EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 December 2014
EMA/COMP/782108/2014
Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 7-9 January 2015 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 treatment of angioedema - EMA/OD/170/14
- For treatment of arginase deficiency - EMA/OD/231/14
- For treatment of argininosuccinate lyase deficiency SA - EMA/OD/230/14
- For treatment of argininosuccinate synthetase deficiency - EMA/OD/229/14
- For treatment of carbamoylphosphate synthetase I deficiency - EMA/OD/233/14
- For treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma - EMA/OD/208/14
- For treatment of Creutzfeldt-Jacob disease - EMA/OD/221/14
- For treatment of diastolic heart failure caused by hypertrophic cardiomyopathy - EMA/OD/153/14
- For treatment of Ebola viral infection - EMA/OD/250/14
- For treatment of Ebola virus disease - EMA/OD/272/14
- For treatment of glioma - EMA/OD/234/14
- For treatment of hyperornithinaemia, hyperammonaemia, homocitrullinuria syndrome - EMA/OD/228/14
- For treatment of lysinuric protein intolerance - EMA/OD/232/14
- For treatment of mantle cell lymphoma - EMA/OD/220/14
- For treatment of N-acetylglutamate synthase deficiency - EMA/OD/227/14
- For treatment of non-infectious uveitis - EMA/OD/236/14
- For treatment of ornithine transcarbamylase deficiency - EMA/OD/226/14
- For treatment of ovarian cancer - EMA/OD/211/14
- For treatment of sickle cell disease - EMA/OD/210/14
- For treatment of Sjogren's syndrome - EMA/OD/235/14
- For treatment of systemic sclerosis - EMA/OD/225/14

2.2. For discussion / preparation for an opinion

- For prevention of bronchopulmonary dysplasia - EMA/OD/270/14
- For prevention of necrotising enterocolitis - EMA/OD/237/14
- For treatment of acute myeloid leukaemia - EMA/OD/258/14
- For treatment of acute myeloid leukaemia - EMA/OD/240/14
- For treatment of adenovirus infections in patients following allogeneic stem cell transplantations - EMA/OD/245/14
- For treatment of Alport syndrome - EMA/OD/238/14
- For treatment of amyotrophic lateral sclerosis - EMA/OD/262/14
- For treatment of biliary tract cancer - EMA/OD/252/14
- For treatment of creatine transporter deficiency - EMA/OD/239/14
- For treatment of cytomegalovirus (CMV) infections in patients following allogeneic stem cell transplantations - EMA/OD/246/14
- For treatment of eosinophilic oesophagitis - EMA/OD/243/14
- For treatment of Epstein-Barr Virus infections in patients following allogeneic stem cell transplantations - EMA/OD/247/14
- For treatment of facioscapulohumeral muscular dystrophy - EMA/OD/268/14
- For treatment of fragile X syndrome - EMA/OD/253/14
- For treatment of glioma - EMA/OD/251/14
- For treatment of graft versus host disease - EMA/OD/267/14
- For treatment of Huntington's disease - EMA/OD/192/14
- For treatment of Huntington's disease - EMA/OD/255/14
- For treatment of idiopathic noncirrhotic portal hypertension - EMA/OD/269/14
- For treatment of narcolepsy - EMA/OD/254/14
- For treatment of Netherton syndrome - EMA/OD/264/14
- For treatment of pancreatic cancer - EMA/OD/242/14
- For treatment of progressive supranuclear palsy - EMA/OD/261/14
- For treatment of retinitis pigmentosa - EMA/OD/271/14
- For treatment of sickle cell disease - EMA/OD/249/14
- For treatment of Smith-Magenis syndrome - EMA/OD/260/14
- For treatment of soft tissue sarcoma - EMA/OD/266/14
- For treatment of uraemic pruritus - EMA/OD/265/14
- For treatment of Wilson disease - EMA/OD/241/14

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 47 applications for orphan designation.

3. Requests for protocol assistance

- For treatment of African trypanosomiasis
- For treatment of Stargardt's disease

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 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)

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

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

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

5.3. On-going procedures

5.3.1 Blinatumomab for treatment of acute lymphoblastic leukaemia; Amgen Europe B.V. (EU/3/09/650)

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 Isavuconazonium sulfate; Basilea Medical Ltd:

a) treatment of invasive aspergillosis (EU/3/14/1284)

b) treatment of mucormycosis (EU/3/14/1276)

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

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

5.3.6 Efmoroctocog alfa for treatment of haemophilia A; Biogen Idec Ltd (EU/3/10/783)

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

5.3.8 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)

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

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

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

5.3.12 Lenvatinib; Eisai Ltd

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

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

5.3.13 Recombinant human parathyroid hormone for treatment of hypoparathyroidism; NPS Pharma UK Ltd (EU/3/13/1210)

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

5.3.15 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)

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

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

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

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

5.3.20 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

5.3.21 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 Working group

7. Any other business

None.