

# Phenylketonuria (PKU) - Pipeline Review, H2 2020

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## **Abstracts**

Phenylketonuria (PKU) - Pipeline Review, H2 2020

#### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Phenylketonuria - Pipeline Review, H2 2020, provides an overview of the Phenylketonuria (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 - Pipeline Review, H2 2020, provides comprehensive information on the therapeutics under development for Phenylketonuria (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 (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 I, IND/CTA Filed, Preclinical and Discovery stages are 1, 3, 1, 1, 10 and 2 respectively.

Phenylketonuria (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 (Metabolic Disorders).

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

The pipeline guide evaluates Phenylketonuria (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 (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 (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 (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.



## **Contents**

Introduction

Global Markets Direct Report Coverage

Phenylketonuria (PKU) - Overview

Phenylketonuria (PKU) - Therapeutics Development

Pipeline Overview

Pipeline by Companies

Products under Development by Companies

Phenylketonuria (PKU) - Therapeutics Assessment

Assessment by Target

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Phenylketonuria (PKU) - Companies Involved in Therapeutics Development

Agios Pharmaceuticals Inc

American Gene Technologies International Inc

APR Applied Pharma Research SA

BioMarin Pharmaceutical Inc

Generation Bio Co

Homology Medicines Inc

MipSalus ApS

Moderna Inc

Nestle Health Science SA

Pluvia AS

PTC Therapeutics Inc

Sangamo Therapeutics Inc

SOM Biotech SL

Synlogic Inc

Vera Therapeutics Inc

Phenylketonuria (PKU) - Drug Profiles

AGT-324 - Drug Profile

**Product Description** 

Mechanism Of Action

R&D Progress

APROD-031 - Drug Profile

**Product Description** 

Mechanism Of Action

**R&D Progress** 



Biologic for Phenylketonuria - Drug Profile

**Product Description** 

Mechanism Of Action

R&D Progress

Phenylketonuria (PKU) - Dormant Projects

Phenylketonuria (PKU) - Discontinued Products

Phenylketonuria (PKU) - Product Development Milestones

Featured News & Press Releases

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 Phenylketonuria (PKU), H2 2020

Number of Products under Development by Companies, H2 2020

Products under Development by Companies, H2 2020

Products under Development by Companies, H2 2020 (Contd..1), H2 2020

Number of Products by Stage and Target, H2 2020

Number of Products by Stage and Mechanism of Action, H2 2020

Number of Products by Stage and Route of Administration, H2 2020

Number of Products by Stage and Molecule Type, H2 2020

Phenylketonuria (PKU) - Pipeline by Agios Pharmaceuticals Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by American Gene Technologies International Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by APR Applied Pharma Research SA, H2 2020

Phenylketonuria (PKU) - Pipeline by BioMarin Pharmaceutical Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by Generation Bio Co, H2 2020

Phenylketonuria (PKU) - Pipeline by Homology Medicines Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by MipSalus ApS, H2 2020

Phenylketonuria (PKU) - Pipeline by Moderna Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by Nestle Health Science SA, H2 2020

Phenylketonuria (PKU) - Pipeline by Pluvia AS, H2 2020

Phenylketonuria (PKU) - Pipeline by PTC Therapeutics Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by Sangamo Therapeutics Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by SOM Biotech SL, H2 2020

Phenylketonuria (PKU) - Pipeline by Synlogic Inc, H2 2020

Phenylketonuria (PKU) - Pipeline by Vera Therapeutics Inc, H2 2020

Phenylketonuria (PKU) - Dormant Projects, H2 2020

Phenylketonuria (PKU) - Discontinued Products, H2 2020



## **List Of Figures**

#### LIST OF FIGURES

Number of Products under Development for Phenylketonuria (PKU), H2 2020

Number of Products under Development by Companies, H2 2020

Number of Products by Targets, H2 2020

Number of Products by Stage and Targets, H2 2020

Number of Products by Mechanism of Actions, H2 2020

Number of Products by Stage and Mechanism of Actions, H2 2020

Number of Products by Routes of Administration, H2 2020

Number of Products by Stage and Routes of Administration, H2 2020

Number of Products by Molecule Types, H2 2020

Number of Products by Stage and Molecule Types, H2 2020

## **COMPANIES MENTIONED**

Agios Pharmaceuticals Inc

American Gene Technologies International Inc

APR Applied Pharma Research SA

BioMarin Pharmaceutical Inc

Generation Bio Co

Homology Medicines Inc

MipSalus ApS

Moderna Inc

Nestle Health Science SA

Pluvia AS

PTC Therapeutics Inc

Sangamo Therapeutics Inc

SOM Biotech SL

Synlogic Inc

Vera Therapeutics Inc



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