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, and for which it can be demonstrated that they maintain the criteria for the designation, are granted 10 years of market protection.

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.

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 medicines in the EU
Finding effective treatment for patients with rare diseases can be very difficult. Since the EU orphan regulation entered into force in 2000, it has played a central role in facilitating the development and authorisation of medicines for rare diseases.