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Committee for Orphan Medicinal Products (COMP)

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

June 2014

The Committee for Orphan Medicinal Products held its 157th plenary meeting on 10-12 June 2014.

## Orphan medicinal product designation

### Positive opinions

The COMP adopted 16 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:

- Carboxy pyrrolidine hexanoyl pyrrolidine carboxylate for treatment of AL amyloidosis, GlaxoSmithKline Trading Services Limited
- Marizomib for treatment of plasma cell myeloma, Richardson Associates Regulatory Affairs Ltd
- Recombinant monoclonal antibody to human serum amyloid P component for treatment of AL amyloidosis, GlaxoSmithKline Trading Services Limited
- Sodium acetate salt of the synthetic peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH for treatment for necrotising soft tissue infections, Dr Ulrich Granzer

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 9 containing the human cardiac calsequestrin gene for treatment of catecholaminergic polymorphic ventricular tachycardia, Fondazione Salvatore Maugeri Clinica del Lavoro e della Riabilitazione
- Cediranib for treatment of ovarian cancer, AstraZeneca AB
- Cysteamine bitartrate for treatment of Huntington's disease, Raptor Pharmaceuticals Europe BV

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- Eculizumab for treatment of myasthenia gravis, Alexion Europe SAS
- Humanised anti-alpha v beta 6 monoclonal antibody for treatment of idiopathic pulmonary fibrosis, Biogen Idec Limited
- Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F for treatment of glioma, AbbVie Ltd
- Oxytocin for treatment of Prader-Willi syndrome, Maïthé Tauber
- Recombinant fusion protein consisting of a modified form of the extracellular domain of human activin receptor IIB linked to the human IgG1 Fc domain for treatment of beta thalassaemia intermedia and major, IDEA Innovative Drug European Associates Limited
- Rilotumumab for treatment of gastric cancer, Amgen Europe BV
- Riociguat for treatment of systemic sclerosis, Bayer Pharma AG
- Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues for treatment of haemophilia B, Alnylam UK Limited
- Synthetic double-stranded siRNA oligonucleotide directed against antithrombin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues for treatment of haemophilia A, Alnylam UK Limited

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 23 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

5 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 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.

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

# 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 product:

- **Gazyvaro** (obinutuzumab) for treatment of chronic lymphocytic leukaemia; Roche Registration Limited (EU/3/12/1054)
- **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)

# Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

# Upcoming meetings

- The 158th meeting of the COMP will be held on 8-10 July 2014

**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[2] authorised | Orphan designations included in authorised therapeutic indication |
|---|---|---|---|---|---|---|---|---|
| 2014 | 110 | 97 | 72 (74%) | 24 (25%) | 1 (1%) | 59 | 8 | 8 |
| 2013 | 201 | 197 | 136 (69%) | 60 (30%) | 1 (1%) | 136 | 7 | 8 |
| 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** | **1908** | **1806** | **1306 (72%)** | **481 (27%)** | **19 (1%)** | **1278** | **93** | **99** |

[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 May 2014 COMP monthly report**

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| (5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]-β-D-glucopyranosyl]-oxy]-5-(4-({[(2,2-dimethyl-1,3-dioxolan-4-yl)methoxy]carbonyl}oxy)-3,5-dimethoxyphenyl)-5,8,8a,9-9 April 2014tetrahydroisobenzofuro[5,6-*f*][1,3]benzodioxol-6(5aH)-one | Treatment of biliary tract cancer | CellAct Pharma GmbH | 9 April 2014 | 4 June 2014 |
| $^{177}$Lu-tetraxetan-tetulomab | Treatment of follicular lymphoma | Nordic Nanovector AS | 9 April 2014 | 4 June 2014 |
| 4-(4-Methoxy-phenylamino)-6-methylcarbamyl-quinoline-3-carboxylic acid | Prevention of scarring post glaucoma filtration surgery | Clanotech AB | 9 April 2014 | 4 June 2014 |
| Adeno-associated viral vector serotype 2 containing the human *CHM* gene encoding human Rab escort protein 1 | Treatment of choroideraemia | Alan Boyd Consultants Ltd | 9 April 2014 | 4 June 2014 |
| Aganirsen | Treatment of central retinal vein occlusion | Gene Signal SAS | 9 April 2014 | 10 June 2014 |
| Autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene | Treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome) | Cochamo Systems Ltd | 9 April 2014 | 10 June 2014 |
| Autologous dendritic cells pulsed with RNA from glioma stem cells | Treatment of glioma | Epitarget AS | 9 April 2014 | 4 June 2014 |
| Isavuconazonium sulfate | Treatment of mucormycosis | Basilea Medical Ltd | 9 April 2014 | 4 June 2014 |
| Lutetium ($^{177}$Lu) edotreotide | Treatment of gastro-entero-pancreatic neuroendocrine tumours | ITG Isotope Technologies Garching GmbH | 9 April 2014 | 4 June 2014 |
| Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser- | Treatment of glioma | CLL Pharma | 9 April 2014 | 4 June 2014 |

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| Arg-Arg-Arg-Phe | | | | |
| Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent | Treatment of cystic fibrosis | Imperial Innovations Limited | 9 April 2014 | 4 June 2014 |
| Recombinant human alpha 1 chain homotrimer of type VII collagen | Treatment of epidermolysis bullosa | Shire Pharmaceuticals (Ireland) Limited | 9 April 2014 | 4 June 2014 |

# Annex 3

**Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the May 2014 COMP monthly report**

| Active substance | Designated orphan indication | Sponsor/applicant | EU designation number |
|---|---|---|---|
| | | | |