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 2-4 September 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 treatment of adenovirus infections in patients following allogeneic stem cell transplantations - EMA/OD/094/14
- For treatment of Cushing's syndrome - EMA/OD/099/14
- 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 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 neuromyelitis optica - EMA/OD/089/14
- For treatment of pancreatic cancer – EMA/OD/081/14
- For treatment of pemphigus - EMA/OD/091/14
- For treatment of pigmented villonodular synovitis - EMA/OD/107/14
- For treatment of short bowel syndrome - EMA/OD/080/14
- For treatment of systemic-onset juvenile idiopathic arthritis - EMA/OD/108/14

2.2. For discussion / preparation for an opinion

- For prevention of angioedema - EMA/OD/115/14
- 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 congenital hyperinsulinism - EMA/OD/128/14
- For treatment of Crigler-Najjar syndrome - EMA/OD/122/14
- For treatment of cystic fibrosis - EMA/OD/036/14
- For treatment of cystic fibrosis - EMA/OD/131/14
- For treatment of erythropoietic protoporphyria - EMA/OD/127/14
- For treatment of essential thrombocythaemia - EMA/OD/124/14
- For treatment of fragile X syndrome - EMA/OD/105/14
- For treatment of fragile X syndrome - EMA/OD/137/14
- For treatment of glioma - EMA/OD/111/14
- For treatment of haemophilia A - EMA/OD/123/14
- For treatment of idiopathic pulmonary fibrosis - EMA/OD/130/14
- For treatment of inhalation anthrax disease - EMA/OD/134/14
- For treatment of mucopolysaccharidosis type I - EMA/OD/138/14
- 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 pyridoxamine 5'-phosphate oxidase deficiency - EMA/OD/104/14
- 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
- For treatment of X-linked hypophosphatemia - EMA/OD/133/14

2.3. COMP opinions adopted via written procedure following previous meeting

- [5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone for treatment of pancreatic cancer, Synovo GmbH - EMA/OD/085/14
- Recombinant human apolipoprotein A-I in a complex with phospholipids (CER-001) for treatment of ATP-Binding Cassette Transporter A1 (ABCA1) deficiency, Cerenis Therapeutics Holding SA - EMA/OD/063/14
- Variant of recombinant human fibroblast growth factor 19 for treatment of primary biliary cirrhosis, Diamond BioPharm Limited - EMA/OD/101/14

- The COMP adopted 1 negative opinion recommending the refusal of the orphan medicinal product designation for a product for treatment of autosomal dominant polycystic kidney disease.

2.4. Appeal procedure

None.

2.5. Evaluation on-going

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

2.6. Validation on-going

Validation is on-going for 25 applications for orphan designation.

3. Requests for protocol assistance

- For treatment of congenital adrenal hyperplasia
- For treatment of cytomegalovirus disease in patients with impaired cell mediated immunity
- For treatment of Dravet syndrome
- 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 Imbruvica (1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one); Janssen-Cilag International N.V

a) for treatment of mantle cell lymphoma (EU/3/13/1115)

b) for treatment of chronic lymphocytic leukaemia (EU/3/12/984)

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

5.2.1 (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)

5.2.2 Ramucirumab for treatment of gastric cancer; Eli Lilly Nederland B.V. (EU/3/12/1004)

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

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

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

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

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 Mifepristone for treatment of hypercortisolism (Cushing's syndrome) of endogenous origin; FGK Representative Service GmbH (EU/3/11/925)

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

5.3.4 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)

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

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

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

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

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

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

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

5.3.17 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

5.3.18 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 COMP Workplan 2015

7. Any other business

7.1 EMA/RCE meeting: Methodology of clinical studies on rare cancers

- Draft Agenda of the 3 October 2014 EMA/RCE meeting