UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **May 17, 2023**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

100 Corporate Court **South Plainfield, NJ** (Address of Principal Executive Offices)	**07080** (Zip Code)

Registrant's telephone number, including area code: **(908) 222-7000**

Not applicable

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01. Regulation FD Disclosure.

PTC Therapeutics, Inc. (the "Company") will host a conference call on May 17, 2023 at 8:00 a.m. eastern time. During this conference call, the Company expects to discuss the results from its Phase 3 registration-directed clinical trial of sepiapterin for phenylketonuria. Directions on how to access the conference call are included in the press release furnished as Exhibit 99.1 hereto. A copy of the slide deck that will be presented during the conference call is furnished as Exhibit 99.2 hereto.

The information in this Item 7.01 of this Current Report on Form 8-K ("Report"), including Exhibits 99.1 and 99.2, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 8.01. Other Events.

On May 17, 2023, the Company announced that the primary endpoint was achieved in its Phase 3 registration-directed clinical trial of sepiapterin in adult and pediatric patients with phenylketonuria.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated May 17, 2023 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – APHENITY Topline Results
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: May 17, 2023

By: /s/ Emily Hill
Name: Emily Hill
Title: Chief Financial Officer

PTC Therapeutics Announces APHENITY Trial Achieved Primary Endpoint with Sepiapterin in PKU Patients

- Highly statistically significant and clinically meaningful results -
- 63% mean blood Phe reduction in primary analysis population (p<0.0001) -
- Conference call and webcast to be held at 8:00 AM EDT -

SOUTH PLAINFIELD, N.J., **May 17, 2023** -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the primary endpoint was achieved in the APHENITY, Phase 3 registration-directed clinical trial of sepiapterin in adult and pediatric patients with phenylketonuria (PKU).

“The positive results from the APHENITY trial bring us one step closer to providing a therapy that could deliver meaningful benefit to PKU patients,” said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. “The Phe reductions observed in the placebo-controlled portion of the study are consistent with, and, in some cases, exceed the magnitude of Phe reductions recorded in the open label portion of the study. We look forward to meeting with regulatory authorities to discuss the path to approval.”

The placebo-controlled portion of the study included 98 patients in the primary analysis population. The mean percent Phe reduction in sepiapterin treated patients was 63%. In the subset of classical PKU patients, the mean percent Phe reduction was 69%. Minimal reductions in Phe levels were observed in the placebo treated patients resulting in a highly statistically significant sepiapterin treatment benefit ($p<0.0001$). Sepiapterin was generally well tolerated with no serious adverse events.

Today's Conference Call and Webcast
PTC will hold a conference call at 8 AM EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About APHENITY
APHENITY was a global double-blind, placebo-controlled, registration-directed study which enrolled 156 children and adults with PKU. Participants were randomized to receive sepiapterin or placebo for six weeks with the primary endpoint being reduction in blood phenylalanine levels. The trial consisted of two parts. Part 1 was a run-in phase, during which all screened subjects received sepiapterin for two weeks. Only those subjects who demonstrated a reduction in phenylalanine levels of 15% or more from baseline in Part 1 were randomized to receive either sepiapterin or placebo in Part 2 of the clinical trial. The primary analysis population consists of those who had greater than 30% reduction in phenylalanine levels from baseline during Part 1 of the trial. The primary outcome measure is the reduction of blood phenylalanine levels from baseline compared to Weeks 5 and 6 in patients from Part 2 of the clinical trial. All patients are eligible to enroll in an open label long term clinical trial designed to further evaluate the long-term safety and durable effect of sepiapterin.

About Sepiapterin
Sepiapterin (formerly PTC923) is an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in the metabolism and synthesis of numerous metabolic products. Sepiapterin is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat the broad range of PKU patients.

About Phenylketonuria
Phenylketonuria (PKU) is a rare, inherited metabolic disease, which affects the brain.[1] It is caused by a defect in the gene that helps create the enzyme needed to break down phenylalanine.[1] If left untreated or poorly managed, phenylalanine – an essential amino acid found in all proteins and most foods – can build up to harmful levels in the body. This causes severe and irreversible disabilities, such as permanent

intellectual disability, seizures, delayed development, memory loss, and behavioral and emotional problems.[1] Newborns with phenylketonuria initially don't have any symptoms, but symptoms are usually progressive, and damage caused by toxic levels of phenylalanine in the first few years of life is irreversible.[2,3] Diagnosis of phenylketonuria usually takes place during newborn screening programs.[4] There are an estimated 58,000 people with phenylketonuria globally.

