

Pompe Disease Treatment Market by Treatment (Enzyme Replacement Therapy (ERT), Substrate Reduction Therapy (SRT), Chaperone-Advanced Replacement Therapy (CART), and Others), Route of Administration (Oral, Intravenous, and Others), Distribution Channel (Hospital and Clinics Pharmacies, Retail Pharmacies, Online Pharmacies, and Others), Indication Type (Infantile-Onset Pompe Disease (IOPD), Classic Infantile Form, Non-Classic Infantile Form, Late-Onset Pompe Disease (LOPD), and Others), and Region 2025-2033

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# **Abstracts**

The global Pompe disease treatment market size reached USD 1.07 Billion in 2024. Looking forward, IMARC Group expects the market to reach USD 1.33 Billion by 2033, exhibiting a growth rate (CAGR) of 2.47% during 2025-2033. The increasing number of clinical research and the growing popularity of enzyme replacement therapy (ERT) are stimulating the market.

Pompe disease is a genetic disorder caused by the buildup of a complex sugar known as glycogen in the cells of the body, which affects organs, tissues, and muscles. It is an autosomal recessive glycogen storage syndrome caused by the deficiency of lysosomal enzyme acid a-glucosidase (GAA). It is associated with weak muscles, enlarged liver, trouble breathing, hearing problem, lung infection, irregular heartbeat, and losing weight. It is diagnosed using various tools and tests, including electromyography, X-rays, and magnetic resonance imaging (MRI). Pompe disease can be treated using



medications, enzyme replacement therapy (ERT), substrate reduction therapy (SRT), chaperone-advanced replacement therapy (CART), and injections, which aid in improving the quality of life.

# Pompe Disease Treatment Market Trends:

There is a considerable rise in the prevalence of genetic abnormalities among individuals and an increase in the number of patients suffering from Pompe disease. This, coupled with the growing Pompe disease among infants and increasing concerns among parents about the health of their newborns, represents one of the key factors bolstering the growth of the market. Moreover, the growing geriatric population that is susceptible to developing these medical disorders is resulting in an increasing demand for Pompe disease treatment. In addition, the expanding number of clinical studies and trials to develop new treatments for Pompe disease is influencing the market positively. Apart from this, the rising awareness among people about ERT for curing Pompe disease is favoring the market growth. Furthermore, there is an increase in awareness about various treatments, including stem cell transplant, bone marrow transplantation, chemotherapy, and immunotherapy, on account of their numerous advantages. This, along with the awareness about the easy availability of ERT treatment alternatives like lumizyme, is creating a positive outlook for the market. Besides this, there is a rise in the adoption of health insurance policies that provide financial assistance and minimize healthcare expenditure for Pompe disease treatment. Additionally, governments and non-governmental organizations (NGOs) of numerous countries are undertaking measures to offer quality healthcare facilities that are accessible to all. This, along with significant improvements in the diagnostic technologies, is anticipated to provide a favorable outlook to the market.

## Key Market Segmentation:

IMARC Group provides an analysis of the key trends in each segment of the market, along with the market forecasts at the global, regional, and country levels for 2025-2033. Our report has categorized the market based on treatment, route of administration, distribution channel, and indication type.

## Treatment Insights:

Enzyme Replacement Therapy (ERT)

Substrate Reduction Therapy (SRT)



Chaperone-Advanced Replacement Therapy (CART)
Others
The report has provided a detailed breakup and analysis of the Pompe disease treatment market based on the treatment. This includes enzyme replacement therapy (ERT), substrate reduction therapy (SRT), chaperone-advanced replacement therapy (CART), and others. According to the report, enzyme replacement therapy (ERT) represented the largest segment.
Route of Administration Insights:
Oral
Intravenous
Others
A detailed breakup and analysis of the Pompe disease treatment market based on the route of administration has also been provided in the report. This includes oral, intravenous, and others.
Distribution Channel Insights:
Hospital and Clinics Pharmacies
Retail Pharmacies
Online Pharmacies
Others
A detailed breakup and analysis of the Pompe disease treatment market based on the

Pompe Disease Treatment Market by Treatment (Enzyme Replacement Therapy (ERT), Substrate Reduction Therapy (SR...

distribution channel has also been provided in the report. This includes hospital and clinics, retail, and online pharmacies, and others. According to the report, hospital and



clinics pharmacies accounted for the largest market share.

