

# Phenylketonuria (PKU) Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

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## Abstracts

Phenylketonuria (PKU) 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 Phenylketonuria (PKU) - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides an overview of the Phenylketonuria (PKU) (Metabolic Disorders) pipeline landscape.

Phenylketonuria (also called PKU) is a rare inherited disorder that causes an amino acid called phenylalanine to build up in body. Symptoms include intellectual disability, psychiatric disorders, hyperactivity, skin rashes, poor bone strength and abnormally small head.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Phenylketonuria (PKU) - Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update, provides comprehensive information on the therapeutics under development for Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Phenylketonuria (PKU) 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, Preclinical and Discovery stages are 2, 1, 3, 11 and 8 respectively.

Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic Disorders).

The pipeline guide reviews pipeline therapeutics for Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic 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 Phenylketonuria (PKU) (Metabolic 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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  - Jnana Therapeutics Inc
  - Maze Therapeutics Inc
  - MipSalus ApS
  - Moderna Inc
  - Nestle Health Science
  - Peg-Bio BioPharma Co Ltd
  - Pluvia AS
  - Prosit Sole Biotechnology (Beijing) Co Ltd
  - PTC Therapeutics Inc
  - Relief Therapeutics Holding AG
  - SOM Biotech SL
  - Synlogic Inc
  - Ultragenyx Pharmaceutical Inc
  - Vera Therapeutics Inc

**Phenylketonuria (PKU) - Drug Profiles****AGT-324 - Drug Profile**

Product Description

Mechanism Of Action

History of Events

**APROD-031 - Drug Profile**

Product Description

Mechanism Of Action

History of Events

**CDX-6114 - Drug Profile**

Product Description

Mechanism Of Action

History of Events

**Gene Therapy for Phenylketonuria - Drug Profile**

Product Description

Mechanism Of Action

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Product Description

Mechanism Of Action

History of Events

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Product Description

Mechanism Of Action

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Phenylketonuria - Drug Profile

Product Description

Mechanism Of Action

PJ-008 - Drug Profile

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Mechanism Of Action

PKU - Drug Profile

Product Description

Mechanism Of Action

PSP-007 - Drug Profile

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sepiapterin - Drug Profile

Product Description

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Small Molecule for Phenylketonuria - Drug Profile

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Mechanism Of Action

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Product Description

Mechanism Of Action

History of Events

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Product Description

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Product Description

Mechanism Of Action

History of Events

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Featured News & Press Releases

Feb 23, 2022: Jnana Therapeutics to advance JNT-517 as development candidate and potential first-in-class oral treatment for PKU

Nov 22, 2021: Synlogic presents data demonstrating reductions in plasma phenylalanine levels in patients with phenylketonuria treated with SYNB1934

Nov 22, 2021: Synlogic presents data demonstrating reductions in plasma phenylalanine levels in patients with phenylketonuria treated with SYNB1618

Nov 10, 2021: Synlogic announces presentation of data on SYNB1934 at 14th International Congress of Inborn Errors of Metabolism Meeting

Nov 10, 2021: Synlogic announces presentation of data on SYNB1618 at 14th International Congress of Inborn Errors of Metabolism Meeting

Oct 28, 2021: Synlogic announces Nature Communications publication highlighting use of synthetic biotic platform to optimize therapies for phenylketonuria

Oct 12, 2021: Homology Medicines announces world's first gene editing clinical trial for PKU

Sep 11, 2021: Synlogic presents at Global PKU Patient Meeting

Jul 22, 2021: Synlogic publishes papers in Nature journals demonstrating proof-of-mechanism and potential of synthetic biotic platform for the treatment of phenylketonuria (PKU)

Jul 15, 2021: Synlogic provides update on SYNB1618 , a next-generation strain for the treatment of Phenylketonuria (PKU)

Jul 15, 2021: Synlogic announces initiation of phase 1 study of SYNB1934, a next-generation strain for the treatment of Phenylketonuria (PKU)

Jun 15, 2021: Synlogic presents additional preclinical data on therapeutic candidate SYNB1934 for Phenylketonuria (PKU) at Synthetic Biology: Engineering, Evolution &



## Design (SEED) Conference

Apr 13, 2021: Synlogic presents data demonstrating activity of a solid oral formulation of SYN1618 at American College of Medical Genetics (ACMG) annual meeting

Oct 07, 2020: BioMarin, pioneer in rare disease treatments for Phenylketonuria (PKU), receives FDA approval of label expansion to allow maximum dose of 60 mg for Palynziq (pegvaliase-pqpz) injection for treatment of adults with PKU

Mar 26, 2020: FDA grants Orphan Drug Designation to APR-OD031 for the treatment of phenylketonuria

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