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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Forward-Looking Statement
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this press release, other than statements of historic fact, are forward-looking statements, including statements with respect to the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward -looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to potential regulatory submissions and commercialization of sepiapterin for phenylketonuria, or PKU, and potential development and regulatory milestone payments that PTC may be obligated to make with regards to sepiapterin, expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of sepiapterin for PKU; PTC's scientific approach and general development progress; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-

K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including sepiapterin.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

References:

1. de Groot MJ, Hoeksma M, Blau N, et al. Mol Genet Metab 2010;99:S86–S89.
2. Phenylketonuria (PKU). Available at: https://www.mayoclinic.org/diseases-conditions/phenylketonuria/symptoms-causes/syc-20376302. Accessed October 2021.
3. Blau N, van Spronsen FJ, Levy HL. Lancet 2010;376:1417–1427.
4. Al Hafid N, Christodoulou J. Transl Pediatr 2015;4(4):304–317.

APHENITY
Topline Results

Matthew Klein, M.D.
CEO

May, 2023

Patient L
with PKU

aphenity

PTC THERAPEUTICS

Forward-Looking Statements

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APHENITY Topline Results Demonstrate Clinical and Statistically Significant Benefit

aphe

Achieved primary endpoint in placebo-controlled portion of study with statistically significant ($p<0.0001$) blood phenylalanine (Phe) reduction

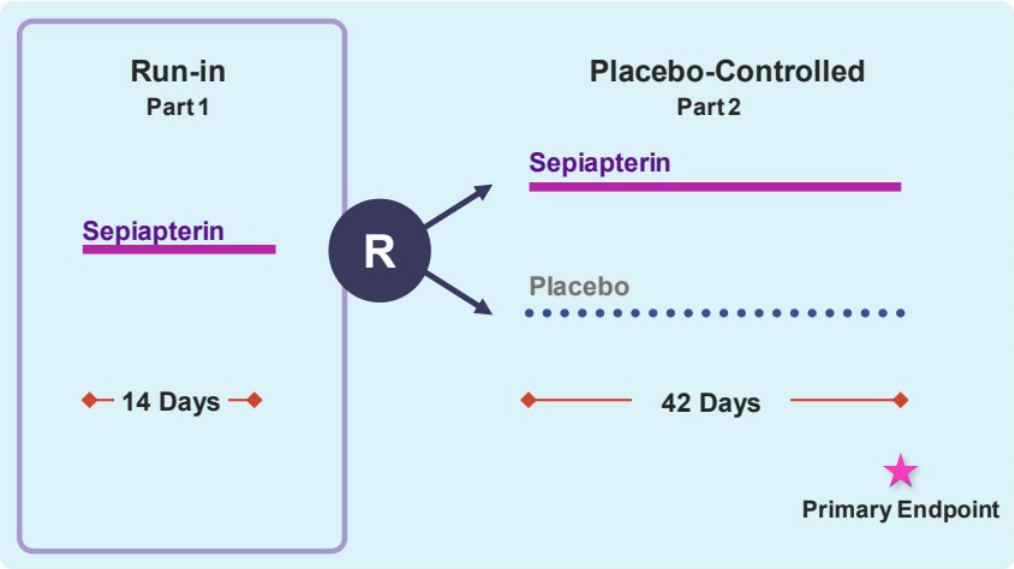
Demonstrated substantial Phe reduction in both the overall primary analysis population (63%) and the subset of classical PKU patients (69%)

Achieved Phe reduction sufficient to bring **84%** of study patients within US guidelines for Phe reduction <360 µmol/L

Well tolerated with no serious adverse events

PTC THERAPEUTICS

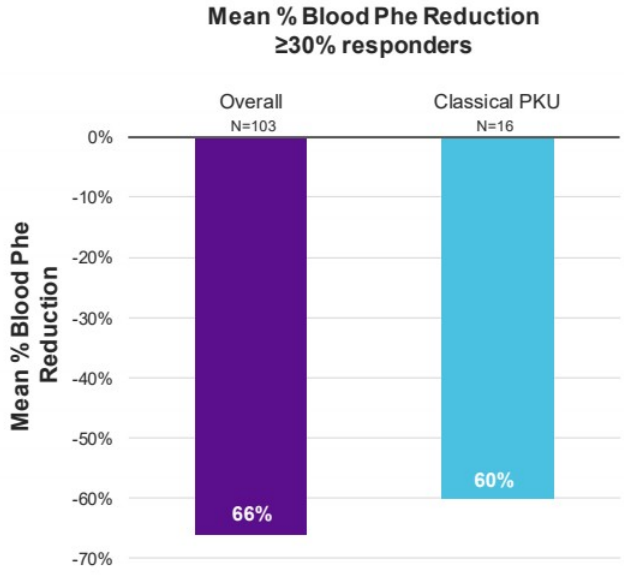
APHENITY Global Registration-Directed Trial of Sepiapterin Study Design

