

EMA-EC multi-stakeholder workshop - Paediatric Regulation

Martina Pitzer – Declaration of Interests

- No direct or indirect interests in the pharmaceutical industry
- Board member of the Drug Commission of the German Medical Association
- Member of the Commission of Drugs for Children and Adolescents at the German Federal Institute for Drugs and Medical Devices



Medicines in the therapeutic area of mental and behavioural disorders

- High burden of disease
- Frequent off-label and off-patent use
- Scarcity of innovations
- Brain maturation extends throughout adolescence well into early adulthood
 - ⇒ Safety and efficacy cannot be readily extrapolated from adults



Suggested criteria to prioritise conditions of unmet medical needs

- **Burden of disease e.g. based on Disability-adjusted-life-years (DALY)**
- **Existence / shortage of non-pharmacological treatment options e.g. as stated in evidence-based practice guidelines**



Methodological considerations

- **widespread public education on the need for paediatric trials in order to reduce recruitment difficulties**
- **compiling data already available to build registries / formularies for off-patent medicines**
- **Starting a MD-PhD programme with a focus on paediatric pharmacology to generate independently thinking and working physician scientists**
 - ⇒ **identify existing gaps of knowledge of old, off-patent medicines**
 - ⇒ **developing own clinical research**

