EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The importance of patient input for the work of the European Medicines Agency

EMSP Annual and Youth Congress

Juan García Burgos
Head of Public Information and Stakeholder Networking - Medical Information
European Medicines Agency (EMA)

Access to medicines in the EU: the role of the EMA

The importance of patient's input

European Medicines Agency's role and responsibilities

The role of the European Medicines Agency

A decentralised Agency of the European Commission, created in 1995.

The Agency provides the Member States and the European Institutions with scientific advice on the evaluation of the quality, safety and efficacy of medicines (Human and Vet).

What does that mean

The Agency is responsible for:

- The evaluation of marketing authorisation applications submitted by pharmaceutical companies, for certain types of products
- Coordination of pharmacovigilance at European level (supervision of the medicines on the market)
- Provision of scientific advice on the development of medicines
- Evaluation of applications for orphan designation in EU
- Evaluation of paediatric investigation plans (or waivers)
- Provision of good quality and independent information on medicines it evaluates to patients and health professionals

The EMA is not responsible for:

- Controlling advertising
- Pricing and reimbursement
- Providing information on diseases (including therapeutic guidelines)

Marketing Authorisation - Key Principles

- The EU is a Single Market for pharmaceuticals - approx. 0.5 billion people.
- In order to sell a medicinal product in the EU, a company needs a Marketing Authorisation
- There are a number of ways (‘Procedures’) for a company to obtain a marketing authorisation.
- The main scientific principle used in the evaluation of medicines is the benefit/risk balance, based mainly on quality, efficacy and safety aspects

Marketing Authorisation Procedures PRE - 1995

15 National Competent Authorities

15 Parallel National Reviews

15 Independent Marketing Authorisations

- Poor resource utilisation
- Divergent scientific opinions
- Divergent patient / doctor information

Marketing approval for medicines today - Two European Systems

Centralised Procedure

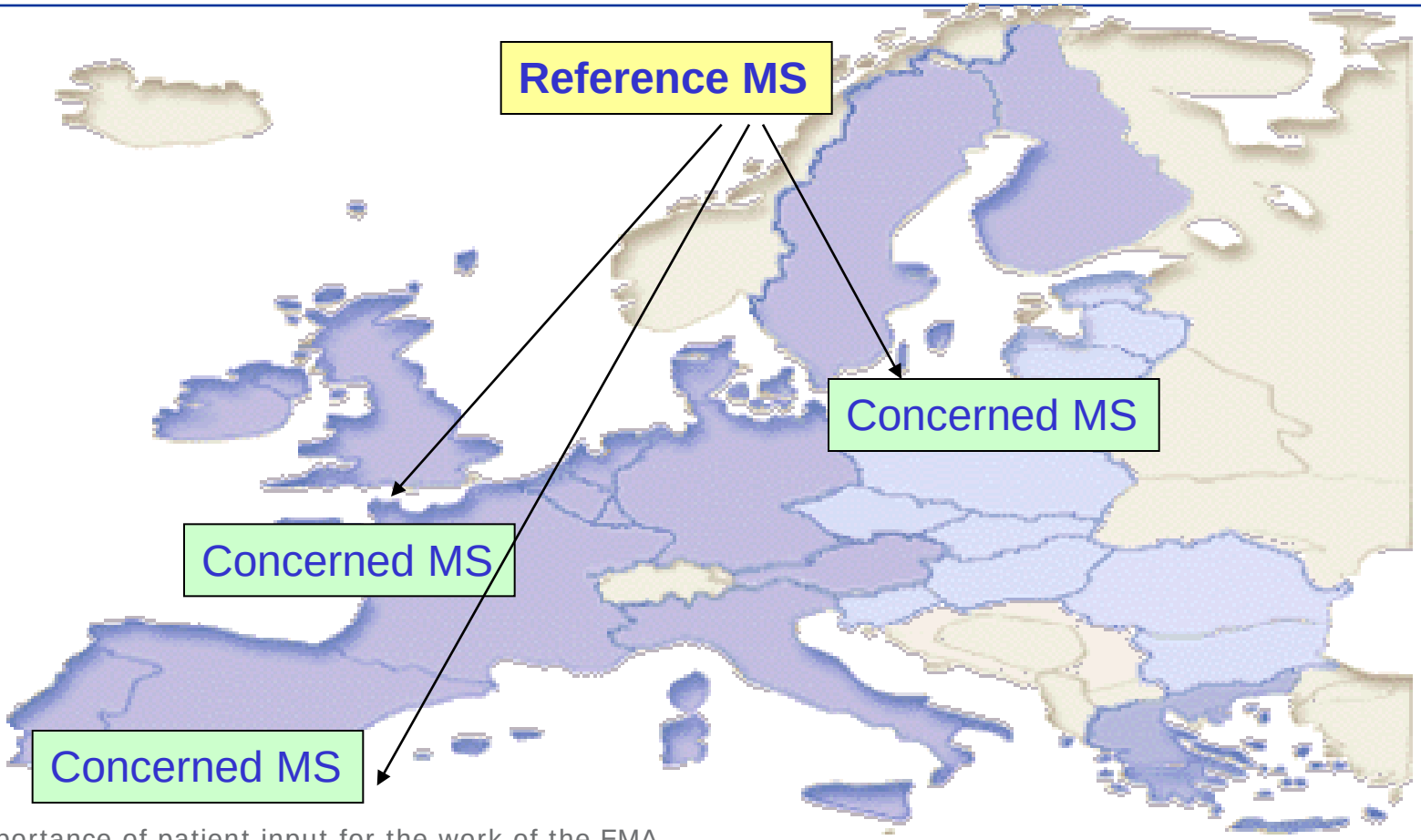
(via EMEA)

