The landscape of Paediatric and Orphan medicines

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Rare diseases and Children in Europe

• Between **5 000-8 000 distinct rare diseases** exist – half of which begin in childhood

• Between **27-36 million people** living in the EU suffer from a debilitating rare disease – 50-75% are children

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• 75 million of EU population in paediatric age group (≤16) (20% of Community population)

• Ca. 4 million live births/year*
Orphan Designation criteria in EU

• Prevalence of the condition not more than **5 in 10 000**

• Medicine must be intended for the treatment, prevention or diagnosis of a disease that is **life-threatening** or **chronically debilitating**

• No satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of **significant benefit** to those affected by the condition
EU Orphan Regulation and children...

- Conditions rare in adults and children
  - Storage diseases, etc.
- Conditions rare in children, not in adults
  - Hypertension, AIDS/HIV
- Conditions rare in adults, not in children
  - RDS, PDA
- Common in adults and children – but children still “orphans”
  - Migraine
ORPHAN MEDICINAL PRODUCT DESIGNATION

Orphan designations adult/paediatric

<table>
<thead>
<tr>
<th>Year</th>
<th>Medical conditions affecting adults only (%)</th>
<th>Medical conditions affecting both children and adults (%)</th>
<th>Medical conditions affecting children only (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>2017</td>
<td>8%</td>
<td>78%</td>
<td>14%</td>
</tr>
<tr>
<td>2018</td>
<td>7%</td>
<td>78%</td>
<td>15%</td>
</tr>
<tr>
<td>2019</td>
<td>5%</td>
<td>76%</td>
<td>19%</td>
</tr>
<tr>
<td>2020</td>
<td>9%</td>
<td>64%</td>
<td>27%</td>
</tr>
<tr>
<td>2021</td>
<td>5%</td>
<td>71%</td>
<td>24%</td>
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</tbody>
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Children, “therapeutic orphans” – also in orphan diseases

- 76% of all designations and 78% of authorised products are for conditions that affect (also) children
- 55 (39%) authorised orphan medicines have been approved for use in children
- 44 products have been authorised for forms of cancer that affect (also) children; 13 of these are approved for use in children.

Data from 2000-2017

Study to support the evaluation of the EU Orphan Regulation; Final report July 2019
Will the relevant OMPs be available for children?

- In a suitable formulation
- Labelled for use in children
- *In all relevant age groups?*
- If yes, *when*?
Problems common for development of Paediatric medicines and Orphan medicines

• Small size clinical trials
  • Methodological problems
  • Regulatory problems