27 March 2013
EMA/COMP/188612/2013
Human Medicines Development and Evaluation

# Committee for Orphan Medicinal Products (COMP)

## Agenda of the 16-17 April 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 2nd discussion / an opinion

- For treatment of adrenocortical carcinoma - EMA/OD/195/12
- For treatment of B-cell acute lymphoblastic leukaemia - EMA/OD/194/12
- For treatment of drug-induced ototoxicity - EMA/OD/193/12
- For treatment of graft versus host disease - EMA/OD/197/12
- For treatment of hepatocellular carcinoma - EMA/OD/187/12
- For treatment of invasive aspergillosis - EMA/OD/189/12
- For treatment of non-dystrophic myotonia - EMA/OD/182/12
- For treatment of ovarian cancer - EMA/OD/192/12
- For treatment of polycythaemia vera - EMA/OD/188/12
- For treatment of zygomycosis/mucormycosis - EMA/OD/190/12

## 2.2. For discussion / preparation for an opinion

- For diagnosis of neuroendocrine tumours - EMA/OD/001/13
- For treatment of (localized) neuroendocrine tumours - EMA/OD/002/13
- For treatment of 5q spinal muscular atrophy - EMA/OD/034/13
- For treatment of adenosine deaminase deficient-severe combined immunodeficiency (ADA-SCID) - EMA/OD/014/13
- For treatment of Alagille syndrome - EMA/OD/007/13
- For treatment of cystic fibrosis - EMA/OD/005/13
- For treatment of cytomegaloviral disease - EMA/OD/013/13
- For treatment of maligmant mesothelioma - EMA/OD/012/13
- For treatment of primary biliary cirrhosis - EMA/OD/010/13
- For treatment of primary sclerosing cholangitis - EMA/OD/008/13
- For treatment of progressive familial intrahepatic cholestasis - EMA/OD/009/13

- For treatment of retinitis pigmentosa - EMA/OD/015/13
- For treatment of Schnitzler syndrome - EMA/OD/006/13
- For treatment of Stargardt's disease - EMA/OD/004/13
- For treatment of transglutaminase 1 deficient autosomal recessive congenital ichthyosis - EMA/OD/003/13

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

- **(S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one** for treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma, Voisin Consulting S.A.R.L. - EMA/OD/196/12

### 2.4. Evaluation on-going

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

### 2.5. Validation on-going

Validation is on-going for forty three applications for orphan designation.

## 3. Requests for protocol assistance

- Treatment of acute myeloid leukaemia
- Treatment of ovarian cancer
- Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy

## 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 Pheburane** (sodium phenylbutyrate) for treatment of carbamoyl-phosphate synthase-1 deficiency; Lucane Pharma SA (EU/3/12/951)

**5.1.2 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

- for treatment of chronic myeloid leukaemia (EU/3/09/716)
- for treatment of acute lymphoblastic leukaemia (EU/3/09/715)

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

- for prevention of hepatic veno-occlusive disease (EU/3/04/211)
- for treatment of hepatic veno-occlusive disease (EU/3/04/212)

## 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]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole) for treatment of tuberculosis; Otsuka Novel Products GmbH (EU/3/07/524) [Co-ordinators: V. Stoyanova / L. Fregonese]

## 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 Cysteamine bitartrate** [Cysteamine bitartrate (gastroresistant)] for treatment of cystinosis; Raptor Pharmaceuticals Europe B.V. (EU/3/10/778)

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

**5.3.7 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 (EU/3/02/092). Type II variation - for the treatment of chronic iron overload due to blood transfusions in patients with beta thalassaemia major aged 6 years and older

**5.3.8 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)

**5.3.9 GPLSCD01** (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)

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

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

**5.3.12 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)

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

**5.3.16 Revlimid** (3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) for treatment of myelodysplastic syndromes; Celgene Europe Limited – UK (EU/3/04/192)

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

**5.3.18 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)

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

**5.3.20 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)

**5.3.21 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 (EU/3/02/115)

# 6. Procedural aspects

**6.1** Report on fee reductions for orphan medicine

**6.2** Wording of the COMP opinions

# 7. Any other business

**7.1** International Summer School "Clinical practice guidelines on rare diseases" to be held at the Italian National Institute of Health in Rome on 8-12 July 2013