Mutual Recognition Decentralised Procedure (national licences)

Both Systems allow

Better Resource Utilisation
Harmonised Scientific Opinions
Harmonised Information to Doctors / Patients

Mutual Recognition/Decentralised



Centralised Procedure

Creation of EMA:

- to manage the centralised procedure
- to formulate scientific opinions

⇩

Sent to the European Commission :

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Commission Decision

(Pan European Marketing Authorisation)

Centralised Procedure

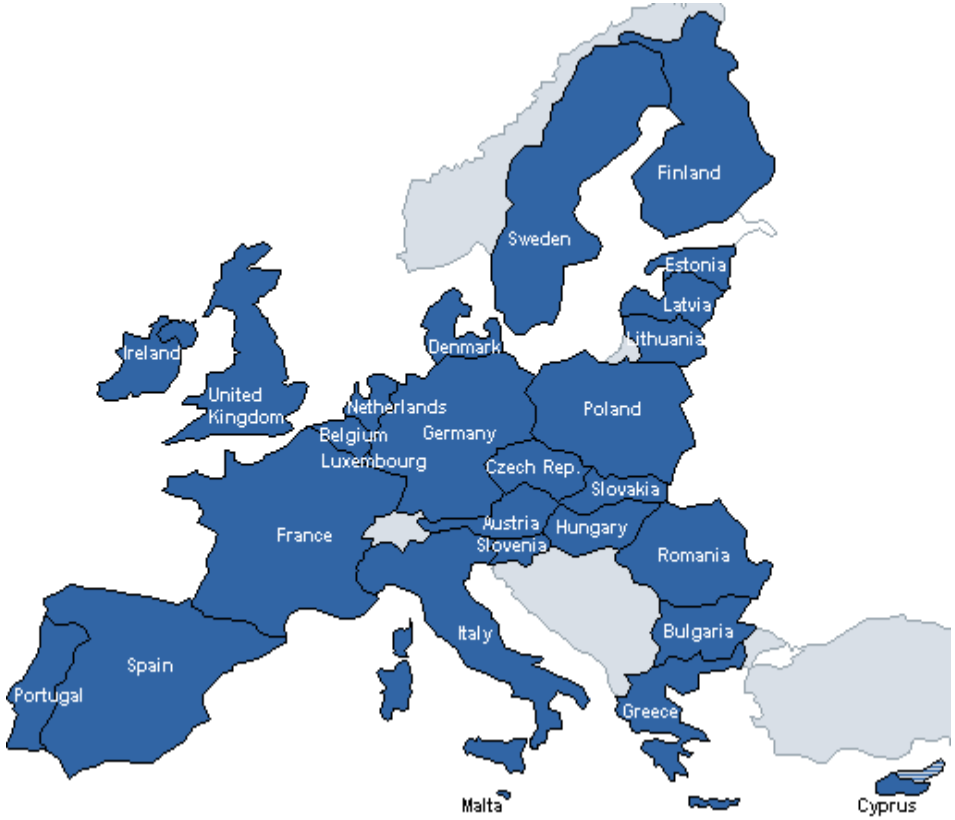
1 application

1 evaluation

1 authorisation for all EU

1 product information (SPC, Labelling, PL)

All EU languages



Which medicines are evaluated at the EMA?

- Rare diseases
- HIV, cancer, neurodegenerative disorders, diabetes
- Auto-immune diseases, viral diseases
- All biotech products
- Gene therapy
- Monoclonal antibodies

+ Other innovative products

How does the Agency work? (1)

Scientific Committees:

- Committee for Medicinal Products for Human Use (CHMP)
- Committee for Medicinal Products for Veterinary Use (CVMP)
- Committee for Orphan Medicinal Products (COMP)
- Committee for Herbal Medicinal Products (HMPC)
- Paediatric Committee (PDCO)
- Committee for Advance Therapies (CAT)

Working parties:

- give support to the Committees

How does the Agency work? (2)

Agency's secretariat:

- gives technical, scientific and administrative support

Experts:

- throughout the EU collaborates with scientific committees and working parties

The European Medicines Agency network

A unique structure

- The Agency partners with:
  - More than 40 national competent authorities
  - 4000 EU Experts
  - European Parliament
  - European Commission
- Establishes relation with non-EU regulatory authorities, international health organisations, industry academia, and the general public

The importance of patient input for the work of the European Medicines Agency

