

# Canavan Disease Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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# **Abstracts**

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### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Canavan Disease - Drugs In Development, 2022, provides an overview of the Canavan Disease (Central Nervous System) pipeline landscape.

Canavan disease is a gene-linked neurological disorder in which the brain degenerates into spongy tissue riddled with microscopic fluid-filled spaces. Cause includes a mutation in the gene which directs the production of the enzyme aspartoacylase (ASPA) allows the buildup of N-acetylaspartic acid (NAA) in the brain. The buildup of NAA causes damage to myelin. Symptoms include lack of motor development, feeding difficulties, abnormal muscle tone, and an abnormally large, poorly controlled head, paralysis, blindness, or hearing loss.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Canavan Disease - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Canavan Disease (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 Canavan Disease (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Canavan 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 and Unknown stages are 2 and 1 respectively. Similarly, the Universities portfolio in Phase I, Preclinical and Discovery stages comprises 1, 3 and 1 molecules, respectively.

Canavan Disease (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 Canavan Disease (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Canavan Disease (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 Canavan Disease



(Central Nervous System) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Canavan Disease (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 Canavan Disease (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 Canavan Disease (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 Canavan Disease (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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Stem Cell Therapy to Activate Aspartoacylase for Canavan Disease - Drug Profile

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Unnamed - Drug Profile

Product Description

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Featured News & Press Releases

Oct 13, 2022: BridgeBio Pharma presents updated positive data from its BBP-812

Canavan disease gene therapy program at the 51st Annual Meeting of the Child Neurology Society

Jun 22, 2022: BridgeBio Pharma announces early positive data for BBP-812, its investigational AAV9 gene therapy for Canavan disease

Nov 03, 2021: BridgeBio Pharma announces dosing of first patient in phase 1/2 trial of investigational gene therapy for Canavan disease

Oct 22, 2019: BridgeBio Pharma presents on its Central Nervous drug candidate

BBP-812 at the European Society of Gene and Cell Therapy Conference

Nov 09, 2017: New gene therapy developed with UNSW researchers targets brain disorders

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