

Usher Syndrome - Drugs in Development, 2021

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Abstracts

Usher Syndrome - Drugs in Development, 2021

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Usher Syndrome - Drugs In Development, 2021, provides an overview of the Usher Syndrome (Genetic Disorders) pipeline landscape.

Usher syndrome is an inherited condition characterized by hearing impairment and progressive vision loss. The major symptoms of Usher syndrome are hearing loss and an eye disorder called retinitis pigmentosa. Risk factors include retinal degeneration, prolonged, unprotected exposure to sunlight may accelerate vision loss. Treatment includes cochlear implants, hearing aids and Vitamin A supplementation.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Usher Syndrome - Drugs In Development, 2021, provides comprehensive information on the therapeutics under development for Usher Syndrome (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 Usher Syndrome (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Usher Syndrome and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase II,



Preclinical and Discovery stages are 1, 13 and 2 respectively. Similarly, the Universities portfolio in Phase I and Preclinical stages comprises 1 and 3 molecules, respectively.

Usher Syndrome (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 Usher Syndrome (Genetic Disorders).

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

The pipeline guide evaluates Usher Syndrome (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 Usher Syndrome (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 Usher Syndrome (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 Usher Syndrome (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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Akouos Inc

Atsena Therapeutics Inc

Clearside BioMedical Inc

Editas Medicine Inc

Eloxx Pharmaceuticals Inc

Ionis Pharmaceuticals Inc

IVERIC bio Inc

Locanabio Inc

Nanoscope Therapeutics Inc

Odylia Therapeutics Inc

ProQR Therapeutics NV

Wave Life Sciences Ltd

Usher Syndrome - Drug Profiles

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BF-844 - Drug Profile

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R&D Progress

EDIT-102 - Drug Profile



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R&D Progress

Usher Syndrome - Dormant Projects

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Featured News & Press Releases

Sep 06, 2021: ProQR announces virtual presentation at EURETINA 2021

May 04, 2021: Editas Medicine to present preclinical data demonstrating progress in



development of gene editing medicines for the treatment of genetic ocular diseases at the American Society Of Gene And Cell Therapy annual meeting

May 01, 2021: Editas Medicine to present preclinical data demonstrating advancements in in vivo gene editing approach for the treatment of genetic ocular diseases at the Association for Research in Vision and Ophthalmology Annual Meeting

Mar 24, 2021: ProQR announces positive results from clinical trial of QR-421a in Usher Syndrome and plans to start pivotal trials

Feb 16, 2021: ProQR announces expert perspectives call on disease education and endpoints in usher syndrome

Jun 12, 2020: ProQR announces expert perspectives conference call series

Mar 31, 2020: ProQR announces positive findings from an interim analysis in the phase 1/2 trial of QR-421a for Usher Syndrome and provides business update

Mar 25, 2020: ProQR to present findings from phase 1/2 interim analysis of QR-421a for Usher Syndrome

Jan 31, 2020: FDA grants RPD designation to ProQR's retinitis pigmentosa therapy

May 02, 2019: Eloxx Pharmaceuticals presents positive new data at the Association for

Research in Vision and Ophthalmology "ARVO" 2019 Annual Meeting

Apr 22, 2019: ProQR announces presentation of abstracts on QR-421a at scientific conferences in April

Mar 11, 2019: ProQR doses first patient in Phase 1/2 stellar trial of QR-421a for Usher Syndrome Type

Jan 02, 2019: ProQR receives fast track designation from FDA for QR-421a for usher syndrome type

Dec 05, 2018: ProQR receives FDA approval for Phase I/II QR-421a trial

Jul 12, 2018: ProQR Announces Presentation on QR-421a in Ophthalmology at Conferences in July

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