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Leber hereditary optic neuropathy - Pipeline Insight, 2021

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Abstracts

This report can be delivered to the clients within 48 hours

DelveInsight's, "Leber hereditary optic neuropathy - Pipeline Insight, 2021," report provides comprehensive insights about 4+ companies and 4+ pipeline drugs in Leber hereditary optic neuropathy 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 hereditary optic neuropathy Understanding

Leber hereditary optic neuropathy: Overview

Leber hereditary optic neuropathy (LHON) is often characterized by bilateral, painless subacute loss of central vision during young adult life. In most cases, symptoms begin with one eye first, followed a few weeks later by visual failure in the other eye. Extremely rarely there may be neurologic abnormalities, such as peripheral neuropathy, postural tremor, nonspecific myopathy, and movement disorders. LHON is caused by mutations in mitochondrial DNA and it is strictly transmitted by maternal inheritance. The prevalence of visual loss from LHON is approximately 1:50,000 people. Many carriers never suffer significant visual loss; males are about four to five times more likely

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than females to lose vision and be affected. Individuals with LHON typically display symptoms in their young adult years. If vision is lost, then it usually occurs before 40 years of age. The acute phase of LHON is characterized by a loss of central vision, including blurring and reduced perception of color. Individuals usually lose vision in one eye first and then lose vision in the other eye after two to three months. The atrophic phase is characterized by bilateral optic atrophy, resulting in lifelong blindness.

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

Leber hereditary optic neuropathy Emerging Drugs Chapters

This segment of the Leber hereditary optic neuropathy 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 hereditary optic neuropathy Emerging Drugs

> GS010: GenSight Biologics

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GS010 is an AAV2 gene therapy vector that encodes the human wild-type ND4 protein, which we are developing as a treatment of LHON caused by mutation of the ND4 gene. The ND4 gene is normally located in the mitochondria where ND4 proteins are synthesized. GS010 allows efficient allotopic expression of the mitochondrial gene ND4 in the nucleus thanks to a proprietary Mitochondrial Targeting Sequence that shuttles the messenger RNA from the nucleus directly to the outer membrane of the mitochondria. There, the ND4 proteins are synthesized and incorporated into the mitochondria. Wild-type ND4 proteins then integrate into Complex I of the respiratory chain and rescue the deficiency.

Elamipretide: Stealth BioTherapeutics Inc.

Elamipretide, is a peptide compound that readily penetrates cell membranes, and targets the inner mitochondrial membrane where it binds reversibly to cardiolipin. In preclinical or clinical studies, the company have observed that elamipretide increases mitochondrial respiration, improves electron transport chain function and ATP production and reduces formation of pathogenic ROS levels. This elamipretide-cardiolipin association has been shown to normalize the structure of the inner mitochondrial membrane, thereby improve ng mitochondrial function. Functional benefit is achieved through improvement of ATP production and interruption and potential reversal of damaging oxidative stress. The company is investigating elamipretide in late stage clinical studies in three primary mitochondrial diseases — rare diseases with cardiomyopathy, Barth syndrome and Leber’s hereditary optic neuropathy – as well as a clinical study in dry age-related macular degeneration.

Further product details are provided in the report.

Leber hereditary optic neuropathy: Therapeutic Assessment

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

Major Players in Leber hereditary optic neuropathy

There are approx. 4+ key companies which are developing the therapies for Leber hereditary optic neuropathy. The companies which have their Leber hereditary optic

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neuropathy drug candidates in the most advanced stage, i.e. preregistration include, GenSight Biologics.

Phases

DelveInsight's report covers around 4+ 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 hereditary optic neuropathy 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

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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.

Leber hereditary optic neuropathy: Pipeline Development Activities

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

Leber hereditary optic neuropathy Report Insights

- Leber hereditary optic neuropathy Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

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

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Key Players

GenSight Biologics

Stealth BioTherapeutics

Neurophth Therapeutics

Fortify Therapeutics

Neuroptika

Mitotech

Key Products

Lenadogene nolparvovec

Elamipretide

NFS 01

Research programme: Leber's hereditary optic neuropathy therapeutics

NRO 1

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