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Beta-thalassaemia - Pipeline Insight, 2021

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Abstracts

This report can be delivered to the clients within 3-4 Business Days

DelveInsight's, "Beta-thalassaemia - Pipeline Insight, 2021," report provides comprehensive insights about 22+ companies and 22+ pipeline drugs in Beta-thalassaemia pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Beta-thalassaemia Understanding

Beta-thalassaemia: Overview

Beta thalassemia is an inherited blood disorder characterized by reduced levels of functional hemoglobin. Hemoglobin is found in red blood cells; it is the red, iron-rich, oxygen-carrying pigment of the blood. A main function of red blood cells is to deliver oxygen throughout the body. Beta thalassemia has three main forms – minor, intermedia and major, which indicate the severity of the disease. Individuals with beta thalassemia minor usually do not have any symptoms (asymptomatic) and individuals often are unaware that they have the condition. Some individuals do experience a very mild anemia. Individuals with beta thalassemia major have a severe expression of the disorder; they often require regular blood transfusions and lifelong, ongoing medical care. The symptoms of beta thalassemia intermedia are widely variable and severity

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falls in the broad range between the two extremes of the major and minor forms. The characteristic finding of beta thalassemia is anemia, which is caused because red blood cells are abnormally small (microcytic), are not produced at the normal amounts, and do not contain enough functional hemoglobin.

'Beta-thalassaemia - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Beta-thalassaemia pipeline landscape is provided which includes the disease overview and Beta-thalassaemia treatment guidelines. The assessment part of the report embraces, in depth Beta-thalassaemia commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Beta-thalassaemia collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

> The companies and academics are working to assess challenges and seek opportunities that could influence Beta-thalassaemia R&D. The therapies under development are focused on novel approaches to treat/improve Beta-thalassaemia.

Beta-thalassaemia Emerging Drugs Chapters

This segment of the Beta-thalassaemia report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Beta-thalassaemia Emerging Drugs

> CTX001: CRISPR Therapeutics

As a therapy, CTX001 involves isolating a patient’s own blood stem cells, editing them with CRISPR/Cas9 to increase HbF expression, and then returning the edited cells to the patient. We believe that over time these edited blood stem cells will generate red

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blood cells that have increased levels of HbF, which may reduce or eliminate patients' symptoms. In 2017, CRISPR therapeutics signed an agreement to co-develop and co-commercialize this program with Vertex Pharmaceuticals. The drug is in Phase I/II clinical evaluation for the treatment of ?-thalassemia. CTX001 has been designated an orphan drug in the U.S. and Europe, and given fast track, rare pediatric disease, and regenerative medicine advanced therapy designations in the U.S. for SCD and transfusion-dependent Beta thalassemia (B-thal) (TDT).

IMR-687: Imara, Inc.

IMR-687 is a highly selective and potent small molecule inhibitor of PDE9. PDE9 selectively degrades cyclic guanosine monophosphate (cGMP), an active signaling molecule that plays a role in vascular biology. Lower levels of cGMP are found in people with SCD and beta-thalassemia and are associated with reduced blood flow, increased inflammation, greater cell adhesion and reduced nitric oxide mediated vasodilation.

Further product details are provided in the report……..

Beta-thalassaemia: Therapeutic Assessment

This segment of the report provides insights about the different Beta-thalassaemia drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Beta-thalassaemia

There are approx. 22+ key companies which are developing the therapies for Beta-thalassaemia. The companies which have their Beta-thalassaemia drug candidates in the most advanced stage, i.e. phase II include, Imara, Inc.

Phases

DelveInsight's report covers around 22+ products under different phases of clinical development like

Late stage products (Phase III)

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Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Beta-thalassaemia pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Oral

Parenteral

intravenous

Subcutaneous

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

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Gene therapy

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Beta-thalassaemia: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Beta-thalassaemia therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Beta-thalassaemia drugs.

Beta-thalassaemia Report Insights

Beta-thalassaemia Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Beta-thalassaemia Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

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Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Beta-thalassaemia drugs?

How many Beta-thalassaemia drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Beta-thalassaemia?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Beta-thalassaemia therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Beta-thalassaemia and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

CRISPR Therapeutics

Imara, Inc.

Sangamo Therapeutics

Vifor Pharma

Bioray Laboratories

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EdiGene (GuangZhou) Inc.

Ionis Pharmaceuticals, Inc.

Agios Pharmaceuticals, Inc.

Silence Therapeutics plc

Aruvant Sciences

Phoenicia Biosciences

San Rocco Therapeutics

Shanghai BDgene

Beam Therapeutics

EmeraMed

Regenacy Pharmaceuticals

Editas Medicine

Fulcrum Therapeutics

Allife Medical Science and Technology

Global Blood Therapeutics, Inc

Orchard Therapeutics

Acceleron Pharma

Disc Medicine

Key Products

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CTX001

IMR-687

ST-400

luspatercept-aamt

VIT-2763

OTL-300

?-globin restored autologous HSC

BEAM 101

Emeramide

FTX 6058

EDIT 301

CRISPR/Cas9 modified human haematopoietic stem cell therapy

Research programme: HDAC1/2 inhibitors

ET-01

IONIS TMPRSS6-LRx

Mitapivat

SLN124

ARU 1801

PB 04

Beta globin transduced bone marrow cells

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BD 211

DISC a

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DISC a: Disc Medicine
  Product Description
  Research and Development

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