

# Ornithine-Transcarbamylase Deficiency Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

<https://marketpublishers.com/r/O81CB99C1514EN.html>

Date: November 2022

Pages: 61

Price: US\$ 2,000.00 (Single User License)

ID: O81CB99C1514EN

## Abstracts

Ornithine-Transcarbamylase Deficiency Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Ornithine-Transcarbamylase Deficiency - Drugs In Development, 2022, 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 - Drugs In Development, 2022, 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 and Preclinical stages are 1, 3 and 9 respectively. Similarly, the Universities portfolio in Preclinical stages comprises 2 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.

## Contents

- Introduction
- Global Markets Direct Report Coverage
- Ornithine-Transcarbamylase Deficiency - Overview
- Ornithine-Transcarbamylase Deficiency - Therapeutics Development Pipeline Overview
- Pipeline by Companies
- Pipeline by Universities/Institutes
- Products under Development by Companies
- Products under Development by Universities/Institutes
- Ornithine-Transcarbamylase Deficiency - Therapeutics Assessment
  - Assessment by Target
  - Assessment by Mechanism of Action
  - Assessment by Route of Administration
  - Assessment by Molecule Type
- Ornithine-Transcarbamylase Deficiency - Companies Involved in Therapeutics Development
  - Arcturus Therapeutics Holdings Inc
  - BioNTech SE
  - Bloomsbury Genetic Therapies Ltd
  - CAMP4 Therapeutics Corp
  - Cellaion SA
  - Gene Therapy Research Institution Co Ltd
  - iECURE
  - Lucane Pharma SA
  - Moderna Inc
  - Poseida Therapeutics Inc
  - Sana Biotechnology Inc
  - Ultragenyx Pharmaceutical Inc
  - Unicyte AG
- Ornithine-Transcarbamylase Deficiency - Drug Profiles
  - ARCT-810 - Drug Profile
    - Product Description
    - Mechanism Of Action
  - avalotcagene ontaparvovec - Drug Profile
    - Product Description
    - Mechanism Of Action
  - BGT-OTCD - Drug Profile

Product Description  
Mechanism Of Action  
BNT-171 - Drug Profile  
Product Description  
Mechanism Of Action  
Gene Therapy to Activate Ornithine Transcarbamylase for Ornithine Transcarbamylase  
Deficiency - Drug Profile  
Product Description  
Mechanism Of Action  
GT-0008X - Drug Profile  
Product Description  
Mechanism Of Action  
GTP-506 - Drug Profile  
Product Description  
Mechanism Of Action  
HepaStem - Drug Profile  
Product Description  
Mechanism Of Action  
HLSC-001 - Drug Profile  
Product Description  
Mechanism Of Action  
mRNA-3139 - Drug Profile  
Product Description  
Mechanism Of Action  
Oligonucleotide to Activate Ornithine Transcarbamylase for Ornithine Transcarbamylase  
Deficiency - Drug Profile  
Product Description  
Mechanism Of Action  
Oligonucleotides for Ornithine Transcarbamylase Deficiency - Drug Profile  
Product Description  
Mechanism Of Action  
POTC-101 - Drug Profile  
Product Description  
Mechanism Of Action  
SG-328 - Drug Profile  
Product Description  
Mechanism Of Action  
sodium benzoate - Drug Profile  
Product Description

## Mechanism Of Action

Ornithine-Transcarbamylase Deficiency - Dormant Projects

Ornithine-Transcarbamylase Deficiency - Discontinued Products

Ornithine-Transcarbamylase Deficiency - Product Development Milestones

## Featured News & Press Releases

Oct 19, 2022: Preclinical data from iECURE's GTP-506 demonstrates potential for the treatment of Ornithine Transcarbamylase (OTC) Deficiency

Aug 24, 2022: FDA grants Rare Pediatric Disease status for iECURE's OTC deficiency therapy

May 19, 2022: Ultragenyx announces positive longer-term durability data from Phase 1/2 Gene Therapy Study of DTX301 at American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting

