

Pompe Disease Market Report 2032: Epidemiology Data, Pipeline Therapies, Latest FDA, EMA, PDMA Approvals by DelveInsight

Pompe Disease Market is expected to rise at a significant CAGR, as per DelveInsight.

LAS VEGAS, NEVADA, UNITED STATES, April 2, 2024 /EINPresswire.com/ -- DelveInsight's "Pompe Disease Market Insights, Epidemiology, and Market Forecast-2032" report offers an in-depth 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.

To Know in detail about the Pompe Disease market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; [Pompe Disease Market Forecast](#)

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 (2019-2032).

As per DelveInsight analysis, the total diagnosed prevalent cases of Pompe disease in the 7MM were found to be approximately 13K cases in 2022, which are expected to increase by 2032.

Key Pompe Disease Companies: Genzyme, Sanofi, Amicus Therapeutics, Actus Therapeutics, Valerion Therapeutics, Astellas Therapeutics, Roche, Lacerta Therapeutics, and others

Key Pompe Disease Therapies: AT-GAA, SPK-3006, 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.

Pompe Disease Overview

Pompe disease, also referred to as glycogen storage disease type 2 (GSD2), is a rare genetic disorder characterized by the abnormal accumulation of glycogen, a complex sugar molecule, within cells. This buildup disrupts the functioning of various organs and tissues, particularly affecting the heart and different types of muscles.

Symptoms of Pompe disease can vary significantly among individuals depending on factors such as age at onset, disease type, and progression rate. Common manifestations include progressive

muscle weakness, reduced muscle tone, respiratory difficulties, frequent respiratory infections, feeding difficulties, enlarged tongue, liver (hepatomegaly), and/or heart (cardiomegaly), and hearing impairment. Pompe disease is classified into three main types: classic infantile-onset form, nonclassic infantile-onset form, and late-onset form.

Glycogen serves as a storage form of sugar primarily found in liver and skeletal muscle cells, providing long-term energy reserves. The breakdown of glycogen into glucose for energy production requires the enzyme acid alpha-glucosidase (GAA). Mutations in the GAA gene, which provides instructions for producing the GAA enzyme, cause Pompe disease. These mutations result in either a dysfunctional GAA enzyme or its absence altogether. Consequently, glycogen cannot be properly broken down, leading to toxic accumulation within cells. Muscle cells, reliant on glycogen for energy to facilitate movement, are particularly affected.

Management of Pompe disease typically involves a multidisciplinary team approach comprising specialists from various fields such as cardiology, neurology, pulmonology, respiratory therapy, dietetics, orthopedics, occupational/speech therapy, genetics, and genetic counseling. Treatment strategies for Pompe disease revolve around disease-specific therapies, including enzyme replacement therapy (ERT), and supportive therapies.

ERT, the primary therapeutic approach for Pompe disease, involves administering a synthetic version of the GAA enzyme to patients to replace the deficient or malfunctioning enzyme. This treatment aims to reduce glycogen accumulation within cells, thereby slowing disease progression. Additionally, several experimental therapies for Pompe disease are under investigation, including gene therapy approaches aimed at providing functional copies of the GAA gene.

Supportive therapies, such as physical therapy, occupational therapy, and speech therapy, are also integral components of Pompe disease management. These non-pharmacological interventions can help manage specific symptoms and improve overall muscle strength and physical function, particularly in respiratory muscles.

Pompe Disease Epidemiology

The epidemiology section provides insights into the historical, current, and forecasted epidemiology trends in the seven major countries (7MM) from 2019 to 2032. 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 2019–2032 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

AT-GAA
SPK-3006

Pompe Disease Key Companies

Genzyme
Sanofi
Amicus Therapeutics
Actus Therapeutics
Valerion Therapeutics
Astellas Therapeutics
Roche
Lacerta Therapeutics

Discover more about therapies set to grab major Pompe Disease market share @ [Pompe Disease Treatment Landscape](#)

Pompe Disease Treatment Market

Treatment for Pompe disease is characterized by its tailored approach addressing the specific disease, managing symptoms, and offering supportive care. This comprehensive method necessitates the collaboration of a multidisciplinary team comprising specialists skilled in neuromuscular disorder management. Pediatricians, internists, neurologists, orthopedists, cardiologists, dietitians, and other healthcare experts work together to devise and execute personalized treatment strategies for affected children. Genetic counseling is integral in providing support to patients and their families, particularly considering the limited number of approved treatment options available.

