

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Drugs in Development, 2021

https://marketpublishers.com/r/L790784CE23AEN.html

Date: March 2021

Pages: 87

Price: US\$ 3,500.00 (Single User License)

ID: L790784CE23AEN

Abstracts

Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) - Drugs in Development, 2021

SUMMARY

According to the recently published report 'Lysosomal Alpha Glucosidase - Drugs in Development, 2021'; Lysosomal Alpha Glucosidase (Acid Maltase or Aglucosidase Alfa or GAA or EC 3.2.1.20) pipeline Target constitutes close to 19 molecules. Out of which approximately 16 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 - Drugs in Development, 2021' 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 Pre-Registration, Phase III, Phase II, Preclinical and Discovery stages are 1, 1, 3, 10 and 1 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

Asklepios BioPharmaceutical Inc

Audentes Therapeutics Inc

AVROBIO Inc.

Denali Therapeutics Inc

eleva GmbH

Etubics Corp

Genzyme Corp

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

Product Description

Mechanism Of Action



R&D Progress

ACTUS-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AT-845 - 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

Fusion Protein to Replace GAA for Pompe Disease - 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 GAA 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

M-021 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

M-023 - 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

RPV-002 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SPK-3006 - 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

Mar 15, 2021: Amicus Therapeutics announces presentation and posters at the 2021 MDA Clinical & Scientific Conference

Feb 11, 2021: Amicus' AT-GAA shows clinically meaningful & significant improvements in both musculoskeletal and respiratory measures in late-onset Pompe disease compared to standard of care in pivotal phase 3 PROPEL study

Feb 02, 2021: Data presented at WORLDSymposium reinforces robust rare disease pipeline and highlights additional clinical data for investigational avalglucosidase alfa in Pompe disease

Feb 01, 2021: Spark Therapeutics announces first participant dosed in phase 1/2 study of investigational gene therapy for late-onset pompe disease

Feb 01, 2021: AVROBIO announces clinical data presentation on AVRRD-03 at 17th Annual WORLDSymposium 2021

Jan 20, 2021: Sanofi announced that it has submitted a new drug application in Japan for avalglucosidase alfa, an investigational enzyme replacement therapy

Dec 01, 2020: Amicus Therapeutics initiates rolling Biologic License Application to the U.S. Food and Drug Administration for AT-GAA in late-onset Pompe disease

Nov 18, 2020: FDA grants priority review for avalglucosidase alfa, a potential new therapy for Pompe disease

Oct 02, 2020: EMA accepts regulatory submission for avalglucosidase alfa, a potentially new standard of care enzyme replacement therapy for Pompe disease

Jun 16, 2020: Sanofi's investigational enzyme replacement therapy shows clinically meaningful improvement in critical manifestations of late-onset Pompe disease

Jun 08, 2020: Sanofi to present Phase 3 results of avalglucosidase alfa in patients with late-onset Pompe disease

May 14, 2020: AVROBIO presents new preclinical data on lentiviral gene therapy program for Pompe Disease at ASGCT 2020

May 05, 2020: Amicus Therapeutics announces presentations on its pompe disease gene therapy at the American Society of Gene & Cell Therapy 23rd Annual Meeting May 05, 2020: AVROBIO to present preclinical data at upcoming American Society of Gene & Cell Therapy (ASGCT) annual meeting, May 12-15, 2020

Feb 07, 2020: Amicus Therapeutics announces presentation on its Pompe Disease gene therapy at 16th Annual WORLDSymposium 2020

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, 2021

Number of Products under Development by Therapy Areas, 2021

Number of Products under Development by Indication, 2021

Number of Products under Development by Companies, 2021

Products under Development by Companies, 2021

Products under Development by Companies, 2021 (Contd..1)

Number of Products under Investigation by Universities/Institutes, 2021

Products under Investigation by Universities/Institutes, 2021

Number of Products by Stage and Mechanism of Actions, 2021

Number of Products by Stage and Route of Administration, 2021

Number of Products by Stage and Molecule Type, 2021

Pipeline by Amicus Therapeutics Inc, 2021

Pipeline by Asklepios BioPharmaceutical Inc, 2021

Pipeline by Audentes Therapeutics Inc, 2021

Pipeline by AVROBIO Inc, 2021

Pipeline by Denali Therapeutics Inc, 2021

Pipeline by eleva GmbH, 2021

Pipeline by Etubics Corp, 2021

Pipeline by Genzyme Corp, 2021

Pipeline by JCR Pharmaceuticals Co Ltd, 2021

Pipeline by M6P Therapeutics, 2021

Pipeline by NanoMedSyn SAS, 2021

Pipeline by Oxyrane Belgium NV, 2021

Pipeline by Pharming Group NV, 2021

Pipeline by Spark Therapeutics Inc, 2021

Dormant Projects, 2021

Discontinued Products, 2021



List Of Figures

LIST OF FIGURES

Number of Products under Development by Stage of Development, 2021

Number of Products by Mechanism of Actions, 2021

Number of Products by Stage and Mechanism of Actions, 2021

Number of Products by Routes of Administration, 2021

Number of Products by Stage and Routes of Administration, 2021

Number of Products by Molecule Types, 2021

Number of Products by Stage and Molecule Types, 2021



I would like to order

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

- Drugs in Development, 2021

Product link: https://marketpublishers.com/r/L790784CE23AEN.html

Price: US\$ 3,500.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer

Service:

info@marketpublishers.com

Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page https://marketpublishers.com/r/L790784CE23AEN.html