EUROPEAN COMMISSION

Directorate-General for Health & Consumers

A Regulation addressing the needs for drug development in rare diseases

10 Years of the Orphan Regulation in Europe Conference, EMA, London

3-4 May 2010

Martin Terberger
Mirjam Söderholm

Pharmaceuticals Unit
European Commission

Regulation (EC) No 141/2000 on orphan medicinal products - the objective:

- Provide same quality of treatment for persons suffering from rare diseases as other patients
- Provide incentives to research and development and placing on the market of medicinal products for rare diseases (orphan medicinal products)
- Provide a Community procedure

Incentives:

- Fee waivers (partial or total)
- Protocol assistance
- 10 years' market exclusivity
- Other incentives (Community and Member States)

Procedure:

- Specific criteria for designation
- Central role of COMP in two-stage procedure (designation / review at time of marketing autorisation)
- Commission adopts legally binding decisions

Facts and figures:

- **April 2000 – April 2005***
  458 applications / 268 designations by Commission / 22 marketing authorisations

- **April 2000 – April 2010****
  1113 applications / 760 positive and 16 negative COMP opinions / 269 withdrawals / 724 designations by Commission /
  60*** marketing authorisations

*Source: Commission Staff Working Document 20.6.2006
** Source: EMA; *** 2 withdrawn from orphan register

2000-2005 designated therapeutic areas*

oncology 36 %, metabolism 11%, immunology 11%, cardiovascular and respiratory disorders 10%, musculoskeletal and nervous system 8%, antiinfectious 4%, others 20 %

2000-2010 COMP opinions on designation**

oncology 46%, metabolism 10%, immunology 10 %, cardiovascular and respiratory disorders 9% musculosskeletal and nervous system 12%, antiinfectious 3%, others 10%

*Source: Commission Staff Working Document 20.6.2006
** Source: EMA

60 authorised* orphan medicines per therapeutic area

- Oncology/immunology: 38%
- Metabolism: 22%
- Haematology: 15%
- Cardiovascular: 12%
- Musculoskeletal and nervous system: 10%
- Others: 3%

* Source EMA (2 products withdrawn from orphan register)

Continuous development of the regulatory framework:

- Commission Regulation (847/2000) on implementation of designation criteria, « similar medicinal product », « clinical superiority »
- Commission Communication (2003/C 178/02) on implementation of designation and market exclusivity
- Commission Communication (C(2008) 4051 final) on Art. 8(2) of Regulation 141/2000 (review of market exclusivity period)
- Commission Communication (C(2008) 4077 final) on Art. 8(1) and (3) of Regulation 141/2000 (similarity of products versus authorised orphan products with market exclusivity; derogations from market exclusivity)
- Commission guideline on the format and content of applications and the transfer of designations (ENTR/6283/00 Rev 3; and guidance documents from COMP and EMA
- Inventory of incentives (Community and MS) (2005); and 2006 report on experiences acquired (based on Art. 10 of Regulation 141/2000

EUROPEAN COMMISSION

Directorate-General for Health & Consumers

Continuous EU policy development on rare diseases

- Rare diseases as one of the priorities in the EU Health Programme 2008-2013
- Commission Communication on rare diseases (11.11.2008) and Council Recommendation (3.7.2009)
  - National plans and strategies
  - Adequate definition, codification and inventorying of rare diseases
  - Encouraging research on rare diseases
  - Centres of expertise and European reference networks
- EU Committee of Experts on Rare Diseases (set up by Commission Decision 30.11.2009)

EUROPEAN COMMISSION

Directorate-General for Health & Consumers

Continuous policy development towards EU cooperation on Health Technology Assessment

Objectives: Avoid duplication of efforts/resources for industry/HTA bodies and national decision makers

Instruments:

- Joint Action Member States/Commission on HTA 2010-2012
- Draft Directive on cross-border health care
- FP7/Research (call 2009: 64Mio€ (22 projects) to research on optimising delivery of health care)
- Call for proposals on development of HTA capacities in Member States
- Call for tender on assessment of clinical added value for orphan medicines

Commission Communication on Safe, Innovative and Accessible Medicines (10.12.2008, COM (2008) 666 final)

- Announces way forward to address major challenges, including unmet medical needs.
- Rare diseases identified as one major unmet medical need.

Conclusions:

- Incentives for orphan product development has triggered research and development
- On several rare diseases new treatment options are now available
- Continuous commitment on EU level to develop existing policies further
- Positive evolution of policy on rare diseases and HTA cooperation

However,

- Products do not reach all markets and patients

  Reasons:
  - Pricing and reimbursement decisions
  - Delays
  - Differences between Member States

- For many rare diseases there is still no treatment under development

Thank you for your attention !