

# Wilson Disease - Pipeline Review, H1 2020

<https://marketpublishers.com/r/W67B36448B0EN.html>

Date: June 2020

Pages: 65

Price: US\$ 2,000.00 (Single User License)

ID: W67B36448B0EN

## Abstracts

Wilson Disease - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Wilson Disease - Pipeline Review, H1 2020, 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 - Pipeline Review, H1 2020, 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, Preclinical and Discovery stages are 1, 2, 5 and 3 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 1 molecules, 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.

## Contents

- Introduction
- Global Markets Direct Report Coverage
- Wilson Disease - Overview
- Wilson Disease - Therapeutics Development
- Pipeline Overview
- Pipeline by Companies
- Pipeline by Universities/Institutes
- Products under Development by Companies
- Products under Development by Universities/Institutes
- Wilson Disease - Therapeutics Assessment
- Assessment by Target
- Assessment by Mechanism of Action
- Assessment by Route of Administration
- Assessment by Molecule Type
- Wilson Disease - Companies Involved in Therapeutics Development
- Alexion Pharmaceuticals Inc
- Aligen Therapeutics SL
- Deep Genomics Inc
- DepYmed Inc
- Generation Bio Co
- GMP-Orphan SA
- Krisani Bio Sciences Pvt Ltd
- LambdaGen Therapeutics
- Nobelpharma Co Ltd
- Ultragenyx Pharmaceutical Inc
- Vivet Therapeutics SAS
- Wilson Disease - Drug Profiles
- CM-1186 - Drug Profile
- Product Description
- Mechanism Of Action
- R&D Progress
- DG-12P1 - Drug Profile
- Product Description
- Mechanism Of Action
- R&D Progress
- DPM-1001 - Drug Profile
- Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Wilson's Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate ATP7B for Wilson Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

KBHD-001 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

methanobactin - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

tiomolibdate choline - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

trientine tetrahydrochloride - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

UX-701 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

VTX-801 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

zinc acetate - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Wilson Disease - Dormant Projects

## Wilson Disease - Product Development Milestones

### Featured News & Press Releases

Apr 23, 2019: Vivet Therapeutics to present on its wilson disease drug candidate VTX-801 at 2019 American Society of Gene and Cell Therapy Annual Meeting

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

Oct 01, 2017: Promising Preliminary Long-term Data from WTX101 Phase 2 Extension Study to be Presented at AASLD Annual Meeting

Sep 26, 2017: Vivet's First Gene Therapy Product, VTX 801 for Wilson's Disease, Receives European and US Orphan Drug Designation

Jun 08, 2017: Wilson Therapeutics presents Phase 2 data for WTX101 at MDS meeting

Apr 25, 2017: Wilson Therapeutics presents promising neurological Phase 2 data for WTX101 at AAN meeting

Apr 24, 2017: Wilson Therapeutics presented positive final Phase 2 data for WTX101 at EASL Annual Meeting

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer

## List Of Tables

### LIST OF TABLES

Number of Products under Development for Wilson Disease, H1 2020  
Number of Products under Development by Companies, H1 2020  
Number of Products under Development by Universities/Institutes, H1 2020  
Products under Development by Companies, H1 2020  
Products under Development by Universities/Institutes, H1 2020  
Number of Products by Stage and Target, H1 2020  
Number of Products by Stage and Mechanism of Action, H1 2020  
Number of Products by Stage and Route of Administration, H1 2020  
Number of Products by Stage and Molecule Type, H1 2020  
Wilson Disease - Pipeline by Alexion Pharmaceuticals Inc, H1 2020  
Wilson Disease - Pipeline by Aligen Therapeutics SL, H1 2020  
Wilson Disease - Pipeline by Deep Genomics Inc, H1 2020  
Wilson Disease - Pipeline by DepYmed Inc, H1 2020  
Wilson Disease - Pipeline by Generation Bio Co, H1 2020  
Wilson Disease - Pipeline by GMP-Orphan SA, H1 2020  
Wilson Disease - Pipeline by Krisani Bio Sciences Pvt Ltd, H1 2020  
Wilson Disease - Pipeline by LambdaGen Therapeutics, H1 2020  
Wilson Disease - Pipeline by Nobelpharma Co Ltd, H1 2020  
Wilson Disease - Pipeline by Ultragenyx Pharmaceutical Inc, H1 2020  
Wilson Disease - Pipeline by Vivet Therapeutics SAS, H1 2020  
Wilson Disease - Dormant Projects, H1 2020

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Wilson Disease, H1 2020  
Number of Products under Development by Companies, H1 2020  
Number of Products by Top 10 Targets, H1 2020  
Number of Products by Stage and Top 10 Targets, H1 2020  
Number of Products by Top 10 Mechanism of Actions, H1 2020  
Number of Products by Stage and Top 10 Mechanism of Actions, H1 2020  
Number of Products by Stage and Top 10 Routes of Administration, H1 2020  
Number of Products by Top 10 Molecule Types, H1 2020  
Number of Products by Stage and Top 10 Molecule Types, H1 2020

### COMPANIES MENTIONED

Alexion Pharmaceuticals Inc  
Aligen Therapeutics SL  
Deep Genomics Inc  
DepYmed Inc  
Generation Bio Co  
GMP-Orphan SA  
Krisani Bio Sciences Pvt Ltd  
LambdaGen Therapeutics  
Nobelpharma Co Ltd  
Ultragenyx Pharmaceutical Inc  
Vivet Therapeutics SAS



## I would like to order

Product name: Wilson Disease - Pipeline Review, H1 2020

Product link: <https://marketpublishers.com/r/W67B36448B0EN.html>

Price: US\$ 2,000.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/W67B36448B0EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970