PTC Therapeutics to Acquire Censa Pharmaceuticals

May 6, 2020

- Acquisition diversifies and strengthens PTC Therapeutics' rare disorders portfolio -
- Compelling Phase 3 ready asset to potentially address unmet need in PKU -

SOUTH PLAINFIELD, N.J., May 6, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has entered into an agreement to acquire 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. The transaction was approved by the Boards of both companies.

"Results from a Phase 2 clinical trial of CNSA-001 demonstrated significant and clinically relevant reductions in phenylanaline levels compared to current first-line treatment," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We believe that CNSA-001 has the potential to address the majority of PKU patients whose condition is not adequately managed by current treatments. We look forward to initiating a Phase 3 study in PKU so that patients diagnosed with this devastating condition can have a new oral treatment option as soon as possible."

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.

"I am proud of the team at Censa and its achievements to date demonstrating the potential role of CNSA-001 in treating diseases of the BH4 pathway," said Jonathan Reis M.D., M.B.A., President and Chief Executive Officer of Censa Pharmaceuticals, Inc. "It is the right time to have an excellent fully integrated, patient-focused biotechnology organization like PTC Therapeutics take over the late-stage development of CNSA-001 so that this promising compound becomes available to patients in the near future."

The transaction is expected to close in the second quarter, pending successful fulfillment of closing conditions. PTC is not obligated to give full time employment to any Censa personnel or acquire any additional facilities in connection with the closing.

Transaction Details
Under the terms of the merger agreement, PTC will pay an upfront consideration of approximately $10 million in cash and up to 850,000 shares of PTC common stock. In addition to the upfront payments, potential future consideration includes $217.5 million in development and regulatory milestones for the two most advanced programs and receipt of a priority review voucher, including $30 million to be paid in either cash or PTC common stock at our option for completing the enrollment of a Phase 3 clinical trial for CNSA-001 for PKU, $109 million in development and regulatory milestones for each additional indication of CNSA-001, net sales milestones up to an aggregate amount of $160 million and a contingent value payment of a percentage of annual net sales ranging from single to low double digits.

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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 and 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 Censa Pharmaceuticals
Censa Pharmaceuticals, Inc. is developing CNSA-001, an oral therapy for phenylketonuria (PKU) patients across all ages and disease severities. Censa's CNSA-001 has demonstrated a strong safety profile and clinical benefit in a Phase 2 study that included a comparison to currently available therapy. CNSA-001 is also being studied in other diseases associated with defects in the tetrahydrobiopterin biochemical pathways. Arkin Bio-Ventures is Censa Pharmaceutical's main institutional investor.

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.

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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 PTC's expectations with respect to the closing of its planned acquisition of Censa; the potential financial impact and benefits to PTC of the acquisition, including with respect to the business of Censa to be acquired and PTC's expectations with respect to contingent payments to the Censa securityholders based on net sales 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: satisfaction of the conditions to closing the acquisition (including the possible failure to obtain necessary Censa stockholder and related approvals) in the anticipated timeframe or at all; 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; negative effects of the announcement of the acquisition on the market price of PTC's common stock; 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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