

Niemann-Pick Type C Disease - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Niemann-Pick Type C Disease – Pipeline Insight, 2021," report provides comprehensive insights about 13+ companies and 13+ pipeline drugs in Niemann-Pick Type 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 Type C Disease Understanding

Niemann-Pick Type 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 Type 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 Type C Disease pipeline landscape is provided which includes the disease overview and Niemann-Pick Type C Disease treatment guidelines. The assessment part of the report embraces, in depth Niemann-Pick Type 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 Type 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 Type C Disease R&D. The therapies under development are focused on novel approaches to treat/improve Niemann-Pick Type C Disease.

Niemann-Pick Type C Disease Emerging Drugs Chapters

This segment of the Niemann-Pick Type 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 Type C Disease Emerging Drugs

Arimoclomol: Orphazyme

Arimoclomol is an investigational drug candidate that amplifies the production of heat-shock proteins (HSPs). HSPs can rescue defective misfolded proteins, clear protein aggregates, and improve the function of lysosomes. Arimoclomol is in clinical development at Orphazyme for the treatment of NPC and Gaucher disease. Arimoclomol has received orphan drug designation for NPC in the US and EU, as well as fast-track designation from the US Food and Drug Administration (FDA) for NPC. In addition, arimoclomol has received breakthrough therapy designation and rare-pediatric disease designation from the FDA for NPC. Orphazyme has received a Complete

Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) following its review of the new drug application for arimoclomol, a heat shock protein amplifier intended for the treatment of Niemann-Pick disease type C (NPC). The FDA issued the CRL based on needing additional qualitative and quantitative evidence to further substantiate the validity and interpretation of the 5-domain NPC Clinical Severity Scale (NPCCSS) and, in particular, the swallow domain. Further, the FDA noted in the CRL that additional data are needed to bolster confirmatory evidence beyond the single phase II/III clinical trial to support the benefit-risk assessment of the NDA.

Adrabetadex: Mandos, LLC

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. In May 2021, Mandos, LLC announced they have entered into an agreement with Vtesse, LLC, a wholly owned subsidiary of Mallinckrodt Pharmaceuticals (Mallinckrodt), a global biopharmaceutical company, to acquire adrabetadex (also known as VTS-270), a drug in development for the treatment of Niemann-Pick Type C1 disease (NPC1).

Further product details are provided in the report.

Niemann-Pick Type C Disease: Therapeutic Assessment

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

Major Players in Niemann-Pick Type C Disease

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

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 Type 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

Oral

Parenteral

Intravenous

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Niemann-Pick Type 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 Type 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 Type C Disease drugs.

Niemann-Pick Type C Disease Report Insights

Niemann-Pick Type C Disease Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Niemann-Pick Type 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 Type C Disease drugs?

How many Niemann-Pick Type 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 Type C Disease?

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

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

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Assessment by Stage and Molecule Type

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Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.

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IB1001: IntraBio

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.

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Research programme: Niemann-Pick disease gene therapies - Sarepta

Therapeutics/StrideBio

Product Description

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