

Urea cycle disorders - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Urea cycle disorders - Pipeline Insight, 2021," report provides comprehensive insights about 8+ companies and 8+ pipeline drugs in Urea cycle disorders 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

Urea cycle disorders Understanding

Urea cycle disorders: Overview

A urea cycle disorder (UCD) is an inherited disease. When we eat protein our bodies break it down into amino acids which we need to grow and stay healthy. Extra amino acids that our body doesn't need are turned into a waste product called ammonia. Enzymes in the liver turn the ammonia into urea so the body can get rid of it through the urine. In someone with a UCD, the enzyme is missing or is not working correctly. Because of this, ammonia builds up in the blood and it can be harmful. It can travel to the brain and cause memory loss, behavior changes, coma, seizures, or brain damage. UCDs affect about 1 in 35,000 births in the United States. Just because it's rare doesn't mean there isn't information to help you or someone you love who is living with a UCD.

'Urea cycle disorders - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Urea cycle disorders pipeline landscape is provided which includes the disease overview and Urea cycle disorders treatment guidelines. The assessment part of the report embraces, in depth Urea cycle disorders 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, Urea cycle disorders 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 Urea cycle disorders R&D. The therapies under development are focused on novel approaches to treat/improve Urea cycle disorders.

Urea cycle disorders Emerging Drugs Chapters

This segment of the Urea cycle disorders 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.

Urea cycle disorders Emerging Drugs

ACER-001: Acer Therapeutics

ACER-001 is a powder formulation of sodium phenylbutyrate (NaPB). The proprietary formulation is designed to be both taste-masked and immediate release. ACER-001 is being developed using a microencapsulation process for the treatment of various inborn errors of metabolism, including UCDs and MSUD. ACER-001 microparticles consist of a core center, a layer of active drug, and a taste-masking coating that quickly dissolves in the stomach, allowing taste to be neutralized while still allowing for rapid systemic release. Currently, it is in phase 3 of development stage for the treatment of inborn urea

cycle disorders.

Avalotcagene ontaparvovec: Dimension Therapeutics

Avalotcagene ontaparvovec is a Ornithine carbamoyltransferase replacement therapy being developed by Dimension Therapeutics for the treatment of Inborn urea cycle disorders. Currently, the therapy is in phase 2 stage of development.

Further product details are provided in the report..

Urea cycle disorders: Therapeutic Assessment

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

Major Players in Urea cycle disorders

There are approx. 8+ key companies which are developing the therapies for Urea cycle disorders. The companies which have their Urea cycle disorders drug candidates in the most advanced stage, i.e. phase III include, Acer Therapeutics.

Phases

DelveInsight's report covers around 8+ 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

Urea cycle disorders 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

intravitreal

Subretinal

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.

Urea cycle disorders: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Urea cycle disorders 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 Urea cycle disorders drugs.

Urea cycle disorders Report Insights

- Urea cycle disorders Pipeline Analysis

- Therapeutic Assessment

- Unmet Needs

- Impact of Drugs

Urea cycle disorders 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 Urea cycle disorders drugs?

How many Urea cycle disorders drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Urea cycle disorders?

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

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

Key Players

Acer Therapeutics

Dimension Therapeutics

Callitas Therapeutics

Poseida Therapeutics

Promethera Biosciences

Arcturus Therapeutics

Kaleido Biosciences

Akaza Biopharma

Evox Therapeutics

Dipharma SA

Sana Biotechnology

Aeglea BioTherapeutics

ERYTECH

Key Products

ACER-001

Avalotcagene ontaparvovec

MACS

P OTC 101

Heparesc

ARCT 810

VS 01

KB195

HepaStem

Resatorvid

EVX-103

SG 328

Pegzilarginase

ERY-ADI

Contents

Introduction

Executive Summary

Urea cycle disorders: Overview

Causes

Mechanism of Action

Signs and Symptoms

Diagnosis

Disease Management

Pipeline Therapeutics

Comparative Analysis

Therapeutic Assessment

Assessment by Product Type

Assessment by Stage and Product Type

Assessment by Route of Administration

Assessment by Stage and Route of Administration

Assessment by Molecule Type

Assessment by Stage and Molecule Type

Urea cycle disorders – DelveInsight's Analytical Perspective

In-depth Commercial Assessment

Urea cycle disorders companies' collaborations, Licensing, Acquisition -Deal Value

Trends

Urea cycle disorders Collaboration Deals

Company-Company Collaborations (Licensing / Partnering) Analysis

Company-University Collaborations (Licensing / Partnering) Analysis

Late Stage Products (Phase III)

Comparative Analysis

ACER-001: Acer Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report..

Mid Stage Products (Phase II)

Comparative Analysis

Avalotcagene ontaparvovec: Dimension Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report..

Inactive Products

Comparative Analysis

Urea cycle disorders Key Companies

Urea cycle disorders Key Products

Urea cycle disorders- Unmet Needs

Urea cycle disorders- Market Drivers and Barriers

Urea cycle disorders- Future Perspectives and Conclusion

Urea cycle disorders Analyst Views

Urea cycle disorders Key Companies

Appendix

List Of Tables

LIST OF TABLES

Table 1 Total Products for Urea cycle disorders

Table 2 Late Stage Products

Table 3 Mid Stage Products

Table 4 Early Stage Products

Table 5 Pre-clinical & Discovery Stage Products

Table 6 Assessment by Product Type

Table 7 Assessment by Stage and Product Type

Table 8 Assessment by Route of Administration

Table 9 Assessment by Stage and Route of Administration

Table 10 Assessment by Molecule Type

Table 11 Assessment by Stage and Molecule Type

Table 12 Inactive Products

List Of Figures

LIST OF FIGURES

- Figure 1 Total Products for Urea cycle disorders
- Figure 2 Late Stage Products
- Figure 3 Mid Stage Products
- Figure 4 Early Stage Products
- Figure 5 Preclinical and Discovery Stage Products
- Figure 6 Assessment by Product Type
- Figure 7 Assessment by Stage and Product Type
- Figure 8 Assessment by Route of Administration
- Figure 9 Assessment by Stage and Route of Administration
- Figure 10 Assessment by Molecule Type
- Figure 11 Assessment by Stage and Molecule Type
- Figure 12 Inactive Products

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