# How are medicines regulated?

## Introduction

A medicine cannot be marketed in the European Union (EU) unless the company has obtained a Marketing Authorisation (MA) for that medicine from the relevant regulatory authority. MAs are only granted for medicines that are proved to be safe, effective and of high quality.

When a pharmaceutical company wants to obtain authorisation to market a new medicine, they must submit a Marketing Authorisation Application (MAA) to the relevant regulatory authority(ies).

## Setting the rules for medicines regulation

The rules for obtaining an MA are laid out in various legislative documents at a European and national level:

- Regulations and directives
- Guidelines

### [glossary_exclude]Regulations and directives

EU regulations and directives are proposed by the European Commission and jointly adopted by the Council and the European Parliament after review and possible amendments.

Once regulations are adopted, they are directly applicable in law in all Member States (MSs).

Directives are instructions to MSs. The requirements of a directive must be introduced into the MS’s national

legislation.

You can find all regulations and directives on the web site of the European Commission in the Eudralex: https://health.ec.europa.eu/medicinal-products/eudralex_en[/glossary_exclude]

## [glossary_exclude]Guidelines

Regulations and national laws are not always very detailed. To facilitate the interpretation of the legislation and its uniform application across the EU, numerous guidelines of regulatory and scientific nature have been adopted. They provide more detailed information for both industry and regulatory authorities on what precisely to do in any given situation.

Draft guidelines are published for open consultation before the final version is adopted by the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) and/or European Commission. https://www.ema.europa.eu/en/human-regulatory-overview/research-and-development/scientific-guidelines

When a company submits documentation for a MAA, the regulatory authorities assess whether the company has complied with the relevant guidelines. It is only acceptable to deviate from a guideline with sound scientific justification.[/glossary_exclude]

# Assessing a medicine for marketing authorisation

The regulatory authorities check the documentation submitted by the company and assess whether it sufficiently establishes that the medicine meets the criteria for:

- Quality

- Safety
- Efficacy

The documentation must also demonstrate that the benefit of taking the medicine outweighs the risk (a positive benefit-risk ratio).

**Quality**

The active substance in the medicine must be of high quality. The company must describe how they produce the active substance. They must also document the nature and amount of any impurities in the active substance and how they control these amounts.

The company must also describe how they manufacture the finished medicinal product, and any test procedures they use to ensure good quality.

**Safety**

During the development of the medicine, the company must demonstrate that the active substance and the finished medicinal product are safe. The medicine should be tested first in animals (non-clinical safety testing) and then in humans (clinical studies).

The company must collect all information on any possible or observed adverse reactions.

**Efficacy**

The results of clinical trials should demonstrate that the medicine has the desired effect.

**Benefit-risk assessment**

The most important task for regulators is to evaluate the balance between the benefits and risks of the medicine. A medicine can never be totally safe. This means that before

authorising a medicine, regulators must consider:

- What are all the good effects of the medicine – for instance, what are the treatment effects on the disease?
- What are the harmful effects of the medicine – for instance, were there any adverse reactions?
- Whether the benefits of taking the medicine outweigh the risks incurred.

Benefit-risk assessments are not straightforward. Careful consideration of any adverse effects caused by the medicine must show that they are acceptable. This depends on many things – for example on the seriousness of the illness.

- If the medicine is meant for treatment of mild pain, only a few mild side effects would be acceptable.
- If the medicine is to treat a serious cancer for which there is no treatment yet available, quite severe side effects may be accepted. This is because the benefits from an increased quality of life or a life-prolonging effect may outweigh the risks incurred by taking the medicine.

# Patient Involvement

Patients are important stakeholders in the approval of new medicines, as they are the ones who actually take the medicines.

At the European level, the European Medicines Agency (EMA) has outlined a number of scenarios in which patients are involved in the regulatory processes.

Patient organisations hold two seats on the EMA management board. In addition, they have formal members in four of the seven scientific committees:

- Committee for Orphan Medicinal Products (COMP)
- Committee for Advanced Therapies (CAT)

- Paediatric Committee (PDCO)
- Pharmacovigilance Risk Assessment Committee (PRAC)

Additionally, the Committee for Human Medicinal Products (CHMP) – which is responsible for the assessment of marketing authorisation applications submitted through the Centralised Procedure (CP) – will consult patients' organisations in specific cases where needed.

The EMA's Patients' and Consumers' Working Party (PCWP), established in 2006, has enabled the Agency to build upon its existing interactions with patients and consumers. It provides recommendations to EMA and its human scientific committees on all matters of interest in relation to medicines.

## References

European Medicines Agency (2019). *Mandate, objectives and composition of the Patients and Consumers Working Party (PCWP)* Retrieved 31 July, 2021, from

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European Medicines Agency (2019). *Mandate, objectives and composition of the Patients and Consumers Working Party (PCWP)* Retrieved 31 July, 2021, from https://www.ema.europa.eu/en/documents/other/mandate-objectives-and-composition-patients-and-consumers-working-party-pcwp_en.pdf[/glossary_exclude]