Indication Type Insights:

Infantile-Onset Pompe Disease (IOPD)

Classic Infantile Form

Non-Classic Infantile Form

Late-Onset Pompe Disease (LOPD)

A detailed breakup and analysis of the Pompe disease treatment market based on the indication type has also been provided in the report. This includes infantile-onset Pompe disease (IOPD), classic infantile form, non-classic infantile form, late-onset Pompe disease (LOPD), and others. According to the report, late-onset Pompe disease (LOPD) accounted for the largest market share.

# Regional Insights:

Others

North America

**United States** 

Canada

Asia Pacific

China

Japan

India

South Korea



Australia
Indonesia
Others
Europe
Germany
France
United Kingdom
Italy
Spain
Russia
Others
Latin America
Brazil
Mexico
Others
Middle East and Africa

The report has also provided a comprehensive analysis of all the major regional markets that include North America (the United States and Canada), Asia Pacific (China, Japan, India, South Korea, Australia, Indonesia, and others), Europe (Germany, France, the United Kingdom, Italy, Spain, Russia, and others), Latin America (Brazil, Mexico, and others), and the Middle East and Africa. According to the report, North America was the largest market for Pompe disease treatment. Some of the factors



driving the North America Pompe disease treatment market included the growing awareness about various alternative treatments, increasing number of clinical studies and trials, and well-established healthcare facilities.

# Competitive Landscape:

The report has also provided a comprehensive analysis of the competitive landscape in the global Pompe disease treatment market. Detailed profiles of all major companies have also been provided. Some of the companies covered include Amicus Therapeutics Inc., Audentes Therapeutics Inc. (Astellas US Holding Inc.), Oxyrane UK Limited, Sanofi S.A., Spark Therapeutics Inc, etc.

Key Questions Answered in This Report

- 1. How big is the pompe disease treatment market?
- 2. What is the future outlook of pompe disease treatment market?
- 3. What are the key factors driving the pompe disease treatment market?
- 4. Which region accounts for the largest pompe disease treatment market share?
- 5. Which are the leading companies in the global pompe disease treatment market?



# **Contents**

#### 1 PREFACE

#### 2 SCOPE AND METHODOLOGY

- 2.1 Objectives of the Study
- 2.2 Stakeholders
- 2.3 Data Sources
  - 2.3.1 Primary Sources
  - 2.3.2 Secondary Sources
- 2.4 Market Estimation
  - 2.4.1 Bottom-Up Approach
  - 2.4.2 Top-Down Approach
- 2.5 Forecasting Methodology

#### **3 EXECUTIVE SUMMARY**

#### **4 INTRODUCTION**

- 4.1 Overview
- 4.2 Key Industry Trends

#### **5 GLOBAL POMPE DISEASE TREATMENT MARKET**

- 5.1 Market Overview
- 5.2 Market Performance
- 5.3 Impact of COVID-19
- 5.4 Market Forecast

# **6 MARKET BREAKUP BY TREATMENT**

- 6.1 Enzyme Replacement Therapy (ERT)
  - 6.1.1 Market Trends
  - 6.1.2 Market Forecast
- 6.2 Substrate Reduction Therapy (SRT)
  - 6.2.1 Market Trends
  - 6.2.2 Market Forecast
- 6.3 Chaperone-Advanced Replacement Therapy (CART)



- 6.3.1 Market Trends
- 6.3.2 Market Forecast
- 6.4 Others
  - 6.4.1 Market Trends
  - 6.4.2 Market Forecast

## 7 MARKET BREAKUP BY ROUTE OF ADMINISTRATION

- 7.1 Oral
  - 7.1.1 Market Trends
  - 7.1.2 Market Forecast
- 7.2 Intravenous
  - 7.2.1 Market Trends
  - 7.2.2 Market Forecast
- 7.3 Others
  - 7.3.1 Market Trends
  - 7.3.2 Market Forecast

#### **8 MARKET BREAKUP BY DISTRIBUTION CHANNEL**

- 8.1 Hospital & Clinics Pharmacies
  - 8.1.1 Market Trends
  - 8.1.2 Market Forecast
- 8.2 Retail Pharmacies
  - 8.2.1 Market Trends
  - 8.2.2 Market Forecast
- 8.3 Online Pharmacies
  - 8.3.1 Market Trends
- 8.3.2 Market Forecast
- 8.4 Others
  - 8.4.1 Market Trends
  - 8.4.2 Market Forecast

#### 9 MARKET BREAKUP BY INDICATION TYPE

- 9.1 Infantile-Onset Pompe Disease (IOPD)
  - 9.1.1 Market Trends
  - 9.1.2 Market Forecast
- 9.2 Classic Infantile Form



- 9.2.1 Market Trends
- 9.2.2 Market Forecast
- 9.3 Non-Classic Infantile Form
  - 9.3.1 Market Trends
  - 9.3.2 Market Forecast
- 9.4 Late-Onset Pompe Disease (LOPD)
  - 9.4.1 Market Trends
  - 9.4.2 Market Forecast
- 9.5 Others
  - 9.5.1 Market Trends
  - 9.5.2 Market Forecast

## 10 MARKET BREAKUP BY REGION

- 10.1 North America
  - 10.1.1 United States
    - 10.1.1.1 Market Trends
    - 10.1.1.2 Market Forecast
  - 10.1.2 Canada
    - 10.1.2.1 Market Trends
    - 10.1.2.2 Market Forecast
- 10.2 Asia-Pacific
  - 10.2.1 China
    - 10.2.1.1 Market Trends
    - 10.2.1.2 Market Forecast
  - 10.2.2 Japan
    - 10.2.2.1 Market Trends
    - 10.2.2.2 Market Forecast
  - 10.2.3 India
    - 10.2.3.1 Market Trends
    - 10.2.3.2 Market Forecast
  - 10.2.4 South Korea
    - 10.2.4.1 Market Trends
    - 10.2.4.2 Market Forecast
  - 10.2.5 Australia
    - 10.2.5.1 Market Trends
    - 10.2.5.2 Market Forecast
  - 10.2.6 Indonesia
  - 10.2.6.1 Market Trends



- 10.2.6.2 Market Forecast
- 10.2.7 Others
  - 10.2.7.1 Market Trends
  - 10.2.7.2 Market Forecast
- 10.3 Europe
  - 10.3.1 Germany
    - 10.3.1.1 Market Trends
    - 10.3.1.2 Market Forecast
  - 10.3.2 France
    - 10.3.2.1 Market Trends
    - 10.3.2.2 Market Forecast
  - 10.3.3 United Kingdom
    - 10.3.3.1 Market Trends
  - 10.3.3.2 Market Forecast
  - 10.3.4 Italy
    - 10.3.4.1 Market Trends
    - 10.3.4.2 Market Forecast
  - 10.3.5 Spain
    - 10.3.5.1 Market Trends
    - 10.3.5.2 Market Forecast
  - 10.3.6 Russia
    - 10.3.6.1 Market Trends
    - 10.3.6.2 Market Forecast
  - 10.3.7 Others
    - 10.3.7.1 Market Trends
    - 10.3.7.2 Market Forecast
- 10.4 Latin America
  - 10.4.1 Brazil
    - 10.4.1.1 Market Trends
    - 10.4.1.2 Market Forecast
  - 10.4.2 Mexico
    - 10.4.2.1 Market Trends
    - 10.4.2.2 Market Forecast
  - 10.4.3 Others
    - 10.4.3.1 Market Trends
    - 10.4.3.2 Market Forecast
- 10.5 Middle East and Africa
  - 10.5.1 Market Trends
  - 10.5.2 Market Breakup by Country



#### 10.5.3 Market Forecast

# 11 DRIVERS, RESTRAINTS, AND OPPORTUNITIES

- 11.1 Overview
- 11.2 Drivers
- 11.3 Restraints
- 11.4 Opportunities

### 12 VALUE CHAIN ANALYSIS

## 13 PORTERS FIVE FORCES ANALYSIS

- 13.1 Overview
- 13.2 Bargaining Power of Buyers
- 13.3 Bargaining Power of Suppliers
- 13.4 Degree of Competition
- 13.5 Threat of New Entrants
- 13.6 Threat of Substitutes

# 14 PRICE ANALYSIS

#### 15 COMPETITIVE LANDSCAPE

- 15.1 Market Structure
- 15.2 Key Players
- 15.3 Profiles of Key Players
  - 15.3.1 Amicus Therapeutics Inc.
    - 15.3.1.1 Company Overview
    - 15.3.1.2 Product Portfolio
    - 15.3.1.3 Financials
  - 15.3.1.4 SWOT Analysis
  - 15.3.2 Audentes Therapeutics Inc. (Astellas US Holding Inc.)
    - 15.3.2.1 Company Overview
    - 15.3.2.2 Product Portfolio
  - 15.3.3 Oxyrane UK Limited
    - 15.3.3.1 Company Overview
    - 15.3.3.2 Product Portfolio
  - 15.3.4 Sanofi S.A.



- 15.3.4.1 Company Overview
- 15.3.4.2 Product Portfolio
- 15.3.4.3 Financials
- 15.3.4.4 SWOT Analysis
- 15.3.5 Spark Therapeutics Inc.
  - 15.3.5.1 Company Overview
  - 15.3.5.2 Product Portfolio

Kindly, note that this only represents a partial list of companies, and the complete list has been provided in the report



# **List Of Tables**

#### LIST OF TABLES

Table 1: Global: Pompe Disease Treatment Market: Key Industry Highlights, 2024 & 2033

Table 2: Global: Pompe Disease Treatment Market Forecast: Breakup by Treatment (in Million USD), 2025-2033

Table 3: Global: Pompe Disease Treatment Market Forecast: Breakup by Route of Administration (in Million USD), 2025-2033

Table 4: Global: Pompe Disease Treatment Market Forecast: Breakup by Distribution Channel (in Million USD), 2025-2033

Table 5: Global: Pompe Disease Treatment Market Forecast: Breakup by Indication Type (in Million USD), 2025-2033

Table 6: Global: Pompe Disease Treatment Market Forecast: Breakup by Region (in Million USD), 2025-2033

Table 7: Global: Pompe Disease Treatment Market: Competitive Structure

Table 8: Global: Pompe Disease Treatment Market: Key Players



# **List Of Figures**

#### LIST OF FIGURES

?Figure 1: Global: Pompe Disease Treatment Market: Major Drivers and Challenges

Figure 2: Global: Pompe Disease Treatment Market: Sales Value (in Billion USD), 2019-2024

Figure 3: Global: Pompe Disease Treatment Market Forecast: Sales Value (in Billion USD), 2025-2033

Figure 4: Global: Pompe Disease Treatment Market: Breakup by Treatment (in %), 2024

Figure 5: Global: Pompe Disease Treatment Market: Breakup by Route of

Administration (in %), 2024

Figure 6: Global: Pompe Disease Treatment Market: Breakup by Distribution Channel (in %), 2024

Figure 7: Global: Pompe Disease Treatment Market: Breakup by Indication Type (in %), 2024

Figure 8: Global: Pompe Disease Treatment Market: Breakup by Region (in %), 2024

Figure 9: Global: Pompe Disease Treatment (Enzyme Replacement Therapy (ERT))

Market: Sales Value (in Million USD), 2019 & 2024

Figure 10: Global: Pompe Disease Treatment (Enzyme Replacement Therapy (ERT))

Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 11: Global: Pompe Disease Treatment (Substrate Reduction Therapy (SRT))

Market: Sales Value (in Million USD), 2019 & 2024

Figure 12: Global: Pompe Disease Treatment (Substrate Reduction Therapy (SRT))

Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 13: Global: Pompe Disease Treatment (Chaperone-Advanced Replacement

Therapy (CART)) Market: Sales Value (in Million USD), 2019 & 2024

Figure 14: Global: Pompe Disease Treatment (Chaperone-Advanced Replacement

Therapy (CART)) Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 15: Global: Pompe Disease Treatment (Other Treatments) Market: Sales Value (in Million USD), 2019 & 2024

Figure 16: Global: Pompe Disease Treatment (Other Treatments) Market Forecast:

Sales Value (in Million USD), 2025-2033

Figure 17: Global: Pompe Disease Treatment (Oral) Market: Sales Value (in Million USD), 2019 & 2024

Figure 18: Global: Pompe Disease Treatment (Oral) Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 19: Global: Pompe Disease Treatment (Intravenous) Market: Sales Value (in



Million USD), 2019 & 2024

Figure 20: Global: Pompe Disease Treatment (Intravenous) Market Forecast: Sales

Value (in Million USD), 2025-2033

Figure 21: Global: Pompe Disease Treatment (Other Rout of Administrations) Market:

Sales Value (in Million USD), 2019 & 2024

Figure 22: Global: Pompe Disease Treatment (Other Rout of Administrations) Market

Forecast: Sales Value (in Million USD), 2025-2033

Figure 23: Global: Pompe Disease Treatment (Hospital & Clinics Pharmacies) Market:

Sales Value (in Million USD), 2019 & 2024

Figure 24: Global: Pompe Disease Treatment (Hospital & Clinics Pharmacies) Market

Forecast: Sales Value (in Million USD), 2025-2033

Figure 25: Global: Pompe Disease Treatment (Retail Pharmacies) Market: Sales Value

