

The World Market for Orphan Drugs

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Abstracts

In recent years, there has been a substantial increase in the number of drugs available to treat rare ("orphan") diseases. This comprehensive report, *The World Market for Orphan Drugs*, looks at this trend and provides estimates for the market opportunity in the U.S., Europe, Japan and globally. With over 400 pages of market analysis and over 120 figures and tables, the report provides in-depth coverage of key competitors and important trends and challenges for makers of orphan drug treatments.

Osteoporosis, arthritis, Alzheimer's, and Parkinson's are major factors affecting quality of life. Mortality from cancer, diabetes, liver and kidney diseases has been slow to change, offering significant areas for research breakthroughs. Cancer, poorly served by traditional chemotherapies, is a major opportunity for biotech firms because the investment compared to return can be favorable: the field has high priority with regulatory authorities who are willing to give it fast track status on the basis of smaller (and therefore cheaper) clinical trials (a few extra months of survival could be enough to win FDA approval); the clinical community is highly concentrated; and the market size is often larger than the approved indication because of a high off-label use (for other cancers). Infectious diseases, the third most common cause of death in many geographic regions, highlight the pressing need for new therapies with novel mechanisms of action to avoid growing issues affecting this industry including a top area of concern—drug resistance.

This report provides estimates for the world market for orphan drugs, as well as the E.U. and Japan markets. Given the size of the U.S. opportunity, the report offers in-depth market data for the U.S. market. Several segments are discussed, including:

Oncology

Hematology

Musculoskeletal

Neurology

Endocrine

Metabolic

Others

These segments are the most common areas of development and success for orphan drug developers/marketers which required providing detailed information about these orphan drug market segments.

In the U.S., a drug may be designated an orphan drug if the disease or condition for which it is indicated, affects less than 200,000 Americans. Orphan medicinal products in the E.U. member states are intended for the diagnosis, prevention or treatment of life-threatening or very serious conditions that affect not more than 5 in 10,000 persons. In Japan an orphan drug may be designated for a patient population of less than 50,000 and provide for an unmet medical need. Other countries have similar designations. These three regional markets for drugs are examined in separate sections of the report.

Analysis of The “Pure” Orphan Drug Market

Some publicized estimates of orphan drug markets include brand revenue that is not truly related to the orphan status of the drug. In this unique research effort, Kalorama Information separates strictly orphan revenue from total brand revenue where necessary to provide a true picture of the pure orphan drug market opportunity. The world orphan drug market is difficult to determine due to the variances in orphan drug status and requirements by country. Products may be available as an orphan drug on one country and be a traditional marketing approval for another country. In addition, some products gain orphan status, are granted approval and then removed from the orphan drug database at the request of the developer. There are a number of factors involved, including reimbursement by country, regulation by country, disease status by country and general development by country.

For this market research report, market numbers discussed in this report are focused on

the global market in brief with in-depth market coverage of the U.S. market. Additionally, there is select coverage for the European Union and Japanese market. All sales are displayed at the manufacturers' level in U.S. dollars.

This report includes orphan drugs. In cases where sales are generated by indications for both orphan indications and non-orphan indications, we have attempted to extract just the orphan portion of sales. The base year is 2012, with forecasts provided for each year through 2017. The market was generated using estimates of individual product sales and compared with market data from a combination of other methods including disease prevalence trends, population trends, pricing trends, government regulations and reimbursements. Both primary and secondary sources were consulted in developing market estimates.

Demographics, Costs, Regulation and Other Trends Examined

The list of trends and factors affecting the orphan drug market remain lengthy. The orphan drug market is affected by both trends that affect the pharmaceutical industry as a whole and trends that specifically affect the orphan drug market:

- Orphan drug pricing
- Aging of the population
- Increasing life expectancy
- Trends in birth rates
- Cost effectiveness
- Regulatory environments
- Orphan drug incentives
- Population demographics
- Product availability by country/region

Due to the wide variety of products to receive orphan designations, there are numerous

developers with products in this area. Some of the developers that have received an orphan designation have followed through to receive a full product approval but others may not gain additional approvals.

