

# **Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genitourinary Disorders) - Drugs In Development, 2021**

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## **Abstracts**

Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genitourinary Disorders) - Drugs In Development, 2021

### **SUMMARY**

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genitourinary Disorders) - Drugs In Development, 2021, provides an overview of the Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) pipeline landscape.

Sandhoff disease is an inherited lipid storage disorder that progressively destroys nerve cells (neurons) in the brain and spinal cord. It is caused by a deficiency of the enzyme beta-hexosaminidase. Symptoms include progressive nervous system deterioration, problems initiating and controlling muscles and movement, increased startle reaction to sound, early blindness, seizures, spasticity, and myoclonus. Treatment includes anticonvulsants to control seizures.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genitourinary Disorders) - Drugs In Development, 2021, provides comprehensive information on the therapeutics under development for Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders), 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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Sandhoff Disease (Jatzkewitz-Pilz Syndrome) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies /Universities /Institutes, the molecules developed by Companies in Phase III, Phase II, Preclinical and Discovery stages are 1, 2, 4 and 1 respectively. Similarly, the Universities portfolio in Phase I stages comprises 1 molecules, respectively.

Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) 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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) 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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) 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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders)

## **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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders).

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 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) (Genetic Disorders) 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 03, 2021: Sio Gene Therapies announces first patient dosed in clinical trial of AXO-AAV-GM2 in patients with Tay-Sachs and Sandhoff Disease (GM2 Gangliosidosis)

Nov 09, 2020: Axovant Gene Therapies announces FDA clearance of IND for AXO-AAV-GM2 gene therapy in Tay-Sachs and Sandhoff Diseases

Oct 13, 2020: Axovant Gene Therapies receives Rare Pediatric Disease Designation for AXO-AAV-GM2 for Tay-Sachs and Sandhoff disease

Mar 25, 2020: IntraBio Fast Track Designation for GM2 Gangliosidosis

Jan 13, 2020: Axovant provides update on gene therapy pipeline program AXO-AAV-GM2

Feb 21, 2019: Axovant to provide update on drug candidate AXO-AAV-GM2 at its first R&D Day March 2019

Feb 11, 2019: ntraBio investigational new drug application approved by the FDA for the treatment of tay-sachs and sandhoff disease

Dec 12, 2018: IntraBio receives Tay-Sachs and Sandhoff Rare pediatric disease

designation from the FDA

Nov 19, 2018: IntraBio receives Tay-Sachs and Sandhoff European Orphan Drug Designation

Oct 01, 2018: IntraBio receives FDA rare pediatric disease designation for ib1000 for GM2 Gangliosidosis

Mar 28, 2018: IntraBio receives FDA orphan drug designation for ib1000 for GM2 Gangliosidosis

Oct 30, 2017: IntraBio receives EMA orphan medicinal drug designation for IB1000s for GM2 Gangliosidosis

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