(in Million USD), 2019 & 2024

Figure 26: Global: Pompe Disease Treatment (Retail Pharmacies) Market Forecast:

Sales Value (in Million USD), 2025-2033

Figure 27: Global: Pompe Disease Treatment (Online Pharmacies) Market: Sales Value

(in Million USD), 2019 & 2024

Figure 28: Global: Pompe Disease Treatment (Online Pharmacies) Market Forecast:

Sales Value (in Million USD), 2025-2033

Figure 29: Global: Pompe Disease Treatment (Other Distribution Channels) Market:

Sales Value (in Million USD), 2019 & 2024

Figure 30: Global: Pompe Disease Treatment (Other Distribution Channels) Market

Forecast: Sales Value (in Million USD), 2025-2033

Figure 31: Global: Pompe Disease Treatment (Infantile-Onset Pompe Disease (IOPD))

Market: Sales Value (in Million USD), 2019 & 2024

Figure 32: Global: Pompe Disease Treatment (Infantile-Onset Pompe Disease (IOPD))

Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 33: Global: Pompe Disease Treatment (Classic Infantile Form) Market: Sales

Value (in Million USD), 2019 & 2024

Figure 34: Global: Pompe Disease Treatment (Classic Infantile Form) Market Forecast:

Sales Value (in Million USD), 2025-2033

Figure 35: Global: Pompe Disease Treatment (Non-Classic Infantile Form) Market:

Sales Value (in Million USD), 2019 & 2024

Figure 36: Global: Pompe Disease Treatment (Non-Classic Infantile Form) Market

Forecast: Sales Value (in Million USD), 2025-2033

Figure 37: Global: Pompe Disease Treatment (Late-Onset Pompe Disease (LOPD))

Market: Sales Value (in Million USD), 2019 & 2024

Figure 38: Global: Pompe Disease Treatment (Late-Onset Pompe Disease (LOPD))

Market Forecast: Sales Value (in Million USD), 2025-2033



Figure 39: Global: Pompe Disease Treatment (Other Indication Types) Market: Sales Value (in Million USD), 2019 & 2024

Figure 40: Global: Pompe Disease Treatment (Other Indication Types) Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 41: North America: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 42: North America: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 43: United States: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 44: United States: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 45: Canada: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 46: Canada: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 47: Asia-Pacific: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 48: Asia-Pacific: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 49: China: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 50: China: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 51: Japan: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 52: Japan: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 53: India: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 54: India: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 55: South Korea: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 56: South Korea: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 57: Australia: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 58: Australia: Pompe Disease Treatment Market Forecast: Sales Value (in



Million USD), 2025-2033

Figure 59: Indonesia: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 60: Indonesia: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 61: Others: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 62: Others: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 63: Europe: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 64: Europe: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 65: Germany: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 66: Germany: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 67: France: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 68: France: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 69: United Kingdom: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 70: United Kingdom: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 71: Italy: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 72: Italy: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 73: Spain: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 74: Spain: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 75: Russia: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 76: Russia: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 77: Others: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024



Figure 78: Others: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 79: Latin America: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 80: Latin America: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 81: Brazil: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 82: Brazil: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 83: Mexico: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 84: Mexico: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 85: Others: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 86: Others: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 87: Middle East and Africa: Pompe Disease Treatment Market: Sales Value (in Million USD), 2019 & 2024

Figure 88: Middle East and Africa: Pompe Disease Treatment Market: Breakup by Country (in %), 2024

Figure 89: Middle East and Africa: Pompe Disease Treatment Market Forecast: Sales Value (in Million USD), 2025-2033

Figure 90: Global: Pompe Disease Treatment Industry: Drivers, Restraints, and Opportunities

Figure 91: Global: Pompe Disease Treatment Industry: Value Chain Analysis

Figure 92: Global: Pompe Disease Treatment Industry: Porter's Five Forces Analysis



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Product name: Pompe Disease Treatment Market by Treatment (Enzyme Replacement Therapy (ERT),

Substrate Reduction Therapy (SRT), Chaperone-Advanced Replacement Therapy (CART), and Others), Route of Administration (Oral, Intravenous, and Others), Distribution Channel (Hospital and Clinics Pharmacies, Retail Pharmacies, Online Pharmacies, and Others), Indication Type (Infantile-Onset Pompe Disease (IOPD), Classic Infantile Form, Non-Classic Infantile Form, Late-Onset Pompe Disease (LOPD), and Others), and Region 2025-2033

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