

# US Orphan Drug Clinical Pipeline Insight 2014

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## Abstracts

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Rare diseases are also known as Orphan diseases. These diseases are generally categorized as chronic, degenerative, and life-threatening in nature. The rare diseases normally include certain cancers, metabolic conditions, diseases of the nervous system and musculoskeletal disorders, which are extremely life-threatening. As per the official definition, rare diseases are classified as those that affect fewer than 200,000 in the US and less than 5 in 10,000 in the EU.

The orphan drugs enjoy significant competitive advantages owing to the market exclusivity period after the drug has been authorized. Through this clause, the manufacturer of orphan drugs is given a monopoly status in the market because according to the law, no other company is allowed to market the orphan drugs during the exclusivity period. Additionally, this monopolistic power is further strengthened with the fact that no other alternative health technology exists for many orphan drugs.

In US, the research & development of orphan drugs is facilitated by public programs, government regulations, and policies. The major incentives for research & development of orphan drug include grants, research support, fee waivers, tax incentives, and orphan drug market exclusivity. In a highly specialized market like that of orphan drugs, though the impact of market exclusivity in the US has been positive yet relatively modest, a special platform for continued orphan drug development has been laid down by the economic incentives and public support mechanisms. In 2013, the US market for orphan drugs was estimated worth US\$ 49 Billion. With factors like government support, high profitability and many more, this market is expected to surpass US\$ 60 Billion by 2018.

“US Orphan Drug Pipeline Insight 2014” Report Highlight:

Reimbursement Policy & FDA Regulatory Framework

US Orphan Drug Pipeline by Phase, Indication & Company

Marketed Orphan Drug Clinical Insight

US Orphan Drug Clinical Pipeline: 570 Drugs

Marketed Orphan Drugs in US: 203 Drugs

Majority Orphan Drugs in PHASE-II Development: 207 Drugs

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