

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Pipeline Review, H2 2017

https://marketpublishers.com/r/F9EC304CB70EN.html

Date: December 2017

Pages: 43

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

ID: F9EC304CB70EN

Abstracts

Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) - Pipeline Review, H2 2017

SUMMARY

According to the recently published report 'Frataxin Mitochondrial - Pipeline Review, H2 2017'; Frataxin Mitochondrial (Friedreich Ataxia Protein or FXN or EC 1.16.3.1) pipeline Target constitutes close to 11 molecules. Out of which approximately 9 molecules are developed by companies and remaining by the universities/institutes.

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 report 'Frataxin Mitochondrial - Pipeline Review, H2 2017' 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; that are being developed by Companies/Universities.

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. Currently, The molecules developed by companies in Phase II, Preclinical and Discovery stages are 1, 6 and 2 respectively. Similarly, the



universities portfolio in Discovery stages comprises 2 molecules, respectively. Report covers products from therapy areas Central Nervous System and Cardiovascular which include indications Friedreich Ataxia and Cardiomyopathy.

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

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Involved in Therapeutics Development

Adverum Biotechnologies Inc

Bio Blast Pharma Ltd

Pfizer Inc

ProQR Therapeutics NV

Voyager Therapeutics Inc

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

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Mechanism Of Action

R&D Progress

AGIL-FA - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BB-FA - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CTI-1601 - Drug Profile

Product Description



Mechanism Of Action

R&D Progress

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

Product Description

Mechanism Of Action

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

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R&D Progress

QRX-604 - 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

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

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

Development Milestones

Featured News & Press Releases

Oct 31, 2017: Agilis Biotherapeutics Announces Orphan Product Designation Approval

in Europe for the Treatment of Friedreich Ataxia

Aug 22, 2017: Jupiter Orphan Therapeutics Receives Orphan Drug Designation for its

Trans-Resveratrol Product JOTROL for Treatment of Friedreich's Ataxia



Aug 03, 2017: Chondrial Announces FDA Orphan Drug Designation for CTI-1601, a Novel Investigational Technology for the Treatment of Friedreich's Ataxia

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

Aug 05, 2015: Key Patent Granted For AAVLife's Gene-Therapy Program to Treat Cardiomyopathy in Friedreich's Ataxia

Jun 18, 2015: BioBlast Pharma to Present Preclinical Proofs of Concept in Friedreich's Ataxia at Two Leading Scientific Meetings in June

Mar 26, 2015: BioBlast Pharma Announces Positive Preclinical Proof-of-Concept Results of Its Novel Mitochondrial Protein Replacement Platform in Friedreich's Ataxia Dec 17, 2014: BioBlast Receives Notice of Patent Issuance From USPTO for Its Novel Fusion Protein-Based Platform for the Treatment of Friedrich's Ataxia

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Number of Products by Stage and Molecule Types, H2 2017

COMPANIES MENTIONED

Adverum Biotechnologies Inc Bio Blast Pharma Ltd Pfizer Inc ProQR Therapeutics NV Voyager Therapeutics Inc



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