EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMA/COMP/46712/2014
Human Medicines Research & Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 4-6 February 2014 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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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 / an opinion

- Prevention of antibody-mediated rejection after solid organ transplantation - EMA/OD/176/13
- Prevention of bronchopulmonary dysplasia - EMA/OD/161/13
- Prevention of bronchopulmonary dysplasia in premature neonates of less than 32 weeks of gestational age - EMA/OD/172/13
- Prevention of graft rejection following solid organ transplantation - EMA/OD/168/13
- Treatment of amyotrophic lateral sclerosis - EMA/OD/178/13
- Treatment of cystic fibrosis - EMA/OD/166/13
- Treatment of familial amyloid polyneuropathy - EMA/OD/098/13
- Treatment of glycogen storage disease type II (Pompe's disease) - EMA/OD/148/13
- Treatment of pancreatic cancer - EMA/OD/164/13
- Treatment of small cell lung cancer - EMA/OD/094/13
- Treatment of systemic sclerosis - EMA/OD/179/13

2.2. For discussion / preparation for an opinion

- Treatment of Leber Congenital Amaurosis type 1 (LCA1) - EMA/OD/182/13
- Treatment of nontuberculous mycobacterial lung disease - EMA/OD/191/13
- Treatment of Recombination Activating Gene 1 deficient Severe Combined Immunodeficiency (RAG1-SCID) - EMA/OD/188/13
- Treatment of sickle cell disease - EMA/OD/184/13
- Treatment of acute lymphoblastic leukaemia - EMA/OD/187/13
- Treatment of mobilisation of progenitor cells prior to stem cell transplantation - EMA/OD/192/13
- Treatment of soft tissue sarcoma - EMA/OD/190/13
- Treatment of Charcot Marie Tooth disease type 1A - EMA/OD/193/13
- Treatment of ovarian cancer - EMA/OD/186/13
- Treatment of osteonecrosis of the jaw - EMA/OD/177/13

- Treatment of argininosuccinic aciduria - EMA/OD/189/13
- Treatment of aneurysmal subarachnoid haemorrhage - EMA/OD/171/13
- Treatment of systemic sclerosis - EMA/OD/165/13
- Treatment of symptomatic transthyretin mediated amyloidosis - EMA/OD/194/13
- Treatment of acute myeloid leukaemia - EMA/OD/181/13

2.3. Appeal procedure

2.3.1 5-Chloro-N2-[2-isopropoxy-5-methyl-4-(4-piperidinyl)phenyl]-N4-[2-(isopropylsulfonyl)phenyl]-2,4-pyrimidinediamine for treatment of non-small cell lung carcinoma (NSCLC) that is anaplastic lymphoma kinase (ALK)-positive, Novartis Europharm Limited - EMA/OD/113/13

2.4. COMP opinions adopted via written procedure following previous meeting

2.4.1 N-({Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide for treatment of optic neuritis, Bionure Farma SL - EMA/OD/175/13

2.4.2 Ruxolitinib for treatment of Polycythaemia vera, Novartis Europharm Limited - EMA/OD/169/13

2.5. Evaluation on-going

Evaluation is on-going for 7 applications which will be discussed at the February meeting.

2.6. Validation on-going

Validation is on-going for 28 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of chronic lymphocytic leukaemia
- Treatment of follicular lymphoma
- Treatment of acute lymphoblastic 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 Adempas (Methyl 4,6-diamino-2-[1-(2-fluorobenzyl)-1H-pyrazolo[3,4-b]pyridine-3-yl]-5-pyrimidinyl(methyl)carbamate) for treatment of pulmonary arterial hypertension including treatment of chronic thromboembolic pulmonary hypertension; Bayer Pharma AG (EU/3/07/518)

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

For revision of the COMP opinion adopted at the December 2013 COMP meeting.

