

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

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## **Abstracts**

Rett Syndrome 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 Rett Syndrome - Drugs In Development, 2022, provides an overview of the Rett Syndrome (Central Nervous System) pipeline landscape.

Rett syndrome is a neurodevelopmental disorder. This is caused by genetic mutation on MECP2 gene. Rett syndrome is described in four stages: Stage I or early onset, Stage II or rapid destruction, Stage III, or the plateau and Stage IV, or the late motor deterioration stage. Symptoms include slowed growth, loss of normal movement and coordination, loss of communication abilities, breathing problems, pain, irregular heartbeat and seizures. Risk factors include family history and gender. Treatment includes disease modifying drugs.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Rett Syndrome - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for Rett Syndrome (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 Rett Syndrome (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Rett Syndrome 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 III, Phase II, Phase I, Preclinical and Discovery stages are 1, 1, 4, 4, 20 and 13 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 1 molecule, respectively.

Rett Syndrome (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 Rett Syndrome (Central Nervous System).

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

The pipeline guide evaluates Rett Syndrome (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 Rett Syndrome (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 Rett Syndrome (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 Rett Syndrome (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

Jul 18, 2022: Acadia Pharmaceuticals submits New Drug Application to the U.S. FDA for Trofinetide for the treatment of Rett Syndrome

May 18, 2022: Neurogene announces new development program in Rett Syndrome utilizing novel EXACT technology platform

May 03, 2022: DepYmed receives FDA Rare Pediatric Disease and Orphan Drug Designations for its lead clinical candidate for the treatment of Rett Syndrome

Mar 29, 2022: Taysha Gene Therapies announces initiation of clinical development of TSHA-102 in Rett Syndrome

Mar 22, 2022: Acadia Pharmaceuticals to present late-breaking data at the 2022 American Academy of Neurology Annual Meeting (AAN)

Mar 10, 2022: DepYmed and Monash Biomedicine Discovery Institute to study the role of a new generation of PTP1B inhibitors against a novel intracellular checkpoint in cancer

Feb 04, 2022: Anavex life sciences provides supplemental information on ANAVEX

2-73 (blarcamesine) AVATAR Phase 3 trial for the treatment of adult patients with Rett syndrome

Feb 01, 2022: ANAVEX 2-73 (Blarcamesine) AVATAR phase 3 trial met primary and secondary efficacy endpoints

Dec 07, 2021: Acadia reports positive data from Phase III Rett Syndrome drug trial

Dec 06, 2021: Acadia Pharmaceuticals announces positive top-line results from the pivotal phase 3 lavender trial of Trofinetide in rett syndrome

Sep 22, 2021: Taysha receives Orphan Drug Designation from the European Commission for TSHA-102 for the treatment of Rett Syndrome

Sep 08, 2021: Taysha Gene Therapies to host key opinion leader webinar on TSHA-102 for the treatment of Rett syndrome

Sep 01, 2021: DAFFODIL Rett syndrome clinical trial for trofinetide in girls aged 2 to 5 years

Aug 05, 2021: Rett syndrome Phase 3 trial enrolment successfully completed - results in Q4 2021

Jun 21, 2021: Anavex Life Sciences announces ANAVEX2-73 (Blarcamesine) biomarker correlated with efficacy endpoints in placebo-controlled U.S. phase 2 clinical trial for the treatment of adult patients with Rett syndrome

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