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# Beta Thalassemia Market Assessment, By Type [Beta Thalassemia Minor, Beta Thalassemia Intermedia, Beta Thalassemia Major], By Therapy [Blood Transfusions, Iron Chelation Therapy, Folic Acid Supplements, Gene Therapy, Bone Marrow Transplants], By Distribution Channel [Hospital Pharmacies, Retail Pharmacies, Online Pharmacies], By Region, Opportunities and Forecast, 2017-2031F

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## Abstracts

Global beta thalassemia market is projected to witness a CAGR of 7.75% during the forecast period 2024-2031F, growing from USD 503 million in 2023, to USD 913.92 million in 2031. The market has experienced significant growth in recent years and is expected to maintain a strong pace of expansion in the coming years.

Beta thalassemia is the most common autosomal recessive disorder inherited from parents and characterized by a reduced production of functional hemoglobin due to a mutation in the HBB gene that leads to the deficient or absent synthesis of beta-globin chains. Based on the severity, the disorder is categorized into minor, intermediate, and major types of beta thalassemia. A variety of therapies like blood transfusion, iron chelation therapy, gene therapy, and bone marrow therapy treat the beta thalassemia condition. Bone marrow transplants are most common in patients under the age of 16 years.

The beta thalassemia marktherapies andly influenced by the growing prevalence of beta thalassemia, escalated advancement in therapies, and increasing demand for point-of-care testing. Growing healthcare infrastructure and increased healthcare spending in

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recent years have significantly supported the beta thalassemia market. Additionally, government initiatives to reduce the blood-related disease burden have positively impacted the demand for relevant products.

Beta thalassemia is most common in low-income regions like Mediterranean, Southeast Asia, the Indian subcontinent, and the Middle East. The migration of people from these regions to high-income countries like the United States has significantly changed the prevalence, for example, the prevalence of beta thalassemia has increased by 7.5% in the last 50 years despite the low number of reported cases. Countries like Italy implemented specific policies for reducing hemoglobinopathies, these initiatives are anticipated to impact the global beta thalassemia market during the assessment period. Alongside, FDA approval of beta thalassemia therapies in recent times has developed healthy competition among the key players.

In January 2024, Vertex Pharmaceuticals Incorporated announced that U.S. Food and Drug Administration (FDA) approved its CASGEVY (exagamglogene autotemcel [exa-cel]), a CRISPR/Cas9 gene-edited cell therapy. The therapy is meant for treating transfusion-dependent beta thalassemia (TDT) in patients above the age of 12 years. The therapy is available in the United States for now in hospitals with expertise in stem cell transplantation. CASGEVY has shown a 91.4% success rate in patients with TDT.

Growing Prevalence of Beta Thalassemia

Beta thalassemia has a significant prevalence in South Asia, Middle East, North Africa, and Southern Europe. Population migration is changing the global distribution of disease. The flow of refugee populations from war-affected zones, like Syria and Iraq, is contributing to the disturbance of disease statistics, but beta thalassemia is considered a rare disease. The global prevalence of beta thalassemia is increasing due to a high carrier rate, migration trends, and cultural preference for consanguineous marriages. With the increasing prevalence of beta thalassemia, the demand for its treatment is anticipated to rise during the forecast period.

As per ‘Global Thalassemia Review - 2023’ published by Thalassemia International Federation estimates, 5-7% of the global population is the carrier of mutated genes affecting the production or function of the hemoglobin in the body. It suggests that over 330,000 affected infants are born annually out of which 83% have sickle cell disorders and 17% are thalassemia (counting around 56,000 infants).

Escalated Advancement in Therapies

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Conventional therapies for beta thalassemia, such as transfusion and iron chelation, have several limitations, however, recent advancements in beta thalassemia therapies have led to improved therapies that can be categorized into three major categories, correction of the globin chain imbalance, addressing ineffective erythropoiesis, and improving iron overload. These emerging therapies include investigational erythroid maturation agents, gene therapy, and embryonic-like stem cells obtained by somatic cell nuclear transfer. Ongoing clinical trials of gene editing and agents modulating iron metabolism promise new improvements. Patients with beta thalassemia have several therapeutic options nowadays, further strengthening the beta thalassemia market.

For example, in August 2023, the U.S. FDA approved Bristol Myers Squibb's Reblozyl (luspatercept-aamt) for providing first-line treatment of anemia in adults with lower-risk myelodysplastic syndromes requiring transfusions. Reblozyl is commercialized through global collaboration between Bristol Mayer Squibb and Merck since November 2021. With this approval, Reblozyl is now approved in the United States for patients of anemia, beta thalassemia, and myelodysplastic syndromes.

