EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The European Medicines Agency (EMA)

Authorization of Medicinal Products

EU Paediatric Regulation

C4C : Train the Trainers Workshop

16 September 2020

***Janina Karres**, Ph.D.*
Scientific Officer, Paediatric Medicines Office
European Medicines Agency

An agency of the European Union

Disclaimer

The presenter does not have any conflict of interests.

European Medicines Agency (EMA)

EUROPEAN MEDICINES AGENCY

- **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

EMA serves market of **>500 million people** in **EU**

Reg. (EC) No 726/2004
[previously Reg. (EEC) No 2309/93]

EMA has recommended **> 1200 human medicines** for licensing in **EU**

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)

Regulatory Interactions-
Research and development support at EMA

https://www.ema.europa.eu/en/human-regulatory/research-development

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.

- Adaptive pathways
- Advanced therapy medicines
- Clinical trials
- Compassionate use
- Compliance
- Data on medicines (ISO IDMP standards)
- Ethical use of animals in medicine testing
- Innovation in medicines
- Medicines for older people
- Non-pharmaceutical products
- Orphan designation: research and development
- Paediatric medicines: research and development
- Pharmacovigilance
- PRIME
- Quality by design
- Scientific advice and protocol assistance
- Scientific guidelines
- Supporting SMEs
- Support for early access

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

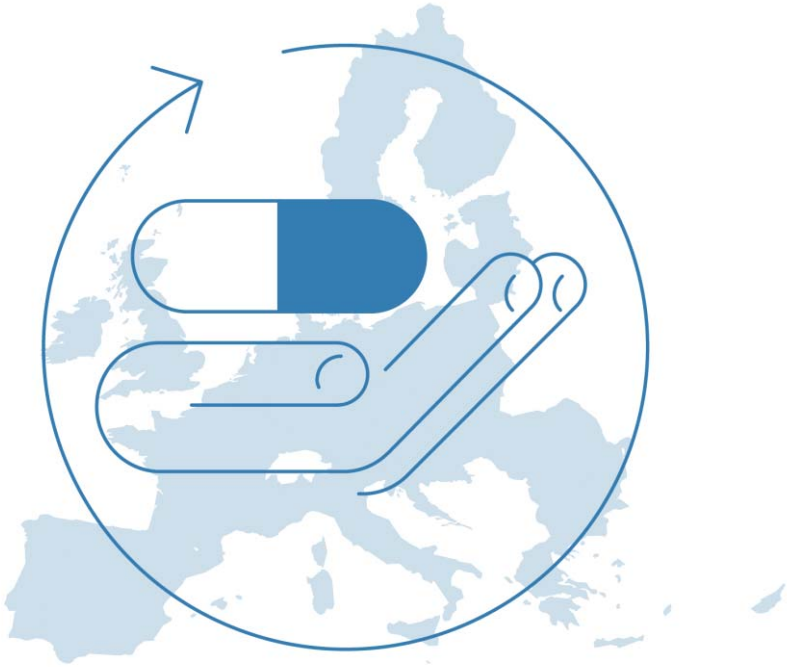
-> Committees are supported by **28 Working Parties**

CHMP Working Parties

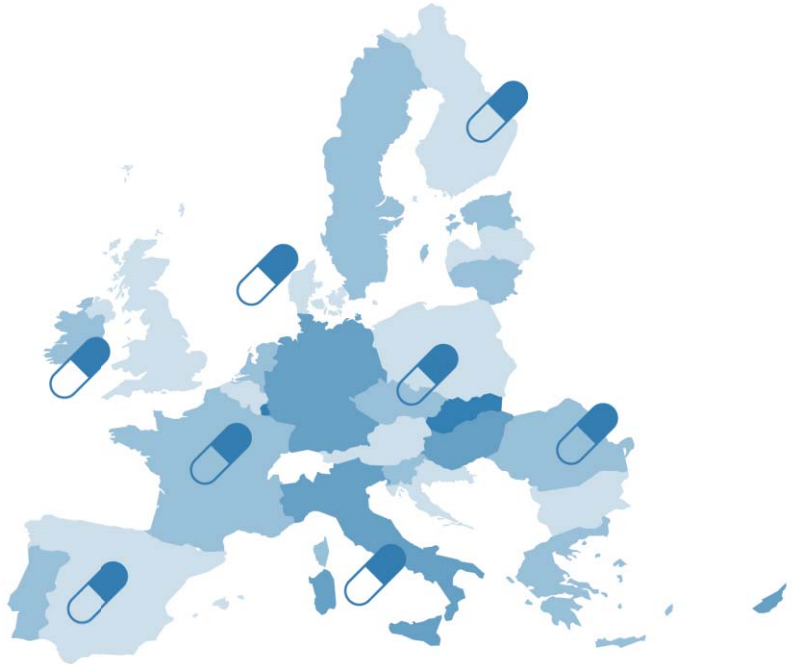
- Healthcare Professionals' Working Party
- Biologics Working Party
- Patients' and Consumers' Working Party
- Quality Working Party
- Safety Working Party
- Scientific Advice Working Party
- Biosimilar Medicinal Products Working Party
- Biostatistics Working Party
- Blood Products Working Party
- Cardiovascular 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

