assistance

- For prevention of graft-versus-host disease
- For treatment of acute myeloid leukaemia
- For treatment of functional gastro-entero-pancreatic endocrine tumours
- For treatment of glioma
- For treatment of hepatocellular carcinoma

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

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

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

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

5.2.1 (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)

5.2.2 Nintedanib for treatment of idiopathic pulmonary fibrosis; Boehringer Ingelheim International GmbH (EU/3/13/1123)

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

5.3.3 Cysteamine hydrochloride for treatment of cystinosis; Orphan Europe S.A.R.L. (EU/3/08/578)

5.3.4 Panobinostat for treatment of multiple myeloma; Novartis Europharm Limited (EU/3/12/1063)

5.3.5 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)

5.3.6 Tasimelteon for treatment of non-24-hour sleep-wake disorder in blind people with no light perception; Vanda Pharmaceuticals Limited (EU/3/10/84)

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 Ruxolitinib for treatment of polycythaemia vera; Novartis Europharm Limited (EU/3/14/1244)

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 Lenvatinib; Eisai Ltd

a) treatment of papillary thyroid cancer (EU/3/13/1121)

b) treatment of follicular thyroid cancer (EU/3/13/1119)

5.3.12 Susoctocog alfa for treatment of haemophilia A; Baxter AG (EU/3/10/784)

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

5.3.14 Glyceryl tri-(4-phenylbutyrate); Hyperion Therapeutics Limited:

a) treatment of carbamoyl-phosphate synthase-1 deficiency (EU/3/10/733)

b) treatment of ornithine carbamoyltransferase deficiency (EU/3/10/734)

c) treatment of citrullinaemia type 1 (EU/3/10/735)

d) treatment of argininosuccinic aciduria (EU/3/10/736)

e) treatment of hyperargininaemia (EU/3/10/737)

f) treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome) (EU/3/10/738)

g) treatment of citrullinaemia type 2 (EU/3/10/739)

5.3.15 Idebenone for treatment of Leber's hereditary optic neuropathy; Santhera Pharmaceuticals (Deutschland) GmbH (EU/3/07/434)

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

5.3.17 Asfotase alfa for treatment of hypophosphatasia; Alexion Europe SAS (EU/3/08/594)

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

5.3.19 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

5.3.20 Herpes simplex 1 virus-thymidine kinase and truncated low affinity nerve growth factor receptor transfected donor lymphocytes for adjunctive treatment in haematopoietic cell transplantation; MolMed S.p.A. (EU/3/03/168)

6. Procedural aspects

6.1 Draft Work plan for the European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP) 2015 (EMA/515424/2014)

6.2 Draft Work plan for the European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) 2015 (EMA/463774/2014)

7. Any other business

7.1 State of the Art Report on RD Activities by European Union Committee of Experts on Rare Diseases (EUCERD)