The Committee for Orphan Medicinal Products held its 204th plenary meeting on 9-11 October 2018.

**Orphan medicinal product designation**

**Positive opinions**

The COMP adopted 22 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:
   
   - 3-((3,5 dimethyl-1H-pyrazol-4-yl)propoxy)-4-fluorobenzoic acid for treatment of ATTR amyloidosis, Pharma Gateway AB;
   
   - 5-((1R,2R)-2-[(cyclopropylmethyl)amino]cyclopropyl)-N-(tetrahydro-2H-pyran-4-yl)thiophene-3-carboxamide monohydrochloride for treatment of Kabuki syndrome, Takeda Pharma A/S;
   
   - Anetumab ravtansine for treatment of ovarian cancer, Bayer AG;
   
   - Apraglutide for treatment of short bowel syndrome, IQVIA RDS Ireland Limited;
   
   - Autologous human adipose perivascular stromal cells genetically modified to secrete soluble tumour necrosis factor-related apoptosis-inducing ligand for treatment of pancreatic cancer, Rigenerand S.r.l.;
   
   
   - Glucagon for treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome, Pharma Gateway AB;
• Humanised IgG1 monoclonal antibody against GD2 for treatment of neuroblastoma, Y-mAbs Therapeutics A/S;
• Imlifidase for treatment of anti-glomerular basement membrane disease, Hansa Medical AB;
• Larotrectinib for treatment of glioma, Bayer AG;
• Larotrectinib for treatment of papillary thyroid cancer, Bayer AG;
• Lisocabtagene maraleucel for treatment of primary mediastinal large B-cell lymphoma, Celgene Europe Limited;
• Propagermanium for treatment of focal segmental glomerulosclerosis, Quality Regulatory Clinical Ireland Limited.

2. Opinions adopted at the first COMP discussion:
• Allogeneic faecal microbiota, pooled for treatment of graft-versus-host disease, MaaT PHARMA;
• Etamsylate for treatment of hereditary haemorrhagic telangiectasia, Consejo Superior de Investigaciones Científicas (CSIC);
• Ex vivo fused normal allogeneic human myoblast with another normal allogeneic human myoblast for treatment of Duchenne muscular dystrophy, Dystrogen Therapeutics S.A.;
• Ex vivo fused normal allogeneic human myoblast with autologous human myoblast derived from Duchenne muscular dystrophy affected donor for treatment of Duchenne muscular dystrophy, Dystrogen Therapeutics S.A.;
• H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O2)-NH2-DOTA-213-bismuth for treatment of glioma, Dr. Regenold GmbH;
• Human apotransferrin for treatment of beta-thalassaemia intermedia and major, Sanquin Plasma Products B.V.;
• Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Phe-Lys-Lys-Glu-Glu-Met-Pro-Ser-Glu-Gly-Tyr-Gln-Asp for treatment of multiple system atrophy, United Neuroscience Limited;
• Fidancagene elaparvovec for treatment of haemophilia B, Pfizer Europe MA EEIG;
• Setmelanotide for treatment of leptin receptor deficiency, TMC Pharma Services Ltd.

3. Opinion following appeal procedures:
None

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

Negative opinion

1. Opinion adopted following the sponsor’s response to the COMP list of questions:
None

1 Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products
2. Opinion following appeal procedures:

None

**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**

18 oral hearings took place.

**Withdrawals of applications for orphan medicinal product designation**

The COMP noted that 12 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

**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 granted by the European Commission since the last COMP meeting is provided in Annex 2.

**Re-assessment of orphan designation at time of marketing authorisation**


When a designated orphan medicinal product receives a positive opinion for marketing authorisation from EMA’s Committee for Medicinal Products for Human Use (CHMP), the COMP has the responsibility to review whether or not the medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation.

1. Opinions adopted at time of CHMP opinion:

   - Luxturna - voretigene neparvovec for treatment of Leber's congenital amaurosis, Spark Therapeutics Ireland Ltd (EU/3/12/981).

2. Opinion following appeal procedures:

None

Details of the designated orphan medicinal products that have been subject of a new European Union (EU) marketing authorisation application since the last COMP monthly report are provided in Annex 3.

Details on the authorised orphan medicinal products can be found on the [EMA website](https://www.ema.europa.eu/en).
Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 205th meeting of the COMP will be held on 6-8 November 2018.

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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E-mail: press@ema.europa.eu
## Annex 1

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

Please also refer to the Community Register of orphan medicinal products for human use.

