

Thalassemia Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

<https://marketpublishers.com/r/T16544CCA89DEN.html>

Date: April 2022

Pages: 156

Price: US\$ 2,000.00 (Single User License)

ID: T16544CCA89DEN

Abstracts

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SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide *Thalassemia - Drugs In Development, 2022*, provides an overview of the Thalassemia (Hematological Disorders) pipeline landscape.

Thalassemia is a group of inherited blood disorders that affect the body's ability to produce hemoglobin and red blood cells. Symptoms include fussiness, paleness, frequent infections, failure to thrive, poor appetite and jaundice. Predisposing factors include family history. Treatment includes blood transfusions and bone marrow transplant.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide *Thalassemia - Drugs In Development, 2022*, provides comprehensive information on the therapeutics under development for Thalassemia (Hematological Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Thalassemia (Hematological Disorders) pipeline guide also reviews of key players involved in therapeutic development for Thalassemia and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II, Phase I, Phase 0, IND/CTA Filed, Preclinical and Discovery stages are 2, 2, 15, 8, 1, 2, 22 and 10 respectively. Similarly, the Universities portfolio in Phase II, Phase 0, Preclinical and Discovery stages comprises 1, 1, 3 and 1 molecules, respectively.

Thalassemia (Hematological Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

Note: Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Thalassemia (Hematological Disorders).

The pipeline guide reviews pipeline therapeutics for Thalassemia (Hematological Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Thalassemia

(Hematological Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Thalassemia (Hematological Disorders) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Thalassemia (Hematological Disorders)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Thalassemia (Hematological Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Thalassemia (Hematological Disorders) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand

business potential and scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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Featured News & Press Releases

Apr 12, 2022: San Rocco Therapeutics pushes clinical trial for a curative treatment for beta-thalassemia and sickle cell disease

Mar 25, 2022: Bristol Myers Squibb announces new Prescription Drug User Fee Act goal date for Reblozyl (luspatercept-aamt) supplemental biologics license application

Jan 25, 2022: Sickle cell disease and transfusion-dependent beta thalassemia: promising results of gene therapy treatment

Jan 18, 2022: bluebird provides update on FDA review timelines for Betibeglogene Autotemcel (beti-cel) for beta-thalassemia

Dec 20, 2021: Editas Medicine announces FDA clearance of investigational new drug (IND) application for EDIT-301 for the treatment of transfusion-dependent beta thalassemia

Dec 14, 2021: Imara presents preclinical data on IMR-261 at the American Society of Hematology (ASH) Annual Meeting 2021

Dec 13, 2021: Agios Presents mitapivat data highlighting long-term safety profile and durable improvement in hemoglobin and markers of hemolysis in non-transfusion-dependent α - and β -thalassemia at 63rd ASH Annual Meeting and Exposition

Dec 12, 2021: New data from SLN124 healthy volunteer study reinforce broad

therapeutic potential in hematological diseases

Dec 11, 2021: New data at ASH21, published in NEJM further demonstrate beti-cel as a potentially curative one-time gene therapy for β -thalassemia patients who require regular transfusions through achievement of durable TI and normal or near-normal adult Hb levels

Dec 06, 2021: Fulcrum Therapeutics announces additional HBG mRNA induction from higher dose cohorts in phase 1 healthy adult volunteer trial of FTX-6058 for sickle cell disease and new preclinical mechanism data

Dec 03, 2021: U.S. Food and Drug Administration accepts for priority review supplemental biologics license application for Reblozyl (luspatercept-aamt) in adults with non-transfusion dependent (NTD) beta thalassemia

Nov 22, 2021: bluebird bio announces FDA priority review of biologics license application for beti-cel gene therapy for patients with β -thalassemia who require regular red blood cell transfusions

Nov 04, 2021: Imara to present preclinical data at the American Society of Hematology (ASH) Annual Meeting 2021

Nov 04, 2021: EdiGene to present latest research on a novel surface marker and migration of hematopoietic stem cell (HSC) that could enhance HSC gene therapy and HSC transplantation at the 63rd American Society of Hematology (ASH) Annual Meeting and Exposition

Nov 04, 2021: Editas Medicine to present data demonstrating progress towards transformative gene editing medicines for the treatment of hemoglobinopathies and cancer at the ASH Annual Meeting and Exposition

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