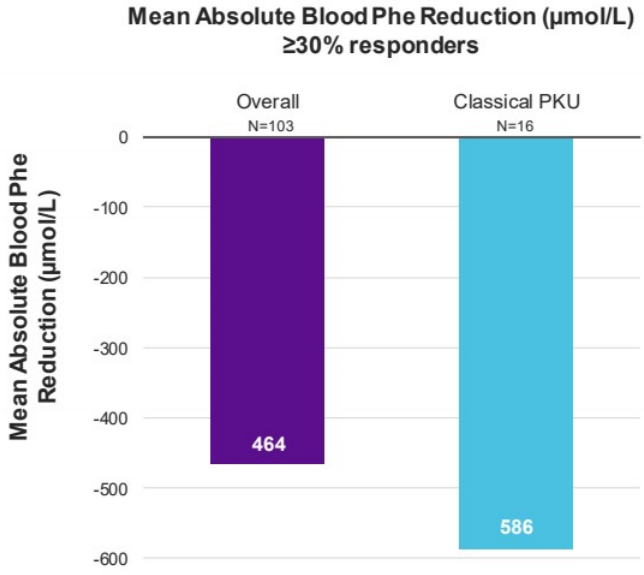


Primary Endpoint

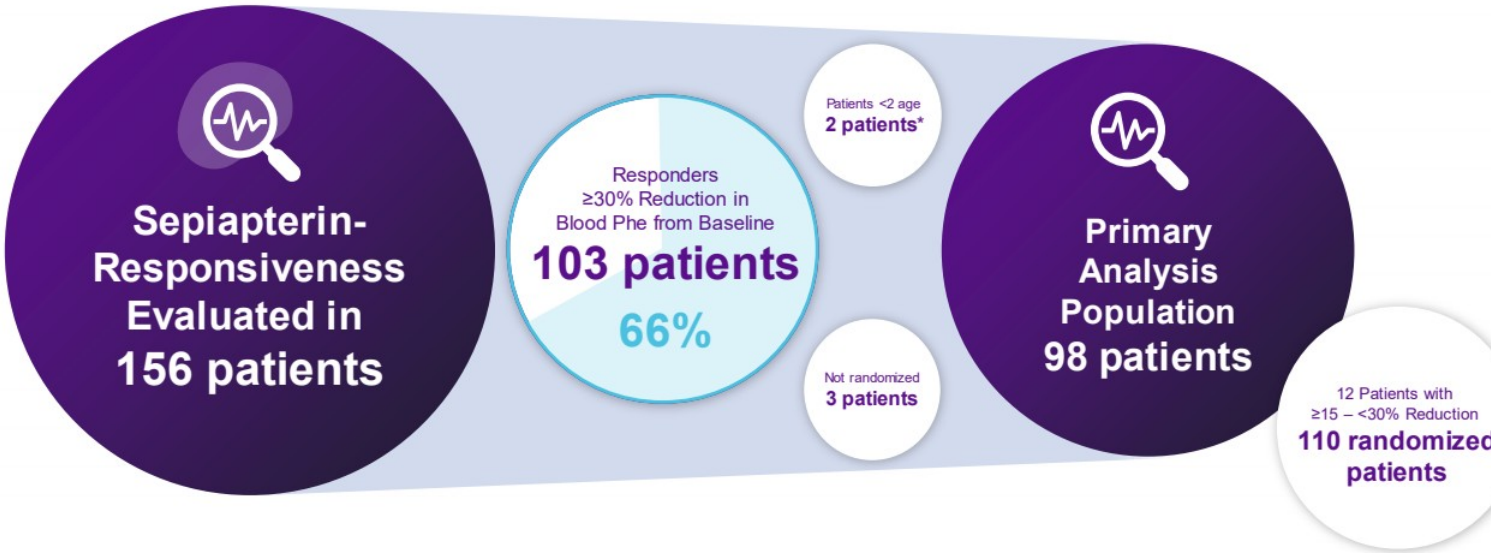
Reduction in blood phenylalanine levels

APHENITY Part 1 Results Demonstrated Marked Blood Phe Reductions



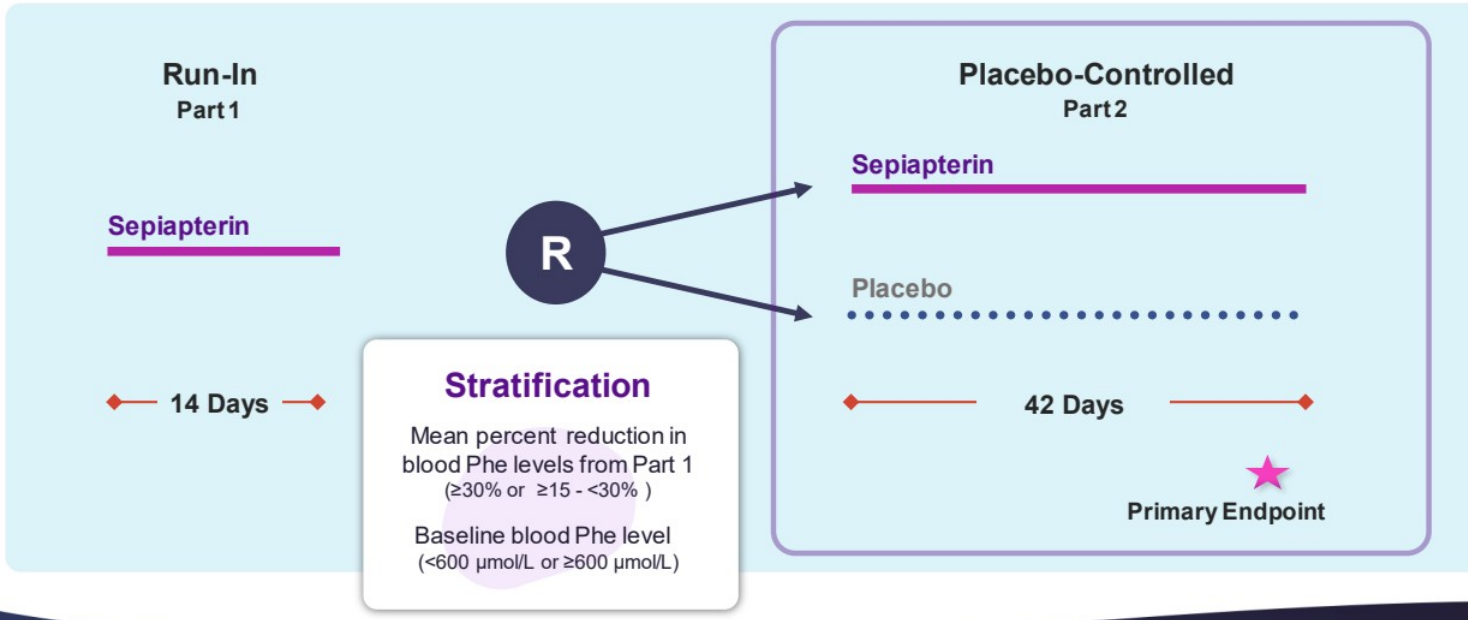


APHENITY Study Population



