PTC Therapeutics Announces Orphan Drug Designations of PTC923 for the Treatment of Hyperphenylalaninemia

May 26, 2021

SOUTH PLAINFIELD, N.J., May 26, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that both the United States Food and Drug Administration (FDA) and European Commission (EC) have granted Orphan Drug Designation (ODD) for PTC923 for the treatment of patients with hyperphenylalaninemia. Phenylketonuria (PKU) accounts for 98% of all hyperphenylalaninemia cases and is a metabolic condition that can lead to cognitive disabilities and seizures. 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. PTC expects to initiate a placebo-controlled Phase 3 global registrational trial, APHENITY, for patients with PKU in mid-2021.

"We are very pleased that orphan drug designations have been granted by both the US and European regulatory bodies," said Stuart W. Peltz, Ph.D. Chief Executive Officer PTC Therapeutics. "PKU is a devastating disease that can have severe and irreversible outcomes, including seizures, intellectual disability, behavioral problems and psychiatric disorders. There still remains a high unmet need for more effective and safe treatment options for patients living with PKU."

In the US, ODD is granted by the FDA's Office of Orphan Products Development to promote the development of products that may offer therapeutic benefits for diseases with a prevalence of fewer than 200,000 individuals per year. Orphan drug designation provides opportunities for grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States, if granted FDA approval.

Similarly, the European Medicines Agency's Orphan Medicinal Product Designation is designed to promote the development of drugs that may provide significant benefit for patients suffering from rare, life-threatening diseases. Each indication with an orphan designation confers ten years of market exclusivity for the particular indication in the EU if it maintains orphan designation at the time of marketing authorization.[1]

About Phenylketonuria (PKU)
Phenylketonuria (PKU) is an inborn error of metabolism caused predominantly by mutations in the phenylalanine hydroxylase (*PAH*) gene resulting in toxic buildup of the amino acid phenylalanine (Phe) in the brain. Gene mutations of PAH result in inefficient Phe metabolism leading to hyperphenylalaninemia. There are at least 1,000 unique variations in the *PAH* gene, resulting in phenotypic variation in the amount of enzyme produced and/or enzyme activity. With the near universal adoption of newborn screening for high plasma phenylalanine PKU is typically diagnosed at birth. PKU has been described in all ethnic groups, and its incidence worldwide varies widely, but is estimated that there are 16,500 patients in the U.S. If left untreated, severe and irreversible disability can occur to include permanent intellectual disability, seizures, delayed development, behavioral problems, and possibly psychiatric disorders. It has been shown that administration of tetrahydrobiopterin improves the function of PAH resulting in reduction in phenylalanine plasma concentration. PTC923 has the potential to address the metabolic and neurological signs and symptoms of a broad range of PKU patients.

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 globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

For More Information:

Investors:
Kylie O'Keefe
+1 (908) 300-0691
kokeefe@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

Forward Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: 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; PTC's strategy, future operations, future financial position, future revenues and 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; the enrollment, conduct and results of PTC's PTC923 clinical trial for PKU; 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 PTC's products and product candidates; PTC's scientific approach and general development progress; 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.

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 press release except as required by law.

[1] European Medicines Agency. (2020, May 13). Market exclusivity: Orphan medicines. Retrieved April 19, 2021, from https://www.ema.europa.eu/en/human-regulatory/post-authorisation/orphan-medicines/market-exclusivity-orphan-medicines

View original content:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-orphan-drug-designations-of-ptc923-for-the-treatment-of-hyperphenylalaninemia-301299619.html

SOURCE PTC Therapeutics, Inc.