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Leber congenital amaurosis - Pipeline Insight, 2021

https://marketpublishers.com/r/L81B0128C0EEEN.html
Date: September 2021
Pages: 60
Price: US$ 1,500.00 (Single User License)
ID: L81B0128C0EEEN

Abstracts

This report can be delivered to the clients within 48 hours

DelveInsight's, "Leber congenital amaurosis - Pipeline Insight, 2021," report provides comprehensive insights about 10+ companies and 10+ pipeline drugs in Leber congenital amaurosis 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

Leber congenital amaurosis Understanding

Leber congenital amaurosis: Overview

Leber congenital amaurosis (LCA) is a rare genetic eye disorder. Affected infants are often blind at birth. Other symptoms may include crossed eyes (strabismus); rapid, involuntary eye movements (nystagmus); unusual sensitivity to light (photophobia); clouding of the lenses of the eyes (cataracts); and/or a cone shape to the front of the eye (keratoconus). LCA is usually inherited as an autosomal recessive genetic condition. Children born with LCA have light-gathering cells (rods and cones) of the retina that do not function properly. Absence or reduction of the electrical activity of the retina is always observed and is necessary for the diagnosis of LCA. A decrease in visual responsiveness at birth is the first sign of the disease. Often the child will poke, press and rub the eyes to stimulate the retina to produce light (Franceschetti's oculo-

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digital sign).This activity may cause the eyes to become sunken or deep set (enophthalmos). LCA is a monogenic disease and at least 27 genes are implicated. Changes (mutations) in these genes can account for about 80-90% of diagnosed cases of LCA. The genes responsible for the remaining 10-20% of diagnoses are not known. LCA is usually inherited as an autosomal recessive genetic condition.

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

Leber congenital amaurosis Emerging Drugs Chapters

This segment of the Leber congenital amaurosis 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.

Leber congenital amaurosis Emerging Drugs

> Sepofarsen (QR-110): ProQR Therapeutics

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Sepofarsen (QR-110) is an RNA therapy that aims to restore vision in Leber congenital amaurosis 10 (LCA10) due to the most common p.Cys998X mutation in the CEP290 gene. Sepofarsen aims to restore vision loss in people with Leber congenital amaurosis due to a specific mutation in the CEP290 gene. This p.Cys998X mutation, also known as c.2991+1655A>G, is the most common mutation causing LCA10. The p.Cys998X mutation causes a mistake in the CEP290 RNA and therefore a process called splicing is not done properly. The cell can therefore not use the RNA to produce a working CEP290 protein that is essential for vision. Currently, it is in Phase II/III stage of development to treat Leber congenital amaurosis.

EDIT-101: Editas Medicine, Inc.

The company's approach to Leber Congenital Amaurosis 10 (LCA10) is to target a disease-causing mutation in the CEP290 gene that causes degeneration in ocular photoreceptor cells—cells critical for experiencing normal vision. They have developed an experimental CRISPR medicine called EDIT-101 designed to remove the CEP290 mutation. This approach may restore normal protein expression, photoreceptor function, and ultimately, vision.

Further product details are provided in the report.

Leber congenital amaurosis: Therapeutic Assessment

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

Major Players in Leber congenital amaurosis

There are approx. 10+ key companies which are developing the therapies for Leber congenital amaurosis. The companies which have their Leber congenital amaurosis drug candidates in the most advanced stage, i.e. phase II/III include, ProQR Therapeutics.

Phases

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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

Leber congenital amaurosis 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

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- Polymer
- Small molecule
- Gene therapy
- Product Type

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

Leber congenital amaurosis: Pipeline Development Activities

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

Leber congenital amaurosis Report Insights

- Leber congenital amaurosis Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

Leber congenital amaurosis Report Assessment

- Pipeline Product Profiles

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- Therapeutic Assessment
- Pipeline Assessment
- Inactive drugs assessment
- Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

- How many companies are developing Leber congenital amaurosis drugs?
- How many Leber congenital amaurosis drugs are developed by each company?
- How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Leber congenital amaurosis?
- What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Leber congenital amaurosis 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 Leber congenital amaurosis and their status?
- What are the key designations that have been granted to the emerging drugs?

Key Players

- ProQR Therapeutics
- Editas Medicine

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Coave Therapeutics

MeiraGTx

Novelion Therapeutics

Applied Genetic Technologies Corporation

Oxford BioMedica

IVERIC bio

Key Products

Sepofarsen (QR-110)

EDIT 101

HORA RPE65

SAR 439483

Cevaretigene ritoparvovec

QLT091001

rAAV2-CB-hRPE65

AAV AIPL1

OXB 204

Research programme: eye disorder gene therapies

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