

# **Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) - Pipeline Review, H2 2018**

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## **Abstracts**

Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) - Pipeline Review, H2 2018

### **SUMMARY**

According to the recently published report 'Glucosylceramidase - Pipeline Review, H2 2018'; Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) pipeline Target constitutes close to 15 molecules. Out of which approximately 11 molecules are developed by companies and remaining by the universities/institutes.

Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) - Glucosylceramidase belong to the family of hydrolases that catalyzes the hydrolysis of the glycolipid, glucocerebroside, to glucose and ceramide as part of the normal degradation pathway for membrane lipids. Deficiency in Beta-glucocerebrosidase enzymatic activity, results in accumulation of lipid glucocerebroside in tissue macrophages (Gaucher cells). Enzyme replacement therapy leads to elevated serum levels of the enzyme and reduction in the accumulation of glucocerebroside.

The report 'Glucosylceramidase - Pipeline Review, H2 2018' outlays comprehensive information on the Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type; that are being developed by Companies/Universities.

It also reviews key players involved in Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics development with respective active and dormant or discontinued projects.

Currently, The molecules developed by companies in Phase II, Phase I, IND/CTA Filed, Preclinical and Discovery stages are 1, 2, 1, 6 and 1 respectively. Similarly, the universities portfolio in Phase I, Preclinical and Discovery stages comprises 1, 1 and 2 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Central Nervous System which include indications Gaucher Disease, Parkinson's Disease, Gaucher Disease Type I, Gaucher Disease Type II, Amyotrophic Lateral Sclerosis and Gaucher Disease Type III.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The report provides a snapshot of the global therapeutic landscape for Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45)

The report reviews Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

The report features descriptive drug profiles for the pipeline products which includes, product description, descriptive MoA, R&D brief, licensing and collaboration details & other developmental activities

The report reviews key players involved in Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics and enlists all their major and minor projects

The report assesses Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) targeted therapeutics

## REASONS TO BUY

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

Identify emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage

Identify and understand the targeted therapy areas and indications for Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45)

Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

Plan mergers and acquisitions effectively by identifying key players and it's most promising pipeline therapeutics

Devise corrective measures for pipeline projects by understanding Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) development landscape

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

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Avrobio Inc

Biosidus SA

GT Gain Therapeutics SA

JCR Pharmaceuticals Co Ltd

Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N

Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) - Drug Profiles

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Featured News & Press Releases

Oct 01, 2018: AVROBIO receives no objection to clinical trial application from Health  
Canada for AVR-RD-02 gene therapy for gaucher disease

Jul 17, 2018: Fetal gene therapy prevents fatal neurodegenerative disease

Mar 22, 2017: AVROBIO Expands Rare Disease Pipeline with Gene Therapy to Treat  
Gaucher Disease

Dec 27, 2016: Protalix BioTherapeutics Receives Confirmation of Order for over \$24  
Million of alfataliglycerase to Treat Gaucher Patients in Brazil

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Dec 14, 2016: Protalix BioTherapeutics Receives Letter Detailing Intended Purchases of Approximately \$24 Million of alfatriglicerol to Treat Gaucher Patients in Brazil

Nov 22, 2016: Alfatriglicerol Approved for Pediatric Indications in Brazil for the Treatment of Gaucher Disease in Children Four years and Older

Oct 25, 2016: Asthma research unexpectedly yields new treatment approach for inherited enzyme disease

Jul 12, 2016: Researchers make advance in possible treatments for Gaucher, Parkinson's diseases

Feb 12, 2015: Safety and Efficacy of ELELYSO (taliglucerase alfa) for Injection in Pediatric Patients with Type 1 Gaucher Disease in Long-term Outcome Study Presented at the WORLDSymposium 2015

Sep 02, 2014: Shire's VPRIV400 units/vial launched in Japan for the improvement of symptoms of Gaucher disease

Aug 28, 2014: Pfizer and Protalix BioTherapeutics Announce FDA Approval of Pediatric Indication for ELELYSO (taliglucerase alfa) for Injection, for Intravenous Use for the Treatment of Type 1 Gaucher Disease

Jul 04, 2014: Shire's VPRIV approved in Japan for the improvement of symptoms of Gaucher disease

Jul 01, 2014: Pfizer Receives Kosher Certification for Elelyso (taliglucerase alfa) for Injection, for the Treatment of Type 1 Gaucher Disease

Jun 27, 2014: Protalix Announces New Data on ELELYSO (taliglucerase alfa) Presented at the European Working Group on Gaucher Disease 2014 11th Meeting

Jun 23, 2014: Protalix BioTherapeutics Announces New Data on ELELYSO (taliglucerase alfa) and Oral GCD to be Presented at the European Working Group on Gaucher Disease 2014 11th Meeting

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### COMPANIES MENTIONED

Avrobio Inc

Biosidus SA

GT Gain Therapeutics SA

JCR Pharmaceuticals Co Ltd

## I would like to order

Product name: Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N Acylsphingosine Glucohydrolase or Beta Glucocerebrosidase or Imiglucerase or GBA or EC 3.2.1.45) - Pipeline Review, H2 2018

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