

# US Orphan Drug Clinical Pipeline Insight 2014

<https://marketpublishers.com/r/UC5DC01FBB5EN.html>

Date: August 2014

Pages: 2000

Price: US\$ 2,000.00 (Single User License)

ID: UC5DC01FBB5EN

## Abstracts

**Please note:** extra shipping charges are applied when purchasing Hard Copy License depending on the location.

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

## Contents

### **1. INTRODUCTION TO US ORPHAN MARKET**

- 1.1 Market Overview
- 1.2 Clinical Pipeline Overview

### **2. US ORPHAN DRUG REGULATORY FRAMEWORK**

- 2.1 Criteria for Orphan Drug Designation
- 2.2 Reimbursement Policy for Orphan Drugs
- 2.3 FDA Regulation
  - 2.3.1 Content & Format for Written Recommendations
  - 2.3.2 Provision for Granting & Refusing Written Recommendations
  - 2.3.3 Content & Format for Orphan Drug Designation
  - 2.3.4 Verification of Orphan Drug Status & Resident Agent for Foreign Sponsor
  - 2.3.5 Requests for Orphan Drug Designation & Designation of Already Approved Drugs
  - 2.3.6 Deficiency Letters & Granting Orphan Drug Designation
  - 2.3.7 Refusal of Grant Orphan Drug Designation
  - 2.3.8 Amendment & Change in Ownership to Orphan Drug Designation
  - 2.3.9 Publication & Revocation of Orphan Drug Designations
  - 2.3.10 Annual Reports of Holder of Orphan Drug Designation
  - 2.3.11 Scope & FDA Recognition of Orphan Drug Exclusive Approval
  - 2.3.12 Protocols for Investigations & Availability of Information

### **3. US ORPHAN DRUG CLINICAL PIPELINE BY PHASE, INDICATION & COMPANY**

- 3.1 Unknown
- 3.2 Research
- 3.3 Preclinical
- 3.4 Clinical
  - 3.5 Phase-I
  - 3.6 Phase-I/II
  - 3.7 Phase-II
  - 3.8 Phase-II/III
  - 3.9 Phase-III
  - 3.10 Preregistration
  - 3.11 Registered

#### **4. MARKETED ORPHAN DRUGS CLINICAL INSIGHT BY INDICATION & COMPANY**

#### **5. SUSPENDED & DISCONTINUED ORPHAN DRUGS IN CLINICAL PIPELINE BY PHASE, INDICATION & COMPANY**

5.1 No development reported

5.2 Discontinued

5.3 Market Withdrawal

5.4 Suspended

#### **6. COMPETITIVE LANDSCAPE**

6.1 Alexion

6.2 Biogen Idec

6.3 Bristol Myers Squibb

6.4 Celgene

6.5 Eli Lilly

6.6 Glaxosmithkline

6.7 Merck

6.8 Novartis Pharmaceuticals

6.9 Pfizer

6.10 Roche

6.11 Rare Disease Therapeutics

6.12 Sanofi

## List Of Figures

### LIST OF FIGURES

Figure 1-1: US Orphan Drug Market (US\$ Billion), 2013-2018

Figure 1-2: US Share in Global Orphan Drug Market, 2013 & 2018

Figure 1-3: Biological & Non Biological Orphan Drug Segment (%), 2013 & 2018

Figure 1-4: Biological & Non Biological Orphan Drug Market (US\$ Billion), 2013-2018

Figure 1-5: US - Orphan Drug Pipeline by Phase (%)

Figure 1-6: US - Orphan Drug Pipeline by Phase (Number)

Figure 1-7: US - No Development Reported in Orphan Drug Pipeline by Phase (%)

Figure 1-8: US - No Development Reported in Orphan Drug Pipeline by Phase  
(Number)

Figure 1-9: US – Discontinued Orphan Drug in Pipeline by Phase (%)

Figure 1-10: US – Discontinued Orphan Drug in Pipeline by Phase (Number)

Figure 1-11: US – Suspended Orphan Drug in Pipeline by Phase (%)

Figure 1-12: US – Suspended Orphan Drug in Pipeline by Phase (Number)

## I would like to order

Product name: US Orphan Drug Clinical Pipeline Insight 2014

Product link: <https://marketpublishers.com/r/UC5DC01FBB5EN.html>

Price: US\$ 2,000.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/UC5DC01FBB5EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970