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

April 2013

The Committee for Orphan Medicinal Products held its 144th plenary meeting on 16-17 April 2013.

## Orphan medicinal product designation

### Positive opinions

The COMP adopted 11 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission (EC):

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

- **Allogeneic bone marrow derived mesenchymal cells expanded *ex vivo* in synthetic media** for treatment of graft-versus-host disease; Cell2B Advanced Therapeutics SA
- **Inotuzumab ozogamicin** for treatment of B-cell acute lymphoblastic leukaemia; Pfizer Limited
- **Mexiletine hydrochloride** for treatment of non-dystrophic myotonia; Prof Michael Hanna
- **N-[2,6-bis(1-methylethyl)phenyl]-N'-[[1-[4-(dimethylamino) phenyl]cyclopentyl]methyl]urea, hydrochloride salt** for treatment of adrenocortical carcinoma; Atterocor Ltd

2. Opinions adopted at the first COMP discussion:

- **5-[1-(2,6-dichlorobenzyl)piperidin-4-ylmethoxy]quinazoline-2,4-diamine dihydrochloride** for treatment of 5q spinal muscular atrophy; Repligen Sweden AB
- **Autologous CD34+ cells transduced with a lentiviral vector containing the human *ADA* gene** for treatment of adenosine deaminase-deficient severe combined immunodeficiency; Prof. Bobby Gaspar

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- **Maribavir** for treatment of cytomegalovirus disease in patients with impaired cell mediated immunity; ViroPharma SPRL
- **N-methyl-4-({4-[({3-methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride** for treatment of malignant mesothelioma; TMC Pharma Services Ltd
- **Recombinant human CXCL8 mutant** for treatment of cystic fibrosis; ProtAffin Biotechnologie AG
- **Recombinant human nerve growth factor** for treatment of retinitis pigmentosa; Dompé S.p.A.
- **Recombinant human transglutaminase 1 encapsulated into liposomes** for treatment of transglutaminase-1-deficient autosomal recessive congenital ichthyosis; Westfälische Wilhelms-Universität Münster.

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[1] by the European Commission.

### Lists of questions

The COMP adopted 8 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

9 oral hearings took place.

### Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 applications for orphan medicinal product designation were withdrawn.

### Detailed information on the orphan designation procedures

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

The list of medicinal products for which decisions on orphan designation 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 of those designated orphan medicinal products that have been subject of a new European Union (EU) marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

Details on the authorised orphan medicinal products can be found on the EMA website.

### Article 5(12) (b) 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 2 opinions recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

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

- **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 acute lymphoblastic leukaemia
  - ✓ for treatment of chronic myeloid leukaemia.

The COMP also adopted 3 opinions recommending the removal of the orphan medicinal designations from the EU registry. The sponsor was informed about the possibility to appeal.

## Other matters

The main topics addressed during the meeting related to:

- 4 Protocol Assistance letters were adopted.

## Upcoming meetings

- The 145th meeting of the COMP will be held on 14-15 May 2013.

### 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

### Contact our press officer

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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[2] authorised | Orphan designations included in authorised therapeutic indication |
|---|---|---|---|---|---|---|---|---|
| 2013 | 68 | 60 | 38 (63%) | 21 (35%) | 1 (2%) | 33 | 1 | 1 |
| 2012 | 197 | 192 | 139 (72%) | 52 (27%) | 1 (1%) | 148 | 10 | 12 |
| 2011 | 166 | 158 | 111 (70%) | 45 (29%) | 2 (1%) | 107 | 5 | 5 |
| 2010 | 174 | 176 | 123 (70%) | 51 (29%) | 2 (1%) | 128 | 4 | 4 |
| 2009 | 164 | 136 | 113 (83%) | 23 (17%) | 0[3] (0%) | 106 | 9 | 9 |
| 2008 | 119 | 118 | 86 (73%) | 31 (26%) | 1 (1%) | 73 | 6 | 7 |
| 2007 | 125 | 117 | 97 (83%) | 19 (16%) | 1 (1%) | 98 | 13 | 13 |
| 2006 | 104 | 103 | 81 (79%) | 20 (19%) | 2 (2%) | 80 | 9 | 11 |
| 2005 | 118 | 118 | 88 (75%) | 30 (25%) | 0 (0%) | 88 | 4 | 4 |
| 2004 | 108 | 101 | 75 (74%) | 22 (22%) | 4 (4%) | 73 | 6 | 6 |
| 2003 | 87 | 96 | 54 (56%) | 41 (43%) | 1 (1%) | 55 | 5 | 5 |
| 2002 | 80 | 75 | 43 (57%) | 30 (40%) | 2[4] (3%) | 49 | 4 | 4 |
| 2001 | 83 | 90 | 62[4] (70%) | 27 (29%) | 1 (1%) | 64 | 3 | 3 |
| 2000 | 72 | 32 | 26 (81%) | 6 (19%) | 0 (0%) | 14 | 0 | 0 |
| **Total** | **1665** | **1572** | **1136 (72%)** | **418 (27%)** | **18 (1%)** | **1116** | **79** | **84** |

[2] Number of authorised orphan medicinal products may cover more than one orphan designation
[3] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[4] 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 March 2013 COMP monthly report**

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| **1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H-pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one** | Treatment of mantle cell lymphoma | Janssen-Cilag International N.V. | 6 February 2013 | 12 March 2013 |
| **2-[4-Methoxy-3-(2-m-tolyl-ethoxy)-benzoylamino]-indan-2-carboxylic acid** | Treatment of systemic sclerosis | Sanofi-Aventis Groupe | 6 February 2013 | 12 March 2013 |
| **4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate** | Treatment of hepatocellular carcinoma | Eli Lilly Nederland B.V. | 6 February 2013 | 12 March 2013 |
| **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** | Treatment of congenital alpha-1 antitrypsin deficiency | Polyphor UK | 6 February 2013 | 20 March 2013 |
| **Gevokizumab** | Treatment of chronic non-infectious uveitis | Les Laboratoires Servier | 6 February 2013 | 12 March 2013 |
| **Mepolizumab** | Treatment of Churg-Strauss syndrome | Glaxo Group Limited (Greenford) | 6 February 2013 | 12 March 2013 |
| **Murine IgM monoclonal antibody binding to alpha beta T-cell receptor** | Prevention of graft rejection following solid organ transplantation | CTI Clinical Trial and Consulting Services | 6 February 2013 | 12 March 2013 |
| **Poloxamer 188** | Treatment of sickle cell disease | Theradex (Europe) Ltd | 6 February 2013 | 12 March 2013 |
| **Ramiprilat** | Treatment of Stargardt's disease | Iris Pharma | 6 February 2013 | 12 March 2013 |

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| **Recombinant adeno-associated viral vector containing the human *retinoschisin* gene** | Treatment of X-linked juvenile retinoschisis | TMC Pharma Services Ltd | 6 February 2013 | 12 March 2013 |
| **Recombinant human heat shock protein 70** | Treatment of Niemann-Pick disease, type C | Orphazyme ApS | 6 February 2013 | 12 March 2013 |
| **Recombinant human tripeptidyl-peptidase 1** | Treatment of neuronal ceroid lipofuscinosis type 2 | BioMarin Europe Ltd | 6 February 2013 | 12 March 2013 |

# Annex 3

**Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the March 2013 COMP monthly report**

| Active substance | Invented name | Sponsor/applicant | EU designation number | Designated orphan indication |
|---|---|---|---|---|
| Ex vivo expanded autologous human corneal epithelium containing stem cells | GPLSCD01 | Chiesi Farmaceutici S.p.A. | EU/3/08/579 | Treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns |