

Rare Disease & Orphan Therapeutic Market Analysis & Forecast to 2025: Antibody Drug Conjugates (ADCs), Bispecific Monoclonal Antibodies, Cancer Vaccines, Cytokines, Interferons, Chimeric Antigen Receptor (CAR) T-Cell Therapy, PD-1/PD-L1 inhibitors, Dendritic Cells, Checkpoint Inhibitors, Adopted Cell Therapy (ACT) & IDO Inhibitors

<https://marketpublishers.com/r/R14D7788EAE2EN.html>

Date: January 2020

Pages: 197

Price: US\$ 4,900.00 (Single User License)

ID: R14D7788EAE2EN

Abstracts

This industry analysis report tackles the major orphan drugs within the rare disease therapeutic market and encompasses the most prevalent and dominant therapies in this space. The market value for the current major orphan therapeutic space is analysed and forecast to 2025 with corresponding CAGR percentages. Market details are provided by drug type, therapeutic area, geography and also specific sales and forecast of the major drugs in the field. Key industry players and developments are covered along with the growing pipeline and clinical trials. Regional insights and approval trends are given, as are the strengths, opportunities and challenges of this growing market.

The scope of the report provides in-depth analysis of the therapeutics space by value and forecast to 2025 that is sub divided into:

Small molecules

Antibodies

Oligonucleotides

Gene therapy

Cell therapy

The report reviews the most dominant therapeutics in each sub-market and provides clinical evaluation, sales forecast and market share to 2025. It gives a comparison between the current market values of the main therapies and how the landscape will change by 2025 with the launch of new pipeline agents. This includes some of the main blockbusters such as

Revlimid

Trikafta

Darzalex

Spinraza

Hemlibra

Venclexta

Soliris

Adcetris

Jakafi

Lenvima

Lynparza

Ultomiris

Yescarta

Pomalyst

Vyndaqel

Ninlaro

Liso-cel

Therefore the reader is provided with data on the current main dominant therapeutics in the area and also the drugs that will dominate the space by 2025. This analysis is provided for each sub-therapeutic area. Geographical breakdown analysis is further provided and is segmented into North America, Europe, Asia Pacific and the Rest of the World.

The scope of the report also includes the orphan drug market by indication and disease type with specific detail given to oncology, hematological disorders, CNS, respiratory, immunomodulation, cardiovascular, musculoskeletal, anti-infectives, endocrine and gastrointestinal disease.

Key players and company profiles in the rare disease market are provided including:

Bristol Myers Squibb

Roche/Genentech

Alexion

Janssen J&J

Biogen

Novartis

AstraZeneca Merck

Eisai

Takeda

Abbvie Genentech

Gilead (Kite)

Pfizer

Vertex

Argenx

The report also includes a detailed description of the following trends and market shapers:

USA (FDA) and European (EMA) Orphan Drug Approvals

Venture Funding Increasing Over Time

Therapeutic Areas Dominating Approval

Orphan Drug Designation Trends

Repurposing Existing Therapeutics

Japan Approval Trends

South Korea Approval Trends

China Approval Trends

The growing rare disease pipeline and clinical trial environment is evaluated with specific consideration given to:

Small Molecules and Multiple Myeloma

Gene and Cell Therapy in Melanoma

Gene and Cell Therapy in ADA-SCID

Gene and Cell Therapy agents in Hemophilia A and B

CAR-T Therapy agents in Pancreatic Cancer

CAR-T Therapy agents in Multiple Myeloma

Gene and Cell Therapies in Duchenne Muscular Dystrophy

RPE65 Genetic Retinal Mutation

Rare Eye Disorders

Spinal Muscular Atrophy

Sickle Cell Anaemia

Cystic Fibrosis

Erythropoietic Protoporphyrria

Guillain-Barre Syndrome

Scleroderma

Alpha-1 Antitrypsin Deficiency

Juvenile Idiopathic Arthritis

Contents

1.0 EXECUTIVE SUMMARY

- 2.1 USA (FDA) and European (EMA) Orphan Drug Approvals
- 2.2 Venture Funding Increasing Over Time
- 2.3 Therapeutic Areas Dominating Approval
- 2.4 Orphan Drug Designation Trends
- 2.5 Repurposing Existing Therapeutics
- 2.6 Japan Approval Trends
- 2.7 South Korea Approval Trends
- 2.8 China Approval Trends

