European Commission

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Orphan medicines: DG Enterprise and Industry perspective

In this talk

- Cooperation – a role for the EU
- Orphan regulation – impact so far
- Key role of market exclusivity
- Looking to the future

Cooperation

- Multiple patients
- Multiple diseases
- Multiple stakeholders
- Multiple member states
- Multiple languages

the way ahead can be confusing

Cooperation

- EU working with stakeholders has provided some solutions

EU solutions

- Public Health Programs – communication, networking, databases, patient groups
- Framework Programs – funding of research on rare diseases
- EU Orphan Regulation – incentives for developing orphan medicines

\+ *the vital role of Member State actions*

EU Orphan regulation

- In force since 2000:
  - If medicine designated as ‘orphan’ – 10-years market exclusivity from ‘similar’ medicines
  - Protocol assistance
  - Fee reductions
  - Access to the centralised procedure

EU Orphan Regulation

- > 250 designations
- 20 authorised medicines
- 24 indications

The successes….so far

New authorisations

- **2 x Fabrazyme & Replagal for Fabry disease**
- **Glivec for chronic myeloid leukaemia**
- **2 x Tracleer & Ventavis for pulmonary hypertension**
- **Somavert for acromegaly**
- **Zavesca for Gaucher disease**
- **Carbaglu for hyperammonaemia**
- **Aldurazyme for Mucopolysaccharidosis**
- **Busilvex (iv) for haematopoietic progenitor cell transplantation**
- **Onsenal for Familial Adenomatous Polyposis**
- **Photobarr for Barrett's Oesophagus**
- **Litak for Hairy Cell Leukaemia**
- **Lysodren for adrenal cortical carcinoma**
- **Pedea for patent ductus arteriosus Wilzin for Wilson's disease**
- **Trisenox for acute promyelocytic leukaemia**
- **Xagrid for Thrombocythaemia**
- **Orfadin for Hereditary tyrosinemia type 1**
- **Prialt for chronic pain**

Extensions of indication

- **Glivec for GIST**
- **Glivec for first line use in CML**
- **Glivec for paediatric use in CML**

The impact on industry

SMEs*:

- 15% of Marketing Authorisations
- 20% of Scientific Advice requests
- 40% of designations

Better data are required

**EMEA figures*

10-years market exclusivity is key

But derogations e.g. article 8(2) of the regulation states:

- "This period may however be reduced to six years if, at the end of the 5th year, it is established……that the product is sufficiently profitable not to justify maintenance of market exclusivity. …. "
- This has caused concern

Commission response

Developing a guideline on sufficient profitability:

- Orphan pricing study
- Working Group of the transparency committee
- Consultation

Orphan pricing study - findings

- Variability of Manufacturer's Price Before Tax is low (mean 122% of minimum price)

- Public Price including Taxes is the main explanation for the variability in prices (ratio 1.7) due to: Tax, Distribution costs, Profit for retail pharmacies, Hospital pharmacy profit

Orphan pricing study - findings

Price comparators

c.f. non orphan indication of same medicine – not possible

c.f. US price – very similar – ratio 1.06 (8 products)

c.f. surgical treatments – 2-3 times cheaper

c.f. non-designated ‘orphan’ drugs in same indication - very variable

c.f. ‘expensive non-orphans’ – x10

Orphan pricing study - findings

Average cost per patient per year

6,000 – 300,000 Euros

Correlates with prevalence but not 'innovation' or 'medical benefit'

Orphan pricing study - findings

Industry revenues

Potential: 100 million – 1.5 billion Euros worldwide

c.f. world top ten blockbusters: 2 – 7 billion Euros worldwide

Orphan pricing study - findings

Calculating sufficient profitability:

- Equation / system for measuring profitability:
  - –will not be easy
  - –various possible methods:
    - revenues only,
    - complete profit calculation etc

Looking foreword….

