

# Acid Sphingomyelinase Deficiency Type C - Pipeline Review, H1 2017

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## **Abstracts**

Acid Sphingomyelinase Deficiency Type C - Pipeline Review, H1 2017

### **SUMMARY**

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Acid Sphingomyelinase Deficiency Type C - Pipeline Review, H1 2017, provides an overview of the Acid Sphingomyelinase Deficiency Type C (Genetic Disorders) pipeline landscape.

Niemann-Pick C disease is one of a group of lysosomal storage diseases that affect metabolism and that are caused by genetic mutations. It involves the accumulation of sphingolipids in cells throughout the body, particularly reticuloendothelial cells (the mononuclear phagocyte system). Symptoms include enlarged liver, brain damage, difficulty walking and swallowing, increased sensitivity to touch, difficulty speaking, loss of muscle tone, learning difficulties.

## **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Acid Sphingomyelinase Deficiency Type C - Pipeline Review, H1 2017, provides comprehensive information on the therapeutics under development for Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) Type C 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, Phase I, Preclinical and Discovery stages are 2, 2, 1, 5 and 2 respectively. Similarly, the Universities portfolio in Preclinical and Discovery stages comprises 3 and 2 molecules, respectively.

Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (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 Acid Sphingomyelinase Deficiency Type C (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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Involved in Therapeutics Development

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Jun 24, 2015: Neurotrope to Conduct Study on Niemann-Pick Type C, a Devastating Rare Pediatric Disease

Jun 12, 2015: Neurotrope to Present Findings of In-vitro Studies of Bryostatin's Effects on Niemann Pick Type C1 Cells at the Annual 'Michael, Marcia & Christa Parseghian Scientific Conference' for Niemann-Pick Type C

Feb 21, 2013: Orphazyme Receives Orphan Drug Designation For OR0005 In EU Appendix

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### **COMPANIES MENTIONED**

CTD Holdings Inc
Merck & Co Inc
Okklo Life Sciences BV
Orphazyme ApS
Sucampo Pharmaceuticals Inc



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