Orphan designation

Orphan designation, also known as orphan drug designation, is a status granted to pharmaceutical drugs or therapy that are intended to treat rare diseases or conditions. The designation is provided by regulatory authorities, such as the U.S. Food and Drug Administration (FDA) in the United States and the European Medicines Agency (EMA) in the European Union.

To qualify for orphan designation, a disease or condition must meet specific criteria, which vary between regulatory agencies. Generally, a rare disease is defined as one that affects a small number of people within a given population. In the United States, it is defined as a disease that affects fewer than 200,000 individuals, while in the European Union, it is a condition that affects fewer than 5 in 10,000 individuals.

Obtaining orphan designation for a drug or therapy provides several benefits to the manufacturer or sponsor:

Market Exclusivity: Orphan designation grants the manufacturer or sponsors a period of market exclusivity, during which competing drugs for the same indication may not be approved. This exclusivity period typically ranges from 7 to 10 years in the United States and 10 years in the European Union.

Financial Incentives: Orphan designation may provide financial incentives, such as tax credits, research grants, and reduced regulatory fees, to encourage the development of therapies for rare diseases.

Regulatory Assistance: Regulatory authorities provide guidance and support throughout the drug development process, including accelerated review and access to scientific advice.

Orphan designation aims to incentivize the development of drugs for rare diseases by providing regulatory and financial advantages to manufacturers or sponsors. This encourages investment in research and development efforts for diseases that would otherwise be commercially unattractive due to the limited patient population.