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EMA/COMP/379025/2013  
Human Medicines Development and Evaluation

## Committee for Orphan Medicinal Products (COMP)

Agenda of the 9-11 July 2013 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 of the previous meeting
- Declaration of conflicts of interest

## **2. Applications for orphan medicinal product designation**

### **2.1. For 2<sup>nd</sup> discussion / an opinion**

- Prevention of recurrent hepatitis C virus induced liver disease in liver transplant recipients - EMA/OD/050/13
- Prevention of rejection of solid organ transplantation - EMA/OD/043/13
- Treatment and management of squamous cell carcinoma of the head and neck, in combination with chemotherapy - EMA/OD/048/13
- Treatment of acromegaly - EMA/OD/042/13
- Treatment of acute myeloid leukaemia - EMA/OD/064/13
- Treatment of autosomal dominant polycystic kidney disease - EMA/OD/066/13
- Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma (CLL/SLL)- EMA/OD/056/13
- Treatment of marginal zone lymphoma - EMA/OD/054/13
- Treatment of osteosarcoma - EMA/OD/020/13
- Treatment of prurigo nodularis - EMA/OD/046/13
- Treatment of small cell lung cancer - EMA/OD/040/13
- Treatment of snakebite envenomation - EMA/OD/068/13
- Treatment of systemic transthyretin-related amyloidosis - EMA/OD/049/13

### **2.2. For discussion / preparation for an opinion**

- Treatment of sickle cell disease - EMA/OD/084/13
- Treatment of Behçets' disease - EMA/OD/076/13
- Treatment of eosinophilic esophagitis - EMA/OD/078/13
- Treatment of pancreatic cancer - EMA/OD/071/13
- Treatment of mastocytosis - EMA/OD/079/13
- Treatment of glioma - EMA/OD/033/13
- Treatment of neuromyelitis optica -EMA/OD/087/13

- Treatment of non-traumatic osteonecrosis - EMA/OD/070/13
- Treatment of chronic sarcoidosis - EMA/OD/081/13
- Treatment of myotonic disorders - EMA/OD/069/13
- Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis - EMA/OD/073/13
- Treatment of cervical insufficiency - EMA/OD/085/13
- Treatment of congenital factor VII deficiency - EMA/OD/051/13
- Treatment of growth hormone deficiency - EMA/OD/074/13
- Treatment of plasma cell myeloma - EMA/OD/072/13
- Treatment of congenital sucrase-isomaltase deficiency - EMA/OD/083/13
- Treatment of parathyroid carcinoma - EMA/OD/080/13

### **2.3. Evaluation on-going**

Evaluation for valid applications will commence on 12 July 2013.

### **2.4. Validation on-going**

Validation is on-going for 21 applications for orphan designation.

## **3. Requests for protocol assistance**

- Treatment of acromegaly
- Treatment of anaplastic thyroid cancer
- Treatment of mercury toxicity
- Treatment of primary myelofibrosis
- Treatment of systemic sclerosis

## **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

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

**5.2.1 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)

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

**5.2.3 Procysbi** (former name: cysteamine bitartrate) [Cysteamine bitartrate (gastroresistant)] for treatment of cystinosis; Raptor Pharmaceuticals Europe B.V. (EU/3/10/778)

**5.3.4 Sirturo** [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. (EU/3/05/314)

### 5.3. On-going procedures

**5.3.1 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)

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

**5.3.3 Defitelio** (Defibrotide); Gentium S.p.A.

- prevention of hepatic veno-occlusive disease (EU/3/04/211)

- treatment of hepatic veno-occlusive disease (EU/3/04/212)

**5.3.4 Folcepri** (N-[4-[[[(2-amino-3,4-dihydro-4-oxo-6-pteridiny)l)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)

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

**5.3.6 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)

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

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

**5.3.9 Neocepri** (Folic acid to be used with N-[4-[[[(2-amino-3,4-dihydro-4-oxo-6-pteridiny]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)

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

**5.3.11 Opsumit** (Macitentan) for treatment of pulmonary arterial hypertension; Actelion Registration Ltd. (EU/3/11/909)

**5.3.12 PAS-GR** (Para-aminosalicylic acid) for treatment of tuberculosis; Lucane Pharma SA (EU/3/10/826)

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

**5.3.14 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)

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

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

**5.3.17 Vynfinit** (Vincalokoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[[(2-amino-3,4-dihydro-4-oxo-6-pteridiny]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)

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

## 6. Procedural aspects

**6.1** Proposals for improvement of the COMP procedures

## 7. Any other business

**7.1** Projects on adaptive licensing

**7.2** Informal COMP meeting to be held on 7 November 2013 (at the EMA) following the 150<sup>th</sup> COMP meeting on 5-6 November 2013

**7.3** Update on international collaboration activities (i.e. FDA and PMDA/MHLW teleconferences)

**7.4** Proposal for a publication strategy

- 7.5** Results on the survey on orphan medicinal products development
- 7.6** Workshop with the PDCO on a definition of conditions for haematological malignancies
- 7.7** Grounds of major contribution to patient care