Commission Guideline on the Format and Content of Applications for Paediatric Investigation Plans (Article 10 of Regulation 1901/2006)

Concept Paper Submitted for Public Consultation

Consultation item No 1: Do you have any comments on the format and content of applications for agreement on or modification of a paediatric investigation plan and requests for waivers or deferrals?

- The detail in the guideline is currently limited. For example, additional information is needed to help understand the requirements of Part A: Administrative and product information, including the section "2.2.4 Details of the medicinal product" which requires a large amount of information to be provided.
- The naming of the subsections of section 2.2 of the guideline does not reflect the actual sections of Part A: Administrative and product information and may lead to confusion. It would be useful to reference the relevant sections of the form.
- Some of the terms stated in the guideline and the application documentation are inconsistent (e.g. EU vs. EEA).
- Additional information should be included on the use of the new concept of extrapolation. Although it is stated that extrapolation between adults and children is possible, there is no reference to further guidance on how, when or where extrapolation could be used.
- Additional information on the content needed in the new 'Application Summary' is needed. It is unclear if the paediatric strategy (waiver, deferral), study design, patient population etc. should be included.

Consultation item No 2: Do you have any comments on the operation of the compliance check and/or the compliance statement?

- Cases when a compliance check is <u>not</u> needed, including when studies have been deferred and not initiated prior to submission of the Marketing Authorisation Application, should be clearly stated in the guideline.

Consultation item No 3: Do you have any comments on the assessment criteria for significant studies?

- The addition of previously conducted studies in adults or older children, which may be deemed significant when the data is extrapolated, should be added to the types of studies considered significant.

Consultation item No 4: Do you have any comments on the key elements of a paediatric investigation plan? Is it appropriate to list key elements in this guideline or should key elements only be specified in the individual decision of the Agency agreeing a specific paediatric investigation plan?

- The key elements of the application seem reasonable although it is unclear the level of detail needed for each element.
- Provision of a set list of the key elements in the guideline is favourable to the applicant as this can help in the planning and creation of the application and future modifications. This will also assist the applicant in preparing the appropriate amount of detail for each section of the application.

Consultation item No 5: Please feel free to raise any other issues or make any comments which have not been addressed in the consultation items above.

General comment:

- The amount of detail required in the application is extensive, flexibility on content is limited and there can be a high amount of repetition and duplication within the application. It can also be difficult to complete certain sections of the application so early in development with limited knowledge of the product. Therefore the expansion of some sections of the guidance is needed to provide further information on the level of detail required to be submitted in the application. This will reduce the burden to the applicant by helping avoid duplication and repetition in the initial application and reduce the number and size of amendments needed once the application is approved.