

# Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) Type C Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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## Abstracts

Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) Type C 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 Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) Type C - Drugs In Development, 2022, provides an overview of the Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) 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 (Niemann-Pick Disease) Type C - Drugs In Development, 2022, provides comprehensive information on the therapeutics under development for

Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) 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 (Niemann-Pick Disease) 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under Development by Companies/Universities/Institutes, the molecules developed by Companies in Filing rejected/Withdrawn, Phase III, Phase I, Preclinical, Discovery and Unknown stages are 1, 3, 2, 14, 5 and 1 respectively. Similarly, the Universities portfolio in Phase 0, Preclinical and Discovery stages comprises 1, 2 and 2 molecules, respectively.

Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) 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 (Niemann-Pick Disease) Type C (Genetic Disorders).

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

The pipeline guide evaluates Acid Sphingomyelinase Deficiency (Niemann-Pick Disease) 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 (Niemann-Pick Disease) 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 (Niemann-Pick Disease) 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 (Niemann-Pick Disease) 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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Jul 13, 2022: Cyclo Therapeutics to present at the World Orphan Drug Congress USA  
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Jun 24, 2022: Cyclo Therapeutics to present at the 2022 NPC Patient and Family  
Conference hosted by the Australian NPC Disease Foundation

May 09, 2022: Cyclo Therapeutics to participate in the virtual investor Niemann-Pick  
Disease Type C spotlight event

Mar 24, 2022: Azafaros announces FDA Grant of Orphan Drug Designation for AZ-3102  
in the treatment of Niemann-Pick Disease

Feb 14, 2022: Azafaros presents positive clinical and preclinical data supporting  
development of lead compound AZ-3102 in lysosomal storage disorders at the 18th  
Annual WORLDSymposium conference

Feb 09, 2022: Cyclo therapeutics selected to present overview of pivotal phase 3 study  
for lead candidate, Trappsol Cyclo, at WORLDSymposium 2022

Feb 03, 2022: Cyclo Therapeutics announces formation of global steering committee

comprised of leading experts to Advise on the global phase 3 clinical development program for Trappsol Cyclo in Niemann-Pick Disease Type C

Oct 18, 2021: Cyclo Therapeutics announces abstract accepted for poster presentation at the 14th International Congress of Inborn Errors of Metabolism (ICIEM)

Oct 06, 2021: Cyclo Therapeutics to participate in the 2021 Virtual Cyclodextrin Conference Hosted by CycloLab

Jul 30, 2021: Cyclo Therapeutics announces new positive safety and efficacy data from ongoing phase 1 open-label extension study of Trappsol Cyclo for the treatment of Niemann-Pick disease type C1

Jul 27, 2021: Cyclo Therapeutics commences commercial-scale manufacturing for Trappsol Cyclo

Jun 21, 2021: Cyclo Therapeutics to present at the 2021 NPC patient and family conference hosted by the Australian NPC Disease Foundation

Jun 18, 2021: Cyclo Therapeutics starts enrolment in Phase III NPC1 treatment trial

Apr 27, 2021: Cyclo Therapeutics announces design of pivotal phase 3 study evaluating Trappsol Cyclo in Niemann-Pick Type C1

Mar 25, 2021: Cyclo Therapeutics meets primary efficacy endpoint in phase 1/2 trial from intravenous Trappsol Cyclo in rare disease Niemann-Pick Type C1 (NPC1)

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