

# Ornithine-Transcarbamylase Deficiency - Pipeline Review, H1 2020

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## Abstracts

Ornithine-Transcarbamylase Deficiency - Pipeline Review, H1 2020

### SUMMARY

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

Ornithine transcarbamylase (OTC) deficiency is a rare X-linked genetic disorder characterized by complete or partial lack of the enzyme ornithine transcarbamylase (OTC). OTC plays an important role in the break down and removal of nitrogen the body (urea cycle). The lack of the OTC enzyme results in excessive accumulation of nitrogen, in the form of ammonia (hyperammonemia), in the blood. Symptoms include vomiting, refusal to eat, progressive lethargy, and coma. Treatment includes nitrogen scavenging agents.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Ornithine-Transcarbamylase Deficiency - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (Genetic Disorders) pipeline guide also

reviews of key players involved in therapeutic development for Ornithine-Transcarbamylase Deficiency 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 and Preclinical stages are 1, 1, 1 and 4 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 3 molecules, respectively.

Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (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 Ornithine-Transcarbamylase Deficiency (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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Featured News & Press Releases

Jun 05, 2020: Arcturus Therapeutics announces first healthy volunteer dosed in phase 1 study of ARCT-810 for Ornithine Transcarbamylase (OTC) Deficiency

Apr 13, 2020: Arcturus Therapeutics announces allowance of IND & approval of clinical trial application (CTA) for ARCT-810, a first-in-class investigational mRNA medicine to

treat ornithine transcarbamylase deficiency

Oct 23, 2019: Selecta Biosciences presents new preclinical data from its gene therapy program at 2019 ESGCT Annual Congress

Apr 16, 2019: Selecta Biosciences presents new preclinical data of SEL-313 from its Gene Therapy Program at the American Society of Gene & Cell Therapy (ASGCT) 22nd Annual Meeting

Oct 05, 2018: Co-administration of AAV Vectors with SVP-Rapamycin Enables Vector Re-administration in Pre-clinical Gene Therapy Study Published in Nature

Communications by Genethon and Selecta Biosciences

Oct 05, 2017: Selecta Biosciences Announces Upcoming Clinical and Scientific Presentations on SEL-313

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### COMPANIES MENTIONED

Arcturus Therapeutics Ltd

Lucane Pharma SA

Promethera Biosciences SA

Selecta Biosciences Inc

Ultragenyx Pharmaceutical Inc

Unicyte AG

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