

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

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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 glygogen 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



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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



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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

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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



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