25 October 2012
EMA/COMP/658591/2012
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 6-7 November 2012 meeting

Chair – Bruno Sepodes, Vice-Chair – Lesley Greene

Note on access to documents

The procedures discussed by the COMP are on-going and therefore are considered confidential. Additional details on these procedures will be disclosed in the COMP meeting reports (after the COMP opinion is adopted). Documents mentioned in the agenda cannot be released at present as they are currently in draft format or are classified as confidential. They will become public when adopted in their final form 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 from the previous meeting.
- Declaration of conflicts of interest.

2. Applications for orphan medicinal product designation

2.1. For opinion

- Treatment of acanthamoeba keratitis, OD/090/12
- Treatment of acquired aplastic anaemia, OD/100/12
- Treatment of acromegaly, OD/107/12
- Treatment of B-cell chronic lymphocytic leukaemia/small lymphocytic lymphoma, OD/083/12
- Treatment of B-cell prolymphocytic leukaemia, OD/074/12
- Treatment of Burkitt lymphoma, OD/075/12
- Treatment of diffuse large B-cell lymphoma, OD/073/12
- Treatment of Extranodal Marginal Zone B-cell Lymphoma of the MALT type, OD/072/12
- Treatment of follicular lymphoma, OD/076/12
- Treatment of hairy cell leukaemia, OD/082/12
- Treatment of lymphoplasmacytic lymphoma, OD/081/12
- Treatment of mantle cell lymphoma, OD/077/12
- Treatment of mature B-cell lymphoma: plasma cell lymphoma/plastocytoma, OD/080/12
- Treatment of nodal marginal zone B-cell lymphoma ± monocytoid, OD/078/12
- Treatment of non-infectious uveitis, OD/118/12
- Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), OD/104/12
- Treatment of splenic marginal zone B-cell lymphoma, OD/079/12
- Treatment of squamous cell carcinoma of the head and neck, OD/120/12.

2.2. For discussion / for opinion

- Treatment of chronic lymphocytic leukaemia, OD/124/12
- Treatment of chronic lymphocytic leukaemia, OD/128/12
- Treatment of complex regional pain syndrome, OD/125/12

- Treatment of Duchenne muscular dystrophy, OD/121/12
- Treatment of Duchenne muscular dystrophy, OD/122/12
- Treatment of Familial Adenomatous Polyposis, OD/130/12
- Treatment of growth hormone deficiency, OD/133/12
- Treatment of lead toxicity, OD/131/12
- Treatment of Long-chain 3-Hydroxyacyl-CoA Dehydrogenase (LCHAD) Deficiency, OD/127/12
- Treatment of malaria, OD/123/12
- Treatment of paracetamol toxicity, OD/132/12
- Treatment of patients with locally advanced, or metastatic malignant pleural mesothelioma (MPM) recurring after a first-line platinum-based chemotherapy, Bayer Pharma AG - OD/063/12
- Treatment of perinatal asphyxia, OD/134/12
- Treatment of Very Long-Chain Acyl-CoA Dehydrogenase (VLCAD) Deficiency, OD/126/12.

2.3. Evaluation on-going

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

2.4. Validation on-going

Validation is on-going for 11 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of corneal cystine crystals deposits in cystinosis.
- Treatment of mercury poisoning.

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 NexoBrid (Purified bromelain) for treatment of partial deep dermal and full thickness burns; Teva Pharma GmbH (OD/012/02, EU/3/02/107).

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

5.2.1 Bosulif (Bosutinib) for treatment of chronic myeloid leukaemia; Pfizer Limited (OD/160/09, EU/3/10/762).

5.2.2 Exjade (4-(3,5-bis(hydroxy-phenyl)-1,2,4) triazol-1-yl) benzoic acid) for treatment of chronic iron overload requiring chelation therapy; Novartis Europharm Limited (OD/061/01, EU/3/02/092).

5.2.3 Jenzyl ((1R, 2R, 4S)-4-{(2R)-2-[(3S,6R,7E,9R,10R,12R,14S,15E,17E,19E,21S,23S,26R, 27R,34aS)-9,27-dihydroxy-10,21-dimethoxy-6,8,12,14,20,26-hexamethyl-1,5,11,28,29-pentaoxo-1,4,5,6,9,10,11,12,13,14,21,22,23,24,25,26,27,28,29,31,32,33,34,34a-tetra-cosahydro-3H-23,27-epoxypyrido[2,1-c][1,4]oxazacyclohentriacontin-3-yl]propyl}-2-methoxy-cyclohexyldimethyl-phosphinate); Merck Sharp & Dohme Limited

- treatment of soft tissue sarcoma (OD/050/05, EU/3/05/312);
- treatment of primary malignant bone tumours (OD/055/05, EU/3/05/321).