Contents

CHAPTER ONE: EXECUTIVE SUMMARY

- Introduction
- Scope and Methodology
 - Market Segments
- Issues and Trends
- World Market Summary
- Competitors

CHAPTER TWO: INTRODUCTION

- Overview
- History of the U.S. Orphan Drug Market
- Pharmaceutical Research & Development Overview
- Pharmaceutical Industry Important Regulatory Terms
- Diseases and Orphan Drug Impact
- Disease Descriptions
 - Blood Disorders
 - Cancer
 - Hormone Disorders
 - Infectious Diseases
- The Cost Side of Orphan Drugs

CHAPTER THREE: U.S. ORPHAN DRUGS

- Overview
- Recent Orphan Drug Designations
- Orphan Drug Approvals by Company
 - Abbott Laboratories
 - Acorda Therapeutics
 - Actelion Pharmaceuticals
 - Aegerion Pharmaceuticals
 - Alexion Pharmaceuticals
 - Allergan, Inc.
 - Amgen
 - Ariad Pharmaceuticals
 - Astellas

AstraZeneca
Auxilium Pharmaceuticals, Inc.
Bausch & Lomb
Baxter International
Bayer AG
Biogen IDEC
BioMarin Pharmaceutical, Inc.
Boehringer Ingelheim
Bristol-Myers Squibb
Cangene Corporation
Celgene Corporation
CSL Behring
Eisai Co., Ltd.
Eli Lilly & Company
EMD Serono
Endo Health Solutions, Inc.
(previously Endo Pharmaceuticals, Inc.)
Exelixis, Inc.
Gilead Sciences, Inc.
GlaxoSmithKline
Grifols, S.A.
Hyperion Therapeutics, Inc.
Incyte Corporation
Ipsen, Inc.
Jazz Pharmaceuticals plc
Johnson & Johnson
KV Pharmaceutical Company
Merck & Company
Novartis
Novo Nordisk
NPS Pharmaceuticals
Octapharma USA, Inc.
Onyx Pharmaceuticals, Inc.
Otsuka Pharmaceutical Co., Inc.
Pacira Pharmaceuticals, Inc.
Pfizer, Inc.
Rare Disease Therapeutics, Inc.
Reckitt Benckiser Group plc
Regeneron Pharmaceuticals, Inc.

Roche
Salix Pharmaceuticals, Inc.
Sanofi
Savient Pharmaceuticals, Inc.
Seattle Genetics, Inc.
Shire Plc
Sigma-Tau Pharmaceuticals, Inc.
Spectrum Pharmaceuticals
Takeda Pharmaceutical Company Ltd.
Talon Therapeutics, Inc.
Teva Pharmaceuticals
United Therapeutics Corporation
Valeant Pharmaceuticals International, Inc.
Vertex Pharmaceuticals, Inc.
ViroPharma, Inc.

CHAPTER FOUR: E.U. ORPHAN DRUGS

Overview
Recent Orphan Drug Designations
Select Company Orphan Drug Activities
Actelion Pharmaceuticals
Adienne Pharma & Biotech
Alexion Pharmaceuticals
Amgen
Bayer AG
BioMarin Pharmaceutical, Inc.
Bristol-Myers Squibb
Celgene Corporation
Eisai Co., Ltd.
Gilead Sciences, Inc.
GlaxoSmithKline
Novartis
Orphan Europe S.A.R.L.
Pfizer, Inc.
Sanofi
Shire Plc
Vertex Pharmaceuticals, Inc.

CHAPTER FIVE: JAPAN ORPHAN DRUGS

Overview

Recent Orphan Drug Designations

Select Company Orphan Drug Activities

Actelion Pharmaceuticals

Alexion Pharmaceuticals

Bristol-Myers Squibb

Celgene Corporation

GlaxoSmithKline

Johnson & Johnson

Merck Serono

Mitsubishi Tanabe

Novartis

Pfizer, Inc.

