

# Primary Hyperoxaluria - Pipeline Review, H1 2019

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### Abstracts

Primary Hyperoxaluria - Pipeline Review, H1 2019

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Primary Hyperoxaluria - Pipeline Review, H1 2019, provides an overview of the Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) pipeline landscape.

Primary hyperoxaluria is a rare condition characterized by recurrent kidney and bladder stones. It results from the overproduction of a substance called oxalate. Symptoms include blood in the urine, pain when urinating, fever, sudden abdominal pain and frequent urge to urinate. Treatment includes Oral preparations of phosphates and diuretics.

### **REPORT HIGHLIGHTS**

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Primary Hyperoxaluria - Pipeline Review, H1 2019, provides comprehensive information on the therapeutics under development for Primary Hyperoxaluria (Genito Urinary System And Sex Hormones), 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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) pipeline guide also reviews of key players involved in therapeutic development for Primary Hyperoxaluria 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, 2, 2 and 1 respectively.

Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) 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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones).

The pipeline guide reviews pipeline therapeutics for Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) 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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) 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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones)

#### **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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones).

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 Primary Hyperoxaluria (Genito Urinary System And Sex Hormones) 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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lumasiran - Drug Profile Product Description Mechanism Of Action **R&D** Progress **Oxabact - Drug Profile Product Description** Mechanism Of Action **R&D** Progress reloxaliase - Drug Profile **Product Description** Mechanism Of Action **R&D** Progress Primary Hyperoxaluria - Dormant Projects Primary Hyperoxaluria - Discontinued Products Primary Hyperoxaluria - Product Development Milestones Featured News & Press Releases May 02, 2019: Dicerna submits updated IND application for DCR-PHXC for treatment of primary hyperoxaluria (PH) for the PHYOX 2 pivotal trial May 02, 2019: Dicerna submits updated IND application for DCR-PHXC for treatment of primary hyperoxaluria (PH) for the PHYOX 2 pivotal trial Apr 16, 2019: Alnylam begins late-stage paediatric trial of lumasiran Apr 15, 2019: Intellia Therapeutics announces Oral Presentation on Gene Therapy to inhibit HAO1 for Primary Hyperoxaluria Type I at the 22nd Annual Meeting of the American Society of Gene and Cell Therapy Mar 29, 2019: Dicerna announces the presentation of updated data demonstrating utility of its lead compound DCR-PHXC in treating primary hyperoxaluria type 1 (PH1) and type 2 (PH2) Mar 29, 2019: Dicerna announces the presentation of updated data demonstrating utility of its lead compound DCR-PHXC in treating primary hyperoxaluria type 1 (PH1) and type 2 (PH2) Nov 15, 2018: OxThera is being granted US patent for oxalobacter secretagogues Oct 31, 2018: OxThera presents encouraging 52-week efficacy and safety data for Oxabact in primary hyperoxaluria type 1 (PH1) patients with ESRD Oct 25, 2018: Dicerna Announces Late-Breaking Data Supporting Use of DCR-PHXC in Adults with Primary Hyperoxaluria Types 1 and 2 (PH1 and PH2) Oct 25, 2018: Dicerna Announces Late-Breaking Data Supporting Use of DCR-PHXC in Adults with Primary Hyperoxaluria Types 1 and 2 (PH1 and PH2) Oct 25, 2018: Alnylam expands Alnylam Act program to improve diagnosis of Primary

Hyperoxaluria Type 1 and aligns with FDA on trial design for ILLUMINATE-B Phase 3



#### pediatric study of Lumasiran

Oct 08, 2018: Alnylam reports new positive Phase I/II results from lumasiran trial Oct 05, 2018: Dicerna Announces Late-Breaking Data from the PHYOX Phase 1 Clinical Trial of DCR-PHXC Will Be Presented at ASN Kidney Week 2018 Oct 05, 2018: Dicerna Announces Late-Breaking Data from the PHYOX Phase 1 Clinical Trial of DCR-PHXC Will Be Presented at ASN Kidney Week 2018 Sep 05, 2018: Dicerna announces proof of concept for DCR-PHXC in the treatment of primary hyperoxaluria Appendix Methodology Coverage Secondary Research Primary Research Expert Panel Validation Contact Us Disclaimer



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#### **COMPANIES MENTIONED**

Allena Pharmaceuticals Inc Alnylam Pharmaceuticals Inc BridgeBio Pharma LLC Dicerna Pharmaceuticals Inc Intellia Therapeutics Inc OxThera AB Precision Biosciences Inc



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