

# Metachromatic Leukodystrophy (MLD) - Pipeline Review, H2 2019

<https://marketpublishers.com/r/M13FF865D53EN.html>

Date: December 2019

Pages: 69

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

ID: M13FF865D53EN

## Abstracts

Metachromatic Leukodystrophy (MLD) - Pipeline Review, H2 2019

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Metachromatic Leukodystrophy - Pipeline Review, H2 2019, provides an overview of the Metachromatic Leukodystrophy (Central Nervous System) pipeline landscape.

Metachromatic leukodystrophy is an inherited disorder characterized by the accumulation of fats called sulfatides in cells. Symptoms include vision problems leading to blindness, personality changes, and motor disturbances such as clumsiness, muscle weakness (hypotonia), rigidity, inability to coordinate movement (ataxia), and/or muscle spasms especially of the neck, spine, arms, and legs. Treatment for metachromatic leukodystrophy is symptomatic and supportive.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Metachromatic Leukodystrophy - Pipeline Review, H2 2019, provides comprehensive information on the therapeutics under development for Metachromatic Leukodystrophy (Central Nervous System), 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 Metachromatic Leukodystrophy (Central Nervous System) pipeline guide also

reviews of key players involved in therapeutic development for Metachromatic Leukodystrophy (MLD) 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 II, Phase I and Preclinical stages are 1, 2, 1 and 3 respectively. Similarly, the Universities portfolio in Phase II and Phase I stages comprises 2 and 1 molecules, respectively.

Metachromatic Leukodystrophy (Central Nervous System) 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 Metachromatic Leukodystrophy (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Metachromatic Leukodystrophy (Central Nervous System) 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 Metachromatic Leukodystrophy (Central Nervous System) therapeutics and enlists all their

major and minor projects.

The pipeline guide evaluates Metachromatic Leukodystrophy (Central Nervous System) 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 Metachromatic Leukodystrophy (Central Nervous System)

## **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 Metachromatic Leukodystrophy (Central Nervous System).

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 Metachromatic Leukodystrophy (Central Nervous System) 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

Metachromatic Leukodystrophy (MLD) - Overview

Metachromatic Leukodystrophy (MLD) - Therapeutics Development

Pipeline Overview

Pipeline by Companies

Pipeline by Universities/Institutes

Products under Development by Companies

Products under Development by Universities/Institutes

Metachromatic Leukodystrophy (MLD) - Therapeutics Assessment

Assessment by Target

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Metachromatic Leukodystrophy (MLD) - Companies Involved in Therapeutics

Development

ArmaGen Inc

EnhanX Biopharm Inc

Homology Medicines Inc

Magenta Therapeutics Inc

Orchard Therapeutics Plc

Recursion Pharmaceuticals Inc

Takeda Pharmaceutical Co Ltd

Metachromatic Leukodystrophy (MLD) - Drug Profiles

AGT-183 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

DUOC-01 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ENX-201 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Metachromatic Leukodystrophy and Adrenoleukodystrophy - Drug

Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Arylsulfatase A for Metachromatic Leukodystrophy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

HMI-202 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

MGTA-456 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

OTL-200 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules for Metachromatic Leukodystrophy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

TAK-611 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Metachromatic Leukodystrophy (MLD) - Dormant Projects

Metachromatic Leukodystrophy (MLD) - Discontinued Products

Metachromatic Leukodystrophy (MLD) - Product Development Milestones

Featured News & Press Releases

Dec 02, 2019: Orchard Therapeutics announces the filing and validation of marketing authorization application by European Medicines Agency for OTL-200 for the treatment of metachromatic leukodystrophy

Nov 18, 2019: European Medicines Agency grants Orchard Therapeutics accelerated assessment of OTL-200 for patients with Metachromatic Leukodystrophy

Oct 22, 2019: Orchard Therapeutics presents data from OTL-200 in patients with

## Metachromatic Leukodystrophy using Cryopreservation

Oct 21, 2019: Homology Medicines presents data from investigational MLD gene therapy program HMI-202 at the American Society of Human Genetics (ASHG) 2019 Meeting

