

Global Orphan Drug Clinical Trials, Patent & Guidelines Insight 2026

<https://marketpublishers.com/r/G1B9FE3023F2EN.html>

Date: March 2020

Pages: 2400

Price: US\$ 7,000.00 (Single User License)

ID: G1B9FE3023F2EN

Abstracts

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

'Global Orphan Drug Clinical Trials, Patent & Guidelines Insight 2026' Report Highlights:

Global Orphan Drug Market Opportunity: US\$ 300 Billion

US Dominates Global Orphan Drug Market: 50% Market Share

US Orphan Drug Opportunity To Surpass: US\$ 150 Billion

Global Orphan Drug Clinical Insight: More Than 900 Drugs

Clinical Insight on Marketed Orphan Drugs: More Than 400 Drugs

Oncology To Dominate Orphan Drug Development: 35% Share

FDA & EMA Regulations For Orphan Drugs

Orphan Drug Designation Criteria & Reimbursement Policy by Country

The research report “Global Orphan Drug Clinical Trials, Patent & Guidelines Insight 2026” discusses about the recent trends and opportunities that the orphan drug market has brought into the pharmaceutical sector. The information related to the current status of the evolving market strategies and ongoing clinical studies by the companies involved in development of the orphan drugs is elaborately discussed in the report. The research

report shares the information related to drugs that have been successfully designated as orphan drugs by respective approval authorities, with an exclusive insight on clinical uniqueness and patent information. In addition to the commercial information, the report brings a deep insight about the efforts that have been put to establish the market as it is now.

' Global Orphan Drug Market Is Estimated To Witness 150% Market Growth By 2026 As Compare To 2018'

Orphan drugs market has been recently recognized as a promising therapeutic market as the diseases that are covered under the market are life-threatening diseases. The FDA & EMA have designated a drug as orphan drug for which the cases in the US are less than 0.2 Million and not more than 5 in 10,000 people across the EU. Earlier the orphan drug segment was overlooked by the big pharmaceutical companies as developing and marketing of these drugs was considered not so profitable. Majority of the research and development activities related to orphan drugs were done by small size pharmaceutical firms and less than 25% of the orphan drugs were being researched and developed by the big firms.

The enactment of 1983 US Orphan Drug Act, as well as similar Acts in 1991 in Singapore, 1993 in Japan, 1997 in Australia and in 2000 by the European Union led to rapid transformation of global orphan drug market landscape which was earlier neglected by the multiple stake holders of the pharmaceutical industry. The structured regulatory and policy framework favoring the research and development of orphan designated drugs resulted in the much needed thrust for the development of global orphan drug market. These laws allowed the various financial incentives, market exclusivity, patent protection, high price allotment and government grants, which resulted in favorable economic environment for the entry of big pharmaceutical companies in the orphan drug segment.

The entry of mid and large size pharmaceutical companies helped in the speeding up the clinical research activities related to orphan drugs. The number of clinical trials increased drastically in last 10 years to more than 500 for orphan drugs as compared to few hundred trials in beginning of 21st century. Currently, more than 400 orphan designated drugs are commercially available in the marketed and close to 1000 drugs are undergoing clinical trials. The number of clinical trials covering the rare diseases has been observed to increase in the recent years with a major participation of the players such as Roche, Celgene, AbbVie, Johnson & Johnson, Shire, Alexion, Novo Nordisk, Sanofi and Bayer. The various major key players and the rising demand of the

orphan drugs clearly depicts about the escalation that the market will experience in the future.

The availability of large number of orphan drugs by limited firms provides an excellent fundamental benefit to the emerged market in the present as well as in future. The dynamic interest scenario that has been delivered by the users since its arrival has completely changed the landscape of the market. The drastic change from few users in the past to millions of users till now has been successful in proving the importance of orphan drugs in the market. It is well witnessed from analyzing the market value of orphan drugs that clinicians as well as rare disease patients are now more inclined towards its use, thereby, promoting a form of treatment that is more mainstream.

As per report findings, the orphan drug market is open to serve the globe with an approach that is about to bring a fresh new era for the life threatening diseases. The ongoing clinical research at preclinical and clinical levels and the major trends followed by the regions such as North America and Europe are about to introduce a drastic change in overall scenario of the approach. The market is driven by the anticipation of the players and the huge commercial success that the market has foreseen in few years. The enhancement and the shape that the market has developed since years is about to get evolved as a serious option for rare diseases.

