

Pompe Disease Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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Abstracts

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SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Pompe Disease - Drugs In Development, 2022, provides an overview of the Pompe Disease (Metabolic Disorders) pipeline landscape.

Pompe disease is an inherited disorder caused by defect in a gene called GAA. The GAA gene is responsible for the production of the GAA enzyme (acid alpha-glucosidase). This enzyme is needed to break down glycogen, a form of sugar stored in muscle cells. When too much glycogen builds up in the muscle cells, the cells become damaged and the muscles cannot function properly. Symptoms include respiratory failure, muscle pain, proximal muscle weakness, respiratory tract infections, headache and difficulty chewing or jaw muscle fatigue.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Pompe Disease - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Pompe Disease (Metabolic Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development

history and latest news and press releases.

The Pompe Disease (Metabolic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Pompe Disease and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II, Phase I, Preclinical and Discovery stages are 1, 1, 2, 1, 22 and 4 respectively. Similarly, the Universities portfolio in Phase I, Preclinical and Discovery stages comprises 1, 1 and 1 molecules, respectively.

Pompe Disease (Metabolic Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's 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. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

Note: Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Pompe Disease (Metabolic Disorders).

The pipeline guide reviews pipeline therapeutics for Pompe Disease (Metabolic Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Pompe Disease (Metabolic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Pompe Disease (Metabolic Disorders) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Pompe Disease (Metabolic Disorders)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Pompe Disease (Metabolic Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Pompe Disease (Metabolic Disorders) pipeline depth and focus of Indication therapeutics.

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.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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Featured News & Press Releases

Aug 11, 2022: FDA grants Orphan Drug status to Aro Biotherapeutics' Pompe disease therapy

Jun 28, 2022: Nexviadyme (avalglucosidase alfa) approved by European Commission as a potential new standard of care for the treatment of Pompe Disease

May 18, 2022: AVROBIO announces preclinical gene therapy data for Pompe Disease at American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

May 11, 2022: Aro Biotherapeutics to present new preclinical data highlighting the potential of Centyrin-siRNA conjugates for the treatment of Pompe disease

May 10, 2022: Amicus Therapeutics receives notification of PDUFA date extensions for AT-GAA

Mar 16, 2022: Amicus Therapeutics announces positive long-term data from phase 1/2 study of AT-GAA in Pompe Disease at the 2022 MDA Clinical & Scientific Conference

Mar 10, 2022: Amicus Therapeutics announces presentation and posters at the 2022 MDA Clinical & Scientific Conference

Feb 18, 2022: Maze Therapeutics initiates dosing in Phase I trial of Pompe disease asset

Feb 10, 2022: M6P Therapeutics presents promising preclinical data in Pompe disease

at the 18th Annual WORLDSymposium 2022

Feb 10, 2022: Maze Therapeutics presents new preclinical data supporting advancement of MZE001 as a potential treatment for Pompe disease

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

Jan 31, 2022: Maze Therapeutics to present preclinical data highlighting MZE001 as a potential treatment for Pompe disease at 2022 WORLD Symposium

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

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