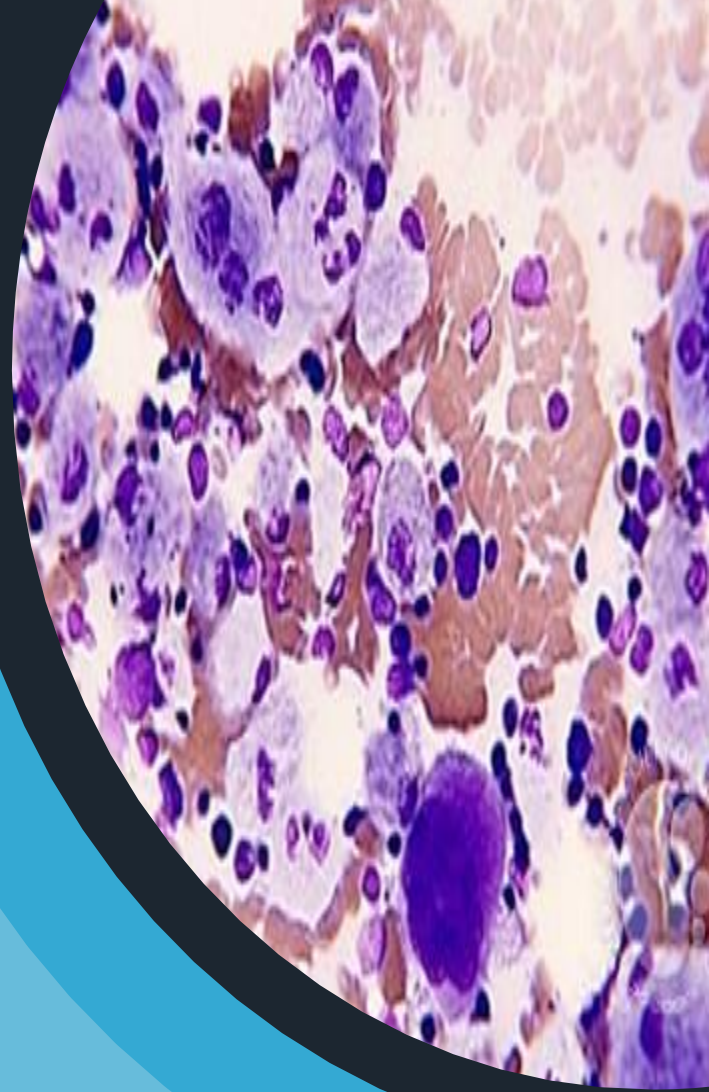




EffeMarket



Gaucher Disease Market Report (7MM), 2026

July 2018

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Executive Summary

Gaucher Disease is the rare multi-systemic metabolic disorder occur due to the defective and insufficient activity of the enzyme glucocerebrosidase. It leads to the accumulation of substrate, glucocerebroside in tissue macrophages and damage to hematological, visceral and bone systems. Painless splenomegaly, anemia, thrombocytopenia, chronic fatigue, hepatomegaly, bone pain, pathologic fractures and others are the most frequent manifestations of Gaucher Disease.

Gaucher Disease is classified as Type 1 Gaucher Disease, Type 2 Gaucher Disease and Type 3 Gaucher Disease based on clinical subtypes. The United States accounts for the highest prevalence of 8,000 cases followed by EU5 (the United Kingdom, Germany, France, and Italy) and Japan. Type 1 Gaucher Disease is more prevalent in comparison to Type 2 Gaucher Disease and Type 3 Gaucher Disease.

Market size of Gaucher Disease in seven major markets is expected to reach USD 1,456 Million in 2026. The United States accounts for the highest market share in comparison to EU5 (United Kingdom, Germany, France and Italy) and Japan owing to the highest prevalent cases, increasing research activities and improved healthcare resources. Among the European countries, Germany accounts for the highest market share followed by France.

Elelyso (taliglucerase alfa), Vpriv (velaglucerase alfa) and Cerezyme (imiglucerase) are the approved enzyme replacement therapy whereas miglustat (Zavesca) and eliglustat (Cerdelga) are the approved substrate reduction therapy for the treatment of Type 1 Gaucher Disease. Use of enzyme replacement therapy has resulted in improving anemia and low platelet counts, reducing enlargement of liver and spleen among the patients. Substrate reduction therapy provides the consistent dosage to patients in comparison to enzyme replacement therapy.

Major players such as Belrose Pharma, Sanofi-Genzyme, AVROBIO, Inc, Lixte Biotechnology Holdings, Inc., Orphazyme A.S and other companies are involved in developing therapies for the treatment of Gaucher Disease. The launch of emerging therapies such as Arimoclomol in 2024 and Venglustat in 2026 shall fuel the market growth. Rising collaborations and agreements between the companies and universities, improved diagnosis and increasing awareness regarding Gaucher Disease would have a positive impact on the market during the forecasted period.

Several factors such as less number of late-stage molecules, lack of awareness among the patients and physicians, high treatment cost, limited access to treatment and approval of generics of marketed therapies are hindering the market growth.

The forecasted market is expected to witness the slow growth due to the less number of late-stage therapies in clinical trials. A large number of therapies are present in pre-clinical and discovery stage of development, which would take several years for the approval in the market. Limited availability of treatment, inaccessibility of certain disease sites and emerging disease manifestations are contributing to the unmet needs of patients. There is immense need of developing novel therapies for the treatment of Type 2 Gaucher Disease and Type 3 Gaucher Disease.

The leading players in the market are Sanofi-Genzyme, Shire Pharmaceuticals, Actelion Pharmaceuticals (Acquired by Johnson & Johnson), and Pfizer. The other prominent players are Belrose Pharma and Orphazyme A.S.

Key Questions Answered

- Prevalent cases of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Sub-type specific prevalent cases of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Diagnosed cases of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Age-Specific diagnosed cases of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Market size of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Therapy wise market size of Gaucher Disease in the US, EU5, and Japan; 2016-2026
- Product Profile of Current Therapies: Cerdelga, Cerezyme, Vpriv, Zavesca, and Elelyso
- Marketed drug analysis of Gaucher Disease
- Pipeline product analysis of Gaucher Disease
- Detailed analysis of growth drivers, and challenges
- Detailed Company Profiling of Key Market Players: Pfizer, Sanofi, Shire Pharmaceuticals, and Johnson & Johnson

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