

Leber Hereditary Optic Neuropathy - Pipeline Review, 2019

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Abstracts

Firstview Insight's Leber Hereditary Optic Neuropathy - Pipeline Review, 2019 provides an overview of the pipeline landscape of Leber Hereditary Optic Neuropathy. It provides comprehensive insights of all the clinical and non-clinical therapeutics in development with detailed description about the collaborations; deals; designations; patent information etc. These reports encourage the clients in distinguishing the upcoming and existing competitors in their separate therapeutic spaces. The report provides detailed description of the competitor profiles with key milestones and evidence along with analysis by mechanism of action; route of administration; molecule type; stage of development. Information obtained from multiple sources will be used to triangulate and update the profiles. The report also provides key events in the last year related to the indication. This report provides detailed analysis of all the products along with the companies involved.

Leber Hereditary Optic Neuropathy (LHON) is the most common inherited mitochondrial disorder and typically affects young males. It typically begins as a unilateral progressive optic neuropathy with sequential involvement of the fellow eye months to years later. LHON is classically associated with mitochondrial base pair mutations G11778A (guanine to adenine at position 11778), T14484C (tyrosine to cytosine), and G3460A. These mutations primarily affect respiratory chain complex I genes, mitochondrial genes ND1, ND4 and ND6 among others. The G11778A is the most common, reported to account for 70% of cases in Northern European populations, and 90% in Asian populations. It is associated with more severe disease, with less chance of significant visual recovery. The T14484C mutation is associated with the best overall prognosis, with some visual recovery in 37-58% of cases. The G3460A mutation usually portends a more intermediate course and has the worst prognosis.

Treatment options are limited, but include the use of antioxidant supplements. Gene therapy trials are currently underway.

LIST OF PRODUCTS:

Catena

Lenadogene nolparvovec

Elamipretide

BVA203

IXC-201

KH176

Research Programme - Fortify Therapeutics

Proprietary Libraray-Khondrion

SPK-LHON

Drug Profile Overview:

The pipeline section provides descriptive drug profiles for the pipeline products including product description, mechanism of action, route of administration, molecule type, technology involved, chemical information.

Clinical Trial Overview:

This section of the report focuses on the clinical activity of the molecule. It includes both clinical and pre-clinical activity which provides detailed information about the safety, efficacy, tolerability, toxicity of pipeline drugs. A graphical representation of the clinical trial landscape of pipeline therapy which includes information about phase of development, trial design, treatment arms, dosage and frequency, formulation of the drug, primary and secondary completion date, enrolment number, exclusion and inclusion criteria, line of therapy. This section also includes the clinical trial results and

analysis based on those results.

Product Development Activity:

This section of the report focuses on detail information about designations, exclusivity details, technology, licensing and collaboration, funding and financing, key milestones and various other development activities.

Company Overview:

Company profile includes the detail about type of company, headquarter, global presence, research focus and key financial

Scope

The report provides a competitive landscape

The report also provides clinical trial landscape of the pipeline drugs including status; trial phase; sponsor type and end-point status

The report provides the list of companies which are the most active in the pipeline

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery

The report provides descriptive drug profiles which includes product description; comprising detailed mechanism of action (MoA); route of administration (RoA); Stage of development; clinical trial status; licensing and collaboration details & other developmental activities

The report features comparative analysis of product profiles based on molecule type; mechanism of action (MoA); route of administration (RoA)

The report summarizes all the dormant and discontinued pipeline projects

The report also provides latest news for the past one year

Reasons To Buy

To identifying prominent players in the treatment landscape

To determine the drivers; barriers and unmet need in the treatment space

Gain strategically significant competitor information; analysis; and insights to formulate effective R&D strategies

Define in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope

To understand the composition of the pipeline in terms of molecule type; molecular target; mechanism of action and route of administration

Contents

1. DISEASE OVERVIEW

- 1.1. Pathophysiology
- 1.2. Symptoms
- 1.3. Etiology
- 1.4. Diagnosis
- 1.5. Etiology
- 1.6. Treatment Algorithm
- 1.7. Treatment Options