*As per protocol this population entered directly into open label extension

APHENITY Global Registration-Directed Trial of Sepiapterin Study Design

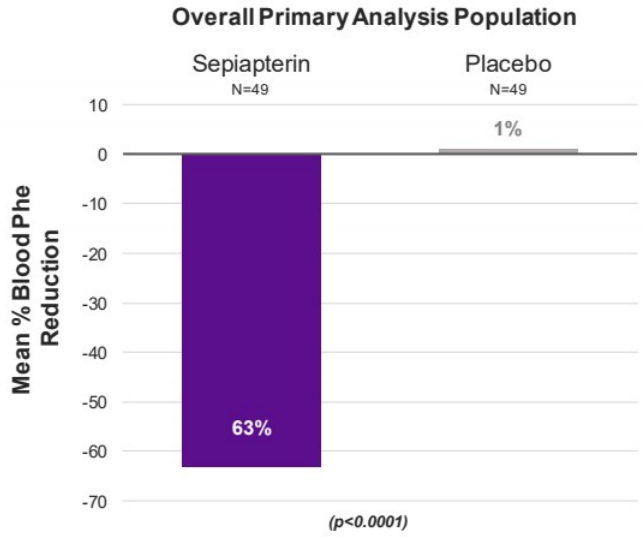


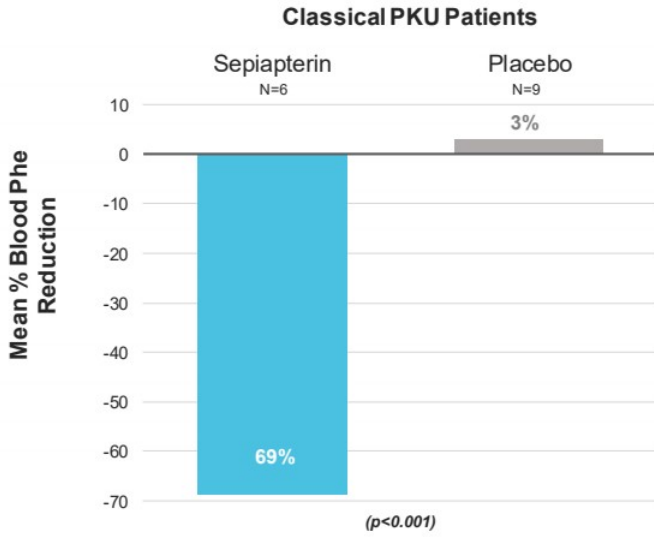
APHENITY Primary Analysis Population Includes Full Spectrum of PKU Patients

