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EMA/COMP/310701/2013 Rev. 1  
Human Medicines Development and Evaluation

## Committee for Orphan Medicinal Products (COMP)

Agenda of the 11-13 June 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**

- For acute lymphoblastic leukaemia - EMA/OD/024/13
- For prevention of graft-versus-host disease - EMA/OD/026/13
- For treatment of amyotrophic lateral sclerosis - EMA/OD/011/13
- For treatment of Hunter syndrome - EMA/OD/021/13
- For treatment of lymphoblastic lymphoma - EMA/OD/027/13
- For treatment of narcolepsy - EMA/OD/029/13
- For treatment of pulmonary alveolar proteinosis - EMA/OD/106/12
- For treatment of renal cell carcinoma - EMA/OD/030/13
- For treatment of renal cell carcinoma in patients with remaining primary tumour or presence of at least one metastasis - EMA/OD/172/12

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

- For prevention of hepatitis B recurrence following liver transplantation - EMA/OD/045/13
- For prevention of ischaemia reperfusion injury associated with solid organ transplantation - EMA/OD/018/13
- For prevention of recurrent hepatitis C virus induced liver disease in liver transplant recipients - EMA/OD/050/13
- For prevention of rejection of solid organ transplantation - EMA/OD/043/13
- For treatment and management of squamous cell carcinoma of the head and neck, in combination with chemotherapy - EMA/OD/048/13
- For treatment of acromegaly - EMA/OD/042/13
- For treatment of acute myeloid leukaemia - EMA/OD/064/13
- For treatment of amyotrophic lateral sclerosis - EMA/OD/044/13
- For treatment of argininosuccinic aciduria - EMA/OD/059/13
- For treatment of ataxia telangiectasia - EMA/OD/052/13

- For treatment of autosomal dominant polycystic kidney disease - EMA/OD/066/13
- For treatment of carbamoylphosphate synthetase I deficiency - EMA/OD/057/13
- For treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma (CLL/SLL)- EMA/OD/056/13
- For treatment of citrullinaemia type 1 - EMA/OD/058/13
- For treatment of citrullinaemia type 2 - EMA/OD/062/13
- For treatment of follicular lymphoma - EMA/OD/047/13
- For treatment of follicular lymphoma - EMA/OD/053/13
- For treatment of hyperargininaemia - EMA/OD/060/13
- For treatment of limbal stem cell deficiency - EMA/OD/065/13
- For treatment of lymphoplasmacytic lymphoma/Waldenström macroglobulinemia (LPL/WM) - EMA/OD/055/13
- For treatment of malignant thymomas - EMA/OD/036/13
- For treatment of marginal zone lymphoma - EMA/OD/054/13
- For treatment of multiple myeloma - EMA/OD/038/13
- For treatment of n-acetylglutamate synthetase deficiency - EMA/OD/061/13
- For treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome) - EMA/OD/063/13
- For treatment of osteosarcoma - EMA/OD/020/13
- For treatment of ovarian cancer - EMA/OD/039/13
- For treatment of pancreatic cancer - EMA/OD/037/13
- For treatment of prurigo nodularis - EMA/OD/046/13
- For treatment of small cell lung cancer - EMA/OD/040/13
- For treatment of snakebite envenomation - EMA/OD/068/13
- For treatment of systemic transthyretin-related amyloidosis - EMA/OD/049/13

### 2.3. COMP opinions adopted via written procedure following previous meeting

- **Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-12,20-dinitroporphyrin** for treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy, MorEx Development Partners LLP - EMA/OD/022/13
- **Immortalised human C3A hepatoblastoma cells** for treatment of acute liver failure, Vital Therapies Limited - EMA/OD/032/13
- **Synthetic double-stranded siRNA oligonucleotide directed against the keratin 6a N171K mutation** for treatment of pachyonychia congenita, Alan Irvine - EMA/OD/028/13

## 2.4. Evaluation on-going

Evaluation for valid applications will commence on 7 June 2013.

## 2.5. Validation on-going

Validation is on-going for 25 applications for orphan designation.

## 3. Requests for protocol assistance

- 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

**5.1.1 Pomalidomide Celgene** (Pomalidomide) for treatment of multiple myeloma; Celgene Europe Ltd. (EU/3/09/672)

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

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

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

### 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 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)

**5.3.3 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)

**5.3.4 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)

**5.3.5 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)

**5.3.6 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)

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

**5.3.8 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)

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

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

**5.3.11 Neocepri** (Folic acid to be used with 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) for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (EU/3/12/1044)

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

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

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

**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 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)

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

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

**5.3.19 Vynfinit** (Vincalculoblastin-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)

**5.3.20 Winfuran** (-)-17(cyclopropylmethyl)-1,14  $\beta$ -dihydroxy-4,5 alpha-epoxy-6 $\beta$ -[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** Appointment of the COMP representative to the European Medicines Agency Human Scientific Committees' Working Party with Healthcare Professionals' Organisations ([HCPWP](#))