



## Session I

# Overview and Summary of Identified Gaps



*Facilitating collaborative research in drug  
development and health policy*

# Overview and Summary of gaps

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- Natural history in pediatric-adolescents
  - 0-2 yrs
  - 2-8 yrs
  - 8-17 yrs
- Impact of puberty
- Immune system



## Gaps-2

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- Clinical trial design
  - Appropriate endpoints
    - Non-invasive
    - Bridging from adults?
  - Follow up
    - Post trial?



## Gaps-3

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- Testing drugs in children
  - Safety
    - Short term/long term
  - PK
    - Infants----youth
    - Different for different drugs
  - PK/PD