

Glucosylceramidase - Pipeline Review, H2 2019

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Abstracts

Glucosylceramidase - Pipeline Review, H2 2019

SUMMARY

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.

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 20 molecules. Out of which approximately 17 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Phase III, Phase II, Preclinical and Discovery stages are 1, 3, 11 and 2 respectively. Similarly, the universities portfolio in Phase I and Discovery stages comprises 1 and 2 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Central Nervous System which include indications Parkinson's Disease, Gaucher Disease, Gaucher Disease Type I, Gaucher Disease Type II, Gaucher Disease Type III and Amyotrophic Lateral Sclerosis.

The latest report Glucosylceramidase - Pipeline Review, H2 2019, 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. 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.

The report is built using data and information sourced from 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.

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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Adienne Pharma & Biotech

Alectos Therapeutics

Apollo Therapeutics LLC

AVROBIO Inc

Belrose Pharma Inc

Bioasis Technologies Inc

Biosidus SA

Blue Turtle Bio Technologies Inc

Erad Therapeutics Inc

Freeline Therapeutics Ltd

GT Gain Therapeutics SA

Lysosomal Therapeutics Inc

Oxyrane Belgium NV

Spedding Research Solutions SAS

Takeda Pharmaceutical Co Ltd

Trucode Gene Repair Inc

Glucosylceramidase (Acid Beta Glucosidase or Alglucerase or D Glucosyl N
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Product Description

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AVRRD-02 - Drug Profile

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FLT-200 - Drug Profile

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Gene Therapy to Activate Glucocerebrosidase for Genetic Disorders - Drug Profile

Product Description

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Product Description

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R&D Progress

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Pcgin - Drug Profile

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Featured News & Press Releases

Nov 06, 2019: Freeline to present data on its Gaucher Disease drug candidate FLT-200 at the 61st ASH Annual Meeting

Oct 24, 2019: AVROBIO receives Orphan-Drug Designation from the U.S. FDA for AVR-RD-02 for the treatment of Gaucher Disease

Sep 23, 2019: Safety and Biomarker effects of novel therapeutic for Parkinson's disease with GBA mutations

Jul 05, 2019: Freeline presents preclinical liver-directed AAV gene therapy data at the European Working Group on Gaucher Disease.

Apr 26, 2019: AVROBIO highlights the plato platform and Gaucher program at the 2019 Annual Meeting of the American Society of Gene and Cell Therapy

Feb 26, 2019: Gain Therapeutics announces award of grant support from leading

Parkinson's Research Foundations to advance its proprietary non competitive molecular chaperones for parkinson's disease

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 alfataliglicerase to Treat Gaucher Patients in Brazil

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

Nov 22, 2016: Alfataliglicerase 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

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Apollo Therapeutics LLC
AVROBIO Inc
Belrose Pharma Inc
Bioasis Technologies Inc
Biosidus SA
Blue Turtle Bio Technologies Inc
Erad Therapeutics Inc
Freeline Therapeutics Ltd
GT Gain Therapeutics SA
Lysosomal Therapeutics Inc
Oxyrane Belgium NV
Spedding Research Solutions SAS
Takeda Pharmaceutical Co Ltd
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