

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Drugs In Development, 2021

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Abstracts

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Drugs In Development, 2021

SUMMARY

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.

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 molecules developed by companies in Phase II, Phase I, Preclinical and Discovery stages are 1, 1, 9 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.

The latest report Frataxin Mitochondrial - Drugs In Development, 2021, 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. It 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.

The report is built using 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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Assessment by Molecule Type

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Involved in Therapeutics Development

AavantiBio Inc

Adverum Biotechnologies Inc

Anima Biotech Inc

Biointaxis SL

Celllivery Therapeutics Inc

CRISPR Therapeutics AG

Design Therapeutics Inc

Fratagene Therapeutics Srl

Fulcrum Therapeutics Inc

Jupiter Orphan Therapeutics Inc

Lacerta Therapeutics Inc

Larimar Therapeutics Inc

Novartis Gene Therapies

PTC Therapeutics Inc

Voyager Therapeutics Inc

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Drug Profiles

ADVM-063 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

AVXS-401 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CTI-1601 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CV-14 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

etravirine - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Frataxin for Friedreich's Ataxia - Drug Profile

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Product Description

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PTC-FA - Drug Profile

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Synthetic Peptide to Activate Frataxin for Friedreich Ataxia - Drug Profile

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Mechanism Of Action

R&D Progress

VYFXN-01 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Product Development Milestones

Featured News & Press Releases

May 25, 2021: Larimar Therapeutics reports FDA clinical hold on CTI-1601 and termination of recently announced private placement financing

May 20, 2021: Larimar Therapeutics receives European Medicines Agency Priority Medicines (PRIME) designation for CTI-1601 in Friedreich's ataxia

May 11, 2021: Larimar Therapeutics reports positive topline phase 1 clinical trial data showing dose-dependent increases in frataxin levels in patients with Friedreich's Ataxia

Dec 08, 2020: Larimar Therapeutics announces completion of dosing of the single ascending dose clinical trial in Friedreich's Ataxia patients and provides program update

Jul 28, 2020: Larimar Therapeutics announces positive opinion on Orphan Drug Designation received from the European Medicines Agency for CTI-1601 for the treatment of Friedreich's Ataxia

Jul 20, 2020: Larimar Therapeutics announces dosing of patients in third cohort of phase 1 SAD trial of CTI-1601 for treatment of Friedreich's ataxia

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

Dec 18, 2019: Chondrial Therapeutics announces dosing of first patients in phase 1 clinical program of CTI-1601 for treatment of Friedreich's ataxia and CTI-1601 granted rare pediatric disease designation and fast track designation by U.S. FDA

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

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