

# Pompe Disease Market Forecasted to Surge in Coming Years, 2024-2034 Analysis by DelveInsight

Pompe Disease Market Forecast-2034 report offers an in-depth understanding of the forecasted epidemiology as well as the Pompe Disease market trends in the 7MM.

LAS VEGAS, NV, UNITED STATES, January 8, 2025 /EINPresswire.com/ --DelveInsight's "Pompe Disease Market Insights, Epidemiology, and Market Forecast-2034" report offers an indepth understanding of the Pompe Disease, historical and forecasted



epidemiology as well as the Pompe Disease market trends in the United States, EU4 (Germany, Spain, Italy, France) the United Kingdom and Japan.

The latest healthcare forecast report provides an in-depth analysis of Pompe Disease, offering comprehensive insights into the Pompe Disease revenue trends, prevalence, and treatment landscape. The report delves into key Pompe Disease statistics, highlighting the current and projected market size, while examining the efficacy and development of emerging Pompe Disease therapies. Additionally, we cover the landscape of Pompe Disease clinical trials, providing an overview of ongoing and upcoming studies that are poised to shape the future of Pompe Disease treatment. This report is an essential resource for understanding the market dynamics and the evolving therapeutic options within the Pompe Disease space.

To Know in detail about the Pompe Disease market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; <u>Pompe Disease Market Forecast</u>

Some of the key facts of the Pompe Disease Market Report:

- The Pompe Disease market size is anticipated to grow with a significant CAGR during the study period (2020-2034)
- In October 2024, Aro Biotherapeutics, a clinical-stage biotechnology company focused on developing targeted, potent medicines, has announced the launch of the Phase 1b portion of its

clinical trial for ABX1100, a potential treatment for late-onset Pompe disease (LOPD). ABX1100 works by targeting the CD71 receptor, delivering siRNA therapy directly to muscle tissue to inhibit the production of the GYS1 enzyme, which is responsible for glycogen synthesis. The Phase 1b study aims to enroll adults with LOPD to assess the safety and bioactivity of ABX1100.

- According to DelveInsight's estimates, there were approximately 13,000 prevalent cases of Pompe disease across the 7MM in 2023, with the number expected to increase by 2034.
- In 2023, approximately 8,600 prevalent cases of Pompe disease were reported in the United States, with 98% of these cases being diagnosed in adult patients.
- Among the EU4 and the UK, Germany recorded the highest market size at around USD 94 million, followed by France, having the smallest market size in 2023.
- Asklepios Biopharmaceutical is working on AAV2/8-LSPhGAA, a gene therapy candidate designed to treat Pompe disease by delivering the GAA gene to restore enzyme function.
   Together with Avalglucosidase alfa from Sanofi, which is a next-generation enzyme replacement therapy, these candidates have the potential to meet the unmet needs in the treatment of Pompe disease.
- The US FDA has approved various enzyme replacement therapies for different types of Pompe disease, including MYOZYME (alglucosidase alfa) and LUMIZYME (alglucosidase alfa) by Genzyme/Sanofi, along with POMBILITI (cipaglucosidase alfa-atga) by Amicus Therapeutics, among others.
- The Pompe disease pipeline is fairly restricted, with only a handful of companies advancing their candidates through various clinical trial phases. Key candidates include AAV2/8-LSPhGAA (ACTUS-101) by Asklepios Biopharmaceutical, currently in Phase I/II, and Avalglucosidase alfa by Sanofi, now in Phase III. The rarity of Pompe disease poses difficulties in recruiting patients for clinical trials, which in turn hampers the development of new treatment options.
- Key Pompe Disease Companies: Asklepios Biopharmaceutical, Genzyme, Sanofi, GeneCradle Inc, Astellas Gene Therapies, Amicus Therapeutics, Spark Therapeutics, Inc., Asklepios Biopharmaceutical, and others
- Key Pompe Disease Therapies: ACTUS-101, MYOZYME (alglucosidase alfa), GC301, zocaglusagene nuzaparvovec, AT2221, SPK-3006, AAV2/8-LSPhGAA (ACTUS-101), and others
- The Pompe Disease market is expected to surge due to the disease's increasing prevalence and awareness during the forecast period. Furthermore, launching various multiple-stage Pompe Disease pipeline products will significantly revolutionize the Pompe Disease market dynamics.
- According to DelveInsight's estimates, there were around 13,000 prevalent cases of Pompe disease in the 7MM in 2023, with projections indicating that this number could increase by 2034.
- The EU4 and the UK comprised roughly 25% of all prevalent cases of Pompe disease in 2023.
- In 2023, approximately 8,600 prevalent cases of Pompe disease were reported in the United States, with 98% of these cases occurring in adult patients.
- In 2023, the incidence of the CRIM-positive phenotype in infantile-onset Pompe disease was significantly higher in the United States, at around 80%. This phenotype is marked by the presence of residual enzyme activity, which can affect the progression of the disease and the response to treatment.