Contents

1. WHAT ARE ORPHAN DRUGS?

2. GLOBAL ORPHAN DRUG DESIGNATION CRITERIA

2.1 US

2.2 Europe

2.3 Asia & Australia

2.3.1 Japan

2.3.2 Taiwan

2.3.3 South Korea

2.3.4 Australia

3. MARKET EXCLUSIVITY & PATENT PROTECTION FOR ORPHAN DRUGS

4. GLOBAL ORPHAN DRUG REIMBURSEMENT POLICY

4.1 US

4.2 Europe

4.3 Asia

5. GLOBAL ORPHAN DRUG MARKET OPPORTUNITY INSIGHT 2026

5.1 Global Orphan Drug Sales Opportunity

5.2 Market by Class Variation

5.3 Market by Therapeutic Application

5.4 Market by Regions

6. FDA REGULATION FOR CLINICAL TRIALS ORPHAN DESIGNATED DRUGS

6.1 Content & Format Of A Request For Written Recommendations

6.2 Provision For Granting & Refusing Written Recommendations

6.3 Content And Format Of A Request For Orphan Drug Designation

6.4 Verification Of Orphan Drug Status & Resident Agent For Foreign Sponsor

6.5 Timing Of Requests For Orphan Drug Designation & Designation Of Already Approved Drugs

6.6 Deficiency Letters And Granting Orphan Drug Designation

6.7 Refusal To Grant Orphan Drug Designation

- 6.8 Amendment & Change In Ownership To Orphan Drug Designation
- 6.9 Publication & Revocation Of Orphan Drug Designations
- 6.10 Annual Reports Of Holder Of Orphan Drug Designation
- 6.11 Scope & FDA Recognition Of Orphan Drug Exclusive Approval
- 6.12 Protocols for Investigations & Availability of Information

7. EMA REGULATIONS FOR CLINICAL TRIALS OF ORPHAN DESIGNATED DRUGS

- 7.1 Committee for Orphan Medicinal Products
- 7.2 How to Apply for Orphan Designation in Europe
- 7.3 Marketing Authorization & Market Exclusivity
- 7.4 Transferring An Orphan Designation To Another Sponsor
- 7.5 Mandatory Submission Of Annual Report On Development
- 7.6 Incentives For Micro, Small And Medium-Sized Enterprises
- 7.7 Fee Reductions For Designated Orphan Medicinal Products
- 7.8 Procedure for Orphan Designation & Incentives for R&D (Regulation (EC) No 141/2000)

8. ASIAN REGULATIONS FOR CLINICAL TRIALS OF ORPHAN DESIGNATED DRUGS

- 8.1 Taiwan Rare Disease and Orphan Drug Act
- 8.2 Japan Orphan Drug Regulation

9. GLOBAL ORPHAN CLINICAL PIPELINE OVERVIEW

- 9.1 By Company
- 9.2 Drug Class
- 9.3 Formulation
- 9.4 Indication
- 9.5 Region
- 9.6 Priority Status
- 9.7 Patient Segment
- 9.8 By Phase

