

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update

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Abstracts

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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 20 molecules. Out of which approximately 17 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Phase II, Preclinical and Discovery stages are 2, 3, 10 and 2 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 - Drugs In Development, 2022, 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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Asklepios BioPharmaceutical Inc

Astellas Gene Therapies

AVROBIO Inc.

Denali Therapeutics Inc

Equaly SA

Genzyme Corp

ImmunityBio Inc

JCR Pharmaceuticals Co Ltd

M6P Therapeutics

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

(cipaglucosidase alfa + miglustat) - Drug Profile

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Mechanism Of Action



History of Events

ACTUS-101 - Drug Profile

Product Description

Mechanism Of Action

History of Events

AT-845 - Drug Profile

Product Description

Mechanism Of Action

History of Events

avalglucosidase alfa - Drug Profile

Product Description

Mechanism Of Action

History of Events

AVRRD-03 - Drug Profile

Product Description

Mechanism Of Action

History of Events

Fusion Protein to Replace GAA for Pompe Disease - Drug Profile

Product Description

Mechanism Of Action

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

Product Description

Mechanism Of Action

History of Events

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

Product Description

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Gene Therapy to Activate GAA for Pompe Disease - Drug Profile

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JR-162 - Drug Profile

Product Description

Mechanism Of Action

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M-021 - Drug Profile

Product Description

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M-023 - Drug Profile

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PGN-004 - Drug Profile

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Product Description

Mechanism Of Action

RPV-002 - Drug Profile

Product Description

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History of Events

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Product Description

Mechanism Of Action

History of Events

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Featured News & Press Releases

Feb 11, 2022: M6P Therapeutics presents promising preclinical data in Pompe disease at the 18th Annual WORLDSymposium 2022

Feb 08, 2022: Nexviazyme (avalglucosidase alfa) shows sustained improvements in respiratory function and mobility in patients with Pompe disease

Feb 07, 2022: Astellas announces positive safety data from the FORTIS study of AT845 in adults with late-onset Pompe disease

Dec 03, 2021: European Medicines Agency validates Amicus Therapeutics marketing authorization applications for AT-GAA for the treatment of Pompe disease

Nov 29, 2021: Sanofi's Pompe Disease drug Nexviazyme now available in Japan

Nov 18, 2021: The Lancet Neurology publishes pivotal Phase 3 PROPEL study results of AT-GAA in late-onset Pompe disease

Nov 15, 2021: Nexviazyme (avalglucosidase alfa for injection) is now approved in Canada for patients with late-onset Pompe disease (acid a-glucosidase deficiency) Nov 12, 2021: Sanofi announces results of CHMP re-examination of the New Active Substance status for avalglucosidase alfa, a potential new standard of care for the

Substance status for avaiglucosidase alfa, a potential new standard of care for the treatment of Pompe disease

Sep 29, 2021: U.S. FDA accepts filings for Amicus' AT-GAA for the treatment of Pompe disease

Sep 27, 2021: Orsini Specialty Pharmacy selected By Sanofi as a limited distribution partner for Nexviazyme

Sep 20, 2021: Amicus Therapeutics announces presentations at the 26th International Annual Congress of the World Muscle Society

Sep 17, 2021: CHMP announces re-examinations of recommendations for Nexviadyme

Aug 09, 2021: FDA approves Sanofi's Nexviazyme for Pompe disease treatment

Aug 06, 2021: FDA approves Nexviazyme (avalglucosidase alfa-ngpt), an important new treatment option for late-onset Pompe disease

Jul 27, 2021: Sanofi provides update on avalglucosidase alfa EU submission for patients with Pompe Disease

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Number of Products by Stage and Molecule Types, 2022



I would like to order

Product name: Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20)

Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and

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