

Phenylketonuria (PKU) (Metabolic Disorder) - Drugs in Development, 2021

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Abstracts

Phenylketonuria (PKU) (Metabolic Disorder) - Drugs in Development, 2021

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Phenylketonuria - Drugs in Development, 2021, 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 - Drugs in Development, 2021, 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 II, Phase I, Preclinical and Discovery stages are 1, 4, 2, 11 and 4 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.

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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

Evox Therapeutics Ltd

Generation Bio Co

Homology Medicines Inc

MipSalus ApS

Moderna Inc

Nestle Health Science SA

Peg-Bio BioPharma Co Ltd

Pluvia AS

Prosit Sole Biotechnology (Beijing) Co Ltd

PTC Therapeutics Inc

Sangamo Therapeutics Inc

SOM Biotech SL

Synlogic Inc

Ultragenyx Pharmaceutical 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

Biologics for Phenylketonuria - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BMN-307 - Drug Profile

Product Description

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CDX-6114 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy for Phenylketonuria and Progressive Familial Intrahepatic Cholestasis -**Drug Profile**

Product Description

Mechanism Of Action

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Product Description

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Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

SOM-1311 - Drug Profile

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Mechanism Of Action

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ST-101 - Drug Profile

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Mechanism Of Action

R&D Progress

SYNB-1618 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

UX-501 - 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

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

Mar 10, 2020: American Gene Technologies to present at 2020 Inborn Errors of Metabolism Drug Development Summit in Boston

Dec 11, 2019: Synlogic bridging study data with solid oral formulation of SYNB1618, a synthetic biotic approach to treat Phenylketonuria, demonstrate improved tolerability over early liquid formulation and guide to next stage of clinical development

Dec 03, 2019: Censa Pharmaceuticals announces CNSA- 001 met primary and secondary endpoints in phase 2 trial in patients with PKU

Sep 04, 2019: Synlogic presents data from phase 1/2a study of SYNB1618 at the Annual Symposium of the Society for the Study of Inborn Errors of Metabolism (SSIEM)

Jul 15, 2019: Synlogic reports positive top-line data from phase 1/2a study of SYNB1618 in patients with phenylketonuria and guides to next phase of development

May 06, 2019: European Commission approves Palynziq (pegvaliase injection) for treatment of phenylketonuria (PKU) in patients aged 16 years or older

Apr 30, 2019: Synlogic presents data describing a Solid Oral Formulation Process for Synthetic Biotic Medicine SYNB1618 for the treatment of PKU at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

Apr 15, 2019: Synlogic to present data from new solid oral formulation process at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

Mar 01, 2019: BioMarin Receives Positive CHMP Opinion in Europe for Palynziq (pegvaliase Injection) for Treatment of Patients with Phenylketonuria (PKU) Aged 16 and Older

Jan 07, 2019: Retrophin provides update on phenylketonuria drug CNSA-001

Jan 07, 2019: BioMarin highlights Palynziq at 37th Annual J.P. Morgan Healthcare Conference in San Francisco

Jan 07, 2019: Homology Medicines advances first gene editing development candidate into IND-enabling studies for pediatric patients with phenylketonuria

Jan 03, 2019: Synlogic provides update on synthetic biotic medicine SYNB-1618

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