Lisata Therapeutics' Certepetide Granted FDA Orphan Drug Designation for the Treatment of Cholangiocarcinoma

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BASKING RIDGE, N.J., Sept. 05, 2024 (GLOBE NEWSWIRE) -- Lisata Therapeutics, Inc. (Nasdaq: LSTA) ("Lisata" or the "Company"), a clinical-stage pharmaceutical company developing innovative therapies for the treatment of advanced solid tumors and other serious diseases, today announced that the U.S. Food and Drug Administration ("FDA") has granted Orphan Drug Designation to certepetide for the treatment of cholangiocarcinoma.

"Cholangiocarcinoma is a rare and aggressive form of cancer that presents a significant challenge for patients due to limited treatment options, especially after initial therapy," said Kristen K. Buck, M.D., Executive Vice President of R&D and Chief Medical Officer of Lisata. "Receiving Orphan Drug Designation for our investigational product, certepetide, is a pivotal step toward addressing the unmet need for cholangiocarcinoma therapies and providing patients with new, innovative treatment options."

Lisata is currently evaluating certepetide in its BOLSTER trial, a Phase 2a double-blind, placebo-controlled, multi-center, randomized study in the U.S. for the treatment of first- and second-line cholangiocarcinoma. For more information on the trial, please visit https://www.lisata.com/bolster-clinical-trials/.

About Orphan Drug Designation

Orphan Drug Designation is granted by the FDA to drugs or biologics intended to treat a rare disease or condition, defined as one that affects fewer than 200,000 people in the United States. The designation can provide up to seven years of exclusive marketing rights post-approval, including exemption from user fees and eligibility for tax credits for qualified clinical trials. In addition to these financial incentives, it may also shorten clinical development timelines due to closer collaboration with the FDA.

About Cholangiocarcinoma

Cholangiocarcinoma, also known as bile duct cancer, is a cancer that forms in the bile ducts, a network of thin tubes that play a crucial role in digestion. Cholangiocarcinoma is a rare and serious cancer that is difficult to diagnose and often misclassified. According to the American Cancer Society, approximately 8,000 people in the United States are diagnosed with cholangiocarcinoma each year, however, the actual number is likely higher due to diagnostic challenges. The five-year survival rate for cholangiocarcinoma is under 5%, highlighting the urgent need for new and effective treatments.

About Certepetide

Certepetide is an investigational drug designed to selectively activate the C-end rule active transport mechanism in a tumor specific manner, resulting in systemically co-administered anti-cancer drugs more efficiently penetrating and accumulating in the tumor. Additionally, certepetide has been shown to modify the tumor microenvironment, diminishing its immunosuppressive nature and inhibiting the metastatic cascade. Lisata and its collaborators have amassed significant non-clinical data demonstrating enhanced delivery of various existing and emerging anti-cancer therapies, including chemotherapies, immunotherapies and RNA-based therapeutics. To date, certepetide has also demonstrated favorable safety, tolerability, and clinical activity in completed and ongoing clinical trials designed to demonstrate its ability to enhance the effectiveness of standard-of-care chemotherapy for pancreatic cancer as well as the combination of chemotherapy and immunotherapy in a variety of solid tumors. Certepetide has been awarded Fast Track designation (U.S.) and Orphan Drug Designation for pancreatic cancer (U.S. and E.U.) as well as Orphan Drug Designation for glioma, osteosarcoma, and cholangiocarcinoma (U.S.). Additionally, certepetide has received Rare Pediatric Disease Designation for osteosarcoma (U.S.).

About Lisata Therapeutics

Lisata Therapeutics is a clinical-stage pharmaceutical company dedicated to the discovery, development and commercialization of innovative therapies for the treatment of advanced solid tumors and other major diseases. Lisata's product candidate, certepetide, is an investigational drug designed to activate a novel uptake pathway that allows co-administered or tethered anti-cancer drugs to selectively target and penetrate solid tumors more effectively. Lisata has already established noteworthy commercial and R&D partnerships based on its CendR Platform® technology. The Company expects to announce numerous milestones over the next 1.5 years and believes that its projected capital will fund operations into early 2026, encompassing anticipated data milestones from its ongoing and planned clinical trials. For more information on the Company, please visit www.lisata.com.

Forward-Looking Statements

This communication contains "forward-looking statements" that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements, other than statements of historical facts, included in this communication regarding the Company's clinical development programs are forward-looking statements. In addition, when or if used in this communication, the words "may," "could," "should," "anticipate," "believe," "estimate," "expect," "intend," "plan," "predict" and similar expressions and their variants, as they relate to Lisata or its management, may identify forward-looking statements. Examples of forward-looking statements include, but are not limited to, the potential efficacy of certepetide as a treatment for patients with metastatic pancreatic ductal adenocarcinoma and other solid tumors; statements relating to Lisata's continued listing on the Nasdaq Capital Market; expectations regarding the capitalization, resources and ownership structure of Lisata; the approach Lisata is taking to discover and develop novel therapeutics; the adequacy of Lisata's capital to support its future operations and

its ability to successfully initiate and complete clinical trials; and the difficulty in predicting the time and cost of development of Lisata's product candidates. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: results observed from a single patient case study are not necessarily indicative of final results and one or more of the clinical outcomes may materially change following more comprehensive reviews of the data and as more patient data becomes available, including the risk that unconfirmed responses may not ultimately result in confirmed responses to treatment after follow-up evaluations; the risk that product candidates that appeared promising in early research and clinical trials do not demonstrate safety and/or efficacy in larger-scale or later clinical trials; the safety and efficacy of Lisata's product candidates, decisions of regulatory authorities and the timing thereof, the duration and impact of regulatory delays in Lisata's clinical programs, Lisata's ability to finance its operations, the likelihood and timing of the receipt of future milestone and licensing fees, the future success of Lisata's scientific studies, Lisata's ability to successfully develop and commercialize drug candidates, the timing for starting and completing clinical trials, rapid technological change in Lisata's markets, the ability of Lisata to protect its intellectual property rights; and legislative, regulatory, political and economic developments. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in Lisata's Annual Report on Form 10-K filed with the SEC on February 29, 2024, and in other documents filed by Lisata with the Securities and Exchange Commission. Except as required by applicable law, Lisata undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

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