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Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 8-10 July 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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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/018/14
- For treatment of acute pancreatitis - EMA/OD/072/14
- For treatment of adrenal insufficiency - EMA/OD/060/14
- For treatment of anti-neutrophil cytoplasmic antibody (ANCA)-associated vasculitis - EMA/OD/050/14
- For treatment of Apolipoprotein A-I (apoA-I) deficiency - EMA/OD/064/14
- For treatment of ATP-Binding Cassette Transporter A1 (ABCA1) deficiency - EMA/OD/063/14
- For treatment of autosomal dominant polycystic kidney disease - EMA/OD/042/14
- For treatment of autosomal dominant polycystic liver disease - EMA/OD/043/14
- For treatment of congenital factor VII deficiency - EMA/OD/057/14
- For treatment of cystic fibrosis - EMA/OD/032/14
- For treatment of Duchenne muscular dystrophy - EMA/OD/049/14
- For treatment of Fabry disease - EMA/OD/052/14
- For treatment of glioma - EMA/OD/055/14
- For treatment of haemophilia A - EMA/OD/024/14
- For treatment of haemophilia A - EMA/OD/069/14
- For treatment of haemophilia B - EMA/OD/073/14
- For treatment of Lecithin Cholesterol Acyltransferase (LCAT) deficiency - EMA/OD/066/14
- For treatment of myelodysplastic syndromes - EMA/OD/048/14
- For treatment of paroxysmal nocturnal haemoglobinuria - EMA/OD/056/14
- For treatment of pigmented villonodular synovitis / giant cell tumour of the tendon sheath - EMA/OD/058/14
- For treatment of retinopathy of prematurity - EMA/OD/040/14
- For treatment of Schnitzler Syndrome - EMA/OD/053/14

2.2. For discussion / preparation for an opinion

- For prevention of ischemia / reperfusion injury associated with solid organ transplantation - EMA/OD/090/14
- For treatment of acute myeloid leukaemia - EMA/OD/100/14
- For treatment of acute myeloid leukemia - EMA/OD/061/14
- For treatment of adenovirus infections in patients following allogeneic stem cell transplantations - EMA/OD/094/14
- For treatment of Crigler-Najjar syndrome - EMA/OD/082/14
- For treatment of Cushing's syndrome - EMA/OD/099/14
- For treatment of cutaneous T-cell lymphoma - EMA/OD/084/14
- For treatment of cystinosis - EMA/OD/106/14
- For treatment of cytomegalovirus (CMV) infections in patients following allogeneic stem cell transplantations - EMA/OD/096/14
- For treatment of diffuse large B cell lymphoma - EMA/OD/071/14
- For treatment of diffuse large B-cell lymphoma - EMA/OD/092/14
- For treatment of Dravet syndrome - EMA/OD/083/14
- For treatment of Epstein-Barr Virus infections in patients following allogeneic stem cell transplantations - EMA/OD/095/14
- For treatment of Leigh syndrome - EMA/OD/068/14
- For treatment of limbal stem cell deficiency - EMA/OD/109/14
- For treatment of mastocytosis - EMA/OD/075/14
- For treatment of Merkel cell carcinoma - EMA/OD/079/14
- For treatment of mitochondrial neurogastrointestinal encephalomyopathy - EMA/OD/093/14
- For treatment of neuromyelitis optica - EMA/OD/089/14
- For treatment of pancreatic cancer – EMA/OD/081/14
- For treatment of pancreatic cancer - EMA/OD/085/14
- For treatment of paroxysmal nocturnal hemoglobinuria - EMA/OD/098/14
- For treatment of Pemphigus - EMA/OD/091/14
- For treatment of pigmented villonodular synovitis - EMA/OD/107/14
- For treatment of primary biliary cirrhosis - EMA/OD/101/14
- For treatment of Pyruvate Kinase Deficiency - EMA/OD/102/14
- For treatment of short bowel syndrome - EMA/OD/080/14
- For treatment of small cell lung cancer - EMA/OD/086/14
- For treatment of systemic-onset juvenile idiopathic arthritis - EMA/OD/108/14

2.3. Revision on the COMP opinion adopted via written procedure

- Adeno-associated viral vector serotype 9 containing the human cardiac calsequestrin gene for treatment of catecholaminergic polymorphic ventricular tachycardia, Fondazione Salvatore Maugeri Clinica del Lavoro e della Riabilitazione - EMA/OD/037/14

2.4. Evaluation on-going

Evaluation for valid applications will commence on 11 July 2014.

2.5. Validation on-going

Validation is on-going for 49 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of congenital adrenal hyperplasia
- Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity
- Treatment of Dravet syndrome
- Treatment of glioma
- 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

None.

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

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

5.2.2 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. 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 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 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)

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

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

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

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

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

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

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

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

5.3.15 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)

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

6. Any other business

6.1 Update on the EURORDIS Summer School and EUPATI training