

# Pompe Disease - Pipeline Review, H1 2020

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## Abstracts

Pompe Disease - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Pompe Disease - Pipeline Review, H1 2020, 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 - Pipeline Review, H1 2020, 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 Phase III, Phase II, IND/CTA Filed, Preclinical and Discovery stages are 2, 2, 1, 14 and 2 respectively. Similarly, the Universities portfolio in Phase II, Phase I and Preclinical 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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### COMPANIES MENTIONED

Abeona Therapeutics Inc  
Actus Therapeutics Inc  
Amicus Therapeutics Inc  
Audentes Therapeutics Inc  
Avidity Biosciences LLC  
AVROBIO Inc  
EpiVax Inc  
Etubics Corp  
Genzyme Corp  
Greenovation Biotech GmbH  
Icagen Inc  
JCR Pharmaceuticals Co Ltd  
NanoMedSyn SAS  
Oxyrane Belgium NV  
Pharming Group NV  
RespireRx Pharmaceuticals Inc  
Sarepta Therapeutics Inc  
Spark Therapeutics Inc  
Valerion Therapeutics LLC

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