

# Leber Congenital Amaurosis (LCA) - Pipeline Review, H1 2020

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## Abstracts

Leber Congenital Amaurosis (LCA) - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Leber Congenital Amaurosis (LCA) - Pipeline Review, H1 2020, provides an overview of the Leber Congenital Amaurosis (LCA) (Genetic Disorders) pipeline landscape.

Leber congenital amaurosis is an eye disorder that primarily affects the retina, which is the specialized tissue at the back of the eye that detects light and color. This condition is the most common cause of inherited blindness in childhood. Symptoms include vision loss, roving eye movements, deep-set eyes, developmental delay, epilepsy, and motor skill impairment.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Leber Congenital Amaurosis (LCA) - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Leber Congenital Amaurosis (LCA) (Genetic Disorders), 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 Congenital Amaurosis (LCA) (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Leber Congenital Amaurosis

(LCA) 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, Preclinical and Discovery stages are 2, 3, 6 and 1 respectively. Similarly, the Universities portfolio in Phase I and Preclinical stages comprises 1 and 3 molecules, respectively.

Leber Congenital Amaurosis (LCA) (Genetic Disorders) 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 Congenital Amaurosis (LCA) (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Leber Congenital Amaurosis (LCA) (Genetic Disorders) 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 Congenital Amaurosis (LCA) (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Leber Congenital Amaurosis (LCA) (Genetic Disorders) 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 Congenital Amaurosis (LCA) (Genetic Disorders)

## **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 Congenital Amaurosis (LCA) (Genetic Disorders).

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 Congenital Amaurosis (LCA) (Genetic Disorders) 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.

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Generation Bio Corp

IVERIC bio Inc

MeiraGTx Holdings Plc

Ocugen Inc

Odylia Therapeutics Inc

Oxford BioMedica Plc

ProQR Therapeutics NV

Retinagenix LLC

Sanofi

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## R&D Progress

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Featured News & Press Releases

Mar 04, 2020: Allergan and Editas Medicine announce dosing of first patient in landmark phase 1/2 clinical trial of CRISPR medicine AGN-151587 (EDIT-101) for the treatment of LCA10

Jan 13, 2020: Editas Medicine reports presentation on EDIT-101 at J.P. Morgan Healthcare Conference

Sep 18, 2019: Ocugen granted FDA orphan drug designation for OCU400 (NR2E3) gene therapy for the treatment of CEP290 mutation associated retinal disease

Jul 26, 2019: Allergan and Editas to assess in-vivo CRISPR editing to treat LCA10

Apr 29, 2019: Allergan and Editas Medicine announce initial data from natural history study to evaluate patients with leber congenital amaurosis 10 (LCA10)

Jan 21, 2019: Editas medicine announces publication in nature medicine of data supporting the development of EDIT-101 to treat Leber Congenital Amaurosis 10 (LCA10)

Jan 07, 2019: Editas Medicine reports on recent progress at J.P. Morgan Healthcare Conference

Nov 30, 2018: Editas Medicine Announces FDA Acceptance of IND Application for EDIT-101

Aug 27, 2018: Editas Medicine announces transition of chief medical officer

Aug 16, 2018: Editas Medicine Announces the Completion of the Recombinant DNA

Advisory Committee (RAC) Registration Process

May 18, 2018: Editas Medicine Reports Data Demonstrating Subretinal Injection of EDIT-101 Well-tolerated in Non-human Primates

Oct 19, 2017: Editas Medicine Demonstrates Dose-Dependent, In Vivo Editing with EDIT-101 in CEP290 Transgenic Mice

Sep 26, 2017: Editas Medicine Receives EMA's Orphan Medicinal Product Designation for EDIT-101 for the Treatment of LCA10

Sep 12, 2017: Editas Medicine Initiates Clinical Natural History Study to Evaluate Patients with Leber Congenital Amaurosis Type 10

Jan 09, 2017: Editas Medicine Reports on Recent Progress and 2017 Goals at J.P. Morgan Healthcare Conference

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Generation Bio Corp

IVERIC bio Inc

MeiraGTx Holdings Plc

Ocugen Inc

Odylia Therapeutics Inc

Oxford BioMedica Plc

ProQR Therapeutics NV

Retinagenix LLC

Sanofi

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