Baseline Characteristic	Sepiapterin (N=49)	Placebo (N=49)
Mean age at enrollment (yrs) [min, max]	16.3 [2, 47]	18.1 [4, 54]
2-17 (%)	34 (69.4)	31 (63.3)
≥18 years (%)	15 (30.6)	18 (36.7)
Sex: %F, %M	F: 46.9 M: 53.1	F: 55.1 M: 44.9
Mean Baseline Blood Phe (µmol/L) (min, max)	646.1 (179.5, 1350.0)	654.0 (289.5, 1650.0)
Mean Baseline Blood Phe in Classical PKU (µmol/L) (min, max)	761.25 (452.0, 1350.0)	771.56 (317.0, 1240.0)

Sepiapterin Treatment Resulted in Clinically Significant Blood Phe Reduction

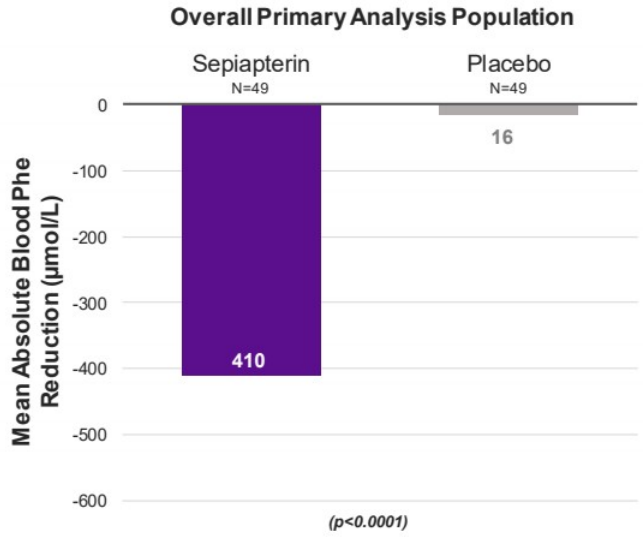
Mean % Blood Phe Reduction

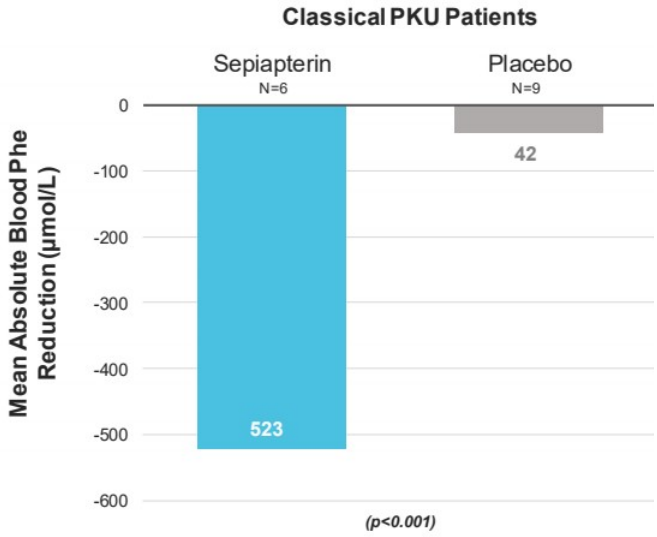




Sepiapterin Treatment Resulted in Clinically Significant Blood Phe Reduction

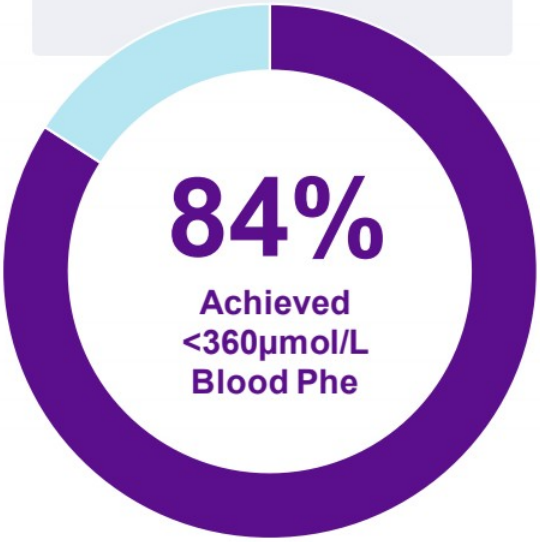
Mean Absolute Blood Phe Reduction (µmol/L)