- Regulation on SME
- Regulation on paediatrics
- Regulation on conditional marketing authorisations
- “2005 Report” of the orphan regulation
- (Public Health programmes)
- (7th Framework Program)

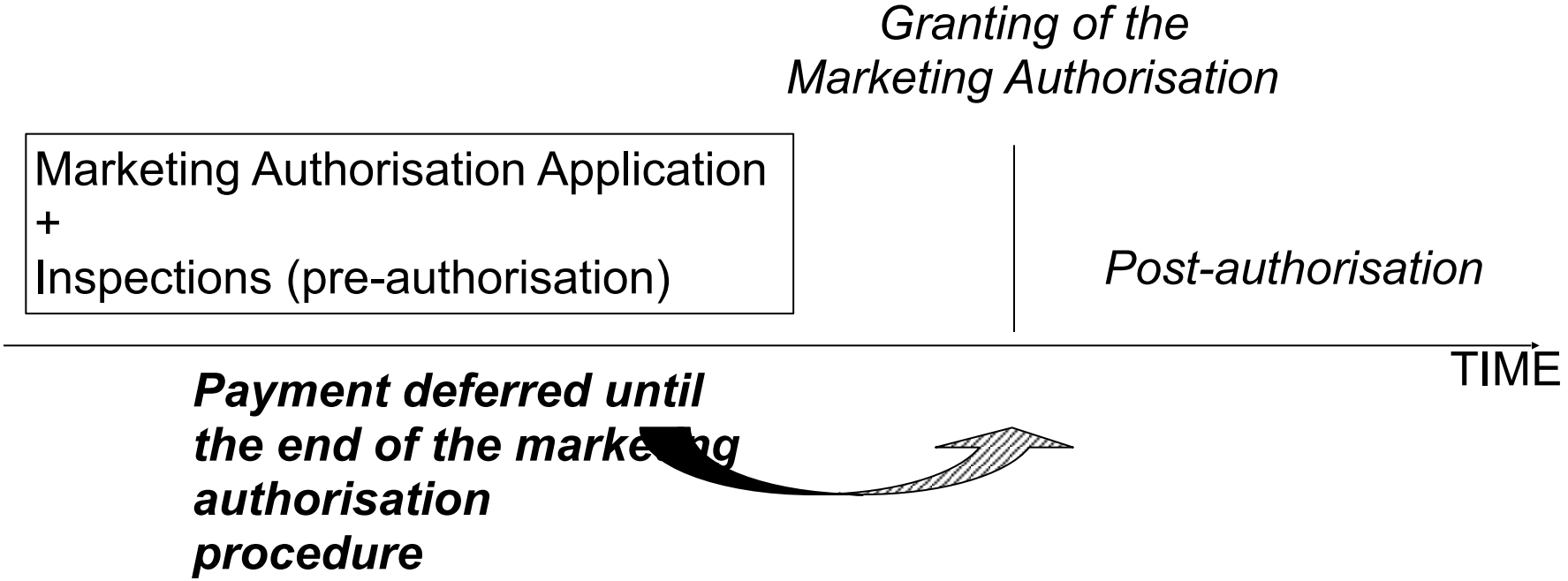
Regulation on SMEs

IF SME + Orphan status :

=>100% ‘reduction’ on :

- ➢Scientific advice/protocol assistance
- ➢Scientific services

+ fee deferrals:



Regulation on paediatrics

- Requirement for studies in children or waiver or deferral
- Reward for orphan medicines 2-years additional market exclusivity (10+2)

In force by end of 2006 ?

Regulation on conditional marketing authorisations

- lays down the conditions and procedure for granting a conditional MA
- orphan medicines within scope
- “public health interest”….”unmet medical need”
- MA with specific obligations and annual renewals

In force before November 2005

“2005 Report” of orphan regulation

Article 10 states:

- “Before 22 January 2006, the Commission shall publish a general report…experience acquired…public health benefits..”
- Plan:
  - Currently collecting information / data
  - Public consultation

Conclusions

- Much has been achieved in the past 10-years – orphan regulation central to this
- DG Enterprise and Industry is committed to:
  - the development of orphan medicines
  - the orphan regulation
  - supporting SMEs
  - promoting public health