May 19, 2022: Ultragenyx announces upcoming data presentations at American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting

Nov 29, 2021: Ultragenyx announces additional positive multi-year durability data from phase 1/2 AAV gene therapy studies of DTX301

Jul 28, 2021: Arcturus announces regulatory approval to proceed with phase 2 study of ARCT-810 mRNA therapeutic candidate for Ornithine Transcarbamylase (OTC) Deficiency

May 14, 2021: Ultragenyx announces presentation on its gene therapy candidate DTX301 at American Society of Gene & Cell Therapy 2021 Annual Meeting

Apr 22, 2021: Ultragenyx completes successful end-of-phase 2 meeting with FDA and finalizes phase 3 study design for DTX301 Ornithine Transcarbamylase (OTC) gene therapy program

Dec 07, 2020: Arcturus Therapeutics announces initiation of dosing ARCT-810 in patients with Ornithine Transcarbamylase (OTC) deficiency

Oct 05, 2020: Arcturus Therapeutics announces completion of first three dose escalation cohorts in phase 1 study of ARCT-810, therapeutic candidate for ornithine transcarbamylase (OTC) deficiency

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

May 13, 2020: Ultragenyx announces positive longer-term results from first three cohorts of phase 1/2 study of DTX301 Gene Therapy in 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

Jan 09, 2020: Ultragenyx announces positive topline cohort 3 results and improved longer-term cohort 2 results from phase 1/2 study of DTX301 gene therapy in ornithine

transcarbamylase (OTC) deficiency

Sep 27, 2018: Ultragenyx announces positive topline cohort 2 results from phase 1/2 clinical study of DTX301 gene therapy in ornithine transcarbamylase (OTC) deficiency and progression to higher dose

[Appendix](#)

[Methodology](#)

[Coverage](#)

[Secondary Research](#)

[Primary Research](#)

[Expert Panel Validation](#)

[Contact Us](#)

[Disclaimer](#)



## List Of Tables

### LIST OF TABLES

Number of Products under Development for Ornithine-Transcarbamylase Deficiency, 2022

Number of Products under Development by Companies, 2022

Number of Products under Development by Universities/Institutes, 2022

Products under Development by Companies, 2022

Products under Development by Universities/Institutes, 2022

Number of Products by Stage and Target, 2022

Number of Products by Stage and Mechanism of Action, 2022

Number of Products by Stage and Route of Administration, 2022

Number of Products by Stage and Molecule Type, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Arcturus Therapeutics Holdings Inc, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by BioNTech SE, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Bloomsbury Genetic Therapies Ltd, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by CAMP4 Therapeutics Corp, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Cellaion SA, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Gene Therapy Research Institution Co Ltd, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by iECURE, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Lucane Pharma SA, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Moderna Inc, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Poseida Therapeutics Inc, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Sana Biotechnology Inc, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Ultragenyx Pharmaceutical Inc, 2022

Ornithine-Transcarbamylase Deficiency - Pipeline by Unicyte AG, 2022

Ornithine-Transcarbamylase Deficiency - Dormant Projects, 2022

Ornithine-Transcarbamylase Deficiency - Discontinued Products, 2022

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Ornithine-Transcarbamylase Deficiency, 2022

Number of Products under Development by Companies, 2022

Number of Products by Stage and Top 10 Targets, 2022

Number of Products by Stage and Top 10 Mechanism of Actions, 2022

Number of Products by Top 10 Routes of Administration, 2022

Number of Products by Stage and Top 10 Routes of Administration, 2022

Number of Products by Top 10 Molecule Types, 2022

Number of Products by Stage and Top 10 Molecule Types, 2022

## I would like to order

Product name: Ornithine-Transcarbamylase Deficiency Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

Product link: <https://marketpublishers.com/r/O81CB99C1514EN.html>

Price: US\$ 2,000.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/O81CB99C1514EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970