3.0 RARE DISEASE MARKET ANALYSIS

- 3.1 Major Orphan Drug Market to 2025
- 3.2 Orphan Small Molecule Market Analysis to 2025
- 3.3 Orphan Antibody Market Analysis to 2025
- 3.4 Orphan Gene Therapy Market Analysis to 2025
- 3.5 Orphan Cell Therapy Market Analysis to 2025
- 3.6 Orphan Oligonucleotide Therapy Market Analysis to 2025
- 3.7 Geographical Breakdown of Rare Disease Market to 2025
- 3.8 Orphan Drug Market by Indication and Disease Type
- 3.9 Key Market Players
 - 3.9.1 Bristol-Myers Squibb
 - 3.9.2 Roche/Genentech
 - 3.9.3 Alexion
 - 3.9.4 J&J/Janssen Biotech
 - 3.9.5 Biogen
 - 3.9.6 Novartis
 - 3.9.7 AstraZeneca
 - 3.9.8 Eisai
 - 3.9.9 Takeda
 - 3.9.10 Abbvie
 - 3.9.11 Gilead
 - 3.9.12 Pfizer
 - 3.9.13 Vertex Pharmaceuticals
 - 3.9.14 Argenix

4.0 PIPELINE AGENTS IN CLINICAL TRIALS BY CONDITION, INTERVENTION AND PHASE

- 4.1 Select Clinical Trials Investigating Small Molecules and Multiple Myeloma
- 4.2 Select Clinical Trials Investigating Gene and Cell Therapy in Melanoma
- 4.3 Select Clinical Trials Investigating Gene and Cell Therapy in ADA-SCID
- 4.4 Current Clinical Trials Investigating the RPE65 Genetic Retinal Mutation
- 4.5 Select Clinical Trials involving Rare Eye Disorders and Orphan Agents
- 4.6 Select Clinical Trials involving Spinal Muscular Atrophy and Orphan Agents
- 4.7 Select Clinical Trials involving Sickle Cell Anaemia and Orphan Agents
- 4.8 Select Clinical Trials involving Gene and Cell Therapy agents in Hemophilia A and B
- 4.9 Select Phase III Clinical Trials in Cystic Fibrosis
- 4.10 Select Clinical Trials involving Gene and Cell Therapy agents in Cystic Fibrosis
- 4.11 Select Clinical Trials involving CAR-T Therapy agents in Pancreatic Cancer
- 4.12 Select Clinical Trials involving CAR-T Therapy agents in Multiple Myeloma
- 4.13 Select Clinical Trials involving Gene and Cell Therapies in Duchenne Muscular Dystrophy
- 4.14 Select Clinical Trials involving Oligonucleotide Therapies in Rare Diseases
- 4.15 Select Clinical Trials Investigating Therapeutics in Erythropoietic Protoporphyrria
- 4.16 Select Clinical Trials Investigating Therapeutics in Guillain-Barre Syndrome
- 4.17 Select Phase III Clinical Trials Investigating Therapeutics in Scleroderma
- 4.18 Select Phase III Clinical Trials Investigating Therapeutics in Alpha-1 Antitrypsin Deficiency
- 4.19 Select Phase III Clinical Trials Investigating Therapeutics in Juvenile Idiopathic Arthritis

5.0 PIPELINE THERAPEUTICS ANALYSIS

6.0 FUTURE OUTLOOK

List Of Tables

LIST OF TABLES

- Exhibit 2.1: Number of FDA and EMA Orphan Drug Approvals from 2001-2018
- Exhibit 2.2: FDA Priority, Accelerated, Breakthrough and Fast Track Regulatory Reviews for Rare Disease from 2008-2017
- Exhibit 2.3: EMA PRIME, Accelerated Assessment, Exceptional Circumstance or Conditional Marketing Approval Reviews for Rare Disease from 2000-2018
- Exhibit 2.4: Venture Funding into Rare Disorders, Excluding Oncology, 2013-2018
- Exhibit 2.5: FDA Orphan Drug Approvals by Therapeutic Area
- Exhibit 2.6: EMA Orphan Drug Approvals by Therapeutic Area
- Exhibit 2.7: Number of FDA Approved Rare Disease Designations by Drug Type 2017-2020
- Exhibit 2.8: Number of FDA Applications for Orphan Designation 2020
- Exhibit 2.9: Number of Orphan FDA Designated Agents 2020
- Exhibit 2.10: Percentage of FDA Applications for Orphan Designation 2020
- Exhibit 2.11: FDA Approved Agents for Rare Disease Indications 2020
- Exhibit 2.12: FDA Approved Agents for Rare Disease Indications 2019
- Exhibit 2.13: FDA Approved Agents for Rare Disease Indications 2018
- Exhibit 2.14: FDA Approved Agents for Rare Disease Indications 2017
- Exhibit 2.15: EMA Approved Rare Disease Therapies to Date
- Exhibit 3.1: Major Rare Disease Therapeutic Market by Drug Class 2018 - 2025
- Exhibit 3.2 Major Rare Disease Therapeutic Market by Small Molecules, Antibodies, Gene Therapies, Cell Therapies and Oligonucleotides, 2018-2025
- Exhibit 3.3: Percentage Market Share of Rare Disease Drugs by Subtype, 2018 Vs 2025
- Exhibit 3.4: Top Rare Disease Drug Sales (\$ Billion) 2018
- Exhibit 3.5: Top Rare Disease Drug Sales Forecast (\$ Billion) 2025
- Exhibit 3.6: Current Small Molecules Dominating the Rare Disease Market and Forecast to 2025
- Exhibit 3.7: Future Small Molecules within the Rare Disease Market and Forecast to 2025
- Exhibit 3.8: Small Molecule Orphan Drug Market 2018-2025
- Exhibit 3.9: Current & Future Antibodies Dominating the Rare Disease Market and Forecast to 2025
- Exhibit 3.10: Orphan Antibody Market Analysis 2018-2025
- Exhibit 3.11: Current & Future Gene Therapies Dominating the Rare Disease Market and Forecast to 2025