5.3. On-going procedures

5.3.1 Bedaquiline ((1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethano) for treatment of tuberculosis; Janssen-Cilag International N.V. (OD/024/05, EU/3/05/314).

5.3.2 Cholic Acid FGK for treatment of inborn errors of primary bile acid synthesis responsive to treatment with cholic acid; FGK Representative Service GmbH (OD/080/09, EU/3/09/683).

5.3.3 Cysteamine bitartrate [Cysteamine bitartrate (gastroresistant)] for treatment of cystinosis; Raptor Pharmaceuticals Europe B.V. (OD/034/10, EU/3/10/778).

5.3.4 Defitelio (Defibrotide); Gentium S.p.A.

- prevention of hepatic veno-occlusive disease (OD/025/04, EU/3/04/211);
- treatment of hepatic veno-occlusive disease (OD/026/04, EU/3/04/212).

5.3.5 Delamanid ((R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole) for treatment of tuberculosis; Otsuka Novel Products GmbH (OD/094/07, EU/3/07/524).

5.3.6 Iclusig (benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]); ARIAD Pharma Ltd

- treatment of acute lymphoblastic leukaemia (OD/122/09, EU/3/09/715);
- treatment of chronic myeloid leukaemia (OD/121/09, EU/3/09/716).

5.3.7 Kinaction (Masitinib mesilate) for treatment of pancreatic cancer; AB Science (OD/063/09, EU/3/09/684).

5.3.8 Loulla (Mercaptopurine) for treatment of acute lymphatic leukaemia, Only For Children Pharmaceuticals (OD/065/07, EU/3/07/496).

5.3.9 PAS-GR (Para-aminosalicylic acid) for treatment of tuberculosis; Lucane Pharma SA (OD/072/10, EU/3/10/826).

5.3.10 Pheburane (Sodium phenylbutyrate) for treatment of carbamoyl-phosphate synthase-1 deficiency; Lucane Pharma SA (OD/098/11, EU/3/12/951).

5.3.11 Pomalidomide Celgene (Pomalidomide) for treatment of multiple myeloma, Celgene Europe Ltd. (OD/053/09, EU/3/09/672).

5.3.12 Raxone (previously SAN Idebenone; Idebenone) for treatment of Leber's hereditary optic neuropathy; Santhera Pharmaceuticals (Deutschland) GmbH (OD/076/06, EU/3/07/434).

5.3.13 Revlimid (3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) for treatment of myelodysplastic syndromes; Celgene Europe Limited – UK (OD/083/03, EU/3/04/192).

5.3.14 Scenesse ([Nle4, D-Phe7]-alfa-melanocyte stimulating hormone, Afamelanotide) for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (OD/108/07, EU/3/08/541).

5.3.15 Masican N-(methyl-diazacyclohexyl-methylbenzamide)-azaphenyl-aminothiopyrrole for treatment of malignant gastrointestinal stromal tumours; AB Science (OD/061/04, EU/3/04/251).

5.3.16 Winfuran (-)-17(cyclopropylmethyl)-1,14 ß-dihydroxy-4,5 alpha-epoxy-6ß-[N-methyl-trans-3-(3-furyl) acrylamido] morphinan hydrochloride for treatment of uremic pruritus; Toray International U.K. Limited (OD/020/02, EU/3/02/115).

6. Procedural aspects

6.1 Appointment of the COMP representative to the European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP).

http://www.ema.europa.eu/ema/index.jsp?curl=pages/contacts/CHMP/people_listing_000017.jsp&mid=WC0b01ac0580028d32

7. Any other business

7.1 FDA/EMA Orphan Designation and Grant Workshop held on 7-13 October 2012 in Washington D.C.

7.2 COMP Work Programme 2013-2015.

7.3 COMP Informal Meeting to be held on 22-23 November 2012 in Rome.

7.4 Adaptive licensing.

7.5 Managing Meeting Documents (MMD).

7.6 Draft reflection paper on biomarkers.