Orphan Medicines Figures

2000-2017

Orphan Medicines - Product Development Scientific Support
## Applications for orphan medicinal product designation

<table>
<thead>
<tr>
<th></th>
<th>2000</th>
<th>2006</th>
<th>2011</th>
<th>2016</th>
<th>2017</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Applications submitted</strong></td>
<td>548</td>
<td>686</td>
<td>1151</td>
<td>329</td>
<td>260</td>
<td>2974</td>
</tr>
<tr>
<td><strong>Positive COMP Opinions</strong></td>
<td>348</td>
<td>500</td>
<td>759</td>
<td>220</td>
<td>144</td>
<td>1971</td>
</tr>
<tr>
<td><strong>Negative COMP Opinions</strong></td>
<td>8</td>
<td>6</td>
<td>7</td>
<td>2</td>
<td>2</td>
<td>25</td>
</tr>
<tr>
<td><strong>EC Designations</strong></td>
<td>343</td>
<td>485</td>
<td>768</td>
<td>209</td>
<td>147</td>
<td>1952</td>
</tr>
<tr>
<td><strong>Withdrawals during assessment</strong></td>
<td>150</td>
<td>144</td>
<td>313</td>
<td>77</td>
<td>100</td>
<td>784</td>
</tr>
</tbody>
</table>
Applications for orphan medicinal product designation


- submitted
- positive opinions
- negative opinions
- withdrawals during assessment
- EC Designations
260 applications submitted for orphan designation in 2017

- SME: 112, 43%
- Non-SME: 148, 57%
New orphan designated conditions

TOTAL DESIGNATIONS (1952)  NEW CONDITIONS (508) - 26%
Orphans designations based on significant benefit

Designations
Percentage based on SB
Distribution of opinions on orphan designation by therapeutic area
Period 2000 – 2017 / Total opinions 1971

- A-alimentary tract & metabolism: 13%
- B - blood & blood forming organs: 8%
- C - cardiovascular system: 3%
- D - dermatology: 1%
- G - genito urinary tract: 2%
- H - systemic hormonal preparations: 1%
- J & P - antiinfectives & antiparasitic: 5%
- L - antineoplastic agents: 2%
- L - immunomodulating agents: 5%
- M & N - musculoskeletal & nervous system: 5%
- R - respiratory system: 7%
- S - sensory organs: 2%
- V - various: 12%
- Total opinions: 1971
Distribution of orphan designations adult/paediatric use
Period 2000 – 2017/ Total designations 1952

- Adult: 32%
- Paediatric: 13%
- Both: 55%
Distribution of orphan designations
adult/paediatric use

Total designations 1952
Prevalence for designated orphan conditions
Period 2000 – 2017 / Total designations 1952

- More than 3 in 10,000: 11%
- Between 1 and 3 in 10,000: 49%
- Less than 1 in 10,000: 40%
Authorised orphan medicinal products

Orphan marketing authorisations
Orphan designations covered by marketing authorisations
Authorisations by type of product

99 authorisations including 5 withdrawals from the register of medicinal products human use
Authorisations by type of product including extensions of indications

162 products including withdrawals from register of orphan medicinal products, register medicinal products human use and expired orphan status
Status of Orphan MAA

- **142** initial marketing authorisations granted and **20** extensions of indication
- Adopted opinions by CHMP awaiting EC decision: **5**
- On-going applications in review process: **28**
- Extensions of indication in review process: **8**
- Expired – end of market exclusivity: **36** initial MAA & **1** extension of indication

Withdrawals
- **65** during evaluation, including 2 extensions of indication
- **3** from register human medicinal products
- **2** from register human and orphan medicinal products
- **13** from register orphan medicinal products after authorisation, including 6 extensions
- **27** from register orphan medicinal products before authorisation, including 2 extensions

Negative outcomes: **14** refusals
142 initial orphan marketing authorisations and 20 extension of indication granted to date

Chart includes:
- 13 authorised extensions of indication
- 13 withdrawals from the register of orphan medicinal products (including 6 ext. of indication)
- 5 withdrawals from register medicinal products human use
- 36 removals of initial MAA from register after expire of the market exclusivity period & 1 ext of indication

Active orphan MA: 94
Active extension of indication: 13

Number of conditions: 111

A Alimentary tract and metabolism
B Haematology
C Cardiovascular system
H Systemic hormonal
J Anti-infectives for systemic use
L Immunology
L Antineoplastic
M Musculo-skeletal system
N Nervous system
R Respiratory system
S Sensory organs
V Various
Prevalence for orphan marketing authorisations period 2000-2017

Prevalence per 10,000 all orphan products
Including extensions of indication, withdrawals and products which market exclusivity has expired

- < 1: 49%
- 1-2: 24%
- 2-3: 13%
- 3-5: 14%
Thank you for your attention

Further information

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