

# Beta Thalassemia Market Size in the 7MM is expected to grow by 2032 | DelveInsight

## *Beta Thalassemia Market*

DELHI, DELHI, INDIA, July 5, 2024 /EINPresswire.com/ -- DelveInsight's 'Beta Thalassemia Market Insights, Epidemiology, and Market Forecast—2032' report deliver an in-depth understanding of Beta Thalassemia, historical and forecasted epidemiology as well as the market trends in the United States and EU5 (Germany, Spain, Italy, France, and United Kingdom).



## Beta Thalassemia Market Insights

### Key Takeaways from the Beta Thalassemia Market Report

- The increase in Beta Thalassemia Market Size is a direct consequence of the increasing patient population and anticipated launch of emerging therapies in the 7MM.
- As per DelveInsight analysis, the Beta Thalassemia market is anticipated to witness growth at a considerable CAGR.
- The leading Beta Thalassemia Companies working in the market include Novartis, Merck, Bristol Myers Squibb, Chiesi Farmaceutici S.p.A, Bluebird Bio, Agios Pharmaceuticals, Imara Inc., CRISPR Therapeutics, Vertex Pharmaceuticals, Vifor Pharma, Ionis Pharmaceuticals, Forma Therapeutics, DisperSol Technologies, SILENCE Therapeutics, and others.
- Promising Beta Thalassemia Pipeline Therapies in the various stages of development include SP-420, VIT-2763 once a day (QD), BRL-101, CS-101, and others.
- April 2024: Celgene announced a study of Phase 2 clinical trials for ACE-536. This is a Phase 2a study to evaluate the safety and pharmacokinetics (PK) of luspatercept in pediatric participants with  $\beta$ -thalassemia.

- April 2024: Agios Pharmaceuticals Inc. announced a study of Phase 3 clinical trials for Mitapivat. The primary purpose of this study is to compare the effect of mitapivat versus placebo on transfusion burden in participants with transfusion-dependent alpha- or beta-thalassemia (TDT).
- April 2024: Vertex Pharmaceuticals announced a study of Phase 3 clinical trials for CTX001. This is a single-dose, open-label study in pediatric participants with TDT. The study will evaluate the safety and efficacy of autologous CRISPR-Cas9 modified CD34+ human hematopoietic stem and progenitor cells (hHSPCs) (CTX001).

Discover which therapies are expected to grab the Beta Thalassemia Market Share @ [Beta Thalassemia Market Outlook](#)

### Beta Thalassemia Overview

Beta thalassemia ( $\beta$ -thalassemia) is a blood disorder characterized by reduced synthesis of the hemoglobin subunit beta (hemoglobin beta chain) that results in microcytic hypochromic anemia, an abnormal peripheral blood smear with nucleated red blood cells, and reduced amounts of hemoglobin A (HbA) on hemoglobin analysis.

### Beta Thalassemia Epidemiology Segmentation Insights

The epidemiology section of Beta Thalassemia offers insights into both historical and current patient populations, as well as forecasted trends across seven major countries. This section aids in understanding the factors behind present and projected trends through analysis of various studies and input from key opinion leaders. Additionally, this portion of the market report provides information on the diagnosed patient pool, trends, and underlying assumptions.

- Total Beta Thalassemia Prevalent Cases
- Beta Thalassemia Type-specific Diagnosed Prevalent Cases
- Beta Thalassemia Diagnosed Prevalent Cases
- Beta Thalassemia Treatable Cases

Download the report to understand which factors are driving Beta Thalassemia Epidemiology trends @ [Beta Thalassemia Prevalence](#)

### Beta Thalassemia Market Insights

To counter unmet market needs and provide better treatment choices for beta thalassemia, several market players are working robustly either on single-agent novel molecules or on combination and dose modification of standard therapies. Several therapies are expected to be launched in the study period, likely to drive market growth during the study period.

### Beta Thalassemia Market Dynamics

The Beta Thalassemia market is anticipated to be driven in the coming years owing to the launch of emerging therapies and improvement in the research and development activities.

The participation of the key players such as Agios Pharmaceuticals, Vertex Pharmaceuticals, Pharmacosmos, Editas Medicine, and others will bring a change in the market dynamics during the forecast period (2023–2032)

