

# **Lysosomal Alpha Glucosidase - Pipeline Review, H1 2020**

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## **Abstracts**

Lysosomal Alpha Glucosidase - Pipeline Review, H1 2020

### **SUMMARY**

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.

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) pipeline Target constitutes close to 17 molecules. Out of which approximately 14 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Phase III, Phase II, IND/CTA Filed and Preclinical stages are 2, 2, 1 and 9 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.

The latest report Lysosomal Alpha Glucosidase - Pipeline Review, H1 2020, 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. 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.

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.

**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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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

Valerion Therapeutics LLC

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

**ACTUS-101 - Drug Profile**

Product Description

Mechanism Of Action

R&amp;D Progress

**AT-845 - Drug Profile**

Product Description

Mechanism Of Action

R&amp;D Progress

**avalglucosidase alfa - Drug Profile**

Product Description

Mechanism Of Action

R&amp;D Progress

**AVRRD-03 - Drug Profile**

Product Description

Mechanism Of Action

R&amp;D Progress

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

Product Description

Mechanism Of Action

R&amp;D Progress

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Product Description

Mechanism Of Action

R&amp;D Progress

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

Product Description

Mechanism Of Action

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Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Number of Products by Stage and Molecule Types, H1 2020

### COMPANIES MENTIONED

Actus Therapeutics Inc

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

Valerion Therapeutics LLC



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