

Niemann Pick C Disease - Pipeline Insight, 2021

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Abstracts

This report can be delivered to the clients within 48-72 Hours

DelveInsight's, "Niemann Pick C Disease – Pipeline Insight, 2021," report provides comprehensive insights about 10+ companies and 10+ pipeline drugs in the Niemann Pick C Disease pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Niemann Pick C Disease Understanding

Niemann Pick C Disease: Overview

Niemann-Pick disease type C (NPC) is a rare progressive genetic disorder characterized by an inability of the body to transport cholesterol and other fatty substances (lipids) inside of cells. NPC belongs to a larger group of more than 50 disorders known as lysosomal storage disorders. This leads to the abnormal accumulation of these substances within various tissues of the body, including brain tissue. Individuals with NPC can have onset of symptoms at different ages that have been grouped historically as: perinatal (shortly before and after birth), early infantile (3 months to

"Niemann Pick C Disease - Pipeline Insight, 2021" report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication.



A detailed picture of the Niemann Pick C Disease pipeline landscape is provided which includes the disease overview and Niemann Pick C Disease treatment guidelines. The assessment part of the report embraces, in depth Niemann Pick C Disease commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Niemann Pick C Disease collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Niemann Pick C Disease R&D. The therapies under development are focused on novel approaches to treat/improve Niemann Pick C Disease.

Niemann Pick C Disease Emerging Drugs Chapters

This segment of the Niemann Pick C Disease report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Niemann Pick C Disease Emerging Drugs

VTS-270(Adrabetadex): Mallinckrodt

VTS-270 (Mallinckrodt) also known as Kleptose; 2-Hydroxypropyl- beta-cyclodextrin; HP-beta- CD; HPBCD) is under development. The drug has received various designations from the United States Food Drug and Administration (FDA)-Rare Pediatric Disease Designation, Promising Innovative Medicine (PIM) Designation, Breakthrough Therapy Designation and Orphan Drug Designation for the treatment of NPC. VTS-270 is currently in phase III stage of development, an intrathecal preparation that can cross the Blood Brain Barrier (BBB) and as a result, it targets the neurological symptoms of NPC such as dementia and cataplexy.

IB1001: IntraBio



IB1001, N-acetyl-L-leucine, is an orally administered modified amino acid. IBI001 is in Phase 2 of clinical study and is being developed by IntraBio. IBI001 acts as calcium channel modulators. In addition to the Fast Track Designation, IB1001 has previously received Orphan Drug Designations in the US (FDA) and EU (European Commission), and granted a Rare Pediatric Disease Designation in the US (FDA) for the treatment of NPC.

Further product details are provided in the report......

Niemann Pick C Disease: Therapeutic Assessment

This segment of the report provides insights about the different Niemann Pick C Disease drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Niemann Pick C Disease

There are approx. 10+ key companies which are developing the therapies for Niemann Pick C Disease. The companies which have their Niemann Pick C Disease drug candidates in the most advanced stage, i.e. phase III include, Mallinckrodt.

Phases

DelveInsight's report covers around 10+ products under different phases of clinical development like

Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

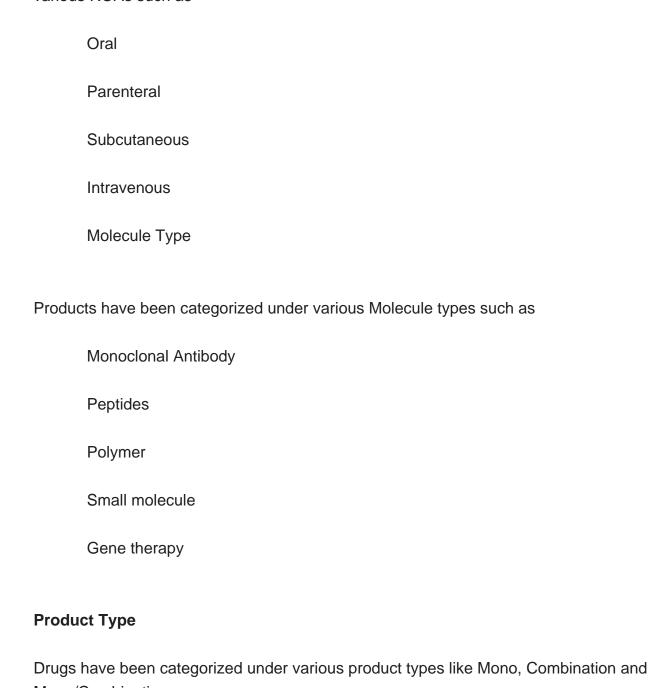
Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration



Niemann Pick C Disease pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as



Mono/Combination.

Niemann Pick C Disease: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Niemann Pick C Disease therapeutic drugs key players involved in developing key drugs.



Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Niemann Pick C Disease drugs.

Niemann Pick C Disease Report Insights

Niemann Pick C Disease Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Niemann Pick C Disease Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Niemann Pick C Disease drugs?

How many Niemann Pick C Disease drugs are developed by each company?



How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Niemann Pick C Disease?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Niemann Pick C Disease therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Niemann Pick C Disease and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

IntraBio

Mallinckrodt

Cyclo Therapeutics

SOM Biotech

Evox therapeutics

Orphazyme

Key Products

SOM0208

IB1001

Trappsol Cyclo



VTS-270

Arimoclomol

Hydroxypropyl-beta-cyclodextrin



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Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Mid Stage Products (Phase II)

Comparative Analysis

IB1001: IntraBio

Product Description

Research and Development

Product Development Activities



Drug profiles in the detailed report.....

Early Stage Products (Phase I/II)

Comparative Analysis

Hydroxypropyl Betadex: Cyclo Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Inactive Products

Comparative Analysis

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Niemann Pick C Disease Key Products

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