

Leber Congenital Amaurosis - Pipeline Review, H1 2017

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Abstracts

Leber Congenital Amaurosis - Pipeline Review, H1 2017

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Leber Congenital Amaurosis - Pipeline Review, H1 2017, provides an overview of the Leber Congenital Amaurosis (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 - Pipeline Review, H1 2017, provides comprehensive information on the therapeutics under development for Leber Congenital Amaurosis (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 (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 Pre-Registration, Phase III, Phase II and Preclinical stages are 1, 1, 2 and 2 respectively. Similarly, the Universities portfolio in Phase I and Preclinical stages comprises 1 and 1 molecules, respectively.

Leber Congenital Amaurosis (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 (Genetic Disorders).

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

The pipeline guide evaluates Leber Congenital Amaurosis (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 (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 (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 (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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Editas Medicine Inc

Novelion Therapeutics Inc

ProQR Therapeutics NV

Spark Therapeutics Inc

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Leber Congenital Amaurosis (LCA) - Product Development Milestones

Featured News & Press Releases

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

Jun 01, 2016: ProQR's Drug Candidate QR-110 for Leber's Congenital Amaurosis Type 10 Receives EMA and FDA Orphan Drug Designation

Apr 25, 2016: ProQR Announces an Investor and Analyst Event and Pre-Clinical Data Presentations for QR-110 for LCA10 at the 2016 ARVO Annual Meeting

May 04, 2015: Penn Study Indicates that Gene Therapy Efficacy for LCA is Dynamic: Improvement is Followed by Decline in Vision

May 01, 2015: Spark Therapeutics to Have Multiple Presentations at 2015 ARVO Meeting

Dec 05, 2014: QLT Announces Results From Proof-of-Concept Trial With Oral Synthetic cis-Retinoid (QLT091001) in Adult Subjects With Impaired Dark Adaptation and/or Impaired Low Luminance Vision

Nov 06, 2014: Spark Therapeutics Receives FDA Breakthrough Therapy Designation for Its Lead Product Candidate, SPK-RPE65

Sep 12, 2014: QLT Announces Positive Final Results From Phase 1b Retreatment Trial With Oral Synthetic cis-Retinoid (QLT091001) in Subjects With LCA or RP Due to Mutations in RPE65 or LRAT

Jul 14, 2014: QLT Announces Publication in The Lancet of Phase 1b Data for QLT091001 in Leber Congenital Amaurosis Due to LRAT or RPE65 Mutations

May 08, 2014: Spark Therapeutics Scientific Advisor to Deliver Keynote at the Association for Research in Vision and Ophthalmology's 2014 Annual Meeting

Feb 27, 2014: QLT Announces Positive Preliminary Results From Phase 1b Retreatment Trial of QLT091001 in Subjects With Leber Congenital Amaurosis and Retinitis Pigmentosa Due to Mutations in LRAT or RPE65

Jan 14, 2014: Spark Therapeutics Achieves Recruitment Goal in Phase 3 Gene Therapy Clinical Study for Inherited Blindness

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COMPANIES MENTIONED

AmpliPhi Biosciences Corp

Editas Medicine Inc

Novelion Therapeutics Inc

ProQR Therapeutics NV

Spark Therapeutics Inc

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