

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..

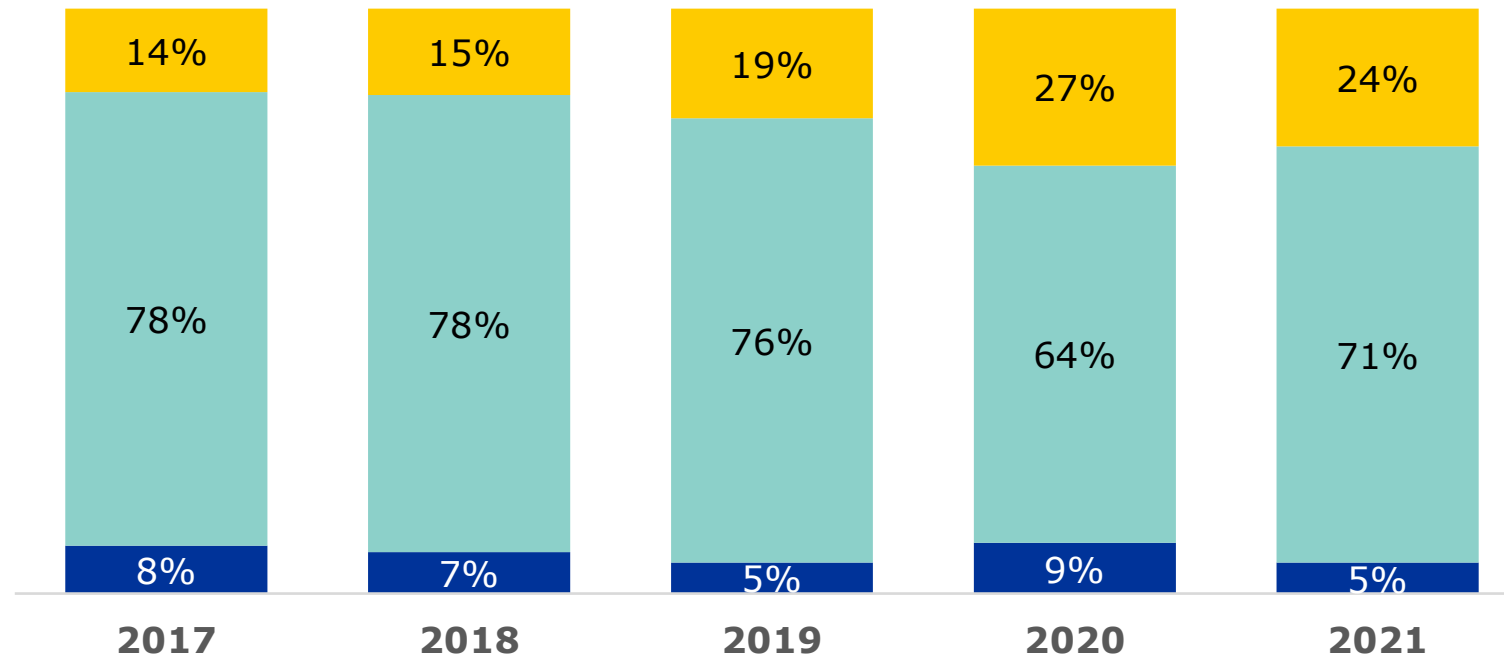
- Conditions rare in adults and children
 - Storage diseases, etc.
 - Conditions rare in children, not in adults
 - Hypertension, AIDS/HIV
 - Conditions rare in adults, not in children
 - RDS, PDA
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- Common in adults and children – but children still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





ORPHAN MEDICINAL PRODUCT DESIGNATION

Orphan designations adult/paediatric



- Medical conditions affecting adults only
- Medical conditions affecting both children and adults
- Medical conditions affecting children only

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

