

## **Final Business Plan**

### **S11: Nonclinical Safety Testing in Support of Development of Pediatric Medicines dated 22 September 2014**

*Endorsed by the ICH Steering Committee on 10 November 2014*

#### **Introduction**

A new guideline on juvenile animal testing for pediatric indications is proposed to be published through the ICH process. This guideline is needed to recommend standards for the conditions under which juvenile animal testing is considered informative and necessary for the safety of pediatric clinical trial subjects and to provide guidance on the design and timing of the studies. This will result in streamlined drug development and higher scientific rigor while minimising the unnecessary use of animals.

#### **1. The issue and its costs**

- *What problem/issue is the proposal expected to tackle?*  
Three regional guidelines on juvenile animal testing exist that address, in varying degrees of scope and concurrence, the need for studies, study designs and timing, and the use of juvenile data in drug development and labelling. The lack of a harmonised approach can lead to default conduct of a study or the repeating of a study, which results in the unwarranted use of animals in direct contradiction to the 3R principles and existing recommendations in ICH M3(R2), as well as inconsistent quality and application of data to the safety of subjects in pediatric clinical trials.
- *What are the costs (social/health and financial) to our stakeholders associated with the current situation or associated with “non action”?*  
The conflicting regional recommendations on the need for and design of juvenile animal testing are leading to unnecessary use of animals and delaying key medicines from expedient pediatric use. The uncertainty around requirements is responsible for increased drug development costs overall and the generation of suboptimal preclinical data to guide the safe evaluation of drugs in pediatric populations.

#### **2. Planning**

- *What are the main deliverables?*  
A new harmonised guideline on juvenile animal testing for pediatric indications that will provide clarity in determining the need for juvenile animal testing and, when testing is warranted, the design and timing of the study.
- *What resources (financial and human) would be required?*  
Formation of an expert working group (two nonclinical experts nominated by EU, EFPIA, FDA, PhRMA, MHLW, JPMA, Health Canada and Swissmedic. One member can also be nominated by WHO Observer, as well as RHIs, DRAs/DoH) and active/dedicated participation by industry, regulatory, and *ad hoc* advisory members.

- *What are the timeframe and key milestones of the project?*

The request will be submitted to the ICH Steering Committee (SC) in September 2014 with the expectation of the EWG meeting face-to-face in the spring 2015. It is anticipated that a *Step 2b* Guideline will be completed by 4Q 2016 and that *Step 5* will be reached in 2018.

### **3. The impacts of the project**

- *What are the likely benefits (social, health and financial) to our key stakeholders of the fulfilment of the objective?*

The proposed guideline will streamline drug development, minimise the unnecessary use of animals, and lead to resource application for those cases where juvenile animal testing is necessary for the safety of the pediatric population. This may result in the savings of two or more animal studies (range-finding and Good Laboratory Practices) for a drug under development. The guideline will provide a harmonised approach on the need and design of preclinical juvenile studies to minimise conflicting opinions from regulatory bodies. The data from animal studies that are conducted will be of higher quality and more informative to the safety of pediatric clinical trial participants.

- *What are the regulatory implications of the proposed work – is the topic feasible (implementable) from a regulatory standpoint?*

The proposal is consistent with current laws and regulations of the ICH regions. Regulatory authorities responsible for reviewing pediatric plans and issuing opinions will need to agree globally on the recommendations for determining the need for juvenile animal studies and on the recommendations for the design and timing of the studies when they are warranted. This guideline will supersede regional guidelines. An overall effect on regulatory resources cannot be established at this time.

### **4. Post-hoc evaluation**

- *How and when will the results of the work be evaluated?*  
Not applicable.