



HAUTE AUTORITÉ DE SANTÉ

# **Towards Timely and Equitable Access to Orphan Medicines Across Europe**

## **The Outcomes of European Workshops**

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**Lisbon 2007**

European Conference on Rare Diseases

## *Introducing a new drug in the healthcare system in Europe :*

- 1st step: Marketing authorisation.
  - *Assessment* : CHMP / EMEA / Afssaps
  - *Decision* : European Commission / Afssaps

- 2nd step: Introduction into national healthcare systems
    - *Assessment*: « **Health Technology Assessment** »  
Some common principles, many national specificities
    - *Decision*
      - Pricing system*
      - Financing*
      - Implementation*
- Country specific**

- ✓ **Assessment**
- ✓ **Appraisal**
- ✓ **Listing**
- ✓ **Pricing**
- ✓ **Financing**
- ✓ **Appropriate  
Healthcare  
Organisation**



**Most of them are and will remain  
at a national or even regional  
level...**



- ✓ **European Platform for Patients' Organisations, Science and Industry: working together to advance health-care **policies** for the prevention and treatment of serious diseases.**
- ✓ **Founded in 1994 on the initiative of patients' organisations, for the exchange of information and the discussion of human health care **policies** in the EU.**
- ✓ **A forum to discuss innovation and **policies** for healthcare, health technology, and the health outcomes for patients, especially those affected by chronic, life-threatening diseases - including rare diseases.**
- ✓ **EPPOSI's ambition is to develop **strategies** that benefit present and future generations.**

## ✓ Objectives

- To encourage timely and regular exchange of information between stakeholders on the latest developments in human health care related to (bio-)medical research, policy and regulations; on the ethical, social, legal and political aspects of this type of research, and on biotechnology, notably for its application to human health care
- To promote a mutual understanding between patients' organisations, science, industry, and EU institutions
- To contribute to equal access for all to human health care products and services in the EU
- To support patients' organisations in presenting timely and effective contributions to the European political debate on all matters that concern them
- To raise public awareness in Europe on the opinion of patients and their organisations
- To help sustain a dialogue within society on progress in medical science through new technologies
- To advocate the development of therapies for unmet medical needs and to facilitate partnerships within society
- To function as an information coordination centre that encourages discussion, opinion-forming, and public debate in the area of human health care

## ***Board Members***

### **✓ Representing Patient Organisations:**

Mary Baker - Rodney Elgie - Michael Griffith - **Alastair Kent, EPPOSI Chair of the Board** and the Executive Committee - Yann Le Cam - Rod Mitchell, EPPOSI Treasurer, Member of the Executive Committee (UK) - Ysbrand Poortman, Former EPPOSI Chair

### **✓ Representing Academic Science:**

Ségolène Aymé - Ivan Baines - Christian Suojanen - Jean-Jacques Cassiman, EPPOSI Secretary, Member of the Executive Committee  
Heinrich Schulte

### **✓ Representing Industry:**

Silvia Matile-Steiner - Thierry Nebout - Andrea Rappagliosi - Erik Tambuyzer, EPPOSI Vice-Chair, Member of the Executive Committee - Jean-Marie Vlassembrouck

## ***Achievements***

- ✓ EPPOSI focuses on building dialogue, consensus positions and policy recommendations for the benefit of EU patients and consumers.
- ✓ These consensus positions have provided building blocks for:
  - **the establishment of the European Orphan Medicinal Products Regulation**
  - **the advancement of biomedical research and the value of innovation**
  - **the timely access to innovative medicines**
  - **several rare-disease therapy developments and partnerships**
  - **East-West European collaboration among patient groups**
  - **bio-banking**

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- ✓ **The Ethical Aspects of Biomedical Research and The Biopharmaceutcial Industry (1994)**
- ✓ **Biomedical Research and Patenting : Ethical, Social, and Legal Aspects (1996)**
- ✓ **Biomedical Research and Orphan Medical Products (1997)**
- ✓ **The Patients' Role in European Health Policy Making (1998)**
- ✓ **EPPOSI Workshops on Partnering for Rare Disease Therapy Development : Nos 1, 2000 and 2, 2001**
  - No 3 : From Research to Development and from Bottlenecks to Partnering (2002)
  - No 4 : Orphan Therapies : From Clinical Development to Equitable Access. (2003)
  - No 5 : A responsible System for Healthcare Innovation and Access to Care (2004)
  - No 6 : People with Rare Diseases - No Longer Alone in the World (2005)
  - 7th Workshop (2006)
  - No 8 : The Reality of Orphan Medicines (2007)

## **Orphan Drugs in the EU: Toward a Common Approach for a Fair and Sustainable Patient Access**

- ✓ **WG 1** The epidemiology of rare diseases to assess the needs
- ✓ **WG2** The assessment of the potential clinical benefit in the premarketing phase and of the real benefit after marketing authorisation
- ✓ **WG3** The economic evaluation of OD
- ✓ **WG4** The sustainability of the system.

