EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 February 2014
EMA/COMP/95442/2014
Human Medicines Research & Development Support

Committee for Orphan Medicinal Products (COMP)

Agenda of the 11-12 March 2014 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 following a request for access to documents under Regulation (EC) No 1049/2001 as 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 / an opinion

- Treatment of acute lymphoblastic leukaemia - EMA/OD/187/13
- Treatment of aneurysmal subarachnoid haemorrhage - EMA/OD/171/13
- Treatment of ovarian cancer - EMA/OD/186/13
- Treatment of sickle cell disease - EMA/OD/184/13
- Treatment of symptomatic transthyretin mediated amyloidosis - EMA/OD/194/13
- Treatment of systemic sclerosis - EMA/OD/165/13

2.2. For discussion / preparation for an opinion

- Treatment of biliary tree cancer - EMA/OD/199/13
- Treatment of dystrophic epidermolysis bullosa - EMA/OD/201/13
- treatment of follicular lymphoma - EMA/OD/200/13
- Treatment of gastro-entero-pancreatic neuroendocrine tumours - EMA/OD/196/13
- Treatment of inherited retinal disease caused by lecithin: retinol acyltransferase (*LRAT*) or retinal pigment epithelium protein 65 (*RPE65*) mutations - EMA/OD/197/13
- Treatment of plasma cell myeloma - EMA/OD/198/13
- Treatment of Waldenström's macroglobulinemia - EMA/OD/185/13

2.3. COMP opinions adopted via written procedure following previous meeting

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. Evaluation on-going

Evaluation is on-going for 16 applications which will be discussed at the April meeting.

2.5. Validation on-going

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

3. Requests for protocol assistance

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

5.2.1 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 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 Sorafenib tosylate Bayer HealthCare AG for:

a) treatment of follicular thyroid cancer (EU/3/13/1199)

b) treatment of papillary thyroid cancer (EU/3/13/1200)

5.2.4 Pasireotide for treatment of Cushing's disease; Novartis Europharm Limited (EU/3/09/671)

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

5.2.6 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)

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

5.3. On-going procedures

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

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

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

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

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

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

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

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

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

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

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

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

5.3.18 (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)

5.3.19 Chimeric monoclonal antibody against GD2 Dinutuximab) for treatment of neuroblastoma; United Therapeutics Europe Ltd (OD/002/11)

5.4. Appeal procedure

5.4.1 **Deltyba** ((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 (EU/3/07/524)

6. Procedural aspects

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

- Draft Agenda of the 25 February 2014 PCWP meeting, EMA/19425/2014

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

- Draft Agenda of the 25 February 2014 HCPWP meeting, EMA/13760/2014

6.3 Joint PCWP and HCPWP Workshop on regulatory and methodological standards to improve benefit/risk evaluation of medicines

- Draft Agenda of the 26 February 2014 Workshop, EMA/796225/2013

7. Any other business

7.1 3rd presentation on the EMA move to 30 Churchill Place

Action: for information / to be presented by A. Brandt (Infrastructure Services), proposed time 12 March at 08.30hrs

7.2 Update on the International Rare Diseases Research Consortium (IRDiRC)