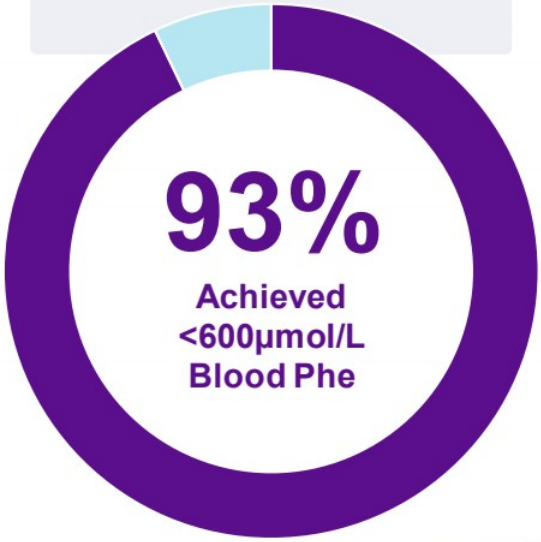


Vast Majority of Patients Achieved Guidelines Target Blood Phe Levels

US guidelines: all ages
EU guidelines: <12 years of age

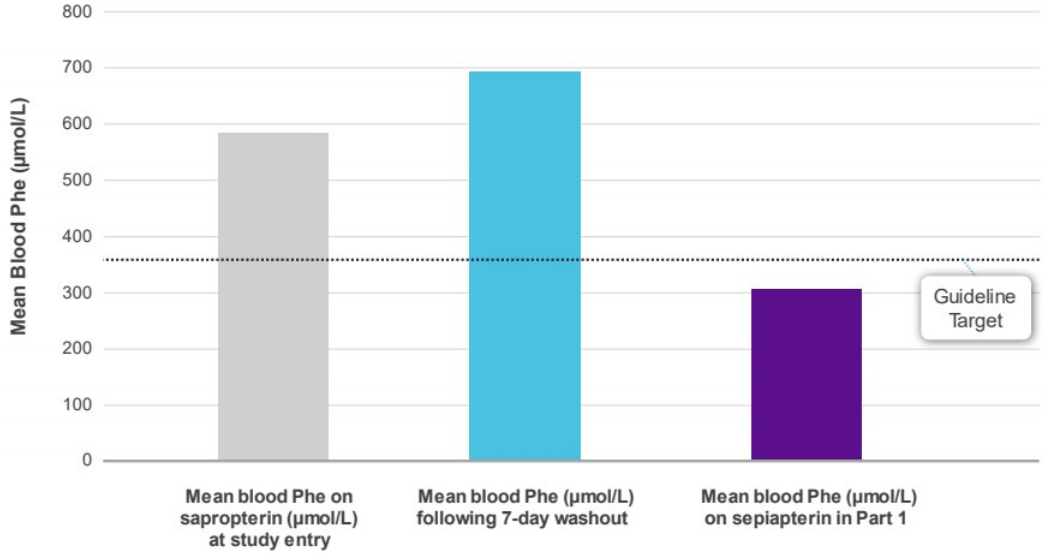


EU guidelines: ≥12 years of age



Sepiapterin Part 1 Treatment Effect in Patients Receiving Sapropterin at Study Entry

(N=27 patients)



48%

lower Phe levels following sepiapterin treatment in those patients receiving sapropterin at study entry

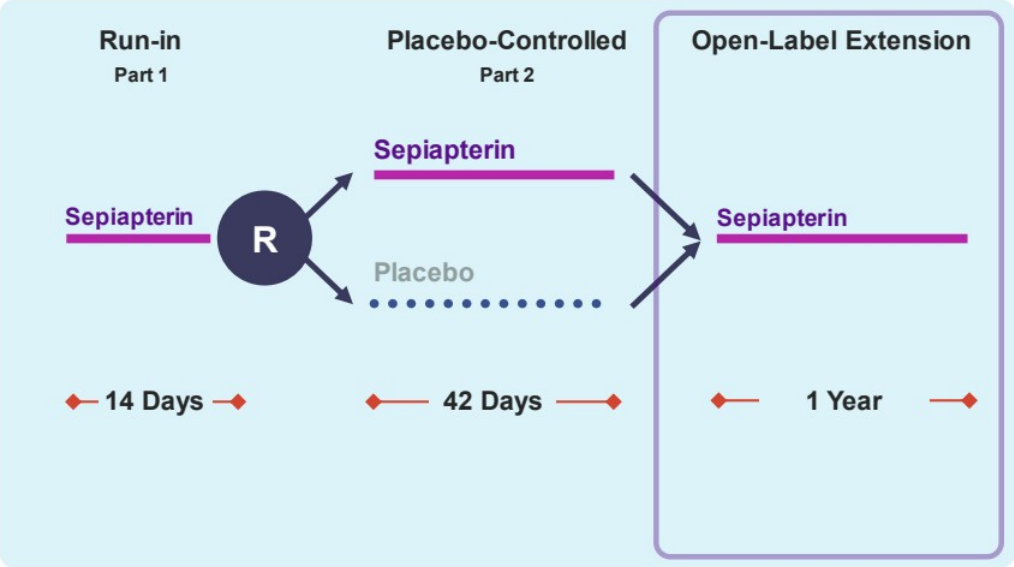
Sepiapterin Demonstrated to be Well Tolerated

