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Revision of the 2003 Communication on orphan medicinal products

75th Pharmaceutical Committee
21 October 2015

Health and Food Safety

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Reflections from the Pharma Committee

- **Heterogeneity of orphan medicinal products**
- **Focus on real "breakthrough"**
- **Clarify the criterion of significant benefit**
  - Based on clinical experience only, major benefit, comparative data
  - Stricter for new pharmaceutical forms
  - Reconsider the inadequate supply
- **Reduce the market exclusivity to 6 years**
- **Clarify the definition of similarity and derogations (impact on market exclusivity)**

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Review of the 2003 Communication from the Commission (2003/C 178/02)

- Communication to be replaced by a Notice from the Commission
- Focus on points in relation to Articles 3 (criteria for designation), 5 (procedure for designation and removal from the register), and 7 (Community marketing authorisation) of the Regulation
- Removal of the interpretation of Article 8 on market exclusivity (already provided in the Commission guidelines C(2008)4077 and 2008/C 242/07)

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Review of the 2003 Communication from the Commission (2003/C 178/02)

1. Facilitating the **entry of innovative products** with a significant benefit over existing treatments and **avoid delaying** the entry of **generics**;

- the major contribution to patients care of the new pharmaceutical form should be justified in all cases with relevant data showing meaningful benefits for the patients;

- Introducing a **review of the orphan criteria** once an applicant modifies therapeutic indication based on Article 7(3) of the orphan Regulation

- Better controlling the **transfer** between companies

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2. **Clarifying** the definition of "**significant benefit**";

For example, "a clinically relevant advantage" may be considered based on :

- An improved efficacy or a better safety profile. The claim should be based on clinical experience;

For example, "a major contribution to patient care" may be considered based on:

– Ease of self-administration or important improvement in compliance

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Significant benefit should not be considered based on:

- A possible **increased supply** due to shortages of existing authorised products or due to a **national marketing authorisation** in one or a limited number of Member States;
- **Enhancement** of the pharmaceutical **quality** of a product in compliance with the relevant Committee on Medicinal Products for Human Use (CHMP) guidelines which is a part of the obligation of every marketing authorisation holder;
- An **alternative mechanism of action** per se, to be sufficient for the assumption of significant benefit it **needs to be translated into a clinically relevant advantage** or a major contribution to patient care.

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3. Encouraging the development of orphan medicinal products for **communicable diseases (e.g. Ebola);**

4. **Facilitating** for the Committee of orphan medicinal products and industry the procedure for reassessment of the orphan criteria when **two orphan** medicinal products are **running in parallel**;

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5. Introducing the **reassessment of the orphan criteria for a new subset** of the condition when a sponsor extends the use of its product after marketing authorisation;

6. **Avoiding the transfer** of orphan designation to ascertain that a sponsor receives only one orphan designation per medicinal product and per condition.

7. **Clarifying** the expectations for the **conditional autorisation**:

- seek protocol assistance
- ensure consistency between the confirmation of the 'unmet medical need' and the 'significant benefit' of the purpose of the orphan designation.
- possible reassessment of the criteria

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Next steps

- Public consultation in October – November 2015
- Review of the comments from EMA, the Member States and the Commission services
- Publication in early 2016

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Thank you for your attention

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