

# Wilson Disease - Drugs in Development, 2021

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## Abstracts

Wilson Disease - Drugs in Development, 2021

### SUMMARY

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

Wilson disease is a rare autosomal recessive inherited disorder of copper metabolism that is characterized by excessive deposition of copper in the liver, brain, and other tissues. Symptoms include abdominal pain, jaundice, problems with speech, swallowing and muscle stiffness. Treatment includes chelators and Vitamin E supplements.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Wilson Disease - Drugs In Development, 2021, provides comprehensive information on the therapeutics under development for Wilson Disease (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 Wilson Disease (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Wilson Disease and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase III, Phase II, Preclinical and Discovery stages are 2, 1, 2, 5 and 4

respectively.

Wilson Disease (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 Wilson Disease (Genetic Disorders).

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

The pipeline guide evaluates Wilson Disease (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 Wilson Disease (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 Wilson Disease (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 Wilson Disease (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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Featured News & Press Releases

Oct 18, 2021: Ultragenyx initiates Cyprus2+, a pivotal clinical trial evaluating UX701 gene therapy for the treatment of Wilson disease

Sep 02, 2021: Orphalan announces FDA acceptance for filing of New Drug Application (NDA) for trientine tetrahydrochloride (TETA 4HCl) for the treatment of Wilson's Disease

Aug 27, 2021: Alexion's drug meets primary goal in Phase III Wilson disease trial

Jun 25, 2021: Orphalan announces positive top line data with Trientine Tetrahydrochloride for maintenance patients with Wilson's disease

Jan 21, 2021: Ultragenyx announces FDA clearance of Investigational New Drug (IND) application for UX701, a new gene therapy for the treatment of Wilson disease

Dec 09, 2020: Ultragenyx announces Orphan Drug Designation for UX701 for the treatment of Wilson disease

Jun 27, 2018: Progress toward improved Wilson's disease drug

Jun 01, 2018: Wilson Therapeutics to present data from WTX101 Phase 2 extension study at 4th Congress of the European Academy of Neurology

Apr 12, 2018: Promising preliminary long-term data for WTX101 in Wilson Disease presented at EASL Annual Meeting

Mar 28, 2018: Preliminary Long-Term Data For WTX101 In Wilson Disease Accepted As A Late-Breaker Presentation At EASL Annual Meeting

Feb 16, 2018: First patient enrolled in pivotal Phase 3 FOCuS trial evaluating WTX101 for the treatment of Wilson Disease

Dec 14, 2017: WTX101 Granted Fast Track Designation by the U.S. FDA for the Treatment of Wilson Disease

Oct 23, 2017: Wilson Therapeutics reaches agreement with the FDA and EMA to initiate pivotal Phase 3 FOCuS study with WTX101 in Wilson Disease

Oct 20, 2017: Promising Preliminary Long-term Data For WTX101 In Wilson Disease Highlighted at The Liver Meeting

Oct 06, 2017: Phase 2 Clinical Trial Data for WTX101 Published in The Lancet Gastroenterology & Hepatology

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