Forthcoming activities on orphan medicinal products

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European Commission
Directorate General for Health and Food Safety
SANCO D5, Medicinal products - Authorisations, EMA

Disclaimer : This presentation only reflects the views of its author and does not necessarily reflect the opinion of the Commission

Health and Food Safety

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Background

- Reg. 141/2000 of the Council and the European Parlement tasked the Commission to adopt the provisions for the interpretation of :
  - **Criteria for designation - Article 3(2)**
  - **Format of the application form and transfer of designation - Article 5 (11)**
  - **Market exclusivity - Article 8(5)**

  To be done **in consultation** with the **MS**, EMA and interested parties

- Other obligations for the Commission: publication of an **inventory** of all incentives by the EU and MS - Article 9 (3); **nomination of patients** representatives - Art 4 (3)

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Current instruments

- Commission Regulation (EC) No 847/2000: criteria and definition of 'similar medicinal products' and 'clinical superiority'
- Guideline on the format and content of application (ENTR/6283/00) *Revised in 2014*
- Communication from the Commission (2003/C 178/02) clarifies the criteria for designation, procedure for designation, Community authorisation and market exclusivity
- Communication from the Commission (C2008)4077 on assessing similarity and applying derogations
- Commission Guideline (2008/C242/07) on the review of the period of market exclusivity to 6 years
- Inventory of incentives last version in 2005

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State of play

- **113 orphan medicinal medicines authorised by the European Commission**
- **1153 products in development designated as orphan medicinal products by the European Commission**
- **2 orphan medicinal products with a paediatric reward**

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Forthcoming revisions (1/3)

- Communication from the Commission (2003/C 178/02)
  - Facilitate entry of innovative products with a significant benefit over existing treatments
  - Reassess the requirements to support significant benefit
  - Adapt the text to technical progress
  - Reassessment of critera at time of MA
- Public consultation second half 2015
- Adoption 1st quarter 2016

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Forthcoming revisions (2/3)

- Assessing similarity and applying derogations
  - Clarify the definition of 'similar products'
  - Ensure the entry of new pharmaceutical forms or better products

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Forthcoming revisions (3/3)

- Inventory of all incentives by the EU and Member States to support research, development and availability of orphans
  - Last update in 2005
- Enquiry to be sent to the MS
- Publication of the updated inventory planned at the end of 2015

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What is your experience ? your views ?

as regards:

- The criteria for orphan designation e.g. criterion of significant benefit
- Procedure for designation, re-evaluation and removal of the orphan designation from the Commission registry
- Orphan Community marketing authorisation
- Market exclusivity and similarity