

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) Drugs in 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

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) pipeline Target constitutes close to 21 molecules. Out of which approximately 15 molecules are developed by companies and remaining by the universities/institutes. The latest report Frataxin Mitochondrial - Drugs In Development, 2022, outlays comprehensive information on the Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type.

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Frataxin is a protein encoded by the FXN gene. It promotes the biosynthesis of heme and assembly and repair of iron-sulfur clusters by delivering Fe²⁺ to proteins involved in these pathways. It plays a role in the protection against iron-catalyzed oxidative stress through its ability to catalyze the oxidation of Fe²⁺ to Fe³⁺. The molecules developed by companies in Phase II, Phase I, IND/CTA Filed, Preclinical and Discovery stages are 1, 1, 1, 8 and 4 respectively. Similarly, the universities portfolio in Preclinical and Discovery stages comprises 3 and 3 molecules, respectively. Report covers products

from therapy areas Central Nervous System and Cardiovascular which include indications Friedreich Ataxia, Cardiomyopathy and Mild Cognitive Impairment.

Furthermore, this report also reviews key players involved in Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) targeted therapeutics development with respective active and dormant or discontinued projects. Driven by 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 Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1)

The report reviews Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) 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 Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) targeted therapeutics and enlists all their major and minor projects

The report assesses Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) 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 Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) 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 Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1)

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 Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) 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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Involved in Therapeutics Development

AavantiBio Inc

Anima Biotech Inc

Astellas Pharma Inc

Biointaxis SL

Cellivery Therapeutics Inc

CRISPR Therapeutics AG

Design Therapeutics Inc

Fratagene Therapeutics Srl

Fulcrum Therapeutics Inc

Jupiter Neurosciences Inc

Lacerta Therapeutics Inc

LEXEO Therapeutics LLC

Novartis Gene Therapies

PTC Therapeutics Inc

Voyager Therapeutics Inc

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Featured News & Press Releases

Mar 30, 2022: Design Therapeutics completes dosing in first patient cohort of phase 1 trial of DT-216 GeneTAC molecule for the treatment of Friedreich Ataxia

Feb 28, 2022: Design Therapeutics announces FDA clearance of investigational New Drug Application for first GeneTAC molecule for Friedreich Ataxia

Feb 16, 2022: LEXEO Therapeutics announces FDA clearance of investigational new drug application for LX2006, an AAV-based gene therapy candidate for Friedreich's Ataxia Cardiomyopathy

Jun 30, 2021: LEXEO Therapeutics receives rare pediatric disease designation and orphan drug designation for LX2006 for the treatment of Friedreich's Ataxia

Apr 28, 2021: LEXEO Therapeutics announces upcoming data presentations at American Society of Gene and Cell Therapy (ASGCT) 24th Annual Meeting, provides update on LX2006

Jan 13, 2020: MDA awards venture philanthropy funding of more than \$1M to AavantiBio to develop gene-targeting therapy for Friedreich's Ataxia

Nov 30, 2019: GoFAR partners with AavantiBio to advance gene therapy for Friedreich's Ataxia

Nov 30, 2019: GoFAR partners with AavantiBio to advance gene therapy for Friedreich's Ataxia

Aug 13, 2019: Ataxia UK funds new FA research project developing new FA animal models to test gene therapy

May 18, 2018: Voyager Therapeutics Announces Additional Data at the American Society of Gene and Cell Therapy 2018 Annual Meeting

Oct 31, 2017: Agilis Biotherapeutics Announces Orphan Product Designation Approval in Europe for the Treatment of Friedreich Ataxia

Oct 25, 2017: Transplanted hematopoietic stem cells reverse damage caused by neuromuscular disorder

Aug 22, 2017: Jupiter Orphan Therapeutics Receives Orphan Drug Designation for its Trans-Resveratrol Product JOTROL for Treatment of Friedreich's Ataxia

Mar 14, 2017: New research front to tackle Friedreich's Ataxia

Aug 02, 2016: Agilis Biotherapeutics Announces FDA Orphan Drug Designation for the Treatment of Friedreich's Ataxia

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