

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

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## **Abstracts**

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

### **SUMMARY**

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Batten Disease - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides an overview of the Batten Disease (Central Nervous System) pipeline landscape.

Batten disease, a rare genetic disorder, belongs to a group of progressive degenerative neurometabolic disorders known as the neuronal ceroid lipofuscinoses. Symptoms of Batten disease usually become apparent between five and 15 years of age. Symptoms include vision loss, lack of muscle coordination, mental retardation or decreasing mental function, emotional disturbances, seizures, muscle spasms, deterioration of muscle tone and movement problems.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Batten Disease - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides comprehensive information on the therapeutics under development for Batten 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 Batten Disease (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Batten 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, Phase I, IND/CTA Filed, Preclinical and Discovery stages are 4, 1, 3, 14 and 6 respectively. Similarly, the Universities portfolio in Phase I, Preclinical and Discovery stages comprises 1, 5 and 3 molecules, respectively.

Batten 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 Batten Disease (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Batten 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 Batten Disease (Central Nervous System) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Batten 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 Batten 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 Batten 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 Batten 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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Featured News & Press Releases

Feb 09, 2022: Taysha Gene Therapies announces positive safety data from UT Southwestern-sponsored clinical trial for the treatment of CLN7 batten disease at 18th annual WORLDSymposium

Dec 16, 2021: Taysha Gene Therapies announces initiation of clinical development of TSHA-118 for the treatment of CLN1 disease

Sep 14, 2021: Neurogene announces FDA clearance of IND for NGN-101 Gene Therapy to Treat CLN5 Batten Disease

Aug 17, 2021: Taysha Gene Therapies to host key opinion leader webinar on TSHA-118 for the treatment of CLN1 disease

Jun 29, 2021: Neurogene announces EMA grants orphan drug designation to CLN5 Batten disease gene therapy

Nov 19, 2020: The Food and Drug Administration (FDA) issues a favorable opinion on the preclinical development plan submitted by Theranexus and BBDF

Nov 12, 2020: Neurogene announces first patient enrolled in Natural History study evaluating two subtypes of Batten Disease including CLN5

Nov 12, 2020: Neurogene announces first patient enrolled in Natural History Study evaluating two subtypes including CLN7 subtype of Batten Disease

Aug 20, 2020: Polaryx Therapeutics receives FDA Fast Track Designation to PLX-200 for the treatment of patients with Juvenile Neuronal Ceroid Lipofuscinosis

Aug 11, 2020: Theranexus and BBDF obtain orphan drug designation (ODD) and rare pediatric disease designation (RPDD) from the Food and Drug Administration (FDA) for BBDF-101 for Batten Disease

Aug 11, 2020: Neurogene announces FDA Orphan Drug Designation for CLN7 Batten Disease Gene Therapy

Jul 07, 2020: FDA grants Orphan Drug Designation to Neurogene's gene therapy for the treatment of CLN5 Batten Disease

Jun 24, 2020: Theranexus provides update on its Batten disease drug candidate BBDF-101

May 05, 2020: Amicus Therapeutics announces presentation on its CLN8 batten disease gene therapy at the American Society of Gene & Cell Therapy 23rd Annual Meeting

Apr 08, 2020: Polaryx Therapeutics receives IND approval from the FDA to study PLX-200 treatment for patients with Juvenile Neuronal Ceroid Lipofuscinosis

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