Recently, Sanofi's NEXVIAZYME (avalglucosidase alfa-ngpt) has received approval from the US FDA, marking a significant advancement in late-onset Pompe disease management. This groundbreaking therapy targets the mannose-6-phosphate (M6P) receptor, a crucial pathway for enzyme replacement therapy. By effectively reducing glycogen buildup in muscle cells, as evidenced by successful Phase III clinical trials, NEXVIAZYME has shown notable benefits. Patients treated with NEXVIAZYME demonstrated remarkable improvements in respiratory function and walking distance, with a significant 2.9-point increase in forced vital capacity (FVC) percent predicted at Week 49, meeting the primary study endpoint. The treatment regimen involves monotherapy enzyme replacement therapy (ERT) administered every two weeks, with dosage determined based on patient weight and administered through a gradual intravenous infusion.

Pompe Disease Market Dynamics

The market for therapies targeting Pompe disease has seen a surge in interest, driven by advancements in understanding the condition and the emergence of innovative treatments. This shift in dynamics is influenced by various factors. Firstly, heightened awareness and improved diagnostic capabilities have facilitated earlier and more precise diagnoses, broadening the patient pool. Consequently, pharmaceutical firms have directed investments towards research and development endeavors aimed at creating new therapies, encompassing both enzyme replacement and gene-based approaches. Regulatory bodies have also played a pivotal role in shaping the market landscape. Streamlined approval pathways and orphan drug designations have incentivized pharmaceutical companies to pursue Pompe disease treatments. These initiatives, coupled with incentives for rare disease research, have cultivated a more competitive environment.

Nevertheless, several challenges hinder the growth of the Pompe disease market. Foremost among these is the substantial cost associated with developing and producing therapies for rare diseases like Pompe. The limited patient population and specialized nature of these treatments translate into high price points, posing obstacles in terms of patient accessibility and reimbursement. Another impediment stems from the complex nature of the disease itself. Pompe disease displays significant variability in clinical presentation and progression, making the development of standardized treatment approaches challenging. While the goal is to tailor

therapies to individual patients, this aspiration presents technical and regulatory complexities.

Moreover, there is a need to raise awareness not only among the general populace but also within the medical community. Many instances of Pompe disease are initially misdiagnosed due to its rarity, leading to delays in appropriate treatment. Additionally, in certain regions, regulatory barriers may impede the introduction of innovative therapies. Local approval processes and reimbursement policies may not be adequately structured to accommodate rare diseases, thereby posing obstacles to market expansion.

Scope of the Pompe Disease Market Report

Study Period: 2019–2032

Coverage: 7MM [The United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom), and Japan]

Key Pompe Disease Companies: Genzyme, Sanofi, Amicus Therapeutics, Actus Therapeutics, Valerion Therapeutics, Astellas Therapeutics, Roche, Lacerta Therapeutics, and others

Key Pompe Disease Therapies: AT-GAA, SPK-3006, 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

Table of Contents

1. Pompe Disease Market Report Introduction
2. Executive Summary for Pompe Disease
3. SWOT analysis of Pompe Disease
4. Pompe Disease Patient Share (%) Overview at a Glance
5. Pompe Disease Market Overview at a Glance
6. Pompe Disease Disease Background and Overview
7. Pompe Disease Epidemiology and Patient Population
8. Country-Specific Patient Population of Pompe Disease
9. Pompe Disease Current Treatment and Medical Practices
10. Pompe Disease Unmet Needs
11. Pompe Disease Emerging Therapies
12. Pompe Disease Market Outlook
13. Country-Wise Pompe Disease Market Analysis (2019–2032)
14. Pompe Disease Market Access and Reimbursement of Therapies
15. Pompe Disease Market Drivers

- 16. Pompe Disease Market Barriers
- 17. Pompe Disease Appendix
- 18. Pompe Disease Report Methodology
- 19. DelveInsight Capabilities
- 20. Disclaimer
- 21. About DelveInsight

Related Reports:

Pompe Disease Pipeline

"Pompe Disease Pipeline Insight, 2024" report by DelveInsight outlines comprehensive insights of present clinical development scenarios and growth prospects across the Pompe Disease market. A detailed picture of the Pompe Disease pipeline landscape is provided, which includes the disease overview and Pompe Disease treatment guidelines.

Pompe Disease Epidemiology

DelveInsight's 'Pompe Disease Epidemiology Forecast to 2032' report delivers an in-depth understanding of the disease, historical and forecasted Pompe Disease epidemiology in the 7MM, i.e., the United States, EU5 (Germany, Spain, Italy, France, and the United Kingdom), and Japan.

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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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