The European Medicines Agency (EMA)
Authorization of Medicinal Products
EU Paediatric Regulation

C4C : Train the Trainers Workshop
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European Medicines Agency (EMA)

- **Agency** of the EU
- Located in **Amsterdam**
- Operational since **1995**
- EMA is a **scientific body**
  (contribution from experts within the EU regulatory network → 27 EU MS),
- EMA coordinates and supports **scientific evaluation, supervision and safety monitoring** of **human and veterinary medicines** in **EU**
  
  **ensuring their safety & efficacy**

Reg. (EC) No 726/2004
[previously Reg. (EEC) No 2309/93]
Main roles of the EMA

- Scientific evaluation of applications for EU marketing authorisations in centralised procedure and supervision of authorised products
- Evaluation of applications for orphan designation in EU and paediatric investigation plans (or waivers)
- Coordination the EU's pharmacovigilance system
- Support of European small- and medium-sized-pharmaceutical enterprises through regulatory & scientific guidance.
- Coordination of inspections requested by the committees
- Provides guidance for innovation and research in the pharmaceutical sector (e.g. scientific advice, guidelines)
The European Medicines Agency (EMA) provides guidance and support to medicine developers. This includes scientific and regulatory information on how to design and run clinical trials, compliance standards, and obligations and incentives for developers of specialised medicines.
Scientific Committees

**CHMP** Committee for Human Medicinal Products

**PRAC** Pharmacovigilance Risk Assessment Committee

**CAT** Committee for Advanced Therapies

**COMP** Committee for Orphan Medicinal Products

**PDCO** Paediatric Committee

**CVMP** Committee for Veterinary Medicinal Products

**HMPC** Committee for Herbal Medicinal Products

-> Committees are supported by **28 Working Parties**
CHMP Working Parties

- Healthcare Professionals' Working Party
- Biologics Working Party
- Patients' and Consumers' Working Party
- Quality Working Party
- Safety Working Party
- Scientific Advice Working Party
- Biosimilar Medicinal Products Working Party
- Biostatistics Working Party
- Blood Products Working Party
- Cardiovascular Working Party
- Central Nervous System Working Party
- Infectious Diseases Working Party
- Oncology Working Party
- Pharmacogenomics Working Party
- Pharmacokinetics Working Party
- Rheumatology/Immunology Working Party
- Vaccines Working Party
How are medicines approved?
Different authorisation routes: one set of common rules

Centralised procedure (via EMA)
National procedures (via Member States)
Which medicines are approved through the centralised procedure?

- Human medicines containing new active substances for the treatment of HIV/AIDS, cancer, diabetes, neurodegenerative diseases, auto-immune, and other immune dysfunctions, and viral diseases
- Medicines derived from biotechnology processes, such as genetic engineering
- Advanced therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines
- Officially designated ‘orphan medicines’ (medicines used for rare human diseases)
- Innovative veterinary medicines and products to be used as growth enhancers
What is the benefit of the centralised procedure for EU citizens?

- Medicines are authorised in all EU countries at the same time
- Centralised safety monitoring
- Product information available in all EU languages at the same time
- Access to the largest network of experts in medicines regulation
Medicines for rare diseases—orphan designation

The EU’s orphan designation programme provides incentives for the development of medicines for rare diseases, including a 10-year period of market exclusivity and reduced fees.

Companies can apply for orphan designation for their medicine, provided certain criteria are met.

The Committee for Orphan Medicinal Products (COMP) reviews applications for orphan designation—orphan designated products are then evaluated by EMA’s Committee for Medicinal Products for Human Use (CHMP) for a marketing authorisation recommendation.

*Affecting fewer than 5 in 10,000 people

~1 in 17 people has a rare disease over 2200 orphan designations granted in the EU over 160 orphan medicines authorised in the EU