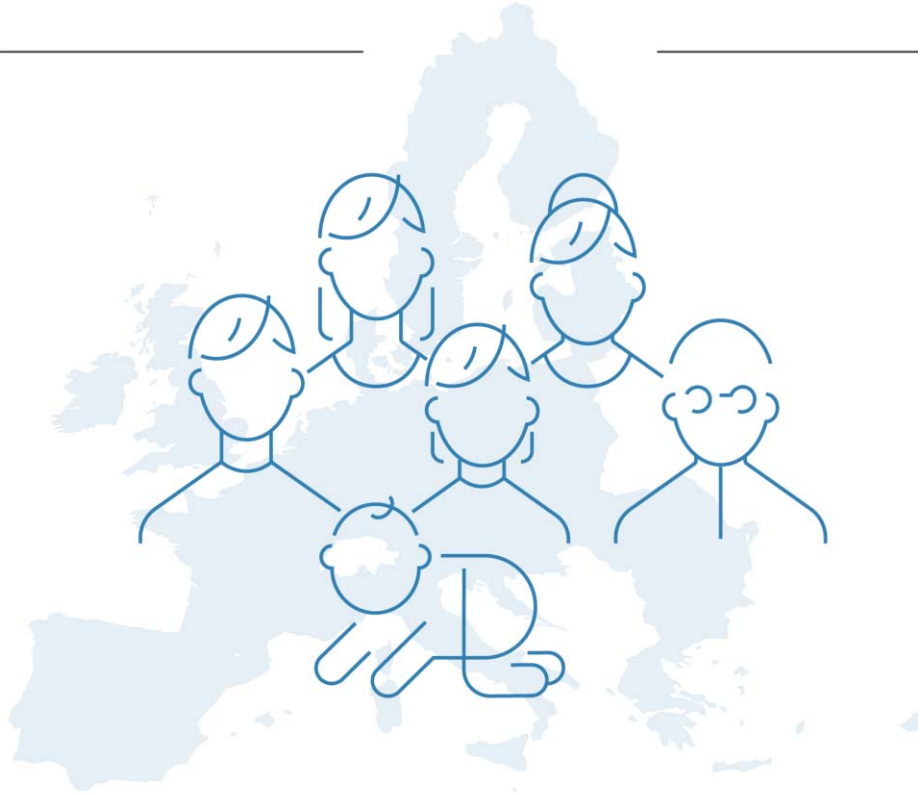
What is the benefit of the centralised procedure for EU citizens?

Medicines are authorised in all EU countries at the same time

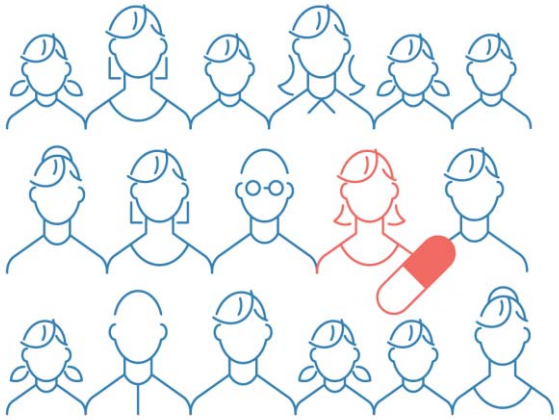
Centralised safety monitoring

ABC
XΨΩ
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



*Affecting fewer than 5 in 10,000 people

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

~1 in 17 people has a rare disease

over 2200 orphan designations granted in the EU

over 160 orphan medicines authorised in the EU