

Around 36 million people in the EU are likely to suffer from a debilitating rare disease. This represents a huge unmet medical need and a significant public health challenge.



Over 6000 rare diseases

1 out of 12 people in the EU has a rare disease<sup>1</sup>

## When is a disease a rare disease?

A disease is considered rare if fewer than 5 in 10,000 people have it. Very few medicines are available for these rare diseases. One of the reasons is that companies are less likely to recover the development cost for medicines for such small numbers of patients.

## **Development of medicines for rare diseases**

The EU offers incentives to encourage companies to research and develop medicines for rare diseases that otherwise would not be developed. To access these incentives, companies can apply for orphan designation for their medicine, provided certain criteria are met.

### Criteria for orphan designation

The medicine must treat, prevent, or diagnose
a disease which is life-threatening or chronically
debilitating, or it is unlikely that the medicine will

- generate sufficient returns to justify the investment needed for its development
- The disease must not affect more than 5 in 10,000 people across the EU
- No satisfactory method of diagnosis, prevention or treatment exists, or if such a method already exists, the medicine must be of significant additional benefit to those affected by the condition

#### **Incentives**

During an orphan medicine's research and development, the company can benefit from incentives such as scientific advice on study protocols, various fee reductions and access to EU grants.

Orphan-designated medicines that eventually make it to the market are granted 10 years of market exclusivity, provided it can be demonstrated that the criteria for their designation still apply.



**Over 260** orphan medicines authorised in the EU

**Over 3000** medicines with orphan designation

# How orphan medicines reach patients

Once an orphan medicine is authorised by the European Commission, it can be marketed in all EU Member States. However, availability and reimbursement are subject to review by the relevant national authorities.

### **ORPHAN DESIGNATION IS NOT AN AUTHORISATION**

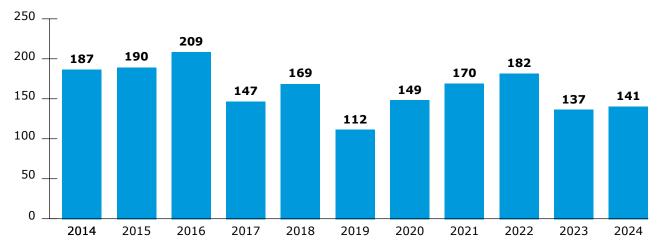
Not all orphan-designated medicines reach the marketing authorisation application stage. Those that do, are evaluated by EMA's Committee for Medicinal Products for Human Use (CHMP) using the same strict safety and efficacy standards that apply to all medicines evaluated by EMA.

### **EMA's Committee for Orphan Medicines**

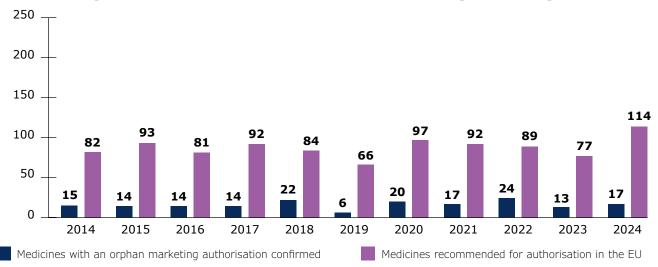
The Committee for Orphan Medicinal Products (COMP) is in charge of reviewing applications for orphan designation.

If a medicine is recommended for marketing authorisation, the COMP will assess it again to check whether the criteria are still met and the orphan designation can be maintained for the authorised medicine.

#### Number of medicines that have received an orphan designation (2014-2024)



#### Number of orphan medicines recommended for authorisation (2014-2024)



#### More information

Luxembourg: Publications Office of the European Union, 2025

ISBN 978-92-9155-138-5 doi:10.2809/1136258 TC-01-25-016-EN-N

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