

Paediatric medicines from a (Eunet)HTA perspective

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Health Technology Assessment (HTA) in Europe

- systematic evaluation and assessment of the properties and effects of a health technology to enable evidence-based decision making to ensure cost-effectiveness („value for money“) in medicine
- Diverse HTA agencies → Impact of the assessment on the decision making differs

EUnetHTA:

- network of government appointed organisations and a large number of relevant regional agencies and non-for-profit organisations that produce or contribute to HTA in Europe



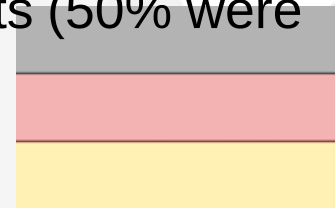
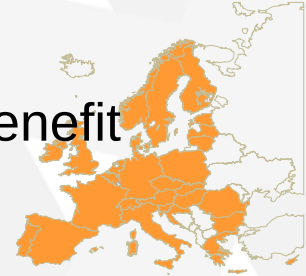
EUnetHTA today

- EUnetHTA collaboration has grown to 80+ organisations from 30 countries
- Work packages address better harmonization of scientific advice and benefit assessment
- Collaboration with EMA: Work plan activities include “Address the specific needs for paediatric medicines”

Work Package 1 Network Coordination - Dutch Health Care Institute 					
Work Package 2 Dissemination	Work Package 3 Evaluation	Work Package 4 Joint Production	Work Package 5 Evidence Generation	Work Package 6 Quality	Work Package 7 Implementation
Lead: AETS-ISCIH 	Lead: TLV 	Lead: NIPHNO Co-lead: LBI ZIN   	Lead: HAS Co-lead: GBA  	Lead: IQWiG Co-lead: KCE  	Lead: NICE Co-lead: Agenas  

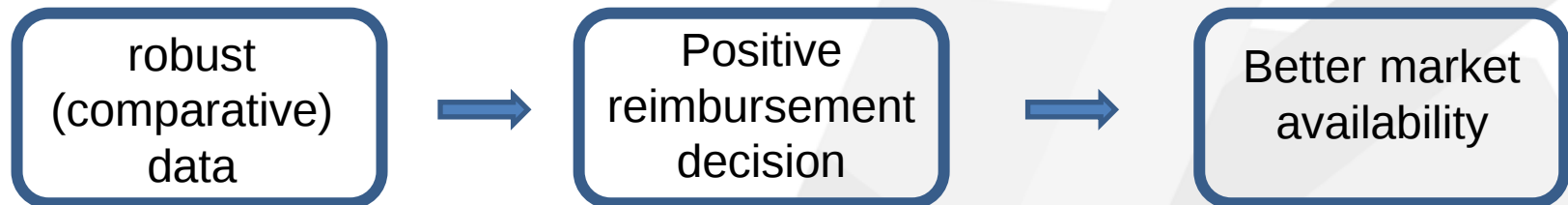
Assessment of paediatric medicines:

- Very limited experience
- In general no specific criteria or incentives for the benefit assessment of paediatric medicines
- Only 10% of new medicinal products for benefit assessment were also or only approved for children and /or adolescents (50% were orphan drugs)
- Additional benefit granted only in less than 25%
- PUMA medicine falls under scope of benefit assessment



Challenges for benefit assessment

1. Currently pediatric medicines are predominantly assessed equally to adult medicines by HTA bodies
 2. Benefit assessment is per se a comparative approach
- Collection of comparative data on patient-relevant endpoints (e.g. mortality, disease symptoms, quality of life, safety) is also important for paediatric studies



Suggestions for the future

In general:

- Define common minimum evidence needs for benefit assessment by European HTA bodies
- Strengthen EMA-EUnetHTA collaboration and understanding of the respective evidence needs in order to allow developers to generate evidence able to address both regulatory and HTA information needs

Specifically:

- Increase awareness of the specificities and possible limitations of evidence generation in pediatric medicines among HTA bodies
- Define requirements for and increase the use of evidence transfer (extrapolation) for benefit assessment of paediatric medicines
 - Example Germany: new law addresses evidence transfer for PUMA

Thank you

Any Questions?