3 June 2015
EMA/COMP/327079/2015
Procedure Management and Committees Support Division

Committee for Orphan Medicinal Products (COMP)

Agenda of the 16-18 June 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 avian influenza A virus - EMA/OD/036/15
- For treatment of acromegaly - EMA/OD/031/15
- 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 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 type I plasminogen deficiency or hypoplasminogenemia - EMA/OD/313/14

2.2. For discussion / preparation for an opinion

- For treatment of acute myeloid leukaemia - EMA/OD/037/15
- For treatment of acute myeloid leukaemia - EMA/OD/045/15
- For treatment of amyotrophic lateral sclerosis - EMA/OD/051/15
- For treatment of carnitine palmitoyl transferase II (CPT II) deficiency - EMA/OD/048/15
- For treatment of craniopharyngioma - EMA/OD/057/15
- For treatment of cystic fibrosis - EMA/OD/039/15
- For treatment of Duchenne muscular dystrophy - EMA/OD/041/15
- For treatment of Fragile X Syndrome - EMA/OD/055/15
- For treatment of long-chain 3-hydroxyacyl-coenzyme A dehydrogenase (LCHAD) deficiency - EMA/OD/046/15
- For treatment of malaria - EMA/OD/043/15
- For treatment of mitochondrial trifunctional protein (TFP) deficiency - EMA/OD/047/15

- For treatment of osteogenesis imperfecta and osteogenesis imperfecta-related disorders - EMA/OD/053/15
- For treatment of pancreatic cancer - EMA/OD/034/15
- For treatment of perinatal asphyxia - EMA/OD/042/15
- For treatment of plasma cell myeloma - EMA/OD/038/15
- For treatment of primary hyperoxaluria type 1 - EMA/OD/052/15
- For treatment of progressive supranuclear palsy - EMA/OD/044/15
- For treatment of retinitis pigmentosa - EMA/OD/040/15
- For treatment of Rett syndrome - EMA/OD/056/15
- For treatment of Rett syndrome - EMA/OD/058/15
- For treatment of short bowel syndrome - EMA/OD/050/15
- For treatment of uveal melanoma - EMA/OD/049/15

2.3. Appeal procedure

None.

2.4. Evaluation on-going

Evaluation for valid applications will commence on 8 June.

2.5. Validation on-going

Validation is on-going for 49 applications for orphan designation.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

- For treatment of Dravet syndrome
- For treatment of follicular lymphoma
- For treatment of Graft-versus-Host disease
- For treatment of Niemann-Pick disease, type C
- For treatment of pancreatic cancer
- For treatment of Urea Cycle Disorders

3.2. Finalised letters

- For treatment of ATTR amyloidosis

- For treatment of glioma
- For treatment of haemophilia A

3.3. New requests

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

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

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

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

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

5.2.3 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)

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

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

5.3. On-going procedures

- Update on on-going procedures

6. Procedural aspects

6.1 Significant Benefit Working group

6.2 EU Medicines Agencies Network Strategy to 2020

6.3 Election of Chair and Vice-Chair - October 2015

6.4 Draft agenda of the EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP)

6.5 Draft Agenda of the EMA Human Scientific Committees' Working Parties with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals' Organisations (HCPWP) joint meeting

6.6 Draft Agenda of the EMA Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP)

7. Any other business

7.1 None