10. UNKNOWN PHASE - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

10.1 Overview

10.2 Clinical Pipeline Insight

11. RESEARCH PHASE - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

11.1 Overview

11.2 Clinical Pipeline Insight

12. PRECLINICAL PHASE - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

12.1 Overview

12.2 Clinical Pipeline Insight

13. CLINICAL PHASE - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

13.1 Overview

13.2 Clinical Pipeline Insight

14. PHASE-I - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

14.1 Overview

14.2 Clinical Pipeline Insight

15. PHASE-I/II - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

15.1 Overview

15.2 Clinical Pipeline Insight

16. PHASE-II - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

16.1 Overview

16.2 Clinical Pipeline Insight

17. PHASE-II/III - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

17.1 Overview

17.2 Clinical Pipeline Insight

18. PHASE-III - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

18.1 Overview

18.2 Clinical Pipeline Insight

19. PREREGISTRATION - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

19.1 Overview

19.2 Clinical Pipeline Insight

20. REGISTERED - ORPHAN DRUGS CLINICAL PIPELINE BY COMPANY, COUNTRY & INDICATION

20.1 Overview

20.2 Clinical Pipeline Insight

21. MARKETED ORPHAN DRUGS CLINICAL INSIGHT BY COMPANY, COUNTRY & INDICATION

21.1 Overview

21.2 Clinical Pipeline Insight

22. COMPETITIVE LANDSCAPE

22.1 AOP Orphan

22.2 Agenus

22.3 Alexion

22.4 Bristol Myers Squibb

22.5 Biogen Idec

22.6 Celgene

22.7 Eli Lilly

- 22.8 Genethon
- 22.9 Genzyme Corporation
- 22.10 Glaxosmithkline
- 22.11 Merck
- 22.12 Novartis Pharmaceuticals
- 22.13 Orphan Europe
- 22.14 Pfizer
- 22.15 Prosensa
- 22.16 Rare Disease Therapeutics
- 22.17 Roche
- 22.18 Sanofi
- 22.19 Shire
- 22.20 Teva Pharmaceutical

List Of Figures

LIST OF FIGURES

- Figure 5-1: Global - Orphan Drugs Market Value (US\$ Billion), 2018-2026
- Figure 5-2: US - Orphan Drugs Market Value (US\$ Billion), 2018-2026
- Figure 5-3: Europe - Orphan Drugs Market Value (US\$ Billion), 2018-2026
- Figure 5-4: Asia* - Orphan Drugs Market Value (US\$ Billion), 2018-2026
- Figure 5-5: Biological & Non Biological Orphan Drug Segment (%), 2018 & 2026
- Figure 5-6: Biological & Non Biological Orphan Drug Market (US\$ Billion), 2018-2026
- Figure 5-7: Global Orphan Drugs Market by Therapeutic Area, 2018 & 2026
- Figure 5-8: Regional Markets for Orphan Drugs, 2018 & 2026
- Figure 8-1: Japan Orphan Drug/Medical Device Designation System
- Figure 8-2: Japan Orphan Drug/Device Designation Process
- Figure 9-1: Global - Orphan Drugs Clinical Pipeline by Company (Number), 2020 till 2026
- Figure 9-2: Global - Orphan Drugs Clinical Pipeline by Drug Class (Number), 2020 till 2026
- Figure 9-3: Global - Orphan Drugs Clinical Pipeline by Formulation (Number), 2020 till 2026
- Figure 9-4: Global - Orphan Drugs Clinical Pipeline by Indication (Number), 2020 till 2026
- Figure 9-5: Global - Orphan Drugs Clinical Pipeline by Region (Number), 2020 till 2026
- Figure 9-6: Global - Orphan Drugs Clinical Pipeline by Priority Status (Number), 2020 till 2026
- Figure 9-7: Global - Orphan Drugs Clinical Pipeline by Patient Segment (Number), 2020 till 2026
- Figure 9-8: Global - Orphan Drugs Clinical Pipeline by Phase (Number), 2020 till 2026
- Figure 9-9: Global - Orphan Drugs Clinical Pipeline by Phase (%), 2020 till 2026
- Figure 10-1: Share of Initial Development Drug in Total Orphan Drug Pipeline, 2020
- Figure 11-1: Share of in Research Phase Orphan Drugs in Total Pipeline, 2020
- Figure 12-1: Share of in Preclinical Phase Orphan Drugs in Total Pipeline, 2020
- Figure 13-1: Share of in Clinical Phase Orphan Drugs in Total Pipeline, 2020
- Figure 14-1: Share of in Phase-I Orphan Drugs in Total Pipeline, 2020
- Figure 15-1: Share of in Phase-I/II Orphan Drugs in Total Pipeline, 2020
- Figure 16-1: Share of in Phase-II Orphan Drugs in Total Pipeline, 2020
- Figure 17-1: Share of in Phase-II/III Orphan Drugs in Total Pipeline, 2020
- Figure 18-1: Share of in Phase-III Orphan Drugs in Total Pipeline, 2020
- Figure 19-1: Share of in Preregistration Orphan Drugs in Total Pipeline, 2020

Figure 20-1: Share of in Registered Orphan Drugs in Total Pipeline, 2020

Figure 21-1: Share of in Marketed Orphan Drugs in Total Pipeline, 2020

I would like to order

Product name: Global Orphan Drug Clinical Trials, Patent & Guidelines Insight 2026

Product link: <https://marketpublishers.com/r/G1B9FE3023F2EN.html>

Price: US\$ 7,000.00 (Single User License / Electronic Delivery)

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

info@marketpublishers.com

Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/G1B9FE3023F2EN.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