

Leigh disease - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Leigh disease - Pipeline Insight, 2021," report provides comprehensive insights about 3+ companies and 3+ pipeline drugs in Leigh 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

Leigh disease Understanding

Leigh disease: Overview

Leigh syndrome is a rare genetic neurometabolic disorder. It is characterized by the degeneration of the central nervous system (i.e., brain, spinal cord, and optic nerve). The symptoms of Leigh syndrome usually begin between the ages of three months and two years, but some patients do not exhibit signs and symptoms until several years later. Symptoms are associated with progressive neurological deterioration and may include loss of previously acquired motor skills, loss of appetite, vomiting, irritability, and/or seizure activity. As Leigh syndrome progresses, symptoms may also include generalized weakness, lack of muscle tone (hypotonia), and episodes of lactic acidosis, which may lead to impairment of respiratory and kidney function.

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

Leigh disease Emerging Drugs Chapters

This segment of the Leigh 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.

Leigh disease Emerging Drugs

Vatiquinone (EPI-743): PTC Therapeutics

EPI-743, an orally bioavailable small molecule being developed by the company for inherited mitochondrial diseases. EPI-743 is a member of the para-benzoquinone class of drugs. The mechanism of action of EPI-743 involves augmenting the synthesis of glutathione, optimizing metabolic control, enhancing the expression of genetic elements critical for cellular management of oxidative stress, and acting at the mitochondria to regulate electron transport. The drug is in phase 3 of clinical trials for the treatment of patients with Leigh disease.

ABI-009: Khondrion

ABI-009 is an MTOR protein inhibitor being developed by Aadi, LLC for the treatment of various indications. The drug is currently in phase 2 of clinical trials for the treatment of patients with Leigh disease.

Further product details are provided in the report

Leigh disease: Therapeutic Assessment

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

Major Players in Leigh disease

There are approx. 3+ key companies which are developing the therapies for Leigh disease. The companies which have their Leigh disease drug candidates in the most advanced stage, i.e. phase III include, PTC Therapeutics.

Phases

DelveInsight's report covers around 3+ 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

Leigh 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

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.

Leigh disease: Pipeline Development Activities

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

Leigh disease Report Insights

Leigh disease Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Leigh 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 Leigh disease drugs?

How many Leigh disease drugs are developed by each company?

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

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

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

Key Players

PTC Therapeutics

Aadi, LLC

Khondrion

Mission therapeutics

Taysha Gene Therapies

Key Products

Vatiquinone (EPI-743)

ABI-009

Sonlicromanol

USP30 MITOCHONDRIAL DISEASE PROGRAM

TSHA-104

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