



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Objectives Expert Meeting List of Questions Preliminary Answers

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- Sophie Olivier, MD, Paediatric Team





# Q1: Can efficacy be extrapolated from adults data?

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- Yes: 3/9
- No: 3/9
- Yes, but...justifying separate studies: 3/9



## Q2: Can same regimen be proposed to all age groups (ie, before or after puberty)?

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- Yes: 3/9
- Before puberty, TBC or TBD: 5/9
- Shorter therapy before puberty: 1/9

**Related question:** should treatment be avoided in a certain age group?



## Q3: Does the risk of growth retardation justify comparative trials vs. SOC?

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- Yes: 4/9
- No: 4/9
- No response: 1/9
  
- **Related question:** can an additive effect on growth retardation with DAA be expected? Of note, no preclinical study has been designed to assess the effect on growth of tri-therapy.



## Q4: Should response guided therapy be validated in children before recommended?

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- Yes: 6/9
  - Probably not: 1/9
  - No: 1/9
  - Controversial: 1/9
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- **Related question:** is the risk of relapse, as seen after 24-w therapy in the boceprevir adult studies acceptable in the paediatric population?



## Q5: Should other sub-groups of the paediatric population be investigated?

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- No: 1/9
- Yes, but deferred: 8/9
  - Such as genotypes 2 to 4 non-responders, co-infected children



## Q6: Study design for investigation of new DAA in combination with SOC in children?

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- Single-arm safety study with efficacy as secondary endpoints (historical control)
  - 2/9
- Superiority trial vs. SOC, 48-w
  - 3/9
- Comparison 48-w tri-therapy vs. response guided therapy
  - 4/9
- Other design?
  - Single-arm RGT tri-therapy: 1/9



## Additional questions proposed by FDA

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- Q7: Should dose finding study in children be requested or strategy to target adult exposure dose is acceptable?
- Q8: Does panel foresee a time when paediatric testing of such drugs may become unfeasible because there are not enough children requiring treatment to study all drugs currently in development?