Exhibit 3.12: Orphan Gene Therapy Market Analysis 2018-2025

Exhibit 3.13: Current & Future CART Cell Therapies Dominating the Rare Disease Market and Forecast to 2025

Exhibit 3.14: Orphan Cell Therapy Market Analysis 2018-2025

Exhibit 3.15: Current & Future Oligonucleotide Therapies Dominating the Rare Disease Market and Forecast to 2025

Exhibit 3.16 Orphan Oligonucleotide Therapy Market Analysis 2018-2025

Exhibit 4.1: Select Clinical Trials Investigating Small Molecules and Multiple Myeloma

Exhibit 4.2: Select Phase III Clinical Trials Investigating Small Antibodies and Multiple Myeloma

Exhibit 4.3: Select Clinical Trials Investigating Gene and Cell Therapy in Melanoma

Exhibit 4.4: Select Clinical Trials Investigating Gene and Cell Therapy in ADA-SCID

Exhibit 4.5: Current Clinical Trials Investigating the RPE65 Genetic Retinal Mutation

Exhibit 4.6: Select Clinical Trials involving Rare Eye Disorders and Orphan Agents

Exhibit 4.7: Select Clinical Trials involving Spinal Muscular Atrophy and Orphan Agents

Exhibit 4.8: Select Clinical Trials involving Sickle Cell Anaemia and Orphan Agents

Exhibit 4.9: Select Clinical Trials involving Gene and Cell Therapy agents in Hemophilia A/B

Exhibit 4.10: Select Phase III Clinical Trials in Cystic Fibrosis

Exhibit 4.11: Select Clinical Trials involving Gene and Cell Therapy agents in Cystic Fibrosis

Exhibit 4.12: Select Clinical Trials involving CAR-T Therapy agents in Pancreatic Cancer

Exhibit 4.13: Select Clinical Trials involving CAR-T Therapy agents in Multiple Myeloma

Exhibit 4.14: Select Clinical Trials involving Gene and Cell Therapies in Duchenne Muscular Dystrophy

Exhibit 4.15: Select Clinical Trials involving Oligonucleotide Therapies in Rare Diseases

Exhibit 4.16: Select Clinical Trials Investigating Therapeutics in Erythropoietic Protoporphyrin

Exhibit 4.17: Select Clinical Trials Investigating Therapeutics in Guillain-Barre Syndrome

Exhibit 4.18: Select Phase III Clinical Trials Investigating Therapeutics in Scleroderma

Exhibit 4.19: Select Phase III Clinical Trials Investigating Therapeutics in Alpha-1 Antitrypsin Deficiency

Exhibit 4.20: Select Phase III Clinical Trials Investigating Therapeutics in Juvenile Idiopathic Arthritis

Exhibit 5.1: Total Number of Orphan Drugs in Phase I, II, III and NDA/BLA Clinical Programs

Exhibit 5.2: Number of Orphan Drugs (Oncology/Ex-Oncology) in Phase I, II, III and

NDA/BLA Clinical Programs

Exhibit 5.3: Number of Therapies in Development for Rare Diseases

Exhibit 5.4: Top Selling Orphan Drugs Forecast to 2025

Exhibit 5.5: Top Orphan Drugs by Type and Mode of Action by 2025

I would like to order

Product name: Rare Disease & Orphan Therapeutic Market Analysis & Forecast to 2025: Antibody Drug Conjugates (ADCs), Bispecific Monoclonal Antibodies, Cancer Vaccines, Cytokines, Interferons, Chimeric Antigen Receptor (CAR) T-Cell Therapy, PD-1/PD-L1 inhibitors, Dendritic Cells, Checkpoint Inhibitors, Adopted Cell Therapy (ACT) & IDO Inhibitors

Product link: <https://marketpublishers.com/r/R14D7788EAE2EN.html>

Price: US\$ 4,900.00 (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/R14D7788EAE2EN.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