Patient Involvement in EMA Activities

An overview

by

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EURORDIS Patient Representative

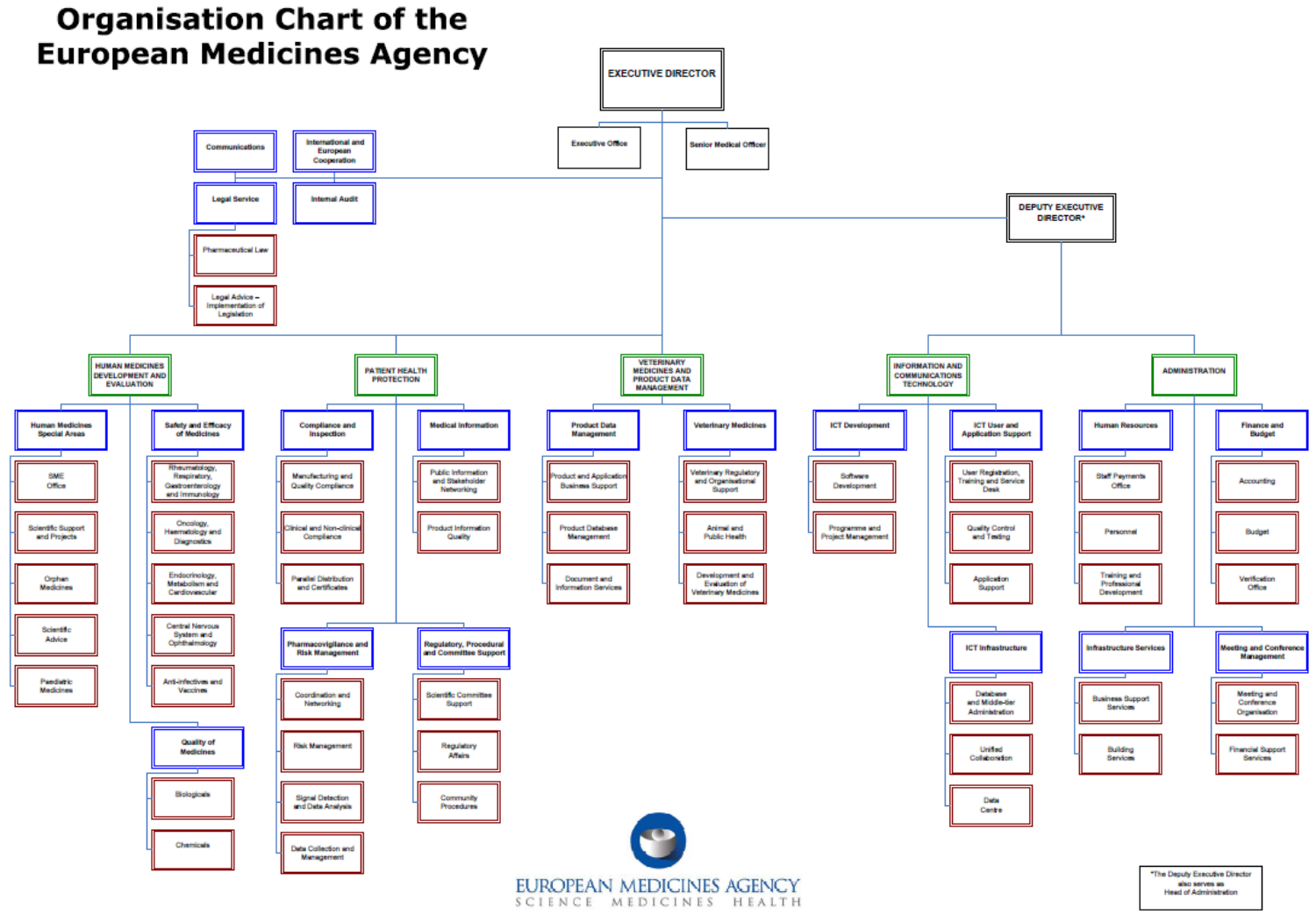
COMP

The European Medicines Agency (EMA)

- is a decentralised body of the European Union created in 1995 with headquarters in London
- Its main responsibility is the protection and promotion of **public and animal health**, through the evaluation and supervision of the quality, safety and efficacy of medicines for human and veterinary use
- also plays a role in stimulating innovation and research in the pharmaceutical sector.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The EMA is headed by the Executive Director and has a secretariat of approximately 530 staff members . A Management Board exists, which includes patient representatives which supervises the EMA's activities and is responsible, in particular, for budgetary matters.