Roche

Sanofi

CHAPTER SIX: COMPETITOR TRENDS AND PRACTICES

Acquisitions and Agreements

U.S. Competitor Market Analysis

Amgen

Roche

Celgene

Novartis

Abbott

Biogen Idec

Bayer

Eli Lilly

Bristol-Myers Squibb

Novo Nordisk

Sanofi

Allergan

Other Companies

CHAPTER SEVEN: ORPHAN DRUG INDUSTRY TRENDS

Introduction

- U.S. Regulation Shaping the Orphan Drug Segment
 - Orphan Drug Act of 1983 Rare Disease Act of 2002
- Office of Orphan Products Development
- Humanitarian Use Device Program
- Positives and Negatives of the Orphan Drug Designation
 - Orphan Drug Designation in the U.S.
 - Orphan Drug Designation in the E.U.
 - Orphan Drug Designation in Japan
 - Orphan Drug Designation in Canada
 - Orphan Drug Designation in Australia
- Factors Affecting the Health Industry
 - Aging of the Population
- Population Statistics
 - Global Population Trends
 - Global Population over 65 U.S. Population Trends
 - U.S. Population over 65 Global Trends in Life Expectancy
 - Global Trends in Birth Rates
- Global Healthcare Spending Trends
 - General Economic Trends by Country
- U.S. Health Industry Trends
 - U.S. Healthcare Challenges
 - Economic Trends in the U.S.
 - U.S. Health Expenditures by Type

CHAPTER EIGHT: U.S. ORPHAN DRUG MARKET SIZE AND OPPORTUNITY

- Overview
- Market Size and Forecast
- Markets by Product Classification
 - Oncology
 - Suppliers Market Share
 - Hematology
 - Suppliers Market Share
 - Musculoskeletal
 - Suppliers Market Share
 - Neurology
 - Suppliers Market Share
 - Endocrinology
 - Suppliers Market Share

Metabolic
Suppliers Market Share
Others
Suppliers Market Share

CHAPTER NINE: WORLD MARKET SUMMARY? ORPHAN DRUGS

Overview
Market Distribution: United States and World Markets

CHAPTER TEN: CORPORATE PROFILES

Introduction
Orphan Drug Designations by Company: A U.S. Analysis
Company Profiles
Abbott Laboratories
Company Overview
Performance Review
AbbVie
Company Overview
Performance Review
Actelion
Company Overview
Performance Review
Overview
Performance Review
Astellas
Company Overview
Performance Review
AstraZeneca
Company Overview
Performance Review
Baxter
Company Overview
Performance Review
Bayer
Company Overview
Performance Review
Bio Products Laboratory

Company Overview
Biogen Idec
Company Overview
Performance Review
BioMarin
Company Overview
Performance Review
Boehringer Ingelheim
Company Overview
Performance Review
Bristol-Myers Squibb
Company Overview
Performance Review
Cangene
Company Overview
Performance Review
Celgene
Company Overview
Performance Review
CSL Behring
Company Overview
Performance Review
Depomed
Company Overview
Performance Review
Eisai
Company Overview
Performance Review
Eli Lilly
Company Overview
Performance Review
EMD Serono
Company Overview
Performance Review
Exelixis
Company Overview
Performance Review
Gilead Sciences
Company Overview

Performance Review
GlaxoSmithKline
Company Overview
Performance Review
ImmunoGen
Company Overview
Performance Review
Insmed
Company Overview
Performance Review
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Performance Review
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Performance Review
Merck & Co
Company Overview
Performance Review
Millennium Pharmaceuticals
Company Overview
Performance Review
NBI Pharmaceuticals
Company Overview
Novartis
Company Overview
Performance Review
Novo Nordisk
Company Overview
Performance Review
NPS Pharmaceuticals
Company Overview
Performance Review
Pfizer
Company Overview
Performance Review
Roche
Company Overview
Performance Review

