

# Phenylketonuria (PKU) - Pipeline Review, H1 2020

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## Abstracts

Phenylketonuria (PKU) - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Phenylketonuria (PKU) - Pipeline Review, H1 2020, 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) - Pipeline Review, H1 2020, 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, IND/CTA Filed, Preclinical and Discovery stages are 1, 3, 2, 1, 9

and 3 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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Agios Pharmaceuticals Inc

American Gene Technologies International Inc

APR Applied Pharma Research SA

BioMarin Pharmaceutical Inc

Censa Pharmaceuticals Inc

Erytech Pharma SA

Generation Bio Corp

Homology Medicines Inc

MipSalus ApS

Moderna Therapeutics Inc

Nestle Health Science SA

Pluvia AS

Rubius Therapeutics Inc

Sangamo Therapeutics Inc

SOM Biotech SL

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Number of Products by Stage and Molecule Types, H1 2020

### COMPANIES MENTIONED

Agios Pharmaceuticals Inc

American Gene Technologies International Inc

APR Applied Pharma Research SA

BioMarin Pharmaceutical Inc

Censa Pharmaceuticals Inc

Erytech Pharma SA

Generation Bio Corp

Homology Medicines Inc

MipSalus ApS

Moderna Therapeutics Inc

Nestle Health Science SA

Pluvia AS

Rubius Therapeutics Inc

Sangamo Therapeutics Inc

SOM Biotech SL

Synlogic Inc

TruCode Gene Repair Inc

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