

# Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Pipeline Review, H2 2018

<https://marketpublishers.com/r/PACB786B867EN.html>

Date: November 2018

Pages: 40

Price: US\$ 3,500.00 (Single User License)

ID: PACB786B867EN

## Abstracts

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Pipeline Review, H2 2018

### SUMMARY

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Phenylalanine 4 hydroxylase or Phenylalanine hydroxylase (PAH) is an enzyme that catalyzes the hydroxylation of the aromatic side-chain of phenylalanine to generate tyrosine. The enzyme works with a molecule called tetrahydrobiopterin (BH4) to carry out this chemical reaction. Tyrosine is used to make several types of hormones, certain chemicals that transmit signals in the brain (neurotransmitters), and a pigment called melanin, which gives hair and skin their color.

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) pipeline Target constitutes close to 9 molecules. The molecules developed by companies in Phase I and Preclinical stages are 1 and 8 respectively. Report covers products from therapy areas Metabolic Disorders which include indications Phenylketonuria (PKU).

The latest report Phenylalanine 4 Hydroxylase - Pipeline Review, H2 2018, outlays comprehensive information on the Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type. It also reviews key players involved in Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics development with respective active and dormant or discontinued projects.

The report is built using data and information sourced from 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.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The report provides a snapshot of the global therapeutic landscape for Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1)

The report reviews Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

The report features descriptive drug profiles for the pipeline products which includes, product description, descriptive MoA, R&D brief, licensing and collaboration details & other developmental activities

The report reviews key players involved in Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics and enlists all their major and minor projects

The report assesses Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics

## REASONS TO BUY

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

Identify emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage

Identify and understand the targeted therapy areas and indications for Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

Plan mergers and acquisitions effectively by identifying key players and it's most promising pipeline therapeutics

Devise corrective measures for pipeline projects by understanding Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) development landscape

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

## Contents

Introduction

Global Markets Direct Report Coverage

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) -  
Overview

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) -  
Therapeutics Development

Products under Development by Stage of Development

Products under Development by Therapy Area

Products under Development by Indication

Products under Development by Companies

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) -  
Therapeutics Assessment

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) -

Companies Involved in Therapeutics Development

American Gene Technologies International Inc

BioMarin Pharmaceutical Inc

Codexis Inc

Homology Medicines Inc

Moderna Therapeutics Inc

Rubius Therapeutics Inc

Synthetic Biologics Inc

Ultragenyx Pharmaceutical Inc

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Drug  
Profiles

BMN-307 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

CDX-6114 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Phenylalanine Hydroxylase for Phenylketonuria - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

HMI-102 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

mRNA-3283 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RTX-134 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules to Activate Phenylalanine Hydroxylase for Phenylketonuria - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SYN-200 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

UX-501 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Dormant Products

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Product Development Milestones

Featured News & Press Releases

Oct 18, 2018: American Gene Technologies granted FDA orphan drug designation for phenylketonuria

Oct 18, 2018: Homology Medicines Presents Long-Term Efficacy Data of a Single Dose of Gene Therapy Development Candidate for Treatment of Phenylketonuria

Jul 11, 2018: Codexis Appoints Dr. Hicham Alaoui, Veteran Drug Discoverer, as Vice President, Biotherapeutics Research & Development

Jul 09, 2018: Codexis Doses First Subjects in Phase 1a Trial of CDX-6114

May 18, 2018: Homology Medicines Presents Data from PKU Gene Therapy Program Demonstrating Long-Term Durability of Treatment in IND-Enabling Studies

Apr 30, 2018: Homology Medicines Announces Upcoming Presentations on its Genetic Medicines Platform at the American Society of Gene & Cell Therapy Meeting

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer

## List Of Tables

### LIST OF TABLES

Number of Products under Development by Stage of Development, H2 2018

Number of Products under Development by Therapy Areas, H2 2018

Number of Products under Development by Indication, H2 2018

Number of Products under Development by Companies, H2 2018

Products under Development by Companies, H2 2018

Number of Products by Stage and Mechanism of Actions, H2 2018

Number of Products by Stage and Route of Administration, H2 2018

Number of Products by Stage and Molecule Type, H2 2018

Pipeline by American Gene Technologies International Inc, H2 2018

Pipeline by BioMarin Pharmaceutical Inc, H2 2018

Pipeline by Codexis Inc, H2 2018

Pipeline by Homology Medicines Inc, H2 2018

Pipeline by Moderna Therapeutics Inc, H2 2018

Pipeline by Rubius Therapeutics Inc, H2 2018

Pipeline by Synthetic Biologics Inc, H2 2018

Pipeline by Ultragenyx Pharmaceutical Inc, H2 2018

Dormant Projects, H2 2018

## List Of Figures

### LIST OF FIGURES

Number of Products under Development by Stage of Development, H2 2018  
Number of Products by Mechanism of Actions, H2 2018  
Number of Products by Stage and Mechanism of Actions, H2 2018  
Number of Products by Molecule Types, H2 2018  
Number of Products by Stage and Molecule Types, H2 2018

### COMPANIES MENTIONED

American Gene Technologies International Inc  
BioMarin Pharmaceutical Inc  
Codexis Inc  
Homology Medicines Inc  
Moderna Therapeutics Inc  
Rubius Therapeutics Inc  
Synthetic Biologics Inc  
Ultragenyx Pharmaceutical Inc



## I would like to order

Product name: Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Pipeline Review, H2 2018

Product link: <https://marketpublishers.com/r/PACB786B867EN.html>

Price: US\$ 3,500.00 (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/PACB786B867EN.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

