

# **Sickle Cell Disease Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update**

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## **Abstracts**

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

### **SUMMARY**

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

Sickle cell anemia is a genetic (inherited) blood disorder in which red blood cells, which carry oxygen around the body, develop abnormally. Signs and symptoms include anemia, delayed growth, vision problems, pain and frequent infections. Treatment includes antibiotics, pain relievers, blood transfusion and stem cell transplant.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Sickle Cell Disease - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Sickle Cell Disease (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 Sickle Cell Disease (Hematological Disorders) pipeline guide also reviews of key

players involved in therapeutic development for Sickle Cell Disease 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, IND/CTA Filed, Preclinical and Discovery stages are 2, 7, 18, 16, 5, 45 and 19 respectively. Similarly, the Universities portfolio in Phase II, Preclinical and Discovery stages comprises 2, 8 and 1 molecules, respectively.

Sickle Cell Disease (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 Sickle Cell Disease (Hematological Disorders).

The pipeline guide reviews pipeline therapeutics for Sickle Cell Disease (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 Sickle Cell Disease (Hematological Disorders) therapeutics and enlists all their major and minor

projects.

The pipeline guide evaluates Sickle Cell Disease (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 Sickle Cell Disease (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 Sickle Cell Disease (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 Sickle Cell Disease (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

Jun 18, 2022: Forma Therapeutics announces presentations at upcoming Hematology Conferences

Jun 11, 2022: Vertex and CRISPR Therapeutics present new data on more patients with longer follow-up treated with exagamglogene autotemcel (exa-cel) at the 2022 European Hematology Association (EHA) Congress

Jun 11, 2022: Vertex and CRISPR Therapeutics announce acceptance of late-breaking abstract for CTX001 at the 2022 Annual European Hematology Association (EHA) Congress

Jun 10, 2022: GBT presents positive new real-world evidence data at EHA2022 Congress further supporting clinical use of Oxbryta (voxelotor) in sickle cell disease

Jun 10, 2022: Fulcrum Therapeutics announces Proof-of-Concept for FTX-6058 in sickle cell disease based on initial data from the ongoing phase 1b trial

Jun 06, 2022: GBT's inclacumab receives U.S. FDA orphan drug and rare pediatric disease designations for the treatment of sickle cell disease

May 12, 2022: Editas Medicine receives FDA Orphan Drug Designation for EDIT-301 for the treatment of beta thalassemia

May 12, 2022: Fulcrum Therapeutics to present initial data from phase 1b trial of

FTX-6058 in adults living with sickle cell disease at the European Hematology Association (EHA) Hybrid Congress in Vienna, Austria

May 12, 2022: Agios to present clinical and translational data at the European Hematology Association Annual Congress

Apr 11, 2022: Emmaus Life Sciences announces launch of full-service telehealth solution

Apr 07, 2022: Emmaus Life Sciences presented positive real-world data on the efficacy of Endari in preventing acute complications from sickle cell disease at the 62nd Annual Scientific Meeting of the British Society for Haematology

Mar 29, 2022: Emmaus Life Sciences announces Endari to be added to the Florida Medicaid Preferred Drug List

Mar 23, 2022: Emmaus Life Sciences receives U.A.E. Marketing Authorization for Endari

Mar 04, 2022: Emmaus Life Sciences' Real World Data on Endari accepted for E-Poster at the 62nd Annual Scientific Meeting of the British Society for Haematology

Mar 02, 2022: Nicox's Partner Fera Pharmaceuticals obtains Orphan Drug Designation from the U.S. FDA for Naproxcinod for the treatment of sickle cell disease

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