

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

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## Abstracts

Gaucher 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 Gaucher Disease - Drugs In Development, 2022, provides an overview of the Gaucher Disease (Genetic Disorders) pipeline landscape.

Gaucher disease is a rare genetic disorder occurs due to lacks an enzyme called glucocerebrosidase. Symptoms include cognitive impairment, enlarged spleen, severe swelling, lung disease, fatigue and seizures. Treatment includes enzyme replacement therapy.

### REPORT HIGHLIGHTS

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

involved in therapeutic development for Gaucher Disease and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase III, Phase II, Phase I, Preclinical and Discovery stages are 2, 8, 1, 23 and 9 respectively. Similarly, the Universities portfolio in Discovery stages comprises 5 molecules, respectively.

Gaucher Disease (Genetic 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 Gaucher Disease (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Gaucher Disease (Genetic 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 Gaucher Disease (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Gaucher Disease (Genetic 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 Gaucher Disease (Genetic 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 Gaucher Disease (Genetic 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 Gaucher Disease (Genetic 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

Dec 07, 2022: AVROBIO to share comprehensive Gaucher disease program update

Oct 27, 2022: AVROBIO receives rare pediatric disease designation from the U.S. FDA for first-in-class gene therapy for Gaucher Disease

Oct 14, 2022: M6P Therapeutics presents Novel AAV Gene Therapy Approach for the Treatment of Gaucher Disease at the ESGCT 29th Annual Congress

May 11, 2022: Gain Therapeutics presents positive preclinical data on its Gaucher Disease program at IWGGD Symposium

Feb 10, 2022: M6P Therapeutics presents promising preclinical data in Gaucher disease at the 18th Annual WORLDSymposium 2022

Feb 08, 2022: Gain Therapeutics presents preclinical data on its structurally targeted allosteric regulators in GBA1 Gaucher Disease at the WORLDSymposium 2022

Feb 08, 2022: Freeline presents on its Gaucher disease AAV-based gene therapies at the 18th Annual WORLDSymposium

Jan 27, 2022: Freeline to present on its gaucher disease AAV-based gene therapies at the 18th Annual WORLDSymposium

Jan 06, 2022: Freeline announces FDA clearance of investigational New Drug Application for FLT201 for Gaucher Disease Type

Nov 08, 2021: AVROBIO to present clinical data on AVRRD-02 at the 14th ICIEM Conference

Oct 19, 2021: AVROBIO reports new interim safety data across investigational gene therapies for Gaucher disease type

Sep 09, 2021: Freeline announces Orphan Drug Designations for FLT201 for the treatment of Gaucher Disease

Feb 08, 2021: Freeline presents data on its Gaucher disease and Fabry disease AAV-based gene therapies at the 17th Annual WORLDSymposium

Feb 01, 2021: AVROBIO announces clinical data presentation on AVRRD-02 at 17th Annual WORLDSymposium 2021

Nov 18, 2020: Prevail Therapeutics granted composition of matter patent for experimental gene therapy program PR001

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