<table>
<thead>
<tr>
<th>Year</th>
<th>Applications submitted</th>
<th>Applications discussed in reporting year</th>
<th>Positive COMP opinions</th>
<th>Applications withdrawn</th>
<th>Negative COMP opinions</th>
<th>EC designations</th>
<th>Orphan medicinal products authorised</th>
<th>Orphan designations included in authorised therapeutic indication</th>
</tr>
</thead>
<tbody>
<tr>
<td>2018</td>
<td>187</td>
<td>210</td>
<td>131 (62%)</td>
<td>77 (37%)</td>
<td>2 (1%)</td>
<td>115</td>
<td>11</td>
<td>14</td>
</tr>
<tr>
<td>2017</td>
<td>260</td>
<td>245</td>
<td>144 (59%)</td>
<td>100 (41%)</td>
<td>2 (1%)</td>
<td>147</td>
<td>14</td>
<td>15</td>
</tr>
<tr>
<td>2016</td>
<td>330</td>
<td>304</td>
<td>220 (72%)</td>
<td>82 (27%)</td>
<td>2 (1%)</td>
<td>209</td>
<td>14</td>
<td>14</td>
</tr>
<tr>
<td>2015</td>
<td>258</td>
<td>272</td>
<td>177 (65%)</td>
<td>94 (35%)</td>
<td>1 (1%)</td>
<td>190</td>
<td>14</td>
<td>21</td>
</tr>
<tr>
<td>2014</td>
<td>329</td>
<td>259</td>
<td>196 (76%)</td>
<td>62 (24%)</td>
<td>2 (1%)</td>
<td>187</td>
<td>15</td>
<td>16</td>
</tr>
<tr>
<td>2013</td>
<td>201</td>
<td>197</td>
<td>136 (69%)</td>
<td>60 (30%)</td>
<td>1 (1%)</td>
<td>136</td>
<td>7</td>
<td>8</td>
</tr>
<tr>
<td>2012</td>
<td>197</td>
<td>192</td>
<td>139 (72%)</td>
<td>52 (27%)</td>
<td>1 (1%)</td>
<td>148</td>
<td>10</td>
<td>12</td>
</tr>
<tr>
<td>2011</td>
<td>166</td>
<td>158</td>
<td>111 (70%)</td>
<td>45 (29%)</td>
<td>2 (1%)</td>
<td>107</td>
<td>5</td>
<td>5</td>
</tr>
<tr>
<td>2010</td>
<td>174</td>
<td>176</td>
<td>123 (70%)</td>
<td>51 (29%)</td>
<td>2 (1%)</td>
<td>128</td>
<td>4</td>
<td>4</td>
</tr>
<tr>
<td>2009</td>
<td>164</td>
<td>136</td>
<td>113 (83%)</td>
<td>23 (17%)</td>
<td>0 (0%)</td>
<td>106</td>
<td>9</td>
<td>9</td>
</tr>
<tr>
<td>2008</td>
<td>119</td>
<td>118</td>
<td>86 (73%)</td>
<td>31 (26%)</td>
<td>1 (1%)</td>
<td>73</td>
<td>6</td>
<td>7</td>
</tr>
<tr>
<td>2007</td>
<td>125</td>
<td>117</td>
<td>97 (83%)</td>
<td>19 (16%)</td>
<td>1 (1%)</td>
<td>98</td>
<td>13</td>
<td>13</td>
</tr>
<tr>
<td>2006</td>
<td>104</td>
<td>103</td>
<td>81 (79%)</td>
<td>20 (19%)</td>
<td>2 (2%)</td>
<td>80</td>
<td>9</td>
<td>11</td>
</tr>
<tr>
<td>2005</td>
<td>118</td>
<td>118</td>
<td>88 (75%)</td>
<td>30 (25%)</td>
<td>0 (0%)</td>
<td>88</td>
<td>4</td>
<td>4</td>
</tr>
<tr>
<td>2004</td>
<td>108</td>
<td>101</td>
<td>75 (74%)</td>
<td>22 (22%)</td>
<td>4 (4%)</td>
<td>73</td>
<td>6</td>
<td>6</td>
</tr>
<tr>
<td>2003</td>
<td>87</td>
<td>96</td>
<td>54 (56%)</td>
<td>37 (40%)</td>
<td>1 (1%)</td>
<td>55</td>
<td>5</td>
<td>5</td>
</tr>
</tbody>
</table>

3 The number of orphan medicinal products authorised includes the products for which the market exclusivity has expired.
4 The market authorisation of an orphan medicinal product may cover more than one orphan designation.
<table>
<thead>
<tr>
<th>Year</th>
<th>Applications submitted</th>
<th>Applications discussed in reporting year</th>
<th>Positive COMP opinions</th>
<th>Applications withdrawn</th>
<th>Final negative COMP opinions</th>
<th>EC designations</th>
<th>Orphan medicinal products authorised</th>
<th>Orphan designations included in authorised therapeutic indication</th>
</tr>
</thead>
<tbody>
<tr>
<td>2002</td>
<td>80</td>
<td>75</td>
<td>43 (57%)</td>
<td>32 (42%)</td>
<td>2 (3%)</td>
<td>49</td>
<td>4</td>
<td>4</td>
</tr>
<tr>
<td>2001</td>
<td>83</td>
<td>90</td>
<td>62 (70%)</td>
<td>26 (29%)</td>
<td>1 (1%)</td>
<td>64</td>
<td>3</td>
<td>3</td>
</tr>
<tr>
<td>2000</td>
<td>72</td>
<td>32</td>
<td>26 (81%)</td>
<td>3 (10%)</td>
<td>0 (0%)</td>
<td>14</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Total</td>
<td>3162</td>
<td>2995</td>
<td>2102 (70%)</td>
<td>866 (29%)</td>
<td>27 (1%)</td>
<td>2067</td>
<td>153</td>
<td>171</td>
</tr>
</tbody>
</table>
Annex 2

Designations granted by the European Commission following COMP opinion on the fulfilment of the orphan designation criteria since last COMP plenary meeting

No new designations were granted by the European Commission since last COMP plenary meeting.
Annex 3

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

Please also refer to the Community Register of orphan medicinal products for human use.

<table>
<thead>
<tr>
<th>Active substance</th>
<th>Designated orphan indication</th>
<th>Sponsor/applicant</th>
<th>EU designation number</th>
</tr>
</thead>
<tbody>
<tr>
<td>Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta^{+}_{T87Q}-globin gene</td>
<td>Treatment of beta-thalassemia intermedia and major</td>
<td>bluebird bio GmbH</td>
<td>EU/3/12/1091</td>
</tr>
<tr>
<td>Larotrectinib</td>
<td>Treatment of salivary gland cancer</td>
<td>Bayer AG</td>
<td>EU/3/18/1995</td>
</tr>
<tr>
<td>Larotrectinib</td>
<td>Treatment of soft tissue sarcoma</td>
<td>Bayer AG</td>
<td>EU/3/15/1606</td>
</tr>
</tbody>
</table>