Pompe Disease Market - Global Outlook by 2030 by Materials, Applications and Regions by Emergen Research

Increasing prevalence of Pompe Disease is one of the key factors driving Pompe Disease market revenue growth

Emergen Research Logo

VANCOUVER, BC, CANADA, June 22, 2022 /EINPresswire.com/ -- The global Pompe Disease market size reached USD 1,386.09 Million in 2021 and is expected to register a revenue CAGR of 3.9% during the forecast period, according to a latest analysis by Emergen Research. Increasing prevalence of Pompe Disease, rising number of clinical studies and trials in the field of Pompe-disease-based treatments, and introduction of novel and advanced screening platforms for early diagnosis are crucial factors driving market revenue growth. Pompe disease is a hereditary, frequently fatal ailment that affects the heart and skeletal muscles. It is thought to affect one in every 40,000 births, according to the National Institute of Neurological Disorders and Stroke. Mutations in a gene that produces an enzyme called acid alpha-glucosidase cause it (GAA). Many researchers are trying to better understand the earliest features and manifestations of late-onset Pompe disease novel screening platforms such as newborn screening.

> Pompe Disease Market Size – USD 1,386.09 Million in 2021, Market Growth – at a CAGR of 3.9%, Market Trends – Rising number of clinical studies and trials in the field of Pompe-disease-based treatments"
>
> *Emergen Research*

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Key Players operating in the industry are:

Sanofi S.A., Amicus Therapeutics, Inc., BioMarin Pharmaceutical, Valerion Therapeutics, Oxyrane, Genzyme Corporation, EpiVax, Inc., Bayer AG, Audentes Therapeutics, and Sangamo Therapeutics, Others

According to the Muscular Dystrophy Association (MDA), 20 states are now testit newborns through newborn screening platforms for Pompe Disease, the latest neuromuscular disease, which is being added to a list of significant genetic disorders for which children can be tested shortly after delivery in the United States. We are investigating if starting people early on the right dose of ERT could impact outcomes. Targeted screening of at-risk individuals, as well as general newborn screening, can lead to early detection and treatment of Pompe disease patients, potentially improving clinical outcomes. Other advanced and creative diagnostic systems, such as enzyme activity assessment using a tandem-mass-spectrometry-based assay for Pompe disease, are being produced, examined, and validated in large screening programs.

Rapid scientific advances in research and development initiatives for treatment of Pompe disease have facilitated sparking significant efforts for their translation into the clinical field. The number of clinical and experimental or research treatments for Pompe disease in various phases of development is steadily increasing. By June 2022, ClinicalTrials.gov includes 12 ongoing clinical research trials for the treatment of Pompe illness. In recent decades, a number of possible treatments for Pompe disease have entered preclinical and clinical development, with a few next-generation ERTs currently in late-stage clinical studies. Many researchers have been studying Pompe disease therapeutic treatment approaches for years, and they have seen the transition from understanding the disease\'s natural history and pathophysiology to recognizing the promise of next-generation medicines, such as second-generation ERTs and gene therapy. These are the key factors, which is significantly contributing to revenue growth of the market.

However, insufficient research funding for such rare diseases as well as delayed clinical diagnosis of Pompe disease, which can take months or even years, are hindering revenue growth of this market. Furthermore, conventional screening methods present a number of drawbacks, including false-positive tests and delayed results, to name a few. These are some of the factors, which is hindering revenue growth of the market.

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Some Key Highlights From the Report

The Chaperone Advanced Replacement Therapy (CHART) segment accounted for a moderate revenue share in 2021. Chaperone therapy for Pompe disease entails the use of small compounds that bind to the defective GAA enzyme, assisting it in assembling correctly and restoring normal enzyme activities. The use of chaperone therapy as an alternative treatment for Pompe\'s illness is being investigated. It employs small-molecule ligands that attach directly to the faulty enzyme, allowing it to fold in the most stable conformation possible. Many companies are actively assessing and analyzing the clinical efficacy of this therapy, which is undergoing clinical development. For instance, Amicus Therapeutics\' application for AT-GAA to be licensed as a therapeutic for late-onset Pompe illness is now being evaluated by the U.S. Food and Drug Administration (FDA) (LOPD). AT-GAA is a two-part therapy that combines cipaglucosidase alfa, a

man-made, enhanced variant of the GAA enzyme, with miglustat, a pharmacological chaperone. Val-1221, a version of GAA coupled with the proprietary delivery antibody 3E10, is being developed by Valerion. This is a sort of pharmacological chaperone therapy that aims to get the GAA enzyme into the cells where it\'s needed. A Phase 1/2 clinical investigation for Val-1221 is currently underway (NCT02898753). These are the major factors driving revenue growth of the segment.

