

# **Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Pipeline Review, H1 2018**

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## **Abstracts**

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Pipeline Review, H1 2018

### **SUMMARY**

According to the recently published report 'Lysosomal Alpha Glucosidase - Pipeline Review, H1 2018'; Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) pipeline Target constitutes close to 15 molecules. Out of which approximately 13 molecules are developed by companies and remaining by the universities/institutes.

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Lysosomal alpha-glucosidase is an enzyme encoded by the GAA gene. It is essential for the degradation of glycogen to glucose in lysosomes. Defects in this gene lead to glycogen storage disease II or Pompe disease.

The report 'Lysosomal Alpha Glucosidase - Pipeline Review, H1 2018' outlays comprehensive information on the Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) 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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) targeted therapeutics development with

respective active and dormant or discontinued projects. Currently, The molecules developed by companies in Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 2, 1, 8 and 1 respectively.

Similarly, the universities portfolio in Phase II and Phase I stages comprises 1 and 1 molecules, respectively. Report covers products from therapy areas Metabolic Disorders which include indications Pompe Disease.

**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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20)

The report reviews Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) 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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) targeted therapeutics and enlists all their major and minor projects

The report assesses Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) 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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) 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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20)

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 Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) 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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Genzyme Corp

greenovation Biotech GmbH

JCR Pharmaceuticals Co Ltd

NanoMedSyn SAS

Oxyrane Belgium NV

Pharming Group NV

Sarepta Therapeutics Inc

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3.2.1.20) - Drug Profiles

(ATB-200 + miglustat) - Drug Profile

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Mechanism Of Action

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Antisense Oligonucleotide to Activate Lysosomal Alpha-Glucosidase for Pompe

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Product Description

Mechanism Of Action

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AT-982 - Drug Profile

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avalglucosidase alfa - Drug Profile

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AVRRD-03 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy 1 to Activate Acid Alpha-Glucosidase for Pompe Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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Product Description

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Featured News & Press Releases

Feb 07, 2018: Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at 14th Annual WORLDSymposium

Feb 06, 2018: Audentes Therapeutics Announces Selection of Optimized Clinical Development Candidate for Pompe Disease Program

Feb 05, 2018: Valerion to Present Initial Clinical Data with VAL-1221 in Pompe Disease

Jan 22, 2018: Amicus Therapeutics Announces Presentations and Posters at 14th Annual WORLDSymposium 2018

Oct 04, 2017: Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at World Muscle Society

Sep 21, 2017: U.S. FDA Grants Orphan Drug Designation for ATB200/AT2221 for Pompe Disease

Sep 20, 2017: AVROBIO Expands Rare Disease Pipeline with Gene Therapy to Treat Pompe Disease

Jul 11, 2017: Valerion Initiates VAL-1221 Dosing in Patients with Pompe Disease

May 15, 2017: Amicus Therapeutics Announces Positive Functional Data from Initial Patients in Pompe Phase 1/2 Study

Mar 03, 2017: Valerion Therapeutics Demonstrates a New Mechanism for Treating

## Pompe Disease

Feb 23, 2017: JCR to Initiate Development of Jr-162, A New Drug Candidate For Pompe Disease Using J-brain Cargo

Feb 15, 2017: Amicus Therapeutics Presents Important New Scientific Findings and Preclinical Data for Pompe Program at WORLDSymposium 2017

Dec 08, 2016: Amicus Therapeutics Announces Positive Preliminary Data from Phase 1/2 Study of Novel Treatment Paradigm for Pompe Disease

Nov 17, 2016: Pivotal Phase 3 Trial of NeoGAA Investigational Second-Generation Therapy for Pompe Disease to Begin in the UK

Nov 04, 2016: Sanofi Genzyme Begins Pivotal Phase 3 Trial of NeoGAA Investigational Second-Generation Therapy for Pompe Disease

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### COMPANIES MENTIONED

Amicus Therapeutics Inc

Audentes Therapeutics Inc

Etubics Corp

Genzyme Corp

greenovation Biotech GmbH

JCR Pharmaceuticals Co Ltd

NanoMedSyn SAS

Oxyrane Belgium NV

Pharming Group NV

Sarepta Therapeutics Inc

## I would like to order

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