Sanofi

Company Overview
Performance Review

Seattle Genetics

Company Overview
Performance Review

Shire

Company Overview
Performance Review

Sigma Tau

Company Overview
Performance Review

Soligenix

Company Overview
Performance Review

Spectrum

Company Overview
Performance Review

Swedish Orphan Biovitrum

Company Overview
Performance Review

TEVA

Company Overview
Performance Review

United Therapeutics

Company Overview
Performance Review

Valeant

Company Overview
Performance Review

XenoPort

Company Overview
Performance Review

REGULATORY AUTHORITIES AND ORGANIZATIONS**LIST OF COMPANIES**

List Of Exhibits

LIST OF EXHIBITS

CHAPTER ONE: EXECUTIVE SUMMARY

Table 1-1 World Orphan Drug Market 2007-2017

Figure 1-1: World Orphan Drug Market

CHAPTER TWO: INTRODUCTION

Table 2-1 Phases of Clinical Drug Development

Table 2-2 Total Cancer Incidence by Sex and Country

Figure 2-1 Total Cancer Incidence by Sex and Country

Table 2-3 Average Annual Drug Costs to Patients, Selected Drugs

Figure 2-2 Average Annual Drug Costs to Patients, Selected Drugs

CHAPTER THREE: U.S. ORPHAN DRUGS

Table 3-1 Orphan Drug Designations Granted and Approvals by the FDA 1983-2013*

Figure 3-1 Orphan Drug Designations Granted and Approvals by the FDA 1983-2013*

Table 3-2 12-Months Orphan Drug Designations Granted by FDA (May 1, 2012 - May 1, 2013)

Figure 3-2 May 2012 - May 2013 FDA Orphan Drug Designations by Target Classification

Table 3-3 Abbott Laboratories' Orphan Drug Approvals Granted by FDA

Table 3-4 Acorda Therapeutics' Orphan Drug Approvals Granted by FDA

Table 3-5 Actelion Pharmaceuticals' Orphan Drug Approvals Granted by FDA

Table 3-6 Aegerion Pharmaceuticals' Orphan Drug Approvals Granted by FDA

Table 3-7 Alexion Pharmaceuticals' Orphan Drug Approvals Granted by FDA

Table 3-8 Allergan's Orphan Drug Approvals Granted by FDA

Table 3-9 Amgen's Orphan Drug Approvals Granted by FDA

Table 3-9 (continued)

