

# **Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Pipeline Review, H2 2018**

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## **Abstracts**

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Pipeline Review, H2 2018

### **SUMMARY**

According to the recently published report 'Lysosomal Alpha Glucosidase - Pipeline Review, H2 2018'; Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) pipeline Target constitutes close to 16 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, H2 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, IND/CTA Filed and Preclinical stages are 1, 2, 1, 1 and 8 respectively. Similarly, the universities portfolio in Phase II, Phase I and Preclinical stages comprises 1, 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

## Contents

Introduction

Global Markets Direct Report Coverage

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC

3.2.1.20) - Overview

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC

3.2.1.20) - Therapeutics Development

Products under Development by Stage of Development

Products under Development by Therapy Area

Products under Development by Indication

Products under Development by Companies

Products under Development by Universities/Institutes

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC

3.2.1.20) - Therapeutics Assessment

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC

3.2.1.20) - Companies Involved in Therapeutics Development

Amicus Therapeutics Inc

Audentes Therapeutics Inc

AvroBio Inc

Etubics Corp

Genzyme Corp

greenovation Biotech GmbH

JCR Pharmaceuticals Co Ltd

NanoMedSyn SAS

Oxyrane Belgium NV

Pharming Group NV

Spark Therapeutics Inc

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC

3.2.1.20) - Drug Profiles

(aglucosidase alfa + miglustat) - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AT-982 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

avalglucosidase alfa - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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

Gene Therapy 2 to Activate Acid Alpha-Glucosidase for Pompe Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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

Product Description

Mechanism Of Action

R&D Progress

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

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate GAA for Pompe Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JR-162 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

MOSS-GAA - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

OXY-2810 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

PGN-004 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Alpha Glucosidase Replacement for Pompe Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SPK-3006 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

VAL-1221 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Dormant Products

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Discontinued Products

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Product Development Milestones

Featured News & Press Releases

Oct 08, 2018: Spark Therapeutics Announces New Preclinical Data for Pompe Disease Gene Therapy Candidate

Oct 05, 2018: Amicus Therapeutics Announces Positive 18-Month Data in Pompe Disease Phase 1/2 Study at 23rd International Annual Congress of the World Muscle Society

Oct 02, 2018: Amicus Therapeutics announces presentation and poster at the 23rd International Congress of the World Muscle Society

Sep 24, 2018: Audentes Therapeutics provides update on AT982 for Pompe disease

Sep 10, 2018: Amicus Therapeutics announces regulatory and clinical updates for AT-GAA in Pompe Disease

Jun 26, 2018: Amicus Therapeutics Announces European Regulatory and Clinical Updates for AT-GAA in Pompe Disease

Apr 30, 2018: Audentes Therapeutics Announces Data on AT982 at the 21st Annual

Meeting of the American Society of Gene and Cell Therapy

Apr 26, 2018: AskBio Spins Out New Gene Therapy Company, Actus Therapeutics

Feb 07, 2018: Sanofi Announces New Safety Data for Investigational Avalglucosidase Alfa in Patients with Pompe Disease

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

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer

## List Of Tables

### LIST OF TABLES

Number of Products under Development by Stage of Development, H2 2018  
Number of Products under Development by Therapy Areas, H2 2018  
Number of Products under Development by Indication, H2 2018  
Number of Products under Development by Companies, H2 2018  
Products under Development by Companies, H2 2018  
Number of Products under Investigation by Universities/Institutes, H2 2018  
Products under Investigation by Universities/Institutes, H2 2018  
Number of Products by Stage and Mechanism of Actions, H2 2018  
Number of Products by Stage and Route of Administration, H2 2018  
Number of Products by Stage and Molecule Type, H2 2018  
Pipeline by Amicus Therapeutics Inc, H2 2018  
Pipeline by Audentes Therapeutics Inc, H2 2018  
Pipeline by AvroBio Inc, H2 2018  
Pipeline by Etubics Corp, H2 2018  
Pipeline by Genzyme Corp, H2 2018  
Pipeline by greenovation Biotech GmbH, H2 2018  
Pipeline by JCR Pharmaceuticals Co Ltd, H2 2018  
Pipeline by NanoMedSyn SAS, H2 2018  
Pipeline by Oxyrane Belgium NV, H2 2018  
Pipeline by Pharming Group NV, H2 2018  
Pipeline by Spark Therapeutics Inc, H2 2018  
Dormant Projects, H2 2018  
Discontinued Products, H2 2018



## List Of Figures

### LIST OF FIGURES

Number of Products under Development by Stage of Development, H2 2018

Number of Products by Mechanism of Actions, H2 2018

Number of Products by Stage and Mechanism of Actions, H2 2018

Number of Products by Routes of Administration, H2 2018

Number of Products by Stage and Routes of Administration, H2 2018

Number of Products by Molecule Types, H2 2018

Number of Products by Stage and Molecule Types, H2 2018

### COMPANIES MENTIONED

Amicus Therapeutics Inc

Audentes Therapeutics Inc

Avrobio Inc

Etubics Corp

Genzyme Corp

greenovation Biotech GmbH

JCR Pharmaceuticals Co Ltd

NanoMedSyn SAS

Oxyrane Belgium NV

Pharming Group NV

Spark Therapeutics Inc

## I would like to order

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- Pipeline Review, H2 2018

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