How are medicines evaluated at the EMA – Part I

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What is the European Medicines Agency (EMA)

The EMA is the EU regulatory body responsible for the scientific evaluation and supervision of medicines developed by pharmaceutical companies for use in the European Union (Human and Veterinary).
European Regulatory Network

The European regulatory system for medicines is a unique model in the global regulatory environment.

This system is based on a network that includes all national medicines authorities (human and veterinary) from EU Member States and the European Economic Area, working closely together in an integrated manner.
What does the EMA do

• Evaluation of **marketing authorisation applications** for **human** and **veterinary** medicines submitted by pharmaceutical companies

• Provision of **scientific advice** on the development of medicines

• Evaluation of applications for **orphan designation** in EU

• Evaluation of **paediatric investigation plans** (or waivers)

• Coordination of European **pharmacovigilance** (supervision of the medicines on the market)

• Evaluation of **arbitration** and **referral** procedures

• Provision of good quality and independent **information on the medicines** it evaluates to patients and healthcare professionals

• Coordination of Member States’ **inspections**
What the EMA does not do

The European Medicines Agency does not control:

- Pricing of medicines
- Access to medicines
- Advertising of medicines
- Patents on medicines
- Medical devices
- Homoeopathic medicines
- Food supplements
- Cosmetics
- Tobacco
Are all medicines approved via the EMA?

No. In the European Union (EU), there are **two ways** of getting a marketing authorisation for a medicine:

1. **Centralised authorisation procedure**, via the European Commission after evaluation by EMA, which results in a single marketing authorisation valid throughout the EU;

2. **National authorisation procedures**, where individual EU Member States authorise medicines for use in their own territory through 3 possible procedures:
   - National authorisation
   - Mutual-recognition procedure (MRP)
   - Decentralised procedure (DCP)
Medicines that are mandatory for evaluation at EMA

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

Medicines outside the mandatory scope can also be evaluated at EMA if they meet certain criteria.
The centralised procedure and the EMA

Marketing Authorisation application

Evaluation

- Authorisation in all EU MS
- Invented name
- Product information
  - Summary of Product Characteristics (SmPC)
  - Labelling
  - Package Leaflet (PL)

ALL

EU languages
EMA and its **scientific committees**

The EMA committees contain members nominated by the medicines regulatory authorities of the EU Member States (the ‘national competent authorities’)

- **Committee for Human Medicinal Products (CHMP)**
- **Committee for Veterinary Medicinal Products (CVMP)**
- **Paediatric Committee (PDCO)**
- **Committee for Herbal Medicinal Products (HMPC)**
- **Committee for Orphan Medicinal Products (COMP)**
- **Committee for Advanced Therapies (CAT)**
- **Pharmacovigilance Risk Assessment Committee (PRAC)**

**Patient members**
Other Working Parties
- Biosimilars
- Biostatistics
- Blood Products
- Cardiovascular
- Central Nervous System
- Infectious Diseases
- Oncology Working
- Pharmacogenomics
- Pharmacokinetics
- Rheumatology/Immu.
- Vaccines

Scientific Advisory Groups
- HIV / Antiviral
- Anti-infectives
- Diabetes Endocrinology
- Psychiatry
- Neurology
- Oncology
- Cardiovascular issues
- Diagnostics
- Vaccines

Ad-hoc Expert Groups
- SAWP: Scientific advice
- QWP: Quality
- CMDh: Co-ordination Group for Mutual Recognition and Decentralised Procedures
- SWP: Safety
- BWP: Biologics
- GCP: Inspectors
- HCPWP: Healthcare professionals
- PCWP: Patients and consumers
- QR:D: Working Group on Quality Review of documents

Working Parties and other Groups
Experts who work with the scientific committees

- National Agencies
- Learned societies
- Academia and Networks
- Healthcare professionals
- Patients and Consumers
Patient/consumer involvement in the EMA
Interaction with patients: the EMA journey... so far

1995
EMA created

1996
Dialogue with HIV patients

2000
Patients join COMP as full members

2003
Working group with patients created

2005
Framework of interaction with patient and consumer organisations

2006
Patients and Consumers Working Party (PCWP) created

2014
• Dedicated Patients and Healthcare Professionals Department created
• Revised Framework

Ongoing...
Systematic inclusion of real life experience EMA regulatory output
How are patients involved at EMA?