EMA Responsibilities

- 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 and protocol assistance to companies on the development of medicines
- Evaluation of applications for orphan designation in the European Union
- Evaluation of PIPS-paediatric investigation plans (or waivers)
- Provision of good quality and independent information on medicines it evaluates to patients and health professional

BUT NOT

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

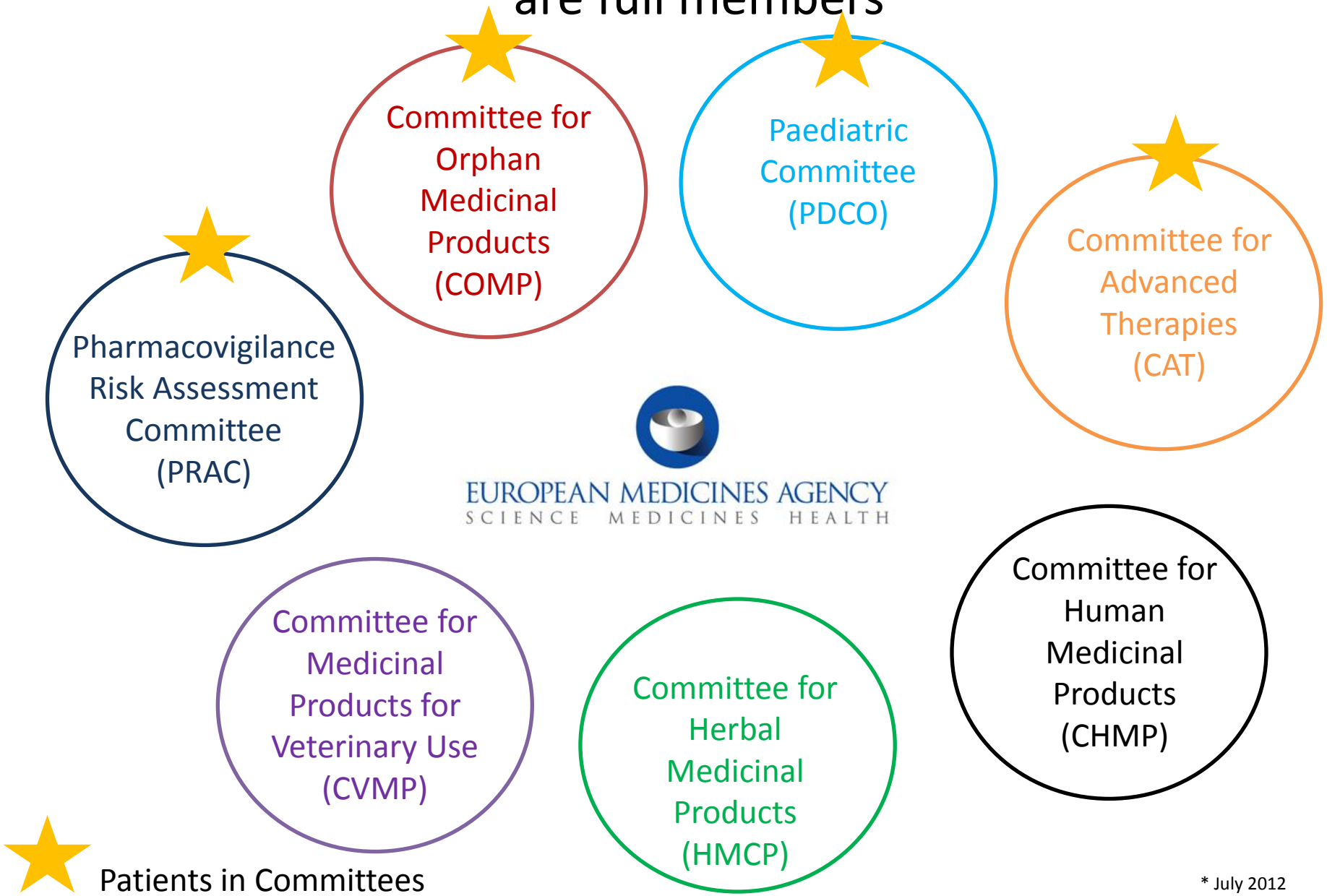
The centralised procedure

- **The EMA** is responsible for the scientific evaluation of applications for European marketing authorisation for medicinal products (**centralised procedure**), the main principle being benefit/risk balance, based on quality, efficacy and safety aspects

- The EU is a Single Market for pharmaceuticals. Therefore the **centralised** (or 'Community') **marketing authorisation (MA) granted by the EC is valid in all European Union** (EU) and EEA-EFTA states (Iceland, Liechtenstein and Norway).