Interaction with patients'/consumers' organisations

- Long experience since the Agency was created

- Unique model of interaction:
  - framework of interaction and,
  - selection criteria

Framework of interaction

The framework comprises:

- The scope of the interaction
- The objectives to be achieved
- The working methodology
- The monitoring (including performance indicators)

Ultimate goal:

- Involve patients in the Agency's activities
- Better inform patients

Framework of interaction

Principle of patient empowerment:

- Patient involvement/patient information/patient safety

Role of patients/consumers' organisations as multipliers of the interaction (promoting patient safety)

Selection criteria for involvement of patients' organisations

- Legitimacy
- Mission/objectives
- Activities
- Representativity
- Structure
- Accountability and consultation modalities
- Transparency

Eligible organisations (1)

Permanent call for interest to work with the EMA:

Launched in 2005 and “continuous”

List of eligible patients’ & consumers’ organisations published on the Agency’s website

Eligible organisations (2)

Patient involvement in the Agency's activities: so-far experience (1)

Full members of:

- Management Board
- Committee for Orphan Medicinal Products (COMP)
- Paediatric Committee (PDCO)
- Committee for Advance Therapies (CAT)

Patient involvement in the Agency's activities: so-far experience (2)

- Patients and Consumers Working Party (PCWP)
- Review of product information:
  - EPAR summaries, package leaflets, safety information–Q&As-)
- CHMP (Ad-hoc collaboration):
  - Input on assessment of products (e.g. thalidomide, tysabri,etc)
  - Experts in scientific advice/protocol assistance
  - Input in guideline preparation
  - Observers in Pharmacovigilance Working Party (pilot phase)
- Regular participation in Agency's workshops and conferences

