

Drug Development in Rare Diseases: Focus on Clinical Trials and the Regulatory Landscape

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Abstracts

Summary:

The orphan drug industry is gaining importance as a significant and rewarding market in the pharmaceutical and biotech sectors. Technological and scientific innovations in specific drug discovery pathways as well as major developments in genetics are cultivating growth in this industry. BCC Research's objective in conducting this report is to provide detailed information on drug development in rare diseases. This report provides a comprehensive analysis and examines the future direction of these drugs as an important means for the treatment of orphan and rare diseases.

Report Scope:

This report analyzes the regulatory framework for the clinical trials of orphan drugs in rare and orphan diseases. The report also analyzes the trends and characteristics of clinical trials conducted on rare diseases from 1999-2018, using information from various sources. This report covers an overview of the global orphan drugs market and its competitive landscape.

Report Includes:

An overview of the drug development in rare diseases with special focus on clinical trial and regulatory landscape

Detailed description of different kinds of orphan drugs and a comparative assessment on biological and chemical or non-biological orphan drugs

Discussion on Orphan Drug Act; insights into general framework for the regulation of drugs and biologics, and regulations for clinical trials of orphan designated drugs

H-ighlights of technological advances and innovations in genetic manufacturing technologies and information on terms such as generic competition, patent expirations, premium pricing and information on orphan drug designation applications

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ASTRAZENECA PLC.
BRISTOL-MYERS SQUIBB
EISAI CO. LTD.
ELI LILLY AND CO.
F. HOFFMANN-LA ROCHE LTD.
GILEAD SCIENCES INC.
JOHNSON & JOHNSON
MERCK & CO.
NOVARTIS AG
PFIZER INC.
SANOFI-AVENTIS
TAKEDA PHARMACEUTICALS LTD.
TEVA PHARMACEUTICAL INDUSTRIES LTD.

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COMPANIES MENTIONED

ABBVIE INC.

ASTELLAS PHARMA INC.

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