- **All medicinal products** for **human and animal use** derived from biotechnology and other high technology processes **must be** approved via the centralised procedure including all **designated orphan medicines** intended for the treatment of **rare diseases**.

EMA Scientific Committees where patients' representatives are full members



* July 2012

Criteria for involvement of organisations

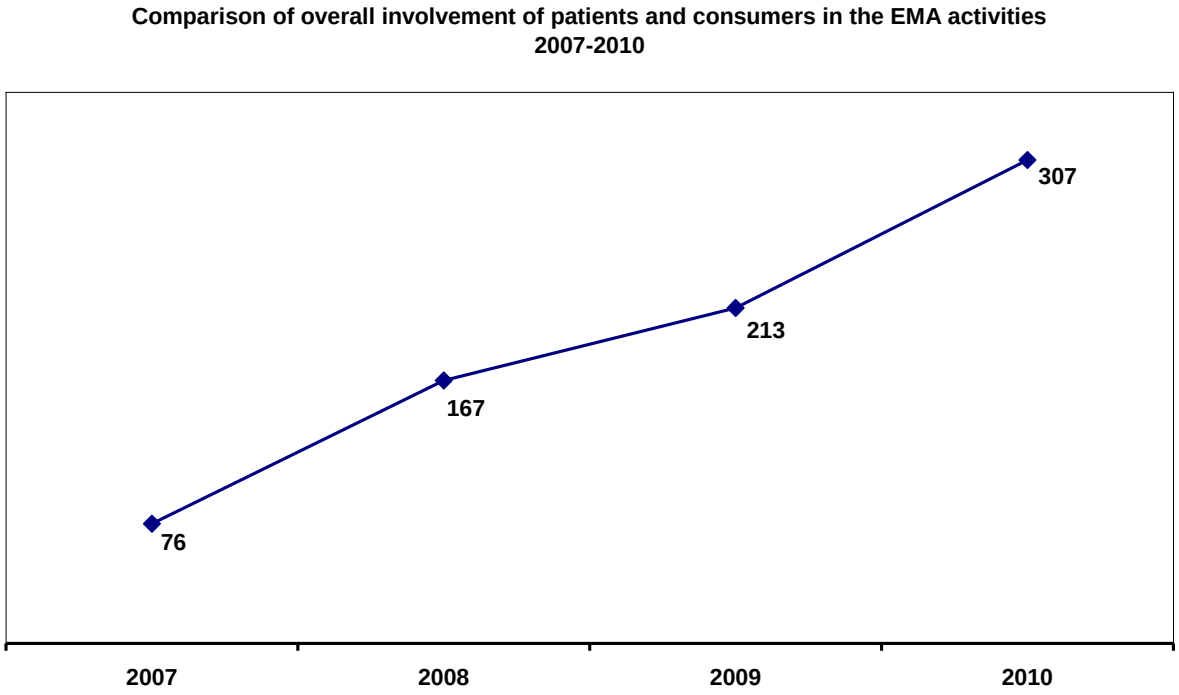
To qualify for consideration , patient organisations must demonstrate

- **Legitimacy**, with statutes registered in the European Union (EU)
- Clear **mission** and **objectives** with an interest in medicines
- European patient or consumer **representation**
- Adequate **structure** and consultation modalities
- **Accountability** and **transparency**

List of eligible patients'/consumers' organisations published on the EMA website
www.ema.europa.eu

The challenges for patient participation

- 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 in finding suitable experts (e.g. language barrier)



Added value of involving patients in EMA

- Bringing a unique and critical input , a “patient perspective”, based on real-life experience of the disease and its current therapeutic environment and/or identifying patients with experience of the disease when necessary, on behalf of those directly affected by regulatory decisions
- Contributing to patient information and communication related to medicines to ensure their stakeholders can access useful and understandable information
- Increasing transparency; building confidence and trust in the regulatory process.
- Disseminating committees’ outcomes when they become public 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.

