Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation
03-05 October 2017

The Committee for Orphan Medicinal Products held its 193rd plenary meeting on 03-05 October 2017.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinion(s) adopted at the second COMP discussion, following the sponsor’s response to the COMP list of questions:
   - 1,4-diamino-2,3-dicyano-1,4-bis[2-aminophenylthio]butadiene for treatment of non-traumatic subarachnoid haemorrhage, Edvince AB;
   - 1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea for treatment of gastrointestinal stromal tumours, Worldwide Clinical Trials Limited;
   - C1-esterase-inhibitor human for treatment in solid organ transplantation, CSL Behring GmbH;
   - Concizumab for treatment of haemophilia B, Novo Nordisk A/S;
   - Diazoxide choline for treatment of Prader-Willi syndrome, Capnia (UK) Ltd;
   - N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide for treatment of peripheral T-cell lymphoma, Celleron Therapeutics Limited.

2. Opinions adopted at the first COMP discussion:
   - (1'R,6'R)-3-(benzylamine)-6-hydroxy-3'-methyl-4-pentyl-6'-(prop-1-en-2-yl)-[1,1'-bi(cyclohexane)]-2',3,6-triene-2,5-dione for treatment of systemic sclerosis, Quintiles Ireland Limited;
   - (R)-troloxamide quinone for treatment of amyotrophic lateral sclerosis, Edison Orphan Pharma BV;
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- 4-amino-1-[(1S,4R,5S)-2-fluoro-4,5-dihydroxy-3-(hydroxymethyl)cyclopent-2-en-1-yl]pyrimidin-2-one for treatment of pancreatic cancer, Quintiles Ireland Limited;
- Antisense oligonucleotide targeting exon 73 in the COL7A1 gene for treatment of epidermolysis bullosa, ProQR Therapeutics VII BV;
- Recombinant adeno-associated viral vector serotype 9 containing human iduronate-2-sulfatase gene for treatment of mucopolysaccharidosis type II (Hunter’s syndrome), REGENXBIO EU Limited;
- Tamoxifen citrate for treatment of Duchenne muscular dystrophy, Duchenne UK;
- Tiratricol for treatment of Allan-Herndon-Dudley syndrome, Medical Need Europe AB.

3. Opinion(s) 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(s)**

1. Opinion(s) adopted following the sponsor’s response to the COMP list of questions:

The COMP adopted 1 negative opinion recommending the refusal of the orphan designation for the following product:

- Melatonin for treatment of subarachnoid haemorrhage, Therapicon Srl. The opinion was adopted by written procedure after the 03-05 October meeting.

2. Opinion(s) following appeal procedures:

None

**Lists of questions**

The COMP adopted 6 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 4 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.

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


1. Opinion adopted at time of CHMP opinion:

   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 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the Community Register of orphan medicinal products for human use:

   - Zejula (niraparib), (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt, for treatment of ovarian cancer, Tesaro UK Limited (EU/3/10/760)

2. Opinion(s) 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.

**Other matters**

The main topics addressed during the meeting related to:

- Protocol assistance advice

**Upcoming meetings**

- The 194th meeting of the COMP will be held on 30-31 October 2017.

**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 details of our press officer**

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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>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>2017</td>
<td>197</td>
<td>198</td>
<td>118 (60%)</td>
<td>79 (40%)</td>
<td>1 (1%)</td>
<td>106</td>
<td>12</td>
<td>13</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>
<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>
</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>
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<th>Orphan designations included in authorised therapeutic indication</th>
</tr>
</thead>
<tbody>
<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>2912</td>
<td>2737</td>
<td>1945 (71%)</td>
<td>768 (28%)</td>
<td>24 (1%)</td>
<td>1911</td>
<td>140</td>
<td>155</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

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

The list includes designation decisions that were revised following the amendment of an existing designated condition (identified by * when applicable)

<table>
<thead>
<tr>
<th>Active substance</th>
<th>Orphan indication</th>
<th>Sponsor</th>
<th>COMP opinion date</th>
<th>EC designation date</th>
</tr>
</thead>
<tbody>
<tr>
<td>None</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
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>Lenvatinib</td>
<td>Treatment of hepatocellular carcinoma</td>
<td>Eisai Ltd</td>
<td>EU/3/15/1460</td>
</tr>
</tbody>
</table>