



European Commission

Dr Peter Arlett

ICORD February 2005

# 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



<http://go.to/funpic>

# 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
- Pedeia 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 5<sup>th</sup> 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)
- (7<sup>th</sup> Framework Program)

# Regulation on SMEs

IF SME + Orphan status :

=> 100% 'reduction' on :

- Scientific advice/protocol assistance
- Scientific services

+ fee deferrals:

*Granting of the  
Marketing Authorisation*

Marketing Authorisation Application  
+  
Inspections (pre-authorisation)

*Post-authorisation*

TIME

***Payment deferred until  
the end of the marketing  
authorisation  
procedure***



# 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