

# Sandhoff Disease (Jatzkewitz-Pilz Syndrome) -Pipeline Review, H2 2020

https://marketpublishers.com/r/SA1421BCE61EN.html

Date: August 2020 Pages: 45 Price: US\$ 2,000.00 (Single User License) ID: SA1421BCE61EN

### **Abstracts**

Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline Review, H2 2020

### Summary

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline Review, H2 2020, 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) - Pipeline Review, H2 2020, 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.



### Contents

Introduction Global Markets Direct Report Coverage Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Overview Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Therapeutics Development **Pipeline Overview** Pipeline by Companies Pipeline by Universities/Institutes Products under Development by Companies Products under Development by Universities/Institutes Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Therapeutics Assessment Assessment by Target Assessment by Mechanism of Action Assessment by Route of Administration Assessment by Molecule Type Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Companies Involved in Therapeutics **Development** Axovant Gene Therapies Ltd Genzyme Corp IntraBio Ltd **Orphi Therapeutics Inc** Polaryx Therapeutics Inc Taysha Gene Therapies Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Drug Profiles acetylleucine - Drug Profile Product Description Mechanism Of Action **R&D** Progress AXO-AAV-GM2 - Drug Profile **Product Description** Mechanism Of Action **R&D** Progress Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Dormant Projects Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Product Development Milestones Featured News & Press Releases Mar 25, 2020: IntraBio Fast Track Designation for GM2 Gangliosidosis 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 Gangliosidoses Mar 28, 2018: Intrabio receives FDA orphan drug designation for ib1000 for GM2 Gangliosidoses Oct 30, 2017: Intrabio receives EMA orphan medicinal drug designation for IB1000s for GM2 Gangliosidoses Appendix Methodology Coverage Secondary Research Primary Research Expert Panel Validation Contact Us Disclaimer



## List Of Tables

### LIST OF TABLES

Number of Products under Development for Sandhoff Disease (Jatzkewitz-Pilz Syndrome), H2 2020 Number of Products under Development by Companies, H2 2020 Number of Products under Development by Universities/Institutes, H2 2020 Products under Development by Companies, H2 2020 Products under Development by Universities/Institutes, H2 2020 Number of Products by Stage and Target, H2 2020 Number of Products by Stage and Mechanism of Action, H2 2020 Number of Products by Stage and Route of Administration, H2 2020 Number of Products by Stage and Molecule Type, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by Axovant Gene Therapies Ltd, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by Genzyme Corp, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by IntraBio Ltd, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by Orphi Therapeutics Inc, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by Polaryx Therapeutics Inc, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline by Taysha Gene Therapies, H2 2020 Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Dormant Projects, H2 2020



## **List Of Figures**

### LIST OF FIGURES

Number of Products under Development for Sandhoff Disease (Jatzkewitz-Pilz Syndrome), H2 2020 Number of Products under Development by Companies, H2 2020 Number of Products by Targets, H2 2020 Number of Products by Stage and Targets, H2 2020 Number of Products by Mechanism of Actions, H2 2020 Number of Products by Stage and Mechanism of Actions, H2 2020 Number of Products by Routes of Administration, H2 2020 Number of Products by Stage and Routes of Administration, H2 2020 Number of Products by Top 10 Molecule Types, H2 2020 Number of Products by Stage and Molecule Types, H2 2020

### **COMPANIES MENTIONED**

Axovant Gene Therapies Ltd Genzyme Corp IntraBio Ltd Orphi Therapeutics Inc Polaryx Therapeutics Inc Taysha Gene Therapies



### I would like to order

Product name: Sandhoff Disease (Jatzkewitz-Pilz Syndrome) - Pipeline Review, H2 2020 Product link: <u>https://marketpublishers.com/r/SA1421BCE61EN.html</u>

> Price: US\$ 2,000.00 (Single User License / Electronic Delivery) If you want to order Corporate License or Hard Copy, please, contact our Customer Service: <u>info@marketpublishers.com</u>

### Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <u>https://marketpublishers.com/r/SA1421BCE61EN.html</u>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name: Last name: Email: Company: Address: City: Zip code: Country: Tel: Fax: Your message:

\*\*All fields are required

Custumer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <u>https://marketpublishers.com/docs/terms.html</u>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970