EMA Committees: Committee for Orphan Medicinal Products (COMP)

Introduction

The European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) reviews applications seeking ‘orphan-medicinal-product designation’ – that is, the medicine is classified as being developed for the diagnosis, prevention, or treatment of rare diseases. The classification of a medicine as an orphan medicinal product comes with specific developmental advantages. The following article contains more information on rare diseases and discusses the regulatory concepts and legislation that guide the COMP’s activities.

EMA Committee for Orphan Medicinal Products (COMP)

Orphan medicines are medicines for rare diseases.

Rare diseases are defined as life-threatening or chronic conditions (long-term conditions that have a significant impact on daily living). They affect no more than 5 in 10,000 people in the EU – which means that there are approximately 30 million people in the EU suffering from a rare disease.

The symptoms (signs) of some rare diseases may appear at birth or in childhood (for instance, spinal muscular atrophy (SMA)) while others may appear during adulthood (for instance, acute myeloid leukaemia (AML)). 80% of rare disease are characterised by degenerative (where the structure or function

of tissues deteriorates) and/or proliferative (where cell production is rapid) mechanisms. The ‘cause’ of rare diseases is generally genetic.

Medical and scientific knowledge of rare diseases is still limited. There continues to be a large number of rare diseases that have still not been sufficiently described.

To stimulate the research and development of treatments for rare diseases, EU legislation (EC 141/2000)[1] has been introduced, including a number of incentives for those companies that choose to develop orphan medicines.

This new legislation has been successful. In the first five years of implementation, 458 applications for orphan designation were submitted.

The main therapeutic areas with orphan medicines were:

- Cancer
- Metabolic disorders (disorders that affect the metabolism, chemical reactions within the body)
- Immunology (disorders of the immune system)
- Cardiovascular disorders (disorders of the heart)
- Respiratory disorders (disorders of the lungs and related structures)

Regulatory concepts and applicable legislation

Since 2000, marketing authorisations for orphan medicines in the EU are required to be submitted through the Centralised Procedure (CP).

An application for orphan designation (which is free of charge) can be done at any stage of the development of the medicine. However, the application for orphan designations must be made before submitting an application for marketing

authorisation to the EMA.

To qualify for orphan designation, a medicine must meet a number of criteria:

- It must be intended for the treatment, prevention, or diagnosis of a disease that is life-threatening or chronically debilitating
- The prevalence of the condition in the EU must not be more than 5 in 10,000 or it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development
- No satisfactory method of diagnosis, prevention, or treatment of the condition concerns or, if such a method exists, the medicine will be of significant benefit to those affected by the condition.

As part of the orphan medicine designation application, some preliminary non-clinical and/or clinical data (proof of concept) are generally required. A request for orphan-medicinal-product designation may be made for an already authorised medicine, if the designation request concerns a new orphan indication that is not currently authorised.

Applications for orphan designation are examined by the EMA COMP using a network of EU experts. The COMP was established in 2000 and was the first committee to include patient representatives as full and equal members of the committee. The patient representatives are nominated by the European Commission. A detailed list of the COMP members is available on the EMA website.

When an orphan designation is obtained for a medicine, the company benefits from a number of incentives, including:

- Protocol assistance (scientific advice specific for designated orphan medicines) at a reduced price
- Market exclusivity for 10 years once the medicine is

granted a marketing authorisation – other companies are not allowed to market a similar medicine for 10 years after approval
- A reduced fee for marketing authorisation applications are also available
- Potential eligibility for grants from EU and Member State programmes and initiatives for research and development.

Sponsors are requested to submit an annual report to the EMA summarising the development status of the medicine.

If the orphan medicine is an advanced therapy, an application to the Committee for Advanced Therapies (CAT) to have the medicine classified as an advanced therapy medicine must also be made.

When the development of a medicine is completed, applications for marketing authorisation are assessed by the Committee for Medicinal Products for Human Use (CHMP). At the time of a positive opinion by the CHMP, if the medicine has an orphan designation, the dossier is returned to the COMP. This enables the committee to determine if the criteria are still valid and if the product maintains its orphan status.

The complete list of orphan medicines adopted in the EU can be found in the Community Register.[2]

The EMA encourages parallel applications for orphan designation with regulatory authorities outside the EU and has special arrangements with regulators in the United States and Japan. A single application has been designed for this purpose.

[glossary_exclude]Further Resources

- European Commission (2015). *Orphan medicinal products*. Retrieved 4 July, 2021 from

https://ec.europa.eu/health/human-use/orphan-medicines

- European Medicines Agency (2013). *EMA/272219/2013 Sixth annual report on the interaction with patients' and consumers' organisations (2012).* Retrieved 3 September, 2015 from http://www.ema.europa.eu/docs/en_GB/document_library/Report/2013/12/WC500158365.pdf[/glossary_exclude]

[glossary_exclude]References

1. European Parliament (2000). *Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products*. Retrieved 3 September, 2015 from http://eur-lex.europa.eu/legal-content/EN/TXT/?qid=1441289843912&uri=CELEX:32000R0141
2. European Commission (2015). *Register of designated Orphan Medicinal Products*. Retrieved 3 September, 2015 from http://eur-lex.europa.eu/legal-content/EN/TXT/?qid=1441289843912&uri=CELEX:32000R0141[/glossary_exclude]

[glossary_exclude]Attachments

- Fact Sheet: Summary of regulatory concepts and legislation, and the role of patient organisations
  Size: 106,232 bytes, Format: .docx
  This factsheet provides an overview of the different regulatory concepts and legislation associated with special medicinal products, and information of the role of patient organisations in these regulatory processes.

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