



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

2018-01-12

## Submission of comments on 'Reflection paper on the use of extrapolation in the development of medicines for paediatrics' (EMA/199678/2016)

### Comments from:

Name of organisation or individual

European Federation of Statisticians in the Pharmaceutical Industry (EFSPI)

*Please note that these comments and the identity of the sender will be published unless a specific justified objection is received.*

*When completed, this form should be sent to the European Medicines Agency electronically, in Word format (not PDF).*



## 1. General comments

Stakeholder number	General comment (if any)	Outcome (if applicable)
<i>(To be completed by the Agency)</i>		<i>(To be completed by the Agency)</i>

## 2. Specific comments on text

Line number(s) of the relevant text <i>(e.g. Lines 20-23)</i>	Stakeholder number <i>(To be completed by the Agency)</i>	Comment and rationale; proposed changes <i>(If changes to the wording are suggested, they should be highlighted using 'track changes')</i>	Outcome <i>(To be completed by the Agency)</i>
Line 200		<p>Comment: This seems to be an incomplete sentence.</p> <p>Proposed change (if any):</p>	
Lines 392-396		<p>Comment: It is unclear what is meant by “adequately powered” here? Adequately powered so that the study shows a statistically significant result or adequately powered so that the study meets a pre-defined success criteria, eg [Prob (treatment difference)<math>&gt;x</math>]<math>&gt;y\%</math>? Doesn't the latter make more sense when there are feasibility constraints around sample size? And also what is an adequate power (90%, 80%, ...)? Of course the success criteria should not be artificial in order to line up with a certain sample size. However, the success criteria that could be met with a feasible sample size should be discussed with regulators to reach agreement on (i) the definition of the success criteria and (ii) the probability of meeting this success criteria.</p> <p>Proposed change (if any):</p>	
Line 401		<p>Comment: When referring to “information” (1<sup>st</sup> word) does this mean treatment difference and if so should this be more explicit?</p> <p>Proposed change (if any):</p>	

Please add more rows if needed.