Sepiapterin was well tolerated with no serious adverse events

Similar related adverse event frequency between sepiapterin and placebo treatment groups

Most common adverse events were headache and diarrhea

APHENITY Open-Label Extension Assesses Long Term Safety and Phe Tolerance

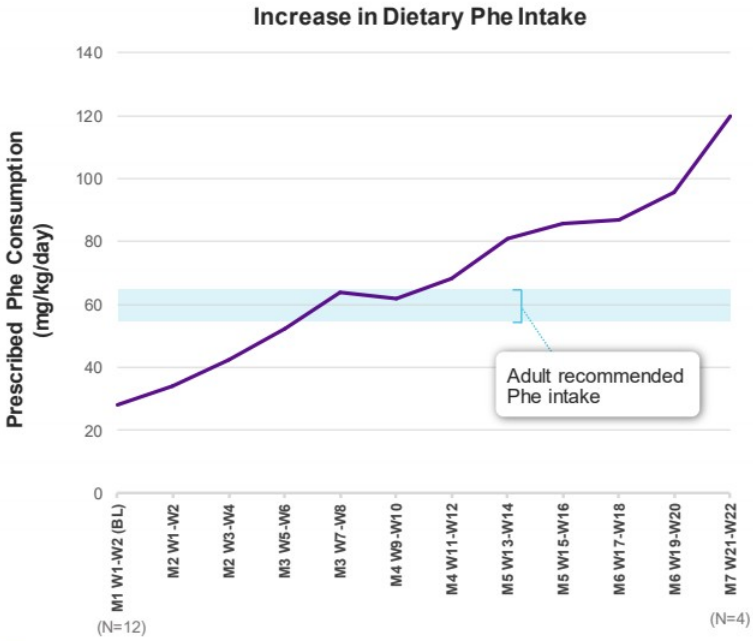


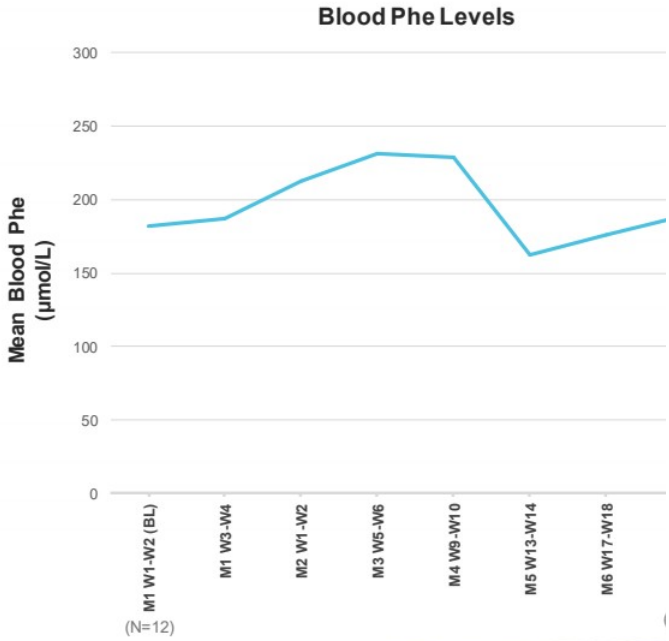
Study Objectives

Long-term safety

Change in dietary Phe/protein consumption

Initial Phe Tolerance Data in Open-Label Extension





Commercial Pillars for Success Already Established

aphe

Newborn screening with ~58,000 patients worldwide[1,2,3]