Sep 30, 2019: Orchard Therapeutics announces presentation of new clinical data for OTL-200 for Metachromatic Leukodystrophy at upcoming European Society of Gene & Cell Therapy Annual Congress

Sep 04, 2019: Orchard Therapeutics presents an integrated data analysis demonstrating sustained clinical benefit of OTL-200 for the treatment of Metachromatic Leukodystrophy

Sep 04, 2019: Magenta Therapeutics announces FDA regenerative medicine advanced therapy (RMAT) designation granted to MGTA-456 for the treatment of Inherited Metabolic Disorders

Aug 27, 2019: Orchard Therapeutics announces presentation on OTL-200 at Upcoming Society for the Study of Inborn Errors of Metabolism (SSIEM) 2019 Annual Symposium

May 09, 2019: Magenta Therapeutics presents updated phase 2 clinical data on MGTA-456 cell therapy at American Academy of Neurology Annual Meeting

Mar 27, 2019: Orchard Therapeutics presents new registrational data demonstrating sustained clinical benefit of OTL-200 for the treatment of MLD

Mar 11, 2019: Orchard Therapeutics announces acceptance of OTL-200 abstract for MLD at the 45th Annual Meeting of the European Society for Blood and Bone Marrow Transplantation (EBMT)

Feb 25, 2019: Magenta Therapeutics Presents Clinical and Preclinical Data on MGTA-456 Cell Therapy in Best Abstracts Sessions at Transplant and Cellular Therapy (TCT) Annual Meeting

Dec 02, 2018: Magenta Therapeutics presents new data from phase 2 study of MGTA-456 cell therapy in patients with inherited metabolic disorders

Nov 07, 2018: Homology Medicines presents data on platform's ability to target the central and peripheral nervous system and therapeutic potential in the rare disease Metachromatic Leukodystrophy

Nov 02, 2018: Magenta presents preclinical data on MGTA-456 at ASH annual meeting 2018

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 Metachromatic Leukodystrophy (MLD), H2 2019

Number of Products under Development by Companies, H2 2019

Number of Products under Development by Universities/Institutes, H2 2019

Products under Development by Companies, H2 2019

Products under Development by Universities/Institutes, H2 2019

Number of Products by Stage and Target, H2 2019

Number of Products by Stage and Mechanism of Action, H2 2019

Number of Products by Stage and Route of Administration, H2 2019

Number of Products by Stage and Molecule Type, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by ArmaGen Inc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by EnhanceX Biopharm Inc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by Homology Medicines Inc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by Magenta Therapeutics Inc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by Orchard Therapeutics Plc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by Recursion Pharmaceuticals Inc, H2 2019

Metachromatic Leukodystrophy (MLD) - Pipeline by Takeda Pharmaceutical Co Ltd, H2 2019

Metachromatic Leukodystrophy (MLD) - Dormant Projects, H2 2019

Metachromatic Leukodystrophy (MLD) - Discontinued Products, H2 2019

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Metachromatic Leukodystrophy (MLD), H2 2019

Number of Products under Development by Companies, H2 2019

Number of Products under Development by Universities/Institutes, H2 2019

Number of Products by Targets, H2 2019

Number of Products by Stage and Targets, H2 2019

Number of Products by Mechanism of Actions, H2 2019

Number of Products by Stage and Mechanism of Actions, H2 2019

Number of Products by Routes of Administration, H2 2019

Number of Products by Stage and Routes of Administration, H2 2019

Number of Products by Molecule Types, H2 2019

Number of Products by Stage and Molecule Types, H2 2019

### COMPANIES MENTIONED

ArmaGen Inc

EnhanX Biopharm Inc

Homology Medicines Inc

Magenta Therapeutics Inc

Orchard Therapeutics Plc

Recursion Pharmaceuticals Inc

Takeda Pharmaceutical Co Ltd

## I would like to order

Product name: Metachromatic Leukodystrophy (MLD) - Pipeline Review, H2 2019

Product link: <https://marketpublishers.com/r/M13FF865D53EN.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/M13FF865D53EN.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