EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 September 2013

Submission of comments on "Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another" (ENTR/6283/00 Rev 4; 12 June 2013) and Annex (Application Form for Orphan Medicinal Product Designation)

Comments from:

Name of organisation or individual
F.Hoffmann-La Roche Ltd

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).

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1. General comments

Stakeholder number *(To be completed by the Agency)*	General comment (if any)	Outcome (if applicable) *(To be completed by the Agency)*
	The overall title of the guidance should be changed to reflect the addition of guidance on changes to existing designations.	
	The proposal to include guidance on "Change of an existing designation" is a welcome addition, as this process has not been described previously. However, it would be helpful if this section could include a level of detail consistent with that in the section on transfer of designations from one sponsor to another, and to provide clarity on the process (for example, information on the steps taken by the EMA upon notification of a change to the designation, and the timelines for these steps [and whether an updated EC Decision would be issued and the Community Register updated] would be informative.	
	In the added section, "Change of an existing designation", it would be helpful if further examples of foreseen changes could be included in addition to the existing example. For instance, minor formulation changes may occur during development (e.g. a change from one salt to another), and it would therefore be helpful to clarify whether this process also allows such minor changes to be made in situations where there is no impact on the criteria that formed the basis for designation.	

Stakeholder number *(To be completed by the Agency)*	General comment (if any)	Outcome (if applicable) *(To be completed by the Agency)*
	Application form and guidance: Clarity on the process for changing from a chemical name used for designation to a designated INN would be welcomed. The Note for Guidance on the format and content of the annual report on the state of development of an orphan medicinal product states that the Sponsor can include a recommended INN in the application form at the time of the annual report (when known), and that this should be indicated in the specific field. However, Section II of the form (Designation Application Particulars) and "Name of the Active Substance" appear to be the only sections where an INN can be included. As it is understood that neither the orphan indication nor the substance name can be changed at the time of the annual report, clarity on the process for replacing a designated chemical name with an INN would be welcome (along with how and when this change would be reflected in the Community Register and on the EMA website, for transparency).	

2. Specific comments on text

Line number(s) of the relevant text *(e.g. Lines 20-23)*	Stakeholder number *(To be completed by the Agency)*	Comment and rationale; proposed changes *(If changes to the wording are suggested, they should be highlighted using 'track changes')*	Outcome *(To be completed by the Agency)*
		Comment: Proposed change (if any):	
		Comment: Proposed change (if any):	
		Comment: Proposed change (if any):	

Please add more rows if needed.