- **Patients representing patients’ organisations**
  - Management Board
  - EMA Scientific Committee(s)

- **Patients representing their organisations**
  - Patients’ and Consumers’ Working Party (PCWP)
  - EMA consultations
  - Workshops

- **Patients as individual experts**
  - Scientific Advice / Protocol Assistance Procedures
  - Scientific Advisory / ad hoc expert Groups
  - Scientific committee consultations
  - Review of documents
Patient involvement as individual experts in EMA activities

**Pre-submission:**

- Participation in scientific advice/protocol assistance procedures

**Evaluation and Post-authorisation**

- Participation in expert meetings (SAG and ad hoc)
- Respond to consultations on assessment of medicines from scientific committees and working parties
- Review information on medicines: Package leaflets, EPAR summaries, safety communications and other Agency documents for the public
Patient involvement along the medicine lifecycle at EMA
Scientific Advice at the EMA
Committees in human Medicines Regulatory process
Scientific Advice

- Pharmaceutical companies can request scientific advice from the EMA regarding the development of a medicine.

- Aimed at ensuring the most appropriate studies are conducted, avoiding major objections related to the study design during evaluation.

- The Scientific Advice Working Party (SAWP) and the Committee for Medicinal Products for Human Use (CHMP) provide scientific advice by answering specific questions posed by the companies.
Types of questions

Scientific Advice can be provided on questions ranging from:

- **Quality** – manufacture of medicines
- **Non-clinical** – animal studies – interpretation and extrapolation of results
  - **Clinical** – discussion of study population, endpoints, feasibility of trial
- **Regulatory** – including statistics
  - **Significant benefit** – for orphan medicines (where applicable)
The role of patients and patient representatives

Patient representatives are invited to participate in EMA scientific advice procedures:

• Either face to face meeting or via written comments

• Share their ‘real-life’ perspective and experience with the SAWP and the pharmaceutical company, in relation to a particular medicine in their disease area.

• Provide comments on the development proposals from the company (e.g. endpoints, population, feasibility etc)
Scientific Advisory/ad hoc expert Group meetings
Committees in human Medicines Regulatory process

- Orphan Designation/ATMP Classification
- Scientific Advice Protocol assistance
- Paediatric Investigation Plan
- Marketing Authorisation Application Evaluation
- Post Marketing Authorisation
Evaluation overview - CHMP

- Evaluation of benefit/risk
- Assessment of Product Information
- Preparation of RMP summary
- Assessment on need for post safety/efficacy studies
- Additional information:
  - Brochure – Applying for marketing authorisation
  - CHMP overview
Type of Approvals

**Standard:**
Comprehensive data

**Conditional Approval:**
- Comprehensive data not available; to be provided after approval
- Must fulfil scope (orphan drugs, emergency threats, serious and life-threatening diseases)
Approval valid for 1 year, renewable

**Exceptional Circumstances:**
- Comprehensive data not available and cannot be provided
- Must meet criteria (rarity, medical ethics, state of scientific knowledge)

For these hyperlinks – please refer to question 56
**Scientific Advisory/Ad hoc expert Groups**

- The CHMP or the Pharmacovigilance and Risk Assessment Committee (PRAC) can convene a SAG during the evaluation of a specific medicine when they encounter specific questions that are best answered by experts in the field, including patients.

- SAGs exist for specific therapeutic areas and when an issue arises for which there is no SAG, an *ad hoc* expert group is organised.

- Two patients, with experience of the disease/condition, are invited to participate in every SAG / ad hoc expert group meeting.

- Patients contribute by providing input to the discussions on the benefits and risks, from their perspective in relation to the questions that the CHMP is asking.
Contact

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