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EMA/COMP/321121/2014
Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 10-12 June 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 for necrotizing soft tissue infections - EMA/OD/028/14
- For treatment of cystinosis - EMA/OD/031/14
- For treatment of Growth Hormone Deficiency in Adults and Children - EMA/OD/030/14
- For treatment of plasma cell myeloma - EMA/OD/035/14
- For treatment of systemic amyloidosis - EMA/OD/020/14
- For treatment of systemic amyloidosis - EMA/OD/021/14

2.2. For discussion / preparation for an 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 beta-thalassemia intermedia and major - EMA/OD/047/14
- For treatment of catecholaminergic polymorphic ventricular tachycardia - EMA/OD/037/14
- 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 Duchenne muscular dystrophy - EMA/OD/067/14
- For treatment of Fabry disease - EMA/OD/052/14
- For treatment of gastric cancer - EMA/OD/012/14

- For treatment of glioma - EMA/OD/055/14
- For treatment of glioma - EMA/OD/065/14
- For treatment of haemophilia A - EMA/OD/024/14
- For treatment of haemophilia A - EMA/OD/039/14
- For treatment of haemophilia A - EMA/OD/069/14
- For treatment of haemophilia B - EMA/OD/041/14
- For treatment of haemophilia B - EMA/OD/073/14
- For treatment of Huntington's disease - EMA/OD/070/14
- For treatment of idiopathic pulmonary fibrosis - EMA/OD/051/14
- For treatment of Lecithin Cholesterol Acyltransferase (LCAT) deficiency - EMA/OD/066/14
- For treatment of myasthenia gravis - EMA/OD/062/14
- For treatment of myelodysplastic syndromes - EMA/OD/048/14
- For treatment of ovarian cancer - EMA/OD/059/14
- 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 Prader-Willi Syndrome - EMA/OD/054/14
- For treatment of retinopathy of prematurity - EMA/OD/040/14
- For treatment of Schnitzler Syndrome - EMA/OD/053/14
- For treatment of systemic sclerosis - EMA/OD/044/14

2.3. Evaluation on-going

None

2.4. Validation on-going

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

3. Requests for protocol assistance

- Treatment of chronic non-infectious uveitis
- Treatment of Dravet syndrome
- Treatment of glioma
- Treatment of ovarian cancer

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 Vantobra (Tobramycin (inhalation use)) for treatment of *Pseudomonas Aeruginosa* lung infection in cystic fibrosis; PARI Pharma GmbH (EU/3/09/613)

5.1.2 Gazyvaro (Obinutuzumab) for treatment of chronic lymphocytic leukaemia; Roche Registration Limited (EU/3/12/1054)

5.1.3 Translarna (3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid) for treatment of Duchenne muscular dystrophy; PTC Therapeutics Ltd (EU/3/05/278)

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)

5.3. 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 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)

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

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

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

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

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

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

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

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

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

5.3.17 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. Any other business

6.1 6th presentation on the EMA move to 30 Churchill Place