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Procedure Management and Business Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 14-16 April 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 oral mucositis in head and neck cancer patients undergoing radiation therapy - EMA/OD/317/14
- For treatment of chronic lymphocytic leukaemia/ small lymphocytic lymphoma - EMA/OD/006/15
- For treatment of Huntington's disease - EMA/OD/325/14
- For treatment of mucopolysaccharidosis IIIC - EMA/OD/322/14
- For treatment of myasthenia gravis - EMA/OD/318/14
- For treatment of myasthenia gravis - EMA/OD/321/14
- For treatment of non-infectious uveitis - EMA/OD/320/14
- For treatment of ovarian cancer - EMA/OD/314/14
- For treatment of plasma cell myeloma - EMA/OD/277/14

2.2. For discussion / preparation for an 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 cystic fibrosis - EMA/OD/018/15
- For treatment of diffuse large B-cell lymphoma - EMA/OD/005/15
- For treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma) - EMA/OD/014/15
- For treatment of follicular lymphoma - EMA/OD/013/15
- For treatment of glucose transporter type-1 deficiency syndrome - EMA/OD/007/15
- For treatment of hepatitis delta virus infection - EMA/OD/329/14
- For treatment of Huntington's disease - EMA/OD/017/15
- For treatment of nodal marginal zone lymphoma - EMA/OD/015/15
- For treatment of non-infectious uveitis - EMA/OD/024/15
- For treatment of oculopharyngeal muscular dystrophy - EMA/OD/008/15

- For treatment of perinatal asphyxia - EMA/OD/004/15
- For treatment of retinal artery occlusion - EMA/OD/011/15
- For treatment of splenic marginal zone lymphoma - EMA/OD/016/15
- For treatment of tromboangiitis obliterans (Buerger's disease) - EMA/OD/019/15

2.3. Appeal procedure

None.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for 44 applications for orphan designation.

3. Requests for protocol assistance

- For diagnosis of gastro-entero-pancreatic neuroendocrine tumours
- For treatment of ATTR amyloidosis
- For treatment of haemophilia A
- For treatment of malignant hyperthermia
- For treatment of paroxysmal nocturnal haemoglobinuria
- For treatment of plasma cell myeloma

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 Lenvima (lenvatinib); Eisai Ltd:

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

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

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

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

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

5.3.4 Isavuconazonium sulfate; Basilea Medical Ltd:

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

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

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

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

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 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)

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

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

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

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

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

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

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

5.3.17 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)

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

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

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

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

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

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

5.3.24 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride for treatment of narcolepsy; Bioprojet (EU/3/07/459)

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

6.3 Evaluation (and communication) of the Benefit/Risk Balance of medicinal products