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Argininosuccinic aciduria - Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 24 - 48 Hours

DelveInsight's, "Argininosuccinic aciduria (ASA) – Pipeline Insight, 2020," report provides comprehensive insights about 5+ companies and 5+ pipeline drugs in Argininosuccinic aciduria 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

Argininosuccinic aciduria Understanding

Argininosuccinic aciduria (ASA): Overview

Argininosuccinic aciduria is an inherited disorder that causes ammonia to accumulate in the blood. Ammonia, which is formed when proteins are broken down in the body, is toxic if the levels become too high. The nervous system is especially sensitive to the effects of excess ammonia. Argininosuccinic aciduria usually becomes evident in the first few days of life. An infant with argininosuccinic aciduria may be lacking in energy (lethargic) or unwilling to eat, and have a poorly controlled breathing rate or body temperature. Some babies with this disorder experience seizures or unusual body movements, or go into a coma. Complications from argininosuccinic aciduria may include developmental delay and intellectual disability. Progressive liver damage, high blood pressure (hypertension), skin lesions, and brittle hair may also be seen.

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Symptoms

The severity and specific symptoms of argininosuccinic aciduria varies from one person to another. A severe form of the disorder, which is characterized by a complete or near complete lack of the ASL enzyme, occurs shortly after birth (neonatal period). A milder form of the disorder, which is characterized by partial lack of the ASL enzyme, affects some individuals later during infancy or childhood or even adulthood (late-onset form). Affected infants may also experience seizures, breathing (respiratory) abnormalities, the accumulation of fluid in the brain (cerebral edema), and an abnormally large liver (hepatomegaly). Less commonly, some individuals develop progressive liver disease and dysfunction such as the buildup of scar tissue (fibrosis) and cirrhosis. In rare instances, chronic kidney (renal) disease has been reported.

Diagnosis

A diagnosis of a urea cycle disorder, such as argininosuccinic aciduria, should be considered in any newborn that has an undiagnosed illness characterized by vomiting, progressive lethargy, and irritability. A diagnosis of argininosuccinic aciduria can be made through a detailed patient/family history, identification of characteristic findings, and a variety of specialized tests. Blood tests may reveal excessive amounts of ammonia in the blood, which is the main criterion for a diagnosis of urea cycles disorders including argininosuccinic aciduria.

Treatment

The treatment of argininosuccinic aciduria is aimed at preventing excessive ammonia from being formed or from removing excessive ammonia during a hyperammonemic episode. Long-term therapy combines dietary restrictions and the stimulation of alternative methods of converting and excreting nitrogen from the body (alternative pathways therapy). Dietary restrictions in individuals with argininosuccinic aciduria are aimed at limiting the amount of protein intake to avoid the development of excess ammonia.

> In 2013, the U.S. Food and Drug Administration (FDA) approved Ravicti (glycerol phenylbutyrate) for the chronic management of urea cycle disorders including argininosuccinic aciduria in affected individuals age 2 years and older.

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Argininosuccinic aciduria Emerging Drugs Chapters

This segment of the Argininosuccinic aciduria 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.

Argininosuccinic aciduria Emerging Drugs

Evox’s exosome-based therapy: Evox Therapeutics Ltd.

Evox is engineering exosomes, the body’s natural vesicular delivery system, to enable a wide variety of drugs to reach previously inaccessible tissues and compartments, such as crossing the blood-brain barrier to deliver drugs to the central nervous system, enabling intracellular delivery of biologics, and allowing for extra-hepatic delivery of RNA therapeutics. UCL has world-leading expertise in urea cycle disorders and will perform testing of Evox’s exosome-based therapy in an in vivo ASL deficiency model.

Further product details are provided in the report.

Argininosuccinic aciduria: Therapeutic Assessment

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

Major Players in Argininosuccinic aciduria

There are approx. 5+ key companies which are developing the therapies for Argininosuccinic aciduria. The companies which have their Argininosuccinic aciduria drug candidates in early stage, i.e. pre-clinical and Discovery include, Evox Therapeutics, ACER Therapeutics etc.

Phases

DelveInsight’s report covers around 5+ products under different phases of clinical

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development like

Mid-stage products (Phase II and Phase I/II)

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

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

Subcutaneous

Intravenous

Oral

Molecule Type

Products have been categorized under various Molecule types such as

Small molecules

Exosomes

Product Type

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

Argininosuccinic aciduria: Pipeline Development Activities

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

Report Highlights

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

March 2019: Evox Therapeutics has secured ?1.5 million in funding from Innovate UK, the UK’s Innovation Agency, through the Biomedical Catalyst Early Stage Award. The funds will be used to support the Company's pre-clinical development of an exosome-based therapy to treat argininosuccinic aciduria (ASA), a rare life-threatening metabolic disease, in collaboration with University College London (UCL).

Argininosuccinic aciduria Report Insights

Argininosuccinic aciduria Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Argininosuccinic aciduria Report Assessment

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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 Argininosuccinic aciduria drugs?

How many Argininosuccinic aciduria drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Argininosuccinic aciduria?

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

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

Key Players

Evox Therapeutics

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Acer Therapeutics

Key Products

ACER-001

Evox’s tissue-targeted exosomes

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