EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

# Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

February 2013

The Committee for Orphan Medicinal Products held its 142nd plenary meeting on 5-6 February 2013.

## Orphan medicinal product designation

The COMP adopted 12 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate** for treatment of hepatocellular carcinoma; Eli Lilly Nederland B.V.
- **Gevokizumab** for treatment of chronic non-infectious uveitis; Les Laboratoires Servier.
- **Murine IgM monoclonal antibody binding to alpha beta T-cell receptor** for prevention of graft rejection following solid organ transplantation; CTI Clinical Trial and Consulting Services.
- **Poloxamer 188** for treatment of sickle cell disease; Theradex (Europe) Ltd.
- **Recombinant human heat shock protein 70** for treatment of Niemann-Pick disease, type C; Orphazyme ApS.
- **Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl] acetate salt** for treatment of congenital alpha-1 antitrypsin deficiency; Polyphor UK.
- **Recombinant adeno-associated viral vector containing the human *retinoschisin* gene** for treatment of X-linked juvenile retinoschisis; TMC Pharma Services Ltd.

2. Opinions adopted at the first COMP discussion:

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- **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.
- **2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid** for treatment of systemic sclerosis; Sanofi-Aventis Groupe.
- **Mepolizumab** for treatment of Churg-Strauss syndrome; Glaxo Group Limited (Greenford).
- **Ramiprilat** for treatment of Stargardt's disease; Iris Pharma.
- **Recombinant human tripeptidyl-peptidase 1** for treatment of neuronal ceroid lipofuscinosis type 2; BioMarin Europe Ltd.

## Negative opinion

Following the appeal to the negative opinion adopted on 6 December 2012 the COMP adopted their final negative opinion recommending the refusal of the orphan medicinal product designation for the following medicine:

- **Zoledronic acid** for treatment of complex regional pain syndrome; Axsome Therapeutics Limited.

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission.

## Other information on the orphan medicinal product designation

### Lists of questions

The COMP adopted 10 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

### Oral hearings

8 oral hearings took place.

### Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 applications for orphan medicinal product designation were withdrawn.

### Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in Annex 1.

The list of medicinal products for which decisions on orphan designation[1] have been given by the European Commission since the last COMP meeting is provided in Annex 2.

### Applications for marketing authorisation for orphan medicinal products

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP meeting reports the EMA website.

1 Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

**Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council**

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted an opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- **Bosutinib (Bosulif)** for treatment of chronic myeloid leukaemia; Pfizer Limited.

## Upcoming meetings

- The 143rd meeting of the COMP will be held on 12-13 March 2013.

## Other matters

The main topics addressed during the meeting related to:

- 1 protocol assistance letter was adopted.

**Note**

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the EMA website: www.ema.europa.eu

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# Annex 1

**Overview for orphan medicinal product designation procedure since 2000**

| Year | Applications submitted | Applications discussed in reporting year | Positive COMP opinions | Applications withdrawn | Final negative COMP opinions | EC designations | Orphan medicinal products authorised |
|---|---|---|---|---|---|---|---|
| 2013 | 16 | 27 | 20 (74%) | 6 (22%) | 1 (4%) | 13 | 0 |
| 2012 | 197 | 192 | 139 (72%) | 52 (27%) | 1 (1%) | 148 | 12 |
| 2011 | 166 | 158 | 111 (70%) | 45 (29%) | 2 (1%) | 107 | 5 |
| 2010 | 174 | 176 | 123 (70%) | 51 (29%) | 2 (1%) | 128 | 4 |
| 2009 | 164 | 136 | 113 (83%) | 23 (17%) | 0[2] (0%) | 106 | 9 |
| 2008 | 119 | 118 | 86 (73%) | 31 (26%) | 1 (1%) | 73 | 6 |
| 2007 | 125 | 117 | 97 (83%) | 19 (16%) | 1 (1%) | 98 | 13 |
| 2006 | 104 | 103 | 81 (79%) | 20 (19%) | 2 (2%) | 80 | 9 |
| 2005 | 118 | 118 | 88 (75%) | 30 (25%) | 0 (0%) | 88 | 4 |
| 2004 | 108 | 101 | 75 (74%) | 22 (22%) | 4 (4%) | 73 | 6 |
| 2003 | 87 | 96 | 54 (56%) | 41 (43%) | 1 (1%) | 55 | 5 |
| 2002 | 80 | 75 | 43 (57%) | 30 (40%) | 2[3] (3%) | 49 | 4 |
| 2001 | 83 | 90 | 62[4] (70%) | 27 (29%) | 1 (1%) | 64 | 3 |
| 2000 | 72 | 32 | 26 (81%) | 6 (19%) | 0 (0%) | 14 | 0 |
| **Total** | **1613** | **1539** | **1118 (73%)** | **403 (26%)** | **18 (1%)** | **1096** | **80** |

[2] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[3] Following a quality assurance exercise it was identified that this figure needed correction

# Annex 2

**Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the January 2013 COMP monthly report**

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| **1,2:5,6-Dianhydrogalactitol** | Treatment of glioma | IDIS Ltd | 6 December 2012 | 24 January 2013 |
| **Adeno-associated viral vector serotype 9 containing the human *N-acetylglucosaminidase alpha* gene** | Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome) | Laboratorios del Dr. Esteve, S.A. | 6 December 2012 | 24 January 2013 |
| **Allogeneic motor neuron progenitor cells derived from human embryonic stem cells** | Treatment of 5q spinal muscular atrophy | California Stem Cell (UK) Ltd | 6 December 2012 | 24 January 2013 |
| **Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta$^{A-T87Q}$-globin* gene** | Treatment of beta-thalassaemia intermedia and major | bluebird bio France | 6 December 2012 | 24 January 2013 |
| **Chimeric monoclonal antibody against claudin 6** | Treatment of ovarian cancer | GANYMED Pharmaceuticals AG | 6 December 2012 | 24 January 2013 |
| **Choline tetrathiomolybdate** | Treatment of Wilson's disease | Medical Need Europe AB | 6 December 2012 | 24 January 2013 |
| **Eflornithine in combination with sulindac** | Treatment of familial adenomatous polyposis | Cancer Prevention Pharma Limited | 6 December 2012 | 24 January 2013 |
| **Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor** | Treatment of retinitis pigmentosa | Enpharma Ltd | 6 December 2012 | 24 January 2013 |
| **Lenalidomide** | Treatment of follicular lymphoma | Celgene Europe Limited | 6 December 2012 | 24 January 2013 |
| **Modified recombinant human C-type natriuretic peptide** | Treatment of achondroplasia | BioMarin Europe Ltd | 6 December 2012 | 24 January 2013 |

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| **Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen** | Treatment of pancreatic cancer | Astellas Pharma Europe B.V. | 6 December 2012 | 24 January 2013 |
| **Recombinant modified human growth hormone** | Treatment of growth hormone deficiency | Richardson Associates Regulatory Affairs Ltd | 6 December 2012 | 24 January 2013 |
| **Terguride** | Treatment of systemic sclerosis | Serodapharm UG (haftungsbeschränkt) | 6 December 2012 | 24 January 2013 |