



# Orphan Drugs – How to share Member States Assessments?

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# Presentation outline

- Current challenges
- The Commission Communication on rare diseases
- The Council recommendations on rare diseases
- The High Level Pharma Forum
- The working party on orphan drugs



## Current challenges

- Orphan Regulation 141/2000 has stimulated the research and development of orphan medicinal products, but:
- Equitable and timely access to approved orphan drugs for rare diseases remains an issue in the EU
- Not all Member States are able or willing to invest in those treatments



# Current challenges

- Member States, when they have access policy for OMP, encounter difficulties to define what a fair price could be for those products and how to manage budgets for treatment of rare diseases in a sustainable way.
- The number of orphan medicinal products with marketing authorisation will increase in the coming years. It doesn't mean that number of European patients having access to OMP will increase at the same rate if prices remain at their current level.



## Commission Communication 08

- The Commission Communication on *rare diseases: Europe's challenges*, sets out a Community strategy for action in three main areas:
  - (i) improving recognition and visibility of rare diseases;
  - (ii) supporting national plans for rare diseases in the Member States;
  - (iii) strengthening cooperation and coordination for rare diseases at European level.

- Among the proposed operational actions are:
  - development of national/regional centres of expertise
  - establishing EU reference network
  - setting up a working party to assess clinical added value of OD
- The Commission is currently setting up a Committee of experts that will support it in implementing the Communication's objectives



## Council recommendations 09

- On 8th June 2009, the Council adopted a Recommendation on an *action in the field of rare diseases*.
- The Recommendation ensures the support of Member States in elaborating the instruments foreseen in the Communication:
  - support and strengthen the adoption before 2013 of national plans and strategies
  - improve recognition and visibility
  - encourage research into rare diseases
  - forge links between centres of expertise and professionals
  - create of European reference networks in order to share knowledge and expertise
  - empowerment of patients organisations



# The High Level Pharmaceutical Forum

- The HLPF welcomed the development of a shared understanding that pricing and reimbursement policies need to balance
  - (1) timely and equitable access to pharmaceuticals for patients all in the EU,
  - (2) control of pharmaceutical expenditure for Member States, and
  - (3) reward for valuable innovation within a competitive and dynamic market that also encourages research & development.

## The WP on orphan drugs

### ■ Commission Communication 08:

- "There are specific bottlenecks in access to orphan drugs through the decision-making process for pricing and reimbursement linked to rarity; the way forward is to increase collaboration at the European level [...].
- **The Commission will set up a Working Party** to exchange knowledge between Member States and European authorities on the scientific assessment of the clinical added value of orphan medicines. These collaborations could lead to non-binding common clinical added value assessment reports with improved information that facilitate the national pricing and reimbursement decisions, **without pre-empting respective roles of the authorities**".

## The WP on orphan drugs (2)

- This Commission objective was confirmed by the Council Reco:  
“gathering the expertise on rare diseases at the European level” (Article 5, d) with a specific action on orphan drugs: **“Sharing Member State’s assessment reports on the therapeutic added value of orphan drugs at Community level, in order to minimise delays for access to orphan drugs for rare disease patients”**.



# The WP on orphan drugs (3): the Eurordis proposal

- The **assessment of the Therapeutic Added Value of Orphan Drugs (TAVOD)** should be performed where the expertise is gathered, and this is not at national level, but within the EMEA.
- A Working Party of the COMP within the European Agency would be in the best position to deliver an expert opinion on the scientific assessment of the TAV, which would support and speed-up decisions on pricing and reimbursement at national level.
- The proposed **Assessment structure for the Therapeutic Added Value of Orphan Drugs** - composed of COMP members, European Commission experts, national Competent Authorities, payers and patient representatives - could perform a **common scientific assessment of the TAV for each Orphan Drug and deliver an “opinion document”**.
- In this way Member States would pool their scarce scientific expertise to assess the TAV and would also recognise the value of this common assessment and opinion document. This system would avoid duplication of procedures at national level.



## The WP on orphan drugs (4): alternative solution

- Creation of a mechanism for the exchange of knowledge between Member States and European authorities on the scientific assessment of the clinical added value for orphan medicines.
- Financial instrument to be used: Call for tenders, within the 2010 public health programme

# Conclusions

- Political momentum
- Sustainable access