5.1.3 Masiviera (Masitinib mesilate) for treatment of pancreatic cancer; AB Science (EU/3/09/684)

5.1.4 Translarna (3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid) for treatment of Duchenne muscular dystrophy; PTC Therapeutics Ltd (EU/3/05/278)

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

5.2.1 Folcepri (N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine to be used with folic acid) for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (EU/3/12/1043)

5.2.2 Neocepri (Folic acid to be used with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine) for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (EU/3/12/1044)

5.2.3 Nexavar (Sorafenib tosylate), Bayer HealthCare AG, (Type II variation):

- treatment of follicular thyroid cancer (EU/3/13/1199)
- treatment of papillary thyroid cancer (EU/3/13/1200)

5.2.4 Vimizim (Recombinant human N-acetylgalactosamine-6-sulfatase) for treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome); BioMarin Europe Ltd (EU/3/09/657)

5.2.5 Vynfinit (Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine) for treatment of ovarian cancer; Endocyte Europe, B.V. (EU/3/12/959)

5.3. On-going procedures

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

5.3.2 Cerdelga ((1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt) for treatment of Gaucher disease; Genzyme Europe BV (EU/3/07/514)

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

5.3.4 Cyramza (Ramucirumab) for treatment of gastric cancer; Eli Lilly Nederland B.V. (EU/3/12/1004)

5.3.5 Gazyva (Obinutuzumab) for treatment of chronic lymphocytic leukaemia; Roche Registration Limited (EU/3/12/1054)

5.3.6 Heparesc (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)

5.3.7 Holoclar (Ex vivo expanded autologous human corneal epithelium containing stem cells) for treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns; Chiesi Farmaceutici S.p.A. (EU/3/08/579)

5.3.8 Imbruvica (1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one) for treatment of mantle cell lymphoma; Janssen-Cilag International N.V. (EU/3/13/1115)

5.3.9 Jinarc (Tolvaptan) for treatment of autosomal dominant polycystic kidney disease; Otsuka Pharmaceutical Europe Ltd (EU/3/13/1175)

5.3.10 Ketoconazole AID-SCFM for treatment of Cushing's syndrome; Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare (EU/3/12/1031)

5.3.11 Levofloxacin – Aptalis (Levofloxacin hemihydrate) for treatment of cystic fibrosis; Aptalis Pharma SAS (EU/3/08/566)

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

5.3.13 Neofordex (Dexamethasone (40 mg tablet) for treatment of multiple myeloma; Laboratoires CTRS (Cell Therapies Research & Services) (EU/3/10/745)

5.3.14 Olaparib AstraZeneca AB (Olaparib) for treatment of ovarian cancer; AstraZeneca AB (EU/3/07/501)

5.3.15 Scenesse ([Nle4, D-Phe7]-alfa-melanocyte stimulating hormone, Afamelanotide) for treatment of erythropoietic protoporphyria; Clinuvel (UK) Limited (EU/3/08/54)

5.3.16 Signifor (Pasireotide) for treatment of Cushing's disease; Novartis Europharm Limited (EU/3/09/671) Type II variation

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

5.3.18 Sylvant (Chimeric-anti-interleukin-6 monoclonal antibody) for treatment of Castleman's disease; Janssen-Cilag International N.V.; (EU/3/07/508)

5.3.19 Unituxin (Chimeric monoclonal antibody against GD2 Dinutuximab) for Treatment of neuroblastoma; United Therapeutics Europe Ltd (EU/3/11/879)

5.3.20 Vantobra, Tobramycin (inhalation use) for treatment of Pseudomonas Aeruginosa lung infection in cystic fibrosis; PARI Pharma GmbH (EU/3/09/613)

5.4. Appeal procedure

5.4.1 Para-aminosalicylic acid Lucane (Para-aminosalicylic acid) for treatment of tuberculosis; Lucane Pharma SA (EU/3/10/826)

5.5. COMP opinions adopted via written procedure following previous meeting

5.5.1 Cometriq [Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt] for treatment of medullary thyroid carcinoma; TMC Pharma Services Ltd (EU/3/08/610)

6. Procedural aspects

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

- Draft Work Plan for the 2014, EMA/552003/2014

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

- Draft Work Plan for the 2014, EMA/549571/2014

7. Any other business

7.1 Orphan medicines survey on designation and development conducted in October-November 2012

7.2 2nd presentation on the EMA move to 30 Churchill Place

7.3 COMP article *Use of biomarkers in the context of orphan medicines designation in the European Union* published in the Orphanet Journal of Rare Diseases on 24 January 2014