3 October 2012
EMA/COMP/600813/2012
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 3-5 October 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 Friedreich's ataxia, OD/096/12
- Treatment of glioma, OD/092/12
- Treatment of hepatic encephalopathy, OD/101/12
- Treatment of macular telangiectasia type 2, OD/160/11
- Treatment of myelodysplastic syndrome, OD/051/12
- Treatment of renal cell carcinoma, OD/094/12
- Treatment of small cell lung cancer, OD/098/12
- Treatment of thymic epithelial tumours, OD/093/12

2.2. For discussion / preparation for an 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 cutaneous T-cell lymphoma, OD/050/12
- Treatment of diffuse large B-cell lymphoma, OD/073/12
- Treatment of dyskeratosis congenita, OD/136/11
- Treatment of Extranodal Marginal Zone B-cell Lymphoma of the MALT type, OD/072/12
- Treatment of follicular lymphoma, OD/076/12
- Treatment of glioma, OD/136/12
- 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 mercury toxicity, OD/119/12
- Treatment of multiple myeloma, OD/113/12
- Treatment of neuroblastoma, OD/112/12
- Treatment of nodal marginal zone B-cell lymphoma ± monocytoid, OD/078/12
- Treatment of non-infectious uveitis, OD/118/12
- Treatment of ovarian cancer, OD/114/12
- Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), OD/104/12
- Treatment of retinitis pigmentosa, OD/135/12
- Treatment of senile systemic amyloidosis, OD/115/12
- Treatment of splenic marginal zone B-cell lymphoma, OD/079/12
- Treatment of squamous cell carcinoma of the head and neck, OD/120/12
- Treatment of systemic light chain amyloidosis, OD/110/12
- Treatment of TNF-receptor associated periodic syndrome (TRAPS), OD/060/12
- Treatment of trypanosomiasis, OD/116/12

2.3. COMP opinions adopted via written procedure following previous meeting

2.3.1 Alpha-1 proteinase inhibitor (for inhalation use) for treatment of cystic fibrosis, Grifols Deutschland GmbH - EMA/OD/058/12.

2.3.2 Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor for treatment of haemophilia A, Novo Nordisk A/S - EMA/OD/095/12.

2.3.3 Obinutuzumab for treatment of chronic lymphocytic leukaemia, Roche Registration Limited - EMA/OD/102/12.

2.3.4 Recombinant human lecithin cholesterol acyltransferase for treatment of lecithin cholesterol acyltransferase deficiency, Alphacore Pharma Limited - EMA/OD/091/12.

2.4. Evaluation on-going

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

2.5. Validation on-going

Validation is on-going for twenty eight applications for orphan designation.

3. Requests for protocol assistance

Protocol assistance letters for two products for treatment of acute myeloid leukaemia.

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 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)

5.2.3 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)

5.2.4 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 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).

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

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

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

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

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

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

5.3.11 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).

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

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

5.3.15 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 Procedure for orphan medicinal product designation - Guidance for sponsors

http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/09/WC500003769.pdf.

6.2 European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP)

- Expression of interest for the COMP representative to the PCWP http://www.ema.europa.eu/ema/index.jsp?curl=pages/contacts/CHMP/people_listing_000017.jsp&mid=WC0b01ac0580028d32
- Draft PCWP Work Plan for 2013.

6.3 European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations (HCPWP)

- Draft HCPWP Work Plan for 2013.

7. Any other business

7.1 EMA - FDA and EU - Japan collaboration.

7.2 COMP Work Programme 2013-2015.

7.3 COMP Information Pack.