

# Glucosylceramide synthase inhibitors - Pipeline Insight, 2022

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## Abstracts

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DelveInsight's, "Glucosylceramide synthase inhibitors - Pipeline Insight, 2022" report provides comprehensive insights about 5+ companies and 5+ pipeline drugs in Glucosylceramide synthase inhibitors 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

### Glucosylceramide synthase inhibitors Understanding

#### Glucosylceramide synthase inhibitors: Overview

Glucosylceramide synthase (GCS) is a glucosyltransferase that processes the sphingolipid ceramide. The product, glucosylceramide, can be further elaborated with a variety of oligosaccharides to become glycosphingolipids called gangliosides (most notably GM1 and GM3). Excess glycosphingolipids have been correlated with a variety of human diseases. The pathologies associated with lysosomal storage disorders (Gaucher, Fabry, Tay-Sachs, and Sandhoff) stem from improper disposal of glucosylceramide and other glycosphingolipids. Gaucher disease (GD) is caused by a deficiency of glucocerebrosidase and the consequent lysosomal accumulation of

unmetabolized glycolipid substrates. Substrate reduction therapy through inhibition of glucosylceramide synthase (GCS) has also been shown to effectively treat the visceral disease.

## Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Glucosylceramide synthase inhibitors R&D. The therapies under development are focused on novel approaches for Glucosylceramide synthase inhibitors.

## Glucosylceramide synthase inhibitors Emerging Drugs Chapters

This segment of the Glucosylceramide synthase inhibitors report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, 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.

## Glucosylceramide synthase inhibitors Emerging Drugs

### Lucerastat: Idorsia Pharmaceuticals

Lucerastat inhibits glycolipid biosynthesis. Lucerastat is an orally bioavailable inhibitor of glucosylceramide synthase. Lucerastat is being developed by the biopharmaceutical company Idorsia for the treatment of Fabry disease. It is currently in Phase III stage of development. It acts as Glucosylceramide synthase inhibitor. In 2018, Lucerastat has received Orphan Drug designation in the US and in the EU for Fabry disease.

### Venglustat: Sanofi

Venglustat is an investigational oral glucosylceramide synthase inhibitor in development for multiple diseases with disruptions in the GSL metabolic pathway. These diseases include three lysosomal storage disorders: Gaucher disease type 3, Fabry disease and GM2 gangliosidosis as well as autosomal dominant polycystic kidney (ADPKD) disease and GBA-associated Parkinson's disease. The FDA has granted Fast Track

designation for the development of venglustat as an investigational therapy for the treatment of Gaucher disease type 3, Fabry disease and GM2 gangliosidosis. Sanofi has been granted Orphan Drug Designation for venglustat in Europe and the U.S. for treatment of patients with Fabry disease, ADPKD, Gaucher disease and GM2 gangliosidosis.

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

## Glucosylceramide synthase inhibitors: Therapeutic Assessment

This segment of the report provides insights about the different Glucosylceramide synthase inhibitors drugs segregated based on following parameters that define the scope of the report, such as:

### Major Players working on Glucosylceramide synthase inhibitors

There are approx. 5+ key companies which are developing the Glucosylceramide synthase inhibitors. The companies which have their Glucosylceramide synthase inhibitors drug candidates in the most advanced stage, i.e. Phase III include, Idorsia Pharmaceuticals.

### Phases

DelveInsight's report covers around 5+ products under different phases of clinical development like

Late-stage products (Phase III and

Mid-stage products (Phase II and

Early-stage products (Phase I/II and Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Glucosylceramide synthase inhibitors pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Infusion

Intradermal

Intramuscular

Intranasal

Intravaginal

Oral

Parenteral

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Vaccines

Monoclonal Antibody

Peptides

Polymer

Small molecule

## Product Type

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

### Glucosylceramide synthase inhibitors: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Glucosylceramide synthase inhibitors 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 Glucosylceramide synthase inhibitors drugs.

### Glucosylceramide synthase inhibitors Report Insights

Glucosylceramide synthase inhibitors Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

### Glucosylceramide synthase inhibitors Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

## Unmet Needs

### Key Questions

#### Current Scenario and Emerging Therapies:

How many companies are developing Glucosylceramide synthase inhibitors drugs?

How many Glucosylceramide synthase inhibitors drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for Glucosylceramide synthase inhibitors?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Glucosylceramide synthase inhibitors 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 Glucosylceramide synthase inhibitors and their status?

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

### Key Players

Idorsia Pharmaceuticals

Sanofi

### Key Products

Lucerastat

Venglustat

Sinbaglustat

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Lucerastat: Idorsia Pharmaceuticals

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Mid Stage Products (Phase II)

Comparative Analysis

Drug Name: Company Name

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Early Stage Products (Phase I)

Comparative Analysis



Sinbaglustat: Idorsia Pharmaceuticals

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Pre-clinical and Discovery Stage Products

Comparative Analysis

Drug Name: Company Name

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Inactive Products

Comparative Analysis

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