Amgen's Orphan Drug Approvals Granted by FDA

Table 3-10 Ariad Pharmaceuticals' Orphan Drug Approvals Granted by FDA

Table 3-11 Astellas' Orphan Drug Approvals Granted by FDA

Table 3-12 AstraZeneca's Orphan Drug Approvals Granted by FDA

Table 3-13 MedImmune's Orphan Drug Approvals Granted by FDA

Table 3-14 Auxilium Pharmaceuticals' Orphan Drug Approvals Granted by FDA

- Table 3-15 Bausch & Lomb's Orphan Drug Approvals Granted by FDA
- Table 3-16 Baxter International's Orphan Drug Approvals Granted by FDA
- Table 3-17 Bayer's Orphan Drug Approvals Granted by FDA
- Table 3-18 Biogen IDEC's Orphan Drug Approvals Granted by FDA
- Table 3-19 BioMarin Pharmaceutical's Orphan Drug Approvals Granted by FDA
- Table 3-20 Boehringer Ingelheim's Orphan Drug Approvals Granted by FDA
- Table 3-21 Bristol-Myers's Orphan Drug Approvals Granted by FDA
- Table 3-22 Cangene's Orphan Drug Approvals Granted by FDA
- Table 3-23 Celgene Corporation's Orphan Drug Approvals Granted by FDA
- Table 3-24 CSL Behring's Orphan Drug Approvals Granted by FDA
- Table 3-25 Eisai's Orphan Drug Approvals Granted by FDA
- Table 3-26 Eli Lilly's Orphan Drug Approvals Granted by FDA
- Table 3-27 EMD Serono's Orphan Drug Approvals Granted by FDA
- Table 3-28 Endo Health Solutions' Orphan Drug Approvals Granted by FDA
- Table 3-29 Exelixis' Orphan Drug Approvals Granted by FDA
- Table 3-30 Gilead Sciences' Orphan Drug Approvals Granted by FDA
- Table 3-31 GlaxoSmithKline's Orphan Drug Approvals Granted by FDA
- Table 3-32 Grifols' Orphan Drug Approvals Granted by FDA
- Table 3-33 Hyperion Therapeutics' Orphan Drug Approvals Granted by FDA
- Table 3-34 Incyte Corporation's Orphan Drug Approvals Granted by FDA
- Table 3-35 Ipsen's Orphan Drug Approvals Granted by FDA
- Table 3-36 Jazz Pharmaceuticals' Orphan Drug Approvals Granted by FDA
- Table 3-37 Johnson & Johnson Pharmaceutical's Orphan Drug Approvals Granted by FDA
- Table 3-38 Alza's Orphan Drug Approvals Granted by FDA
- Table 3-39 Janssen's Orphan Drug Approvals Granted by FDA
- Table 3-40 KV Pharmaceutical's Orphan Drug Approvals Granted by FDA
- Table 3-41 Merck & Co.'s Orphan Drug Approvals Granted by FDA
- Table 3-41 (continued)
- Merck & Co.'s Orphan Drug Approvals Granted by FDA
- Table 3-42 Novartis' Orphan Drug Approvals Granted by FDA
- Table 3-43 Alcon's Orphan Drug Approvals Granted by FDA
- Table 3-44 Novo Nordisk's Orphan Drug Approvals Granted by FDA
- Table 3-45 NPS Pharmaceuticals' Orphan Drug Approvals Granted by FDA
- Table 3-46 Octapharma's Orphan Drug Approvals Granted by FDA
- Table 3-47 Onyx Pharmaceuticals' Orphan Drug Approvals Granted by FDA
- Table 3-48 Otsuka Pharmaceutical's Orphan Drug Approvals Granted by FDA
- Table 3-49 Pacira Pharmaceutical's Orphan Drug Approvals Granted by FDA
- Table 3-49 (continued) Pfizer's Orphan Drug Approvals Granted by FDA

Table 3-49 (continued) Pfizer's Orphan Drug Approvals Granted by FDA
Table 3-50 Rare Disease Therapeutics' Orphan Drug Approvals Granted by FDA
Table 3-51 Reckitt Benckiser's Orphan Drug Approvals Granted by FDA
Table 3-52 Regeneron Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-53 Roche's Orphan Drug Approvals Granted by FDA
Table 3-54 Genentech's Orphan Drug Approvals Granted by FDA
Table 3-55 Salix Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-56 Sanofi's Orphan Drug Approvals Granted by FDA
Table 3-57 Genzyme's Orphan Drug Approvals Granted by FDA
Table 3-58 Savient Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-59 Seattle Genetics' Orphan Drug Approvals Granted by FDA
Table 3-60 Shire's Orphan Drug Approvals Granted by FDA
Table 3-61 Sigma Tau's Orphan Drug Approvals Granted by FDA
Table 3-62 Spectrum Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-63 Millennium Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-64 Talon Therapeutics' Orphan Drug Approvals Granted by FDA
Table 3-65 Teva's Orphan Drug Approvals Granted by FDA
Table 3-66 Cephalon's Orphan Drug Approvals Granted by FDA
Table 3-67 United Therapeutics' Orphan Drug Approvals Granted by FDA
Table 3-68 Valeant Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-69 Vertex Pharmaceuticals' Orphan Drug Approvals Granted by FDA
Table 3-70 ViroPharma's Orphan Drug Approvals Granted by FDA

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