### Beta Thalassemia Drugs Uptake

- PYRUKYND (mitapivat), developed by Agios Pharmaceuticals, is an oral, smallmolecule, allosteric activator of the RBC-specific form of pyruvate kinase (PK-R). It is the first FDA-approved disease-modifying therapy for pyruvate kinase deficiency. Pyruvate kinase-R is a key enzyme for maintaining energy homeostasis in RBCs. In healthy adults, mitapivat activates wild-type pyruvate kinase-R and increases ATP levels in RBCs. Currently, the company is evaluating mitapivat in two Phase III ENERGIZE and ENERGIZE-T (NCT04770753 and NCT04770779) studies in adults with non-transfusiondependent and transfusion-dependent  $\alpha$ - or  $\beta$ -thalassemia, respectively.
- Exagamglogene autotemcel (exa-cel), formerly known as CTX001, is an investigational, autologous, ex vivo CRISPR/Cas9 gene-edited therapy which aims to edit a person's hematopoietic stem cells to produce fetal hemoglobin (hemoglobin F). Vertex Pharmaceuticals is collaborating with CRISPR Therapeutics to investigate the use of a gene-editing technology, known as CRISPR/Cas9, to discover and develop a potential one-time treatment for transfusion-dependent beta thalassemia. HbF is a form of the oxygen-carrying hemoglobin that is naturally present at birth, which then switches to the adult form of hemoglobin. In June 2023, FDA accepted the BLA of exagamglogene autotemcel (exa-cel) for transfusion-dependent beta thalassemia (TDT). The PDUFA target action date for TDT is March 30, 2024. The company is currently conducting trials in Phase III (NCT05356195, NCT05477563) and Phase II/III (NCT03655678) to evaluate CTX001 in adults and pediatric patients with transfusiondependent  $\beta$ -Thalassemia.
- SP-420 belongs to a novel class of orally active iron chelators called desferrithiocins. The drug has the ability to cross the blood-retinal and blood-brain barriers, requisite for retinal and neurological indications. In November 2021, Pharmacosmos acquired AbFero Pharmaceuticals and continued with the development of SP-420 in order to bring new treatment options for patients suffering from transfusional iron overload and other iron mediated. Recently, in September 2023, Pharmacosmos has initiated a Phase II trial (NCT05693909) of SP-420 in patients with transfusion-dependent  $\beta$ -thalassemia.
- EDIT-301, developed by Editas Medicine, is an experimental gene editing medicine that consists of patient-derived CD34+ hematopoietic stem and progenitor cells edited at the gamma globin gene (HBG1 and HBG2) promoters. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin production, which has the potential to provide a one-time, durable treatment benefit for people living with severe SCD and TDT. Currently, the drug is being studied in a Phase I/II trial (NCT05444894) in adult participants with transfusion dependent beta thalassemia.

To know more about Beta Thalassemia treatment guidelines, visit @ [New Beta Thalassemia Drugs](#)

### Beta Thalassemia Market Outlook

The report's outlook on the Beta Thalassemia market aids in developing a comprehensive understanding of historical, current, and projected trends. This is achieved by examining the influence of existing Beta Thalassemia therapies, unmet needs, as well as drivers, barriers, and the demand for advanced technology. This section provides detailed insights into the trends of each marketed Beta Thalassemia drug and late-stage pipeline therapy. It assesses their impact based on various factors such as annual therapy costs, inclusion/exclusion criteria, mechanism of action, compliance rates, market demand, patient population growth, covered patient segments, anticipated launch year, competition with other therapies, brand value, and input from key opinion leaders. The analyzed Beta Thalassemia market data are presented concisely through relevant tables and graphs to offer a clear overview of the market dynamics.

### Beta Thalassemia Companies

Several companies working in the market include Novartis, Merck, Bristol Myers Squibb, Chiesi Farmaceutici S.p.A, Bluebird Bio, Agios Pharmaceuticals, Imara Inc., CRISPR Therapeutics, Vertex Pharmaceuticals, Vifor Pharma, Ionis Pharmaceuticals, Forma Therapeutics, DisperSol Technologies, SILENCE Therapeutics, and others.

Learn more about the FDA-approved drugs for Beta Thalassemia @ [Drugs for Beta Thalassemia Treatment- https://www.delveinsight.com/sample-request/beta-thalassemia-market-forecast?utm\\_source=einpresswire&utm\\_medium=pressrelease&utm\\_campaign=ypr](#)

### Scope of the Beta Thalassemia Market Report

- Coverage- 7MM
- Study Period- 2019-2032
- Beta Thalassemia Companies- Novartis, Merck, Bristol Myers Squibb, Chiesi Farmaceutici S.p.A, Bluebird Bio, Agios Pharmaceuticals, Imara Inc., CRISPR Therapeutics, Vertex Pharmaceuticals, Vifor Pharma, Ionis Pharmaceuticals, Forma Therapeutics, DisperSol Technologies, SILENCE Therapeutics, and others
- Beta Thalassemia Pipeline Therapies- SP-420, VIT-2763 once a day (QD), BRL-101, CS-101, and others.
- Beta Thalassemia Market Dynamics: Beta Thalassemia Market Drivers and Barriers
- Beta Thalassemia Market Access and Reimbursement, Unmet Needs, KOL's Views, and Analyst's Views

Discover more about Beta Thalassemia Drugs in development @ [Beta Thalassemia Clinical Trials Assessment- https://www.delveinsight.com/sample-request/beta-thalassemia-market-forecast?utm\\_source=einpresswire&utm\\_medium=pressrelease&utm\\_campaign=ypr](#)

## Table of Content

1. Key Insights
2. Executive Summary
3. Competitive Intelligence Analysis
4. Beta-thalassemia: Market Overview at a Glance
5. Beta-thalassemia: Disease Background and Overview
6. Patient Journey
7. Beta-thalassemia Epidemiology and Patient Population
8. Treatment Algorithm, Current Treatment, and Medical Practices
9. Beta-thalassemia Unmet Needs
10. Key Endpoints of Beta-thalassemia Treatment
11. Beta-thalassemia Marketed Products
12. Beta-thalassemia Emerging Therapies
13. Beta-thalassemia: Seven Major Market Analysis
14. Attribute analysis
15. 7MM: Beta Thalassemia Market Outlook
16. Access and Reimbursement Overview of Beta-thalassemia
17. KOL Views
18. Beta Thalassemia Market Drivers
19. Beta Thalassemia Market Barriers
20. Appendix
21. DelveInsight Capabilities
22. Disclaimer
23. About DelveInsight

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