#### Pompe Disease Overview

Pompe disease is a rare, inherited genetic disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA). This enzyme is essential for breaking down glycogen, a stored form of sugar, into glucose, which is used for energy. When GAA is deficient, glycogen accumulates in the body's cells, particularly in muscle tissue, leading to progressive muscle weakness and damage.

Get a Free sample for the Pompe Disease Market Forecast, Size & Share Analysis Report: <a href="https://www.delveinsight.com/report-store/pompe-disease-">https://www.delveinsight.com/report-store/pompe-disease-</a>
<a href="mailto:market?utm">market?utm</a> source=einpresswire&utm</a> medium=pressrelease&utm</a> campaign=gpr

## Pompe Disease Epidemiology

The epidemiology section provides insights into the historical, current, and forecasted epidemiology trends in the seven major countries (7MM) from 2020 to 2034. It helps to recognize the causes of current and forecasted trends by exploring numerous studies and views of key opinion leaders. The epidemiology section also provides a detailed analysis of the diagnosed patient pool and future trends.

## Pompe Disease Epidemiology Segmentation:

The Pompe Disease market report proffers epidemiological analysis for the study period 2020–2034 in the 7MM segmented into:

- Total Prevalence of Pompe Disease
- Prevalent Cases of Pompe Disease by severity
- Gender-specific Prevalence of Pompe Disease
- Diagnosed Cases of Episodic and Chronic Pompe Disease

Download the report to understand which factors are driving Pompe Disease epidemiology trends @ Pompe Disease Epidemiology Forecast

# Pompe Disease Drugs Uptake and Pipeline Development Activities

The drugs uptake section focuses on the rate of uptake of the potential drugs recently launched in the Pompe Disease market or expected to get launched during the study period. The analysis covers Pompe Disease market uptake by drugs, patient uptake by therapies, and sales of each drug.

Moreover, the therapeutics assessment section helps understand the drugs with the most rapid uptake and the reasons behind the maximal use of the drugs. Additionally, it compares the drugs based on market share.

The report also covers the Pompe Disease Pipeline Development Activities. It provides valuable insights about different therapeutic candidates in various stages and the key companies involved in developing targeted therapeutics. It also analyzes recent developments such as collaborations, acquisitions, mergers, licensing patent details, and other information for emerging therapies.

Pompe Disease Therapies and Key Companies

- ACTUS-101: Asklepios Biopharmaceutical
- MYOZYME (alglucosidase alfa): Genzyme/ Sanofi
- GC301: GeneCradle Inc
- zocaglusagene nuzaparvovec: Astellas Gene Therapies
- AT2221: Amicus Therapeutics
- SPK-3006: Spark Therapeutics, Inc.
- AAV2/8-LSPhGAA (ACTUS-101): Asklepios Biopharmaceutical

## Pompe Disease Market Drivers

- Increasing Awareness and Diagnosis
- Advancements in Gene Therapy
- Emerging Therapies
- Supportive Regulatory Environment

## Pompe Disease Market Barriers

- High Treatment Costs
- · Limited Patient Population
- Complexity of Treatment Administration
- Regulatory Challenges
- Variable Disease Progression

## Scope of the Pompe Disease Market Report

- Study Period: 2020-2034
- Coverage: 7MM [The United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom), and Japan]
- Key Pompe Disease Companies: Asklepios Biopharmaceutical, Genzyme, Sanofi, GeneCradle Inc, Astellas Gene Therapies, Amicus Therapeutics, Spark Therapeutics, Inc., Asklepios Biopharmaceutical, and others
- Key Pompe Disease Therapies: ACTUS-101, MYOZYME (alglucosidase alfa), GC301, zocaglusagene nuzaparvovec, AT2221, SPK-3006, AAV2/8-LSPhGAA (ACTUS-101), and others
- Pompe Disease Therapeutic Assessment: Pompe Disease current marketed and Pompe Disease emerging therapies
- Pompe Disease Market Dynamics: Pompe Disease market drivers and Pompe Disease market barriers
- Competitive Intelligence Analysis: SWOT analysis, PESTLE analysis, Porter's five forces, BCG Matrix, Market entry strategies
- Pompe Disease Unmet Needs, KOL's views, Analyst's views, Pompe Disease Market Access and Reimbursement

To know more about Pompe Disease companies working in the treatment market, visit @ <u>Pompe Disease Clinical Trials and Therapeutic Assessment</u>

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## About DelveInsight

DelveInsight is a leading Healthcare Business Consultant, and Market Research firm focused exclusively on life sciences. It supports Pharma companies by providing comprehensive end-to-end solutions to improve their performance.

It also offers Healthcare Consulting Services, which benefits in market analysis to accelerate the business growth and overcome challenges with a practical approach.

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