EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMA/COMP/45848/2015
Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 10-12 February 2015 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/270/14
- For treatment of Alport syndrome - EMA/OD/238/14
- For treatment of amyotrophic lateral sclerosis - EMA/OD/262/14
- For treatment of biliary tract cancer - EMA/OD/252/14
- For treatment of creatine transporter deficiency - EMA/OD/239/14
- For treatment of eosinophilic oesophagitis - EMA/OD/243/14
- For treatment of fragile X syndrome - EMA/OD/253/14
- For treatment of graft-versus-host disease - EMA/OD/267/14
- For treatment of Huntington's disease - EMA/OD/255/14
- For treatment of idiopathic noncirrhotic portal hypertension - EMA/OD/269/14
- For treatment of Netherton syndrome - EMA/OD/264/14
- For treatment of Smith-Magenis syndrome - EMA/OD/260/14
- For treatment of uraemic pruritus - EMA/OD/265/14
- For treatment of Wilson's disease - EMA/OD/241/14

2.2. For discussion / preparation for an opinion

- For diagnosis of gastro-entero-pancreatic neuroendocrine tumours - EMA/OD/219/14
- For diagnosis of glioma - EMA/OD/280/14
- For prevention of graft rejection following solid organ transplantation - EMA/OD/308/14
- For the treatment of tularaemia - EMA/OD/301/14
- For treatment of amyotrophic lateral sclerosis - EMA/OD/278/14
- For treatment of amyotrophic lateral sclerosis - EMA/OD/283/14
- For treatment of cholangiocarcinoma - EMA/OD/305/14

- For treatment of congenital venous malformations - EMA/OD/282/14
- For treatment of cryptococcosis - EMA/OD/300/14
- For treatment of Duchenne muscular dystrophy - EMA/OD/257/14
- For treatment of Duchenne muscular dystrophy - EMA/OD/307/14
- For treatment of Ebola virus disease - EMA/OD/310/14
- For treatment of epidermolysis bullosa - EMA/OD/297/14
- For treatment of epidermolysis bullosa - EMA/OD/298/14
- For treatment of epidermolysis bullosa - EMA/OD/299/14
- For treatment of extranodal marginal zone lymphoma of mucosa associated lymphoid tissue - EMA/OD/286/14
- For treatment of follicular lymphoma - EMA/OD/275/14
- For treatment of Gaucher disease - EMA/OD/303/14
- For treatment of hepatocellular carcinoma - EMA/OD/287/14
- For treatment of Huntington's disease - EMA/OD/256/14
- For treatment of invasive candidiasis - EMA/OD/294/14
- For treatment of Leber's congenital amaurosis - EMA/OD/309/14
- For treatment of nodal marginal zone lymphoma - EMA/OD/284/14
- For treatment of ovarian cancer - EMA/OD/281/14
- For treatment of ovarian cancer - EMA/OD/304/14
- For treatment of pancreatic cancer - EMA/OD/302/14
- For treatment of plasma cell myeloma - EMA/OD/293/14
- For treatment of primary sclerosing cholangitis - EMA/OD/288/14
- For treatment of retinitis pigmentosa - EMA/OD/289/14
- For treatment of splenic marginal zone lymphoma - EMA/OD/285/14
- For treatment of Stargardt's disease - EMA/OD/295/14
- For treatment of systemic sclerosis - EMA/OD/296/14
- For treatment of systemic sclerosis - EMA/OD/306/14
- For treatment of tenosynovial giant cell tumour, localised and diffuse type - EMA/OD/279/14
- For treatment of trigeminal neuralgia - EMA/OD/244/14
- For treatment of Wilson's disease - EMA/OD/001/15

2.3. Appeal procedure

None.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for 30 applications for orphan designation.

3. Requests for protocol assistance

- For treatment of African trypanosomiasis
- For treatment of amyloid light-chain amyloidosis
- For treatment of mastocytosis
- For treatment of pancreatic cancer
- For treatment of Pemphigus
- For treatment of Pseudomonas aeruginosa lung infection in cystic fibrosis
- For treatment of Stargardt's disease

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

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

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

5.2.1 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)

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

5.2.3 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. 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 Isavuconazonium sulfate; Basilea Medical Ltd:

a) treatment of invasive aspergillosis (EU/3/14/1284)

b) treatment of mucormycosis (EU/3/14/1276)

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

5.3.5 Cysteamine hydrochloride for treatment of cystinosis; Lucane Pharma (EU/3/14/1341)

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

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

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

5.3.9 Ibrutinib for treatment of lymphoplasmacytic lymphoma; Janssen-Cilag International NV (EU/3/14/1264)

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

5.3.11 Lenvatinib; Eisai Ltd

a) treatment of papillary thyroid cancer (EU/3/13/1121)

b) treatment of follicular thyroid cancer (EU/3/13/1119)

5.3.12 Recombinant human parathyroid hormone for treatment of hypoparathyroidism; NPS Pharma UK Ltd (EU/3/13/1210)

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

5.3.14 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)

5.3.15 Lenalidomide for treatment of mantle cell lymphoma; Celgene Europe Limited (EU/3/11/924)

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 Draft Minutes of Italian CHMP/CAT/COMP Presidency meeting

6.3 Update from the European Commission

6.4 NCA/COMP Consultation on proposed process improvements for Orphan procedures

6.5 EMA communication on public consultation on application of transparency rules of EU Clinical Trial Regulation

- Questions and answers - Public consultation on implementation of transparency requirements of the European Clinical Trial Regulation EMA/36398/2015

http://www.ema.europa.eu/docs/en_GB/document_library/Other/2015/01/WC500180632.pdf

- Press release - Public consultation on application of transparency rules of EU Clinical Trial Regulation EMA/35075/2015

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2015/01/news_detail_002253.jsp&mid=WC0b01ac058004d5c1

6.6 Draft Agenda - PCWP and HCPWP joint meeting – 4 March 2015

6.7 Draft Agenda - PCWP and HCPWP joint meeting - Information session on Biosimilars – 5 March 2015

7. Any other business

7.1 Change of the COMP meeting dates for July, September and December 2015