The hospitals segment accounted for a significant revenue share in 2021. Many hospitals are working on novel and improved ways to facilitate in the early detection of Pompe disease, with the goal of quickly transferring patients with mild symptoms to expert centers. Patients would be able to be properly tested and diagnosed, allowing them to begin treatment as soon as possible. In addition, hospital doctors and medical specialists are providing individualized methods for all forms of Pompe disease, including Enzyme Replacement Therapy (ERT) and additional supportive drugs that address the condition\'s symptoms and problems.

The market in Europe accounted for a moderate revenue share in 2021. This is attributed to increased funding and partnerships between major companies to conduct clinical studies on the effectiveness of novel therapeutic options for the treatment of Pompe disease is another factor, which is expected to significantly contribute to revenue growth of the region. On 9 January 2022 for instance, Hansa and AskBio signed an agreement for evaluating the potential application of imlifidase as a pre-treatment just before the administration of AskBio’s investigational gene therapy in Pompe disease in a preclinical and clinical feasibility program for patients with pre-existing neutralizing antibodies (NAbs). As a part of the partnership, Hansa received a USD 5 million upfront payment, while AskBio was given the exclusive right to negotiate a comprehensive development and commercialization agreement.

On 6 August 2022, Nexviazyme (avalglucosidase alfa-ngpt) was licensed by the FDA for intravenous infusion to treat individuals with late-onset Pompe illness aged one year and older. This application received Fast Track, Priority Review, and Breakthrough Therapy designations from the FDA.

To know more about the report, visit @ https://www.emergenresearch.com/industry-report/pompe-disease-market

Emergen Research has segmented global Pompe Disease market on the basis of type, treatment type, end-use and region:

Type Outlook (Revenue, USD Million; 2019-2030)

Infantile-Onset Pompe Disease (IOPD)

Late-Onset Pompe Disease (LOPD)

Treatment Type Outlook (Revenue, USD Million; 2019-2030)

Enzyme Replacement Therapy (ERT)

Chaperone Therapy

Substrate Replacement Therapy (SRT)

Others

End-Use Outlook (Revenue, USD Million; 2019-2030)

Hospitals

Diagnostic Centers

Others

Regional Outlook (Revenue, USD Million; 2019-2030)

North America

U.S.

Canada

Mexico

Europe

Germany

France

U.K.

Italy

Spain

Benelux

Rest of Europe

Asia Pacific

China

India

Japan

South Korea

Rest of APAC

Latin America

Brazil

Rest of LATAM

Middle East & Africa

Saudi Arabia

U.A.E.

South Africa

Turkey

Rest of MEA

Major benefits of the Pompe Disease report:

The report discusses in detail the changing dynamics of the competitive landscape

The report provides detail-oriented futuristic prospects of factors driving the growth of the market and limitations affecting the market growth

The report gives a comprehensive analysis of the changing dynamics of the market owing to the current scenario

The report encompasses a detailed forecast for the years 2021-2030

The report provides valuable insights on key market growth driving trends and monetary competence in the forecast timeline

The report also provides a detailed analysis of consumption and market value depending on each geographical region. The report aims to provide beneficial information that might help in formulating new business strategies and expansion plans. SWOT analysis is also offered in this report. Research findings and conclusions are offered through detailed graphs, tables, charts, and figures.

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About Emergen Research

At Emergen Research, we believe in advancing with technology. We are a growing Marketresearch and strategy consulting company with an exhaustive knowledge base of cutting-edge and potentially market-disrupting technologies that are predicted to become more prevalent in the coming decade.

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