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EMA/COMP/719717/2014
Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 9-11 December 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 bronchopulmonary dysplasia - EMA/OD/183/14
- 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 aspartylglucosaminuria - EMA/OD/172/14
- For treatment of calciphylaxis - EMA/OD/191/14
- For treatment of glioma - EMA/OD/181/14
- For treatment of haemolytic uremic syndrome caused by Shiga toxin-producing bacteria - EMA/OD/194/14
- For treatment of hypogonadotropic hypogonadism - EMA/OD/126/14
- For treatment of interstitial cystitis - EMA/OD/179/14
- For treatment of neuroblastoma - EMA/OD/199/14
- 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 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 Wilson's disease - EMA/OD/201/14

2.2. For discussion / preparation for an opinion

- For prevention of graft versus host disease - EMA/OD/217/14
- For treatment of angioedema - EMA/OD/170/14
- For treatment of arginase deficiency - EMA/OD/231/14
- For treatment of argininosuccinate lyase deficiency - 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 congenital factor VII deficiency - EMA/OD/224/14
- For treatment of Creuztfeldt-Jacob Disease - EMA/OD/221/14
- For treatment of diastolic heart failure caused by hypertrophic cardiomyopathy - EMA/OD/153/14
- For treatment of diffuse large B-cell lymphoma - EMA/OD/215/14
- 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 hypophosphatasia - EMA/OD/218/14
- For treatment of lysinuric protein intolerance - EMA/OD/232/14
- For treatment of malignant gastro intestinal stromal tumors - EMA/OD/212/14
- For treatment of malignant mesothelioma - EMA/OD/076/14
- For treatment of mantle cell lymphoma - EMA/OD/220/14
- For treatment of mucopolysaccharidosis type III B (Sanfilippo B syndrome) - EMA/OD/213/14
- 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 ovarian cancer - EMA/OD/223/14
- For treatment of plasma cell myeloma - EMA/OD/214/14
- For treatment of sickle cell disease - EMA/OD/210/14
- For treatment of Sjogren's syndrome - EMA/OD/235/14
- For treatment of spinocerebellar ataxia - EMA/OD/216/14
- For treatment of systemic sclerosis - EMA/OD/225/14

- For treatment of Wolfram syndrome - EMA/OD/222/14

2.3. Appeal procedure

None.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for 39 applications for orphan designation.

3. Requests for protocol assistance

- For prevention of graft-versus-host disease
- For treatment of glioma
- For treatment of soft tissue sarcoma

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

5.1.2 (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)

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

5.2.1 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)

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

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

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

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

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

5.3.7 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)

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

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

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

6.2 ITF briefing meeting. Call for expression of interest in participation to the task force briefing meeting (for COMP members).

7. Any other business

None.