Focus of Regulatory Bodies to Catalyze the Market

Regulatory bodies play a crucial role in catalyzing the beta thalassemia market by evaluating and approving novel therapies. The focus of regulatory bodies on emerging treatments can improve the availability of novel treatment options for patients. For instance, the approval of gene therapies such as ZYNTEGLO, which introduces functional, engineered copies of beta-globin genes, represents a significant milestone in the treatment of beta thalassemia. Additionally, the regulatory support for novel genetic therapies and the combination of CRISPR/Cas9 and induced pluripotent stem cell (IPSC) technologies in gene therapy demonstrate the potential for regulatory bodies to foster advancements in the field. By providing a clear pathway for developing and approving these innovative therapies, regulatory bodies can improvise the research and development, ultimately expanding the treatment landscape for beta thalassemia.

For example, in March 2023, Bristol Myers Squibb received European Commission approval for Reblozyl (luspatercept) for anemia in adult patients with non-transfusion-dependent beta thalassemia. After the approval, Reblozyl is approved and available in the European Union (EU), the United States, and Canada for treating beta thalassemia patients. The European Commission approved it based on the phase 2 BEYOND study, which proved the efficacy and safety of Reblozyl versus placebo in 145 patients.

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## Blood Transfusion Segment Dominate the Market

Blood transfusion is the most common type of therapy for beta thalassemia. In more severe cases, such as beta thalassemia major, some patients may require periodic blood transfusions known as transfusion-dependent thalassemia. Blood transfusions help to increase the number of red blood cells in the body and improve oxygen delivery to the tissues. However, regular blood transfusions can lead to iron overload, which can damage vital organs like the liver, heart, and pancreas, but is proven effective in beta thalassemia patients. Therefore, iron chelation therapy may be necessary to remove excess iron from the body. Several conjunctive therapies are provided in addition to blood transfusion to increase efficacy. For instance, as per an article published in PubMed in April 2023, the study conducted among transfusion-dependent thalassemia patients showed that conjunctive therapy of hydroxyurea significantly increases the time interval between transfusions as well as improves the hemoglobin level in these patients.

## Hospital Pharmacies are the Leading Distribution Channel

The hospital pharmacies segment is anticipated to be the leading distribution channel for beta thalassemia treatments. Hospitals are major centers for beta thalassemia drugs due to the complex medical management required for this condition. Patients with beta thalassemia often need routine blood transfusions, iron chelation therapy, and potentially curative treatments, which demand extensive medical supervision. Additionally, hospitals are equipped to handle the specialized care and monitoring associated with these treatments, making them primary location for administering and managing beta thalassemia therapies.

## North America Dominates Beta Thalassemia Market

North America is anticipated to be the leading region in the beta thalassemia market with the highest market share. The dominance of North America is governed by factors like the strong presence of major players in the market and is characterized by increasing healthcare expenditure, rising cases of thalassemia, a growing number of research activities, high disposable income of the region, high healthcare expenditure, and government initiatives to reduce hemoglobinopathies. Presently, the most approved gene therapies are approved in the United States and Canada, contributing the major share of revenue to the beta thalassemia market. Asia-Pacific and Middle East and Africa are anticipated to grow with high CAGR due to large opportunities in untapped markets and the rising prevalence of beta thalassemia.

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Future Market Scenario (2024 – 2031F)

Growing prevalence of beta thalassemia due to migration trends and changes in disease distribution patterns are majorly driving the growth in the beta thalassemia market.

Advancements in beta thalassemia therapies such as investigational erythroid maturation agents, gene therapy, and embryonic-like stem cells drastically contribute to the beta thalassemia market.

Regulatory emphasis on the development and approval of novel therapies for beta thalassemia is leading to an increase in the number of commercialized products which is anticipated to contribute towards the future growth of the market.

Blood transfusion segment is anticipated to dominate therapy type in the beta thalassemia market due to high efficacy and cost-effectiveness.

Key Players Landscape and Outlook

Key participants in the beta thalassemia market include Bluebird Bio, Inc., Bristol-Myers Squibb Co., and Vertex Pharmaceuticals Incorporated. The market caters to several pharmaceutical and biotechnology companies and startups. These companies are involved in the development and commercialization of various treatments and therapies for beta thalassemia. The players in this market are actively involved in product development and approvals, collaboration, and other market tactics. Several players have promising product pipelines and therapies under different phases of clinical trials.

For instance, Shanghai BDgene Co., Ltd., Shenzhen Hemogen, Editas Medicine, Inc., Vertex Pharmaceuticals Incorporated, Bristol-Myers Squibb, Editas Medicine, Inc., CorrectSequence Therapeutics Co., Ltd, are some market players that have genetic and biological products under clinical investigation, presently.

In November 2023, MHRA authorized the world's first gene therapy (Casgevy) to cure sickle-cell anemia and transfusion-dependent ?-thalassemia. The therapy was developed by CRISPR therapeutics. Casgevy is the first approved medicine that utilizes the innovative gene-editing technique CRISPR, for which its inventors were awarded

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the Nobel Prize in 2020.

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