2. FEATURED NEWS AND PRESS RELEASES, 2018-2019

3. LIST OF COMPANIES DEVELOPING LEBER HEREDITARY OPTIC NEUROPATHY

4. TREATMENT ALGORITHM

5. CURRENT UNMET NEEDS

6. MARKET TRENDS

7. PIPELINE THERAPEUTICS

8. THERAPEUTICS UNDER DEVELOPMENT BY COMPANIES **9. LATE STAGE PRODUCTS (PHASE III)**

- 9.1 Lenadogene nolparvovec
 - 9.1.1. Product Description
 - 9.1.2. Research and Development
 - 9.1.3. Product Development Activities
- 9.2. XXX
 - 9.2.1. Product Description
 - 9.2.2. Research and Development
 - 9.2.3. Product Development Activities
- 10. Mid Stage Products (Phase II)
 - 10.1 Elamipretide
 - 10.1.1. Product Description
 - 10.1.2. Research and Development

- 10.1.3. Product Development Activities
- 10.2. XXX
 - 10.2.1. Product Description
 - 10.2.2. Research and Development
 - 10.2.3. Product Development Activities

11. EARLY STAGE AND PRECLINICAL PRODUCTS (PHASE II)

- 11.1 XXX
 - 11.1.1. Product Description
 - 11.1.2. Research and Development
 - 11.1.3. Product Development Activities
- 11.2. BVA203
 - 11.2.1. Product Description
 - 11.2.2. Research and Development
 - 11.2.3. Product Development Activities

12. DRIVERS OF LEBER HEREDITARY OPTIC NEUROPATHY

13. CONSTRAINTS OF LEBER HEREDITARY OPTIC NEUROPATHY

14. THERAPEUTIC ASSESSMENT

- 14.1. Assessment by Route of Administration
- 14.2. Assessment by Phase & Route of Administration
- 14.3. Assessment by Molecule Type
- 14.4. Assessment by Phase & Molecule type
- 14.5. Assessment by Target
- 14.6. Assessment by Mechanism of Action

15. COLLABORATIONS AND ACQUISITIONS DETAILS

16. DORMANT PRODUCTS

17. DISCONTINUED PRODUCTS

18. MARKET TRENDS

19. MARKET OPPORTUNITY ASSESSMENT

20. UNMET NEEDS

21. SWOT ANALYSIS

22. APPENDIX

23. REPORT METHODOLOGY

24. CONSULTING SERVICES

25. DISCLAIMER

List Of Tables

LIST OF TABLES

Table 1: Marketed Leber Hereditary Optic Neuropathy drugs, 2019

Table 2: Sales of Marketed drugs

Table 3: Patent expiration details – marketed drugs

Table 4: Number of Products Under Development for Leber Hereditary Optic Neuropathy

Table 5: Products under Development by Companies

Table 6: Late Stage Products

Table 7: Mid Stage Products

Table 8: Early Stage Products

Table 9: Pre-Clinical and Discovery Products

Table 10: Assessment by Route of Administration

Table 11: Assessment by Stage and Route of Administration

Table 12: Assessment by Molecule Type

Table 13: Assessment by Stage and Molecule Type

Table 14: Assessment by Target

Table 15: Assessment by Mechanism of Action

Table 16: Dormant Products

Table 17: Discontinued Products

List Of Figures

LIST OF FIGURES

Figure 1: Marketed and Pipeline Leber Hereditary Optic Neuropathy - Pipeline Review, 2019 drugs, 2019

Figure 2: Sales of Marketed drugs

Figure 3: Patent expiration details – marketed drugs

Figure 4: Number of Products Under Development for Leber Hereditary Optic Neuropathy - Pipeline Review, 2019

Figure 5: Products under Development by Companies

Figure 6: Late Stage Products

Figure 7: Mid Stage Products

Figure 8: Early Stage Products

Figure 9: Pre-Clinical and Discovery Products

Figure 10: Assessment by Route of Administration

Figure 11: Assessment by Stage and Route of Administration

Figure 12: Assessment by Molecule Type

Figure 13: Assessment by Stage and Molecule Type

Figure 14: Assessment by Target

Figure 15: Assessment by Mechanism of Action

Figure 16: Dormant Products

Figure 17: Discontinued Products

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