

Globoid Cell Leukodystrophy (Krabbe Disease) (Central Nervous System) - Drugs in Development, 2021

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Abstracts

Globoid Cell Leukodystrophy (Krabbe Disease) (Central Nervous System) - Drugs in Development, 2021

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Globoid Cell Leukodystrophy - Drugs In Development, 2021, 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 - Drugs In Development, 2021, 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 2, 7 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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Bial - Portela & Ca SA

BioXcel Corp

Forge Biologics Inc

Gain Therapeutics Inc

Kyorin Pharmaceutical Co Ltd

M6P Therapeutics

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Vascumab LLC

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Featured News & Press Releases

Sep 20, 2021: Forge Biologics announces regulatory updates from FDA and EMA, accelerating manufacturing and clinical trial momentum

Apr 05, 2021: Passage Bio receives European Commission Orphan Designation for PBKR03 for treatment of Krabbe Disease

Mar 08, 2021: Passage Bio announced that U.S. Food and Drug Administration grants Fast Track Designation to its gene therapy candidate PBKR03

Feb 16, 2021: Forge Biologics Receives FDA Fast Track, Orphan Drug, and Rare Pediatric Disease Designations for FBX-101 Gene Therapy for Patients with Krabbe Disease

Feb 10, 2021: Polaryx Therapeutics receives both Rare Pediatric Disease and Orphan Drug Designations for the treatment of Krabbe Disease with PLX-300

Feb 08, 2021: FDA clears IND application for Passage Bio's gene therapy candidate PBKR03 for treatment of patients with early infantile Krabbe disease, a rare pediatric disorder with no approved disease-modifying treatment options

Jan 04, 2021: Forge Biologics announces FDA clearance of Investigational New Drug application for phase 1/2 clinical trial (RESKUE) of FBX-101 gene therapy for patients with Krabbe disease

Oct 28, 2020: Passage Bio's PBKR03 receives Orphan Drug and Rare Pediatric Disease Designations from FDA for treatment of Krabbe Disease

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

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

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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