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 7-9 October 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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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 treatment of acute myeloid leukaemia - EMA/OD/103/14
- For treatment of acute peripheral arterial occlusion - EMA/OD/117/14
- For treatment of acute respiratory distress syndrome - EMA/OD/110/14
- For treatment of cleft lip and palate - EMA/OD/136/14
- For treatment of cystic fibrosis - EMA/OD/131/14
- For treatment of erythropoietic protoporphyria - EMA/OD/127/14
- For treatment of haemophilia A - EMA/OD/123/14
- For treatment of idiopathic pulmonary fibrosis - EMA/OD/130/14
- For treatment of myotonic disorders - EMA/OD/074/14
- For treatment of plasma cell myeloma - EMA/OD/087/14
- For treatment of post-essential thrombocythaemia myelofibrosis - EMA/OD/116/14
- For treatment of post-polycythaemia vera myelofibrosis - EMA/OD/139/14
- For treatment of primary myelofibrosis - EMA/OD/140/14
- For treatment of refractory and/or relapsed Richter's transformation - EMA/OD/078/14
- For treatment of systemic lupus erythematosus - EMA/OD/097/14
- For treatment of systemic sclerosis - EMA/OD/129/14

2.2. For discussion / preparation for an 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 dermatomyositis - EMA/OD/146/14
- For treatment of Duchenne muscular dystrophy - EMA/OD/166/14
- For treatment of familial cerebral cavernous malformations - EMA/OD/161/14

- For treatment of fibrodysplasia ossificans progressiva - EMA/OD/145/14
- For treatment of Gaucher disease - EMA/OD/152/14
- For treatment of glioma - EMA/OD/132/14
- For treatment of glioma - EMA/OD/159/14
- For treatment of granulomatosis with polyangiitis - EMA/OD/150/14
- For treatment of hepatocellular carcinoma - EMA/OD/160/14
- For treatment of hereditary haemorrhagic telangiectasia - EMA/OD/144/14
- For treatment of Huntington's disease - EMA/OD/114/14
- For treatment of malignant hyperthermia - EMA/OD/162/14
- For treatment of mantle cell lymphoma - EMA/OD/151/14
- For treatment of microscopic polyangiitis - EMA/OD/149/14
- For treatment of mucopolysaccharidosis type I - EMA/OD/121/14
- For treatment of mucopolysaccharidosis type I - EMA/OD/165/14
- 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 Niemann-Pick's disease, type C - EMA/OD/158/14
- For treatment of pancreatic cancer - EMA/OD/143/14
- For treatment of Pleural Infection - EMA/OD/125/14
- For treatment of polymyositis - EMA/OD/147/14
- For treatment of systemic sclerosis - EMA/OD/148/14
- For treatment of xeroderma pigmentosum - EMA/OD/155/14

2.3. Appeal procedure

None.

2.4. Evaluation on-going

45 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 treatment of functional gastro-entero-pancreatic endocrine tumours
- For treatment of gastro-entero-pancreatic neuroendocrine tumours
- For treatment of glioma
- For treatment of hepatocellular carcinoma
- For treatment of mantle cell lymphoma

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

5.1.3 Signifor (Pasireotide) for treatment of acromegaly; Novartis Europharm Limited (Type II variation) (EU/3/09/670)

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

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

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

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

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

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

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

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

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

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

5.3.18 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

5.3.19 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 Significant Benefit ad hoc Working Group

7. Any other business

7.1 SAWP/COMP interaction