

Methylmalonic academia - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Methylmalonic academia - Pipeline Insight, 2021," report provides comprehensive insights about 6+ companies and 6+ pipeline drugs in Methylmalonic academia 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

Methylmalonic academia Understanding

Methylmalonic academia: Overview

Methylmalonic acidemia refers to a group of inherited conditions in which the body can't breakdown certain parts of proteins and fats. This leads to a build-up of toxic substances and bouts of serious illness called decompensation events or metabolic crises. Symptoms of a decompensation event include poor feeding, vomiting, trouble breathing, and lack of energy (lethargy). These can occur at different ages and can range from mild to severe. Methylmalonic acidemia is caused by changes in several different genes and is inherited in an autosomal recessive fashion. Treatment includes aggressive management of decompensation events, a low-protein diet, certain medications, antibiotics and, in some cases, liver and kidney transplantation. Some subtypes of methylmalonic acidemia respond to vitamin B12. Long-term complications

can include growth delay, intellectual disability, kidney disease, and pancreatitis. Methylmalonic acidemia can be isolated or may occur along with another condition called homocystinuria.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Methylmalonic acidemia R&D. The therapies under development are focused on novel approaches for Methylmalonic acidemia.

Methylmalonic acidemia Emerging Drugs Chapters

This segment of the Methylmalonic acidemia report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, 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.

Methylmalonic acidemia Emerging Drugs

HST5040: HemoShear Therapeutics

HST5040 is an investigational oral small molecule therapy developed by HemoShear to address metabolic abnormalities associated with MMA and PA. Because HST5040 is a small molecule, it has the ability to distribute to multiple affected tissues and thus has the potential to be active throughout the body, including the brain, heart, liver, kidneys, and muscles. HST5040 is designed for convenient daily administration at home as a liquid formulation taken either orally or through a gastric feeding tube.

In December, 2020, HemoShear Therapeutics announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designations to HemoShear's HST5040 oral small molecule for treatment of methylmalonic acidemia (MMA) and propionic acidemia (PA). HST5040 was granted Fast Track and Rare Pediatric Disease designations to treat MMA and PA earlier in 2020.

LB-001: LogicBio Therapeutics

LB-001 is being developed by LogicBio Therapeutics for Methylmalonic academia. It is currently in phase I/II stage of development. LB-001 acts as Gene transference; Methylmalonyl-CoA mutase replacements.

In November, 2020, LogicBio Therapeutics announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to its clinical candidate, LB-001 for the treatment methylmalonic acidemia (MMA). According to the FDA, the purpose of Fast Track designation is to get important new drugs to patients earlier by facilitating the development, and expediting the review, of drugs to treat serious conditions and fill an unmet medical need.

Further product details are provided in the report

Methylmalonic academia: Therapeutic Assessment

This segment of the report provides insights about the different Methylmalonic academia drugs segregated based on following parameters that define the scope of the report, such as:

Major Players working on Methylmalonic academia

There are approx. 6+ key companies which are developing the Methylmalonic acidemia. The companies which have their Methylmalonic acidemia drug candidates in the most advanced stage, i.e. Phase II include, HemoShear Therapeutics.

Phases

DelveInsight's report covers around 6+ products under different phases of clinical development like

Late-stage products (Phase III and

Mid-stage products (Phase II and

Early-stage products (Phase I/II and Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Methylmalonic academia pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Infusion

Intradermal

Intramuscular

Intranasal

Intravaginal

Oral

Parenteral

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Vaccines

Monoclonal Antibody

Peptides

Polymer

Small molecule

Product Type

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

Methylmalonic academia: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Methylmalonic academia 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 Methylmalonic academia drugs.

Methylmalonic academia Report Insights

Methylmalonic academia Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Methylmalonic academia Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Scenario and Emerging Therapies:

How many companies are developing Methylmalonic academia drugs?

How many Methylmalonic academia drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for Methylmalonic academia?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Methylmalonic academia 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 Methylmalonic academia and their status?

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

Key Players

HemoShear Therapeutics

LogicBio Therapeutics

Selecta Biosciences

Asklepios BioPharmaceutical

Moderna Therapeutics

Poseida Therapeutics

Key Products

HST5040

LB-001

AAV8

MMA-101

mRNA-3705

P-MMUT-101

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Research and Development

Product Development Activities

Drug profiles in the detailed report

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Comparative Analysis

HST5040: HemoShear Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

Early Stage Products (Phase I/II)

Comparative Analysis

LB-001: LogicBio Therapeutics

Product Description

Research and Development

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Pre-clinical and Discovery Stage Products

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mRNA-3705: Moderna Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

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Comparative Analysis

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