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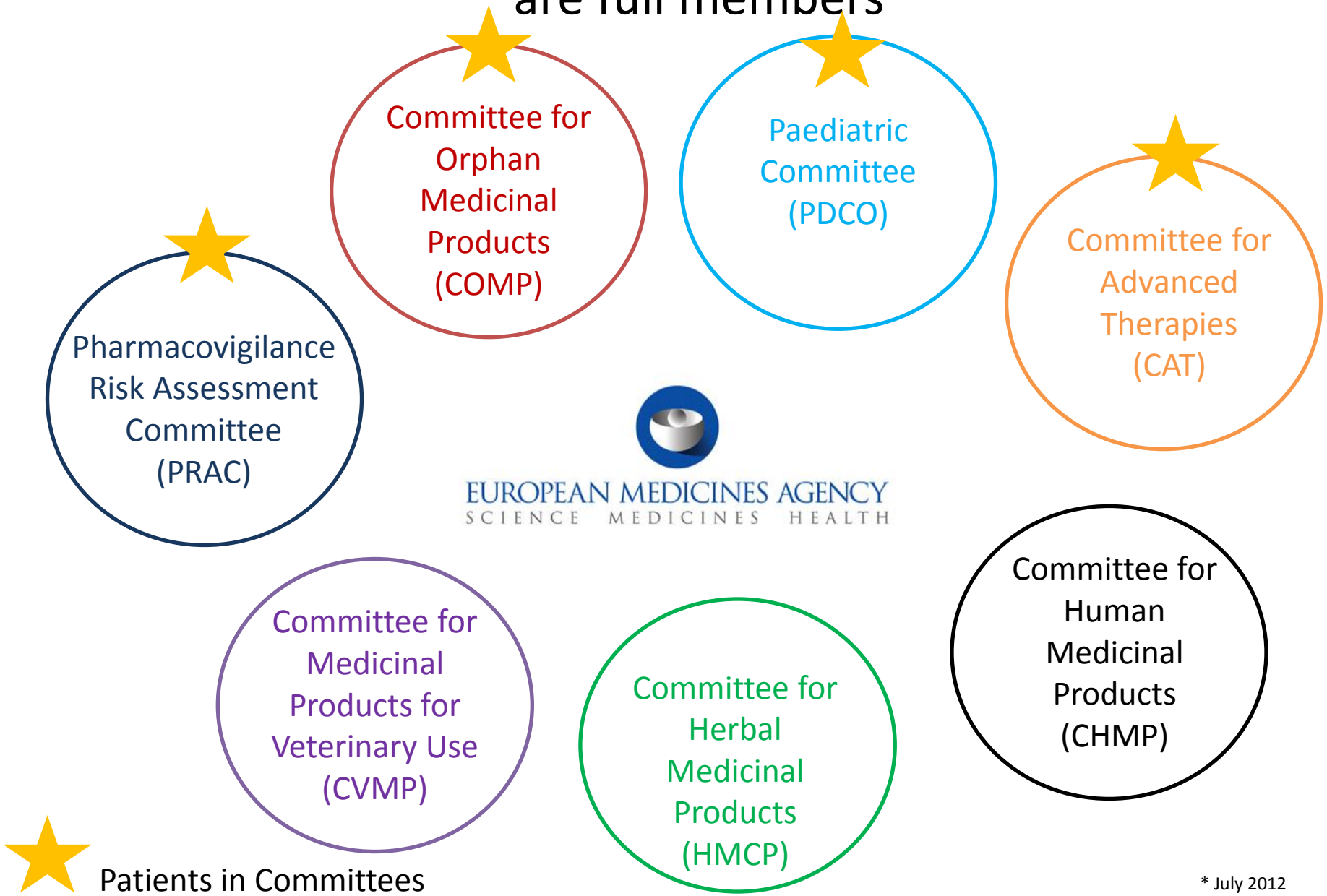

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Patient involvement in EMA activities: 2000-2012

- Full members of Management Board
- Frontline advocate for EU Orphan Drug Regulation (1999), Pharma Legislation (2003), Paediatric Regulation (2006), Advanced Therapies (2007)
- Two EURORDIS Patients' Representatives in each of:

  Committee for Orphan Medicinal Products (COMP) and one Observer - since 2000
- Paediatric Committee (PDCO) - since 2008
- Committee for Advanced Therapies (CAT) - since 2000
- Patients' and Consumers' Working Party (PCWP) - since 2006
- Protocol Assistance, Scientific Advice, Risk Management Plans
- Future Committee on Pharmacovigilance (PRAC) to be formed July 1, 2012








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