20 August 2013
EMA/COMP/418589/2013
Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP)

Agenda of the 3-4 September 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).

Contents

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1. Introduction

- Adoption of the draft Agenda
- Adoption of the draft Minutes of the previous meeting
- Revised Final Minutes from June meeting for information
- Declaration of conflicts of interest

2. Applications for orphan medicinal product designation

2.1. For 2nd discussion / an opinion

- Treatment of glioma - EMA/OD/033/13
- Treatment of congenital factor VII deficiency - EMA/OD/051/13
- Treatment of plasma cell myeloma - EMA/OD/072/13
- Treatment of cervical insufficiency - EMA/OD/085/13
- Treatment of chronic sarcoidosis - EMA/OD/081/13
- Treatment of myotonic disorders - EMA/OD/069/13
- Treatment of parathyroid carcinoma - EMA/OD/080/13

2.2. For discussion / preparation for an opinion

- Treatment of neurotrophic keratitis - EMA/OD/184/12
- Treatment of acromegaly - EMA/OD/082/13
- Treatment of the Allan-Herndon-Dudley Syndrome - EMA/OD/089/13
- Treatment of cystic fibrosis - EMA/OD/096/13
- Treatment of glioma - EMA/OD/086/13
- Prevention of graft versus host disease - EMA/OD/103/13
- Treatment of Duchenne muscular dystrophy - EMA/OD/090/13
- Treatment of Fabry disease - EMA/OD/100/13
- Treatment of mucopolysaccharidosis type II (Hunter's syndrome) - EMA/OD/091/13
- Treatment of follicular thyroid cancer - EMA/OD/092/13
- Treatment of papillary thyroid cancer - EMA/OD/093/13
- Treatment of Wiskott-Aldrich-Syndrome - EMA/OD/104/13
- Treatment of Adult Onset Still's Disease - EMA/OD/099/13

- Treatment of Pseudomonas aeruginosa infection in cystic fibrosis - EMA/OD/101/13
- Treatment of complex regional pain syndrome - EMA/OD/088/13

2.3. Evaluation on-going

Evaluation is on-going for 18 applications for orphan designation.

2.4. Validation on-going

Validation is on-going for 17 applications for orphan designation.

3. Requests for protocol assistance

- Treatment of systemic sclerosis
- Treatment of acromegaly
- Treatment of anaplastic thyroid cancer
- Treatment of primary myelofibrosis
- Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukemic/disseminated)
- Treatment of graft-versus-host disease
- Treatment of chronic lymphocytic leukaemia
- Treatment of Fabry disease
- Treatment of ovarian cancer
- Treatment of mercury toxicity

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 Defitelio (Defibrotide) for treatment of hepatic veno-occlusive disease; Gentium S.p.A (EU/3/04/212)

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

5.2.1 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)

5.2.2 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)

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

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

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

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

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

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

5.2.9 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. 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 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)

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

5.3.4 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)

5.3.5 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)

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

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

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

5.3.9 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)

5.3.10 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 Proposal for improvement of the COMP procedures

6.2 Prevention of graft rejection following solid organ transplantation - EMA/OD/043/13

6.3 PCWP/HCPWP joint meeting 25 September 2013

6.4 Workshop on patient's voice in the evaluation of medicines 26 September 2013

7. Any other business

7.1 Projects on adaptive licensing

7.2 Proposal for a publication strategy (including book on rare diseases)

7.3 Results on the survey on orphan medicinal products development

7.4 Workshop with the PDCO on a definition of conditions for haematological malignancies

7.5 Grounds of major contribution to patient care

7.6 Similarity group

7.7 Scientific Coordination Board