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EMA/COMP/281296/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 12-13 May 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/010/15
- For prevention of scarring post glaucoma filtration surgery - EMA/OD/021/15
- For treatment of non-infectious uveitis - EMA/OD/024/15
- For treatment of hepatitis delta virus infection - EMA/OD/329/14
- For treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma) - EMA/OD/014/15
- For treatment of nodal marginal zone lymphoma - EMA/OD/015/15
- For treatment of splenic marginal zone lymphoma - EMA/OD/016/15
- For treatment of follicular lymphoma - EMA/OD/013/15

2.2. For discussion / preparation for an opinion

- For prevention of avian influenza A virus - EMA/OD/036/15
- For treatment of acromegaly - EMA/OD/031/15
- For treatment of amyotrophic lateral sclerosis - EMA/OD/032/15
- For treatment of autosomal dominant polycystic kidney disease - EMA/OD/027/15
- For treatment of avian influenza A virus - EMA/OD/012/15
- For treatment of cutaneous T cell lymphoma - EMA/OD/033/15
- For treatment of Ebola virus disease - EMA/OD/030/15
- For treatment of haemophilia B - EMA/OD/003/15
- For treatment of hepatoblastoma - EMA/OD/023/15
- For treatment of hepatocellular carcinoma - EMA/OD/022/15
- For treatment of neurotrophic keratitis - EMA/OD/029/15
- For treatment of spinal muscular atrophy - EMA/OD/028/15
- For treatment of spinocerebellar ataxia - EMA/OD/009/15

- For treatment of type I plasminogen deficiency or hypoplasminogenemia - EMA/OD/313/14
- For treatment of Very Long-chain Acyl-coenzyme A Dehydrogenase (VLCAD) Deficiency - EMA/OD/026/15

2.3. Appeal procedure

None.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for 21 applications for orphan designation.

3. Requests for protocol assistance

- For treatment of ATTR amyloidosis
- For treatment of glioma
- For treatment of haemophilia A
- For treatment of Niemann-Pick disease, type C

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 Tasimelteon for treatment of non-24-hour sleep-wake disorder in blind people with no light perception; Vanda Pharmaceuticals Limited (EU/3/10/841)

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

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

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

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

5.2.4 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)

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

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

5.3. On-going procedures

5.3.1 Amikacin; Insmed Limited:

a) treatment of Pseudomonas aeruginosa lung infection in cystic fibrosis (EU/3/06/387)

b) treatment of nontuberculous mycobacterial lung disease (EU/3/14/1259)

5.3.2 Blinatumomab for treatment of acute lymphoblastic leukaemia; Amgen Europe B.V. (EU/3/09/650)

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

5.3.7 Recombinant fusion protein linking human coagulation factor IX with human albumin for treatment of haemophilia B, CSL Behring GmbH (EU/3/09/723)

5.3.8 Carfilzomib for treatment of multiple myeloma; Amgen Europe B.V. (EU/3/08/548)

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

5.3.10 Dexamethasone acetate for treatment of multiple myeloma; LABORATOIRES CTRS (EU/3/10/745)

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

5.3.12 Lumacaftor / ivacaftor for treatment of cystic fibrosis; Vertex Pharmaceuticals (U.K.) Ltd., (EU/3/14/1333)

5.3.13 Sirolimus for treatment of chronic non-infectious uveitis; Santen Oy (EU/3/11/898)

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 Recombinant human lysosomal acid lipase for treatment of lysosomal acid lipase deficiency; Synageva BioPharma Ltd (EU/3/10/827)

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

5.3.18 Selexipag for treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension; Actelion Registration Ltd. (EU/3/05/316)

5.3.19 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

5.3.20 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 Significant Benefit Working group subgroups

6.3 COMP workplan: presentation of COMP survey results

6.4 Election of Chair and Vice-Chair - October 2015

6.5 Pharmacovigilance Programme

6.6 EU Medicines Agencies Network Strategy to 2020