

# **Globoid Cell Leukodystrophy (Krabbe Disease) - Pipeline Review, H1 2020**

<https://marketpublishers.com/r/G57E43F6F00EN.html>

Date: June 2020

Pages: 77

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

ID: G57E43F6F00EN

## **Abstracts**

Globoid Cell Leukodystrophy (Krabbe Disease) - Pipeline Review, H1 2020

### **SUMMARY**

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Globoid Cell Leukodystrophy - Pipeline Review, H1 2020, provides an overview of the Globoid Cell Leukodystrophy (Central Nervous System) pipeline landscape.

Krabbe disease also called globoid cell leukodystrophy is a degenerative disorder caused by the deficiency of an enzyme called galactosylceramidase. This enzyme deficiency impairs the growth and maintenance of myelin. Symptoms include seizures, Muscle spasms, Loss of head control, vomiting, fevers, irritability and excessive crying. Risk factors include family history. Treatment focuses on managing symptoms (anticonvulsant, muscle relaxer) and providing supportive care.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Globoid Cell Leukodystrophy - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Globoid Cell 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 Globoid Cell Leukodystrophy (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Globoid Cell Leukodystrophy (Krabbe Disease) 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, 5 and 3 respectively. Similarly, the Universities portfolio in Phase I and Preclinical stages comprises 1 and 1 molecules, respectively.

Globoid Cell 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 Globoid Cell Leukodystrophy (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Globoid Cell 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 Globoid Cell Leukodystrophy (Central Nervous System) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Globoid Cell 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 Globoid Cell 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 Globoid Cell 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 Globoid Cell 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.



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Featured News & Press Releases

May 12, 2020: Passage Bio announces presentation of data from animal models of Krabbe disease at the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting

Feb 20, 2020: Magenta Therapeutics announces updated phase 2 data on MGTA-456 cell therapy, demonstrating continued durability in inherited metabolic disorders

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

May 30, 2019: Passage Bio announces third gene therapy development program in Krabbe Disease and supports million dreams fundraising gala

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

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

Apr 05, 2018: Magenta Therapeutics Announces First Patient Transplanted with MGTA-456 in Phase 2 Study in Inherited Metabolic Disorders

Jan 18, 2016: FDA Grants Rare Pediatric Disease Designation to MediciNova's MN-166 (ibudilast) for the Treatment of Krabbe Disease

Jun 03, 2015: MediciNova Announces FDA Granted Orphan Drug Designation to MN-166 (ibudilast) for Krabbe Disease

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BioXcel Corp

Commence Bio Inc

GT Gain Therapeutics SA

Kyorin Pharmaceutical Co Ltd

Magenta Therapeutics Inc

Passage Bio Inc

Polaryx Therapeutics Inc

Vascumab LLC

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