

# Gaucher's Disease - Pipeline Insight, 2021

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## Abstracts

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DelveInsight's, "Gaucher's Disease - Pipeline Insight, 2021," report provides comprehensive insights about 14+ companies and 16+ pipeline drugs in Gaucher's Disease 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

### Gaucher's Disease Understanding

#### Gaucher's Disease: Overview

Gaucher disease (GD) is an inborn error of metabolism that affects the recycling of cellular glycolipids. It is an autosomal recessive inherited disorder of metabolism. The major clinical symptoms of GD include enlargement of the liver and spleen (hepatosplenomegaly), low number of red blood cells (anemia), easy bruising, and bone disease (bone pain and fractures). The diagnosis of GD is based on clinical symptoms and laboratory testing. A diagnosis of Gaucher disease is suspected in individuals who have bone problems, enlarged liver and spleen (hepatosplenomegaly), changes in red blood cell levels, easy bleeding and bruising from low platelets or signs of nervous system problems. Treatment is individualized for each patient depending on the type of Gaucher disease. Enzyme replacement therapy (ERT) has proven effective for

individuals with Gaucher disease type 1.

'Gaucher's Disease - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Gaucher's Disease pipeline landscape is provided which includes the disease overview and Gaucher's Disease treatment guidelines. The assessment part of the report embraces, in depth Gaucher's Disease 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, Gaucher's Disease 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 Gaucher's Disease R&D. The therapies under development are focused on novel approaches to treat/improve Gaucher's Disease.

### Gaucher's Disease Emerging Drugs Chapters

This segment of the Gaucher's Disease 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.

### Gaucher's Disease Emerging Drugs

#### GPH301: Graphite Bio

GPH301 is a next generation gene-edited autologous HSC product candidate leveraging the CCR5 locus technology for the treatment of Gaucher disease. With GPH301, a functional copy of the gene for glucocerebrosidase (GCase) is inserted into the chromosomal location of the CCR5 gene. The drug is in preclinical studies for the treatment of Gaucher's Disease.

## AVR-RD-02: AVROBIO

AVR-RD-02 is an ex vivo lentiviral gene therapy, designed using Avrobio's plato platform. The drug is in Phase I/II clinical studies for the treatment of Gaucher's Disease.

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

### Gaucher's Disease: Therapeutic Assessment

This segment of the report provides insights about the different Gaucher's Disease drugs segregated based on following parameters that define the scope of the report, such as:

#### Major Players in Gaucher's Disease

There are approx. 14+ key companies which are developing the therapies for Gaucher's Disease. The companies which have their Gaucher's Disease drug candidates in the most advanced stage, i.e. Phase I/II include, AVROBIO.

#### Phases

DelveInsight's report covers around 16+ products under different phases of clinical development like

Late stage products (Phase III)

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

Gaucher's Disease 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

Intravitreal

Subretinal

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

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

Gaucher's Disease: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Gaucher's Disease 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 Gaucher's Disease drugs.

### Gaucher's Disease Report Insights

- Gaucher's Disease Pipeline Analysis

- Therapeutic Assessment

- Unmet Needs

- Impact of Drugs

### Gaucher's Disease Report Assessment

- Pipeline Product Profiles

- Therapeutic Assessment

- Pipeline Assessment

- Inactive drugs assessment

- Unmet Needs

### Key Questions

Current Treatment Scenario and Emerging Therapies:

- How many companies are developing Gaucher's Disease drugs?

How many Gaucher's Disease drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Gaucher's Disease?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Gaucher's Disease 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 Gaucher's Disease and their status?

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

## Key Players

CANbridge Life Sciences

AVROBIO

Gain Therapeutics

M6P Therapeutics

Denali Therapeutics

Graphite Bio

Sanofi

Freeline Therapeutics

Orphazyme

Sanofi

Prevail Therapeutics

Yuhan

## Key Products

CAN103

AVR-RD-02

GT-02287

GT-02329

M-011

M-013

ETV:GCase

GPH 301

Venglustat

FLT 201

Arimoclomol

Eliglustat

PR-001

YHC-1116

## Contents

Introduction

Executive Summary

Gaucher's Disease: Overview

Causes

Mechanism of Action

Signs and Symptoms

Diagnosis

Disease Management

Pipeline Therapeutics

Comparative Analysis

Therapeutic Assessment

Assessment by Product Type

Assessment by Stage and Product Type

Assessment by Route of Administration

Assessment by Stage and Route of Administration

Assessment by Molecule Type

Assessment by Stage and Molecule Type

Gaucher's Disease – DelveInsight's Analytical Perspective

Late Stage Products (Phase III)

Comparative Analysis

Eliglustat: Sanofi

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

Mid Stage Products (Phase II)

Comparative Analysis

Arimoclomol: Orphazyme

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

Early Stage Products (Phase I/II)

Comparative Analysis

AVR-RD-02: AVROBIO

Product Description

Research and Development



Product Development Activities  
Drug profiles in the detailed report  
Preclinical and Discovery Stage Products  
Comparative Analysis  
CAN103: CANbridge Life Sciences  
Product Description  
Research and Development  
Product Development Activities  
Drug profiles in the detailed report  
Inactive Products  
Comparative Analysis  
Gaucher's Disease Key Companies  
Gaucher's Disease Key Products  
Gaucher's Disease- Unmet Needs  
Gaucher's Disease- Market Drivers and Barriers  
Gaucher's Disease- Future Perspectives and Conclusion  
Gaucher's Disease Analyst Views  
Appendix

## List Of Tables

### LIST OF TABLES

Table 1 Total Products for Gaucher's Disease

Table 2 Late Stage Products

Table 3 Mid Stage Products

Table 4 Early Stage Products

Table 5 Pre-clinical & Discovery Stage Products

Table 6 Assessment by Product Type

Table 7 Assessment by Stage and Product Type

Table 8 Assessment by Route of Administration

Table 9 Assessment by Stage and Route of Administration

Table 10 Assessment by Molecule Type

Table 11 Assessment by Stage and Molecule Type

Table 12 Inactive Products

## List Of Figures

### LIST OF FIGURES

Figure 1 Total Products for Gaucher's Disease

Figure 2 Late Stage Products

Figure 3 Mid Stage Products

Figure 4 Early Stage Products

Figure 5 Preclinical and Discovery Stage Products

Figure 6 Assessment by Product Type

Figure 7 Assessment by Stage and Product Type

Figure 8 Assessment by Route of Administration

Figure 9 Assessment by Stage and Route of Administration

Figure 10 Assessment by Molecule Type

Figure 11 Assessment by Stage and Molecule Type

Figure 12 Inactive Products

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