PTC Therapeutics Successfully Completes Acquisition of Censa Pharmaceuticals

June 1, 2020

SOUTH PLAINFIELD, N.J., June 1, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has successfully completed the acquisition of Censa Pharmaceuticals, Inc., a biopharmaceutical company focused on the development of CNSA-001 (sepiapterin), a clinical-stage investigational therapy for orphan metabolic diseases, including phenylketonuria (PKU) and other diseases associated with defects in the tetrahydrobiopterin (BH4) biochemical pathways diagnosed at birth.

"We're thrilled to complete the acquisition of Censa," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This acquisition adds a well-established late-stage PKU clinical program to our growing rare disease portfolio. Given PTC's established development and commercial capabilities, we're well-positioned to bring this therapy to patients with unmet medical need expeditiously."

With the acquisition, PTC adds CNSA-001 to its robust pipeline of treatments for rare disorders. CNSA-001 has been pursued as a possible treatment for orphan metabolic diseases associated with defects in the tetrahydrobiopterin biochemical pathways. CNSA-001 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. The Phase 2 trial evaluating CNSA-001 for PKU met its primary and secondary endpoints in December 2019[1], and the program is Phase 3 ready.

1 - Bratkovic et al., A Phase 2 Randomized, Double Crossover, Open-label, Active Controlled Study Of CNSA-001 In Patients With Phenylketonuria, American College of Medical Genetics and Genomics (ACMG) Annual Meeting 2020.

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 mutations 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. CNSA-001 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. 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:
Alex Kane
+1 (908) 912-9643
akane@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, other than those of historical fact, contained in this release are forward-looking statements, including statements related to the potential financial

impact and benefits to PTC of its acquisition of Censa, including with respect to the business of Censa and PTC's expectations with respect to contingent payments to the Censa securityholders based on net sales and sublicense fees and the potential achievement of development, regulatory and sales milestones and contingent payments to the Censa securityholders with respect thereto; the future expectations, plans and prospects for PTC; PTC's strategy, future operations, future financial position, future revenues or projected costs; the integration of Censa's operations; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "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: PTC's ability to realize the anticipated benefits of the acquisition, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; significant transaction costs; unknown liabilities; the risk of litigation and/or regulatory actions related to the acquisition; other 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 CNSA-001; the integration of Censa's operations and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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.

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 or product candidate 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.

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