- ✓ Discussion on the **adequacy of the economic toolbox** to address rare diseases issues. Proposal of a seminar with leading health economists to see how to best address some of these issues.
- ✓ The collective choices that are implied if one adopts a collective view are extremely difficult and raise many ethical issues.
- ✓ It is therefore important that the grounds on which they are made and the criteria used should be made explicit.
- ✓ Research into **society's values** with respect to rarity compared to severity has to be fostered, with a comparative analysis between Member States.
- ✓ The issue of **risk-sharing** in research and pricing should be discussed more thoroughly. Workshop at EU level, bearing in mind that every decision will have a significant impact on national governments' strategies.

- ✓ **Clarify key concepts** such as “market exclusivity” and “significant benefit”. Improve communication (COMP-EMEA)
- ✓ **Improve transparency** on medicines costs/prices from pharmaceuticals companies. Database ?
- ✓ Envisage an **informal network** of national authorities in charge of orphan drug pricing. Use the same dossier and same set of criteria when discussing the pricing of orphan drugs. European ex-factory reference price.
- ✓ Nevertheless, the **decision making** has to remain with individual member states for final negotiation and price (other elements such as taxes, distribution mode, volume, other products in portfolio of the marketing holder, etc.)
- ✓ Greater public investment in **research** and **risk sharing** on development costs should be promoted to contribute to ensure long term sustainability of the system.

## ✓ **Improve knowledge of the natural history of Rare Diseases**

Before availability of OMPs, without delaying their development

## ✓ **Improve available data on OMPs**

Clinically significant end points in clinical trials

Methodology : RCTs versus alternative methods

## ✓ **Improve cooperation in the field of Health Technology Assessment**

Common assessment of some OMPs within existing networks: MEDEV?

Develop networking within HTA (EUnetHTA project).

## ✓ **Managing uncertainty**

- Post marketing data collection

**Cooperation between EMEA (COMP-CHMP) and HTA agencies : significant benefit - relative effectiveness, choice of the endpoints for clinical trials, pharmaco-epidemiological studies..**

## ✓ France

- 100% of drugs are evaluated before decision
- 2 step procedure:
- HTA (performed by HAS) focused on clinical effectiveness. Price not (yet) taken into account.
- Price decided by Economic Committee after negotiation with the company, and based on the results of HAS' assessment.
- National Budget

## ✓ England

- Limited number of drugs evaluated, topic selection
- One step procedure
- Freedom of pricing
- Assessment/appraisal of the drug includes cost-effectiveness (based on the price decided by the company)
- Cost/QALY, threshold ?
- Budget at local (Primary Care Trusts) level

The screenshot shows the EUnetHTA website homepage. At the top left is the HAS logo. The main header features the EUnetHTA logo and the text "Welcome to EUnetHTA European network for Health Technology Assessment". A navigation menu includes "HOME", "ABOUT EUNETHTA", "HTA", "WORK PACKAGES", "COMMUNICATION", "CONTACT", and "SEARCH".

Key content areas include:

- EUnetHTA presence at the upcoming conferences:** A callout bubble points to the "December 13-14, 2007 - 3rd European Symposium on Pharmaceutical Law, Brussels, Belgium" section.
- EUnetHTA - what it is about...:** Describes the network's role in coordinating HTA efforts across 24 EU member states.
- EUnetHTA Conference 2008 in Paris:** A callout bubble points to the "HTA's Future in Europe" section, which includes a photo of the Eiffel Tower and a registration link.
- E-News from EUnetHTA:** Lists previous newsletters from September 2007, July 2007, and March 2007.
- EUnetHTA at the HTAi Meeting in Barcelona 17-20 June:** Announces the launch of preliminary results, including a Core HTA model and adaptation toolkit.
- EUnetHTA Stakeholder Open Forum:** Invites stakeholders to provide input on the future development of EUnetHTA.
- Latest News:** Features articles such as "Deadline extended - Public consultation EUnetHTA Collaboration 2009+" and "Future EUnetHTA Collaboration - Years 2009+".

At the bottom, it notes that the project is supported by a grant from the European Commission.

**Final Conference Nov.2008**

**Public Consultation: Future EUnetHTA Collaboration 2009 +**



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