

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

<https://marketpublishers.com/r/F0C25197E528EN.html>

Date: June 2021

Pages: 77

Price: US\$ 3,500.00 (Single User License)

ID: F0C25197E528EN

## 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 Fe<sup>2+</sup> 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 Fe<sup>2+</sup> to Fe<sup>3+</sup>.

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

## Contents

Introduction

Global Markets Direct Report Coverage

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

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

Development

Products under Development by Stage of Development

Products under Development by Therapy Area

Products under Development by Indication

Products under Development by Companies

Products under Development by Universities/Institutes

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

Assessment

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

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

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&amp;D Progress

## CTI-1601 - Drug Profile

Product Description

Mechanism Of Action

R&amp;D Progress

## CV-14 - Drug Profile

Product Description

Mechanism Of Action

R&amp;D Progress

## etravirine - Drug Profile

Product Description

Mechanism Of Action

R&amp;D Progress

## Gene Therapy to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&amp;D Progress

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

Product Description

Mechanism Of Action

R&amp;D Progress

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

Product Description

Mechanism Of Action

R&amp;D Progress

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

Product Description

Mechanism Of Action

R&amp;D Progress

## Gene Therapy to Activate FXN for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&amp;D Progress

## Gene Therapy to Activate FXN for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate FXN for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate FXN for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JOT-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

PTC-FA - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Proteins to Replace Frataxin Mitochondrial for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecule to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Synthetic Peptide to Activate Frataxin for Friedreich Ataxia - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

VYFXN-01 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Dormant Products

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

[Appendix](#)

[Methodology](#)

[Coverage](#)

[Secondary Research](#)

[Primary Research](#)

[Expert Panel Validation](#)

[Contact Us](#)

[Disclaimer](#)



## List Of Tables

### LIST OF TABLES

Number of Products under Development by Stage of Development, 2021  
Number of Products under Development by Therapy Areas, 2021  
Number of Products under Development by Indication, 2021  
Number of Products under Development by Companies, 2021  
Products under Development by Companies, 2021  
Number of Products under Investigation by Universities/Institutes, 2021  
Products under Investigation by Universities/Institutes, 2021  
Number of Products by Stage and Mechanism of Actions, 2021  
Number of Products by Stage and Route of Administration, 2021  
Number of Products by Stage and Molecule Type, 2021  
Pipeline by AavantiBio Inc, 2021  
Pipeline by Adverum Biotechnologies Inc, 2021  
Pipeline by Anima Biotech Inc, 2021  
Pipeline by Biointaxis SL, 2021  
Pipeline by Cellivery Therapeutics Inc, 2021  
Pipeline by CRISPR Therapeutics AG, 2021  
Pipeline by Design Therapeutics Inc, 2021  
Pipeline by Fratagene Therapeutics Srl, 2021  
Pipeline by Fulcrum Therapeutics Inc, 2021  
Pipeline by Jupiter Orphan Therapeutics Inc, 2021  
Pipeline by Lacerta Therapeutics Inc, 2021  
Pipeline by Larimar Therapeutics Inc, 2021  
Pipeline by Novartis Gene Therapies, 2021  
Pipeline by PTC Therapeutics Inc, 2021  
Pipeline by Voyager Therapeutics Inc, 2021  
Dormant Projects, 2021

## List Of Figures

### LIST OF FIGURES

Number of Products under Development by Stage of Development, 2021

Number of Products under Development by Therapy Areas, 2021

Number of Products under Development by Top 10 Indications, 2021

Number of Products by Mechanism of Actions, 2021

Number of Products by Stage and Mechanism of Actions, 2021

Number of Products by Routes of Administration, 2021

Number of Products by Stage and Routes of Administration, 2021

Number of Products by Molecule Types, 2021

Number of Products by Stage and Molecule Types, 2021

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