

# Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline Review, H1 2018

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## Abstracts

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline Review, H1 2018

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline Review, H1 2018, provides an overview of the Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) pipeline landscape.

Leber's hereditary optic neuropathy (LHON) is an inherited form of vision loss. This condition usually begins in a person's teens or twenties, rare cases may appear in early childhood or later in adulthood. Males are affected much more often than females. Symptoms include eye pain or discomfort, numbness, tingling and clouding of vision. Predisposing factors include age, tobacco smoke, alcohol, cancer treating drugs, stress related chemicals, some antibiotics and cyanogens.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline Review, H1 2018, provides comprehensive information on the therapeutics under development for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development

history and latest news and press releases.

The Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) pipeline guide also reviews of key players involved in therapeutic development for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase III, Phase II, Phase I, Preclinical, Discovery and Unknown stages are 1, 3, 2, 4, 1 and 1 respectively. Similarly, the Universities portfolio in Phase I stages comprises 1 molecules, respectively.

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology).

The pipeline guide reviews pipeline therapeutics for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products

which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology)

## **REASONS TO BUY**

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) (Ophthalmology) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and @Scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

## Contents

- Introduction
- Global Markets Direct Report Coverage
- Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Overview
- Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Therapeutics Development
- Pipeline Overview
- Pipeline by Companies
- Pipeline by Universities/Institutes
- Products under Development by Companies
- Products under Development by Universities/Institutes
- Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Therapeutics Assessment
- Assessment by Target
- Assessment by Mechanism of Action
- Assessment by Route of Administration
- Assessment by Molecule Type
- Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Companies Involved in Therapeutics Development
- Alkeus Pharmaceuticals Inc
- Biovista Inc
- GenSight Biologics SA
- Ixchel Pharma LLC
- Khondrion BV
- Mitobridge Inc
- Mitotech SA
- Spark Therapeutics Inc
- Stealth BioTherapeutics Inc
- Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Drug Profiles
- BVA-202 - Drug Profile
- Product Description
- Mechanism Of Action
- R&D Progress
- BVA-203 - Drug Profile
- Product Description
- Mechanism Of Action
- R&D Progress
- Drug for Lebers Hereditary Optic Neuropathy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

elamipretide - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate ND4 for Leber's Hereditary Optic Neuropathy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

GS-010 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

GS-011 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

IXC-201 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

IXC-203 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

KH-176 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

MA-0211 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

plastoquinone decyl triphenylphosphonium bromide - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

## SPK-LHON - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Dormant Projects

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Product

Development Milestones

Featured News & Press Releases

Feb 20, 2018: GenSight Biologics Announces Publication of Positive Data from Phase I/II Trial and Long-Term Follow-up of GS010 in Ophthalmology, the Journal of the American Academy of Ophthalmology

Feb 02, 2018: Bascom Palmer Study Shows Gene Therapy Safe for Rare Eye Disease

Jan 25, 2018: A potential drug for Leber's hereditary optic neuropathy and Barth syndrome discovered at the IRCM

Dec 18, 2017: Stealth BioTherapeutics Granted Fast Track Designation for Elamipretide for the Treatment of Leber's Hereditary Optic Neuropathy

Dec 05, 2017: GenSight Biologics reports positive long-term visual acuity gains and safety results from Phase I/II Study of GS010 for the treatment of Leber Hereditary Optic Neuropathy (LHON)

Oct 16, 2017: GenSight Biologics to Present Data on GS010 at 2017 Congress of the European Society of Gene & Cell Therapy

Aug 01, 2017: GenSight Biologics Completes Enrollment of RESCUE Phase III Study of GS010 in the Treatment of Leber's Hereditary Optic Neuropathy

Jun 14, 2017: GenSight Biologics Reports Long-Term Positive Safety and Visual Acuity Results at Week 96 in Phase I/II Study of GS010 for the Treatment of Leber's Hereditary Optic Neuropathy

May 08, 2017: GenSight Biologics to Present Data on GS010 at the Annual Meeting of ARVO

Feb 21, 2017: GenSight Biologics Completes Enrollment of REVERSE Phase III Study of GS010 in the treatment of Leber's Hereditary Optic Neuropathy

Dec 20, 2016: GenSight Biologics Reports Sustained Visual Acuity Gain at 78 Weeks in Its Phase I/II Study with GS010 for the Treatment of Leber's Hereditary Optic Neuropathy (LHON)

Sep 08, 2016: GenSight Biologics Receives Approval to Include Teenage Patients in RESCUE and REVERSE Phase III Trials with GS010 in Leber's Hereditary Optic Neuropathy

Jun 08, 2016: GenSight Biologics Announces Additional Promising Results of Its Phase I/II Study with GS010 for the Treatment of Leber's Hereditary Optic Neuropathy (LHON)

Jun 01, 2016: Stealth BioTherapeutics Initiates Phase 2 Study of Elamipretide in

Leber's Hereditary Optic Neuropathy

Feb 29, 2016: GenSight Biologics Enrolls First Patient in Pivotal Phase III Program of GS010 in Leber's Hereditary Optic Neuropathy

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer



## List Of Tables

### LIST OF TABLES

Number of Products under Development for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy), H1 2018

Number of Products under Development by Companies, H1 2018

Number of Products under Development by Universities/Institutes, H1 2018

Products under Development by Companies, H1 2018

Products under Development by Universities/Institutes, H1 2018

Number of Products by Stage and Target, H1 2018

Number of Products by Stage and Mechanism of Action, H1 2018

Number of Products by Stage and Route of Administration, H1 2018

Number of Products by Stage and Molecule Type, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Alkeus Pharmaceuticals Inc, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Biovista Inc, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by GenSight Biologics SA, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Ixchel Pharma LLC, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Khondrion BV, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Mitobridge Inc, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Mitotech SA, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Spark Therapeutics Inc, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Pipeline by Stealth BioTherapeutics Inc, H1 2018

Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy) - Dormant Projects, H1 2018

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Leber's Hereditary Optic Neuropathy (LHON) (Leber optic atrophy), H1 2018

Number of Products under Development by Companies, H1 2018

Number of Products by Targets, H1 2018

Number of Products by Stage and Targets, H1 2018

Number of Products by Mechanism of Actions, H1 2018

Number of Products by Stage and Mechanism of Actions, H1 2018

Number of Products by Routes of Administration, H1 2018

Number of Products by Stage and Routes of Administration, H1 2018

Number of Products by Molecule Types, H1 2018

Number of Products by Stage and Molecule Types, H1 2018

### COMPANIES MENTIONED

Alkeus Pharmaceuticals Inc

Biovista Inc

GenSight Biologics SA

Ixchel Pharma LLC

Khondrion BV

Mitobridge Inc

Mitotech SA

Spark Therapeutics Inc

Stealth BioTherapeutics Inc

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