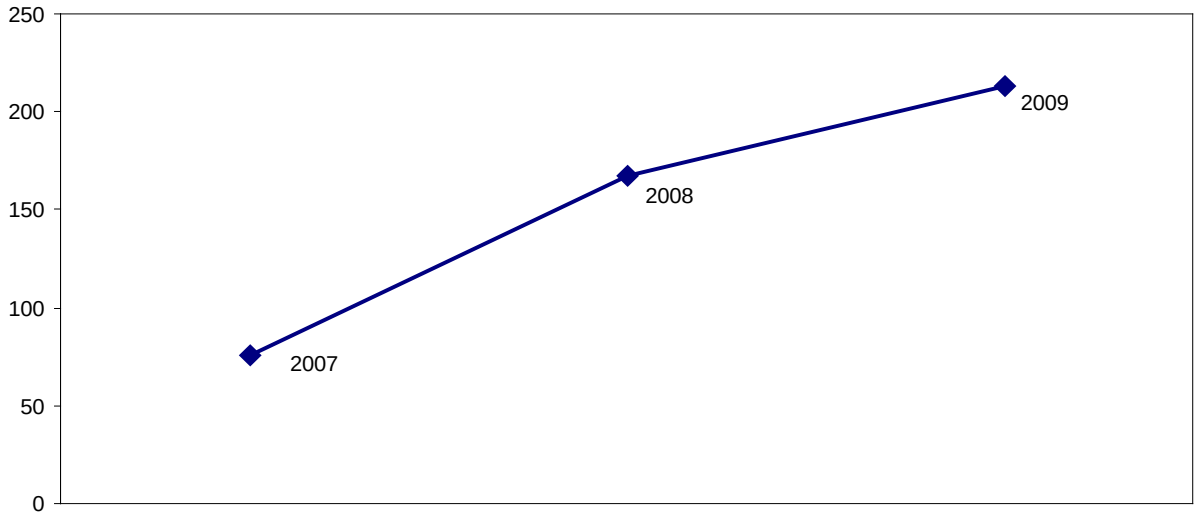
EMA Scientific Committees Working Party with Patients' and Consumers' Organisations (PCWP)

PCWP Members: 15/30 Eligible Organisations + representatives from Agency's scientific committees (CHMP, COMP, HMPC, PDCO and CAT)

Co-Chair: Isabelle Moulon (EMA)/ Lise Murphy (Eurordis)

4 meetings per year (one joint with Healthcare Professionals Working Group)

Number of patients/consumers involved in EMA activities in recent years



Added value of involving patients in EMA (1)

- Bring a unique and critical input based on their real-life experience of the disease and its current therapeutic environment.
- Increase transparency and build confidence and trust in the regulatory process.
- Representing patients interests and providing a “patient perspective” view, on behalf of those directly affected by regulatory decisions
- Bringing experience of the disease and/or identifying patients with experience of the disease when necessary

Added value of involving patients in EMA(2)

- Actively contributing to patient information and communication related to medicines. Ensure that patients and patient's organisations can access to useful and understandable information.
- Disseminating committees' outcomes when they become public; passing on information to other patients and patients' organisations.
- Advising and supporting regulators in its dialogue with industry and other stakeholders when identifying areas of medical need for target research.

The challenges

- Lack of resources in the organisations
- Need for training to understand the regulatory environment
- Need to define the roles of the patient in the different activities/scientific committees
- Difficulty to find suitable experts (e.g. language barrier)

The way forward in involving patients in the work of the agency

- Revision of the current framework of interaction is ongoing:
  - Define the role of patients/consumers in the agency's scientific committees
  - Further involve patients in benefit/risk evaluation
  - Foster involvement in the preparation and dissemination of EMA information intended to the public (including safety communication)
  - Participation in the Pharmacovigilance Working Party
  - Provision of specific (financial) support

Thank you for your attention