29 April 2014
EMA/COMP/257297/2014
Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 13-14 May 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

- Treatment of amyotrophic lateral sclerosis - EMA/OD/007/14
- Treatment of cystic fibrosis - EMA/OD/002/14
- Treatment of invasive aspergillosis - EMA/OD/009/14
- Treatment of non-infectious uveitis - EMA/OD/014/14
- Treatment of Stargardt's disease - EMA/OD/005/14
- Treatment of Usher syndrome - EMA/OD/004/14

2.2. For discussion / preparation for an opinion

- Treatment for necrotizing soft tissue infections - EMA/OD/028/14
- Treatment of choroideremia - EMA/OD/033/14
- Treatment of chronic lymphocytic leukemia/small lymphocytic lymphoma - EMA/OD/022/14
- Treatment of cystinosis - EMA/OD/031/14
- Treatment of diffuse large B-cell lymphoma - EMA/OD/029/14
- Treatment of familial benign chronic pemphigus (Hailey-Hailey disease) - EMA/OD/019/14
- Treatment of Growth Hormone Deficiency in Adults and Children - EMA/OD/030/14
- Treatment of plasma cell myeloma - EMA/OD/035/14
- Treatment of Prader-Willi syndrome - EMA/OD/023/14
- Treatment of Preeclampsia - EMA/OD/027/14
- Treatment of primary sclerosing cholangitis - EMA/OD/026/14
- Treatment of systemic amyloidosis - EMA/OD/020/14
- Treatment of systemic amyloidosis - EMA/OD/021/14
- Treatment of thrombocytopenia caused by chronic idiopathic thrombocytopenia purpura - EMA/OD/025/14

2.3. Evaluation on-going

Evaluation is on-going for 37 applications which will be discussed at the June meeting.

2.4. Validation on-going

Validation is on-going for 13 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of acute lymphoblastic leukaemia
- Treatment of Dravet syndrome
- Treatment of glioma
- Treatment of ovarian cancer
- Treatment of primary myelofibrosis

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 Nexavar (Sorafenib tosylate), Bayer HealthCare AG, (Type II variation):

a) treatment of follicular thyroid cancer (EU/3/13/1199)

b) treatment of papillary thyroid cancer (EU/3/13/1200)

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

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

5.2.2 Masitinib mesylate for treatment of pancreatic cancer; AB Science (EU/3/09/684)

5.2.3 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)

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

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

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

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

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

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

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

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. Any other business

6.1. Informal CHMP/CAT/COMP meeting to be held on 28-29 October 2014 in Rome

6.2. 5th presentation on the EMA move to 30 Churchill Place

6.3. EMA/COMP publications

- Significant Benefit
- Medical plausibility