

Fabry 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 Fabry Disease - Drugs In Development, 2022, provides an overview of the Fabry Disease (Genetic Disorders) pipeline landscape.

Fabry disease is an X-linked lysosomal disorder that results in abnormal deposits of globotriaosylceramide in blood vessel walls throughout the body. It is caused due to mutations in GLA gene. The GLA gene controls the production of a particular enzyme called alpha-galactosidase A (this enzyme is responsible for breaking down of globotriaosylceramide). Symptoms include skin rash, cramps, gas, diarrhea, heart enlargement, angina, dizziness, headache, nausea, and heat intolerance. Treatment includes enzyme replacement therapy (ERT) and pain management.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Fabry Disease - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Fabry Disease (Genetic 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 Fabry Disease (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Fabry 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, IND/CTA Filed, Preclinical and Discovery stages are 1, 3, 3, 2, 1, 15 and 5 respectively. Similarly, the Universities portfolio in Preclinical, Discovery and Unknown stages comprises 1, 1 and 1 molecules, respectively.

Fabry Disease (Genetic 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 Fabry Disease (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Fabry Disease (Genetic 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 Fabry Disease (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Fabry Disease (Genetic 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 Fabry Disease (Genetic 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 Fabry Disease (Genetic 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 Fabry Disease (Genetic 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

Apr 04, 2022: Protalix BioTherapeutics and Chiesi Global Rare Diseases announce topline results from the 24-month Phase III BALANCE clinical trial of PRX-102 for the treatment of Fabry disease

Mar 24, 2022: Freeline announces updated development plan and timelines for FLT190 for people with Fabry disease

Mar 21, 2022: Protalix, Chiesi report data from Phase III Fabry disease trial

Feb 24, 2022: Protalix BioTherapeutics and Chiesi Global Rare Diseases announce the submission of a marketing authorization application to the European Medicines Agency for PRX-102 for the treatment of Fabry Disease

Feb 09, 2022: 4D Molecular Therapeutics announces updated interim results from the 4D-310 phase 1/2 clinical trial in patients with Fabry Disease at the 18th Annual WORLDSymposium

Feb 08, 2022: Freeline presents on its Fabry disease AAV-based gene therapies at the 18th Annual WORLDSymposium

Feb 07, 2022: Sangamo Therapeutics announces updated preliminary phase 1/2 data showing tolerability and sustained elevated a-Gal a enzyme activity in patients With Fabry Disease

Feb 03, 2022: Protalix BioTherapeutics to participate in the 18th Annual WORLDSymposium 2022

Feb 02, 2022: 4D Molecular Therapeutics to present updated clinical data with 4D-310 in Fabry Disease patients in platform presentation at the 18th Annual WORLDSymposium

Jan 27, 2022: Freeline to present on its fabry disease AAV-based gene therapies at the 18th Annual WORLDSymposium

Dec 13, 2021: Idorsia to further characterize lucerastat for the treatment of Fabry disease by continuing the open-label extension of the Phase 3 MODIFY study

Nov 04, 2021: Sangamo Therapeutics announces preliminary phase 1/2 data showing tolerability and sustained elevated a-Gal A enzyme activity in patients with Fabry disease

Oct 25, 2021: 4D Molecular Therapeutics reports interim results from the 4D-310 phase 1/2 clinical trial in patients with fabry disease

Oct 15, 2021: Protalix Biotherapeutics and Chiesi Global Rare Diseases announce final dosing of last patient in phase III BALANCE clinical trial PRX-102 for the treatment of Fabry disease

Oct 11, 2021: Idorsia announces the results of MODIFY, a Phase 3 study of lucerastat in Fabry disease

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