Well-known metabolic centers of excellence worldwide

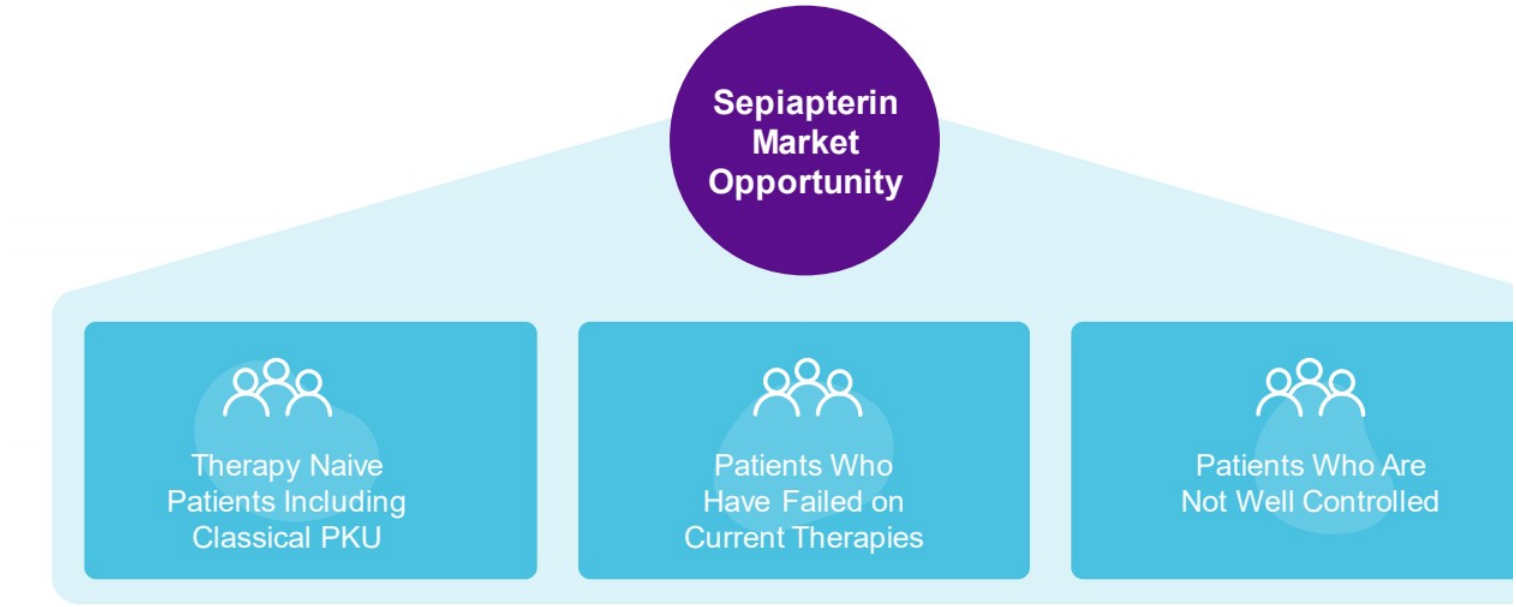
Disease pathology well understood and documented

Connected and coordinated patient advocacy community

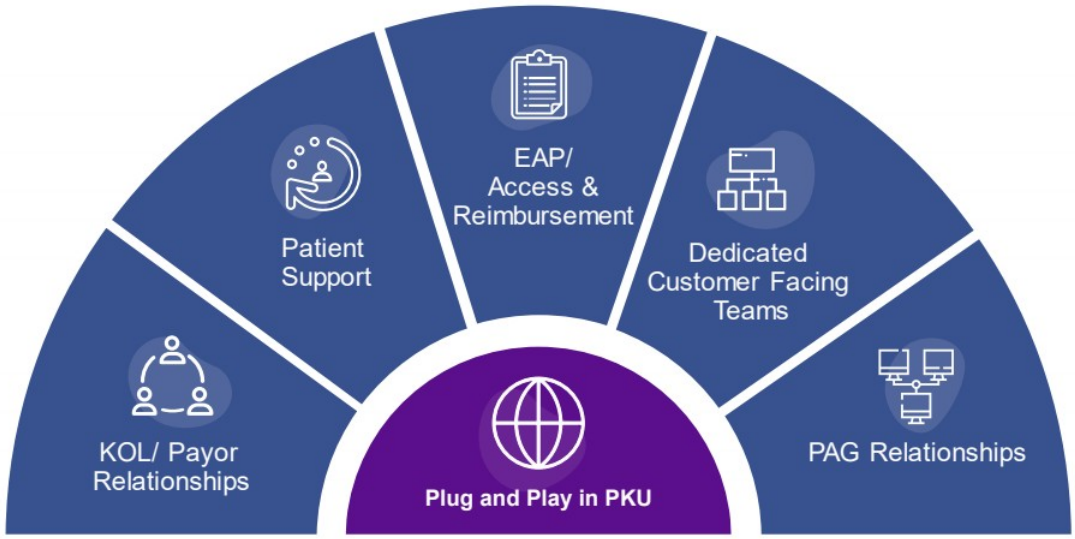
1. NPKUA; https://www.npkua.org/, 2. Shoraka et. Al, Clin Exp Pediatr. 2020; 63(2): 34–43., 3. Borrajo G., Acta Ped Mex. 2012; 33(6): 279-287.

PTC THERAPEUTICS

APHENITY Results Support Potential for Sepiapterin to Address Majority of PKU Segments



PTC Global Commercial Infrastructure Will Allow for Rapid Worldwide Launch



APHENITY Results Support Next Steps in Regulatory Process and Commercial Planning