TransCode Therapeutics Announces Orphan Drug Designation Status for TTX-MC138 for Treatment of Pancreatic Cancer

February 28, 2023

BOSTON, Feb. 28, 2023 (GLOBE NEWSWIRE) -- TransCode Therapeutics, Inc. (NASDAQ: RNAZ), the RNA Oncology Company™ committed to more effectively treating cancer using RNA therapeutics, announced today that it has received Orphan Drug Designation (ODD) from the U.S. Food and Drug Administration (FDA) for its lead therapeutic candidate, TTX-MC138, in pancreatic cancer.

Pancreatic cancer is the seventh leading cause of cancer-related deaths worldwide and fourth in the United States. The survival rate of pancreatic cancer is less than 10%, with exocrine pancreatic adenocarcinoma (PDAC), having a 5-year survival rate of only 1% when diagnosed at an advanced inoperable stage, which occurs in 80% of cases. Despite continued research, the prognosis for people with this malignancy has not improved in over 40 years.

“We are pleased to have received Orphan Drug Designation from FDA with our lead therapeutic candidate in pancreatic cancer,” said TransCode’s Chief Executive Officer and Co-founder, Michael Dudley. “This is the second drug in our pipeline to receive such status. In June 2022, FDA granted ODD status for our checkpoint inhibitor, TTX-siPDL1, also for treatment of pancreatic cancer,” added Dudley.

The Company believes that TTX-MC138 has the potential to dramatically improve clinical outcomes in patients with pancreatic cancer. TTX-MC138 is designed to inhibit the pro-metastatic RNA, microRNA-10b, described as the master regulator of metastasis in a number of advanced solid tumors. TransCode believes that TTX-MC138 could be used as a treatment for many of these cancers. In a preclinical model of pancreatic adenocarcinoma, administration of TTX-MC138 as monotherapy resulted in complete responses, manifested as regression without recurrence, in 40% of treated animals. This study comes on the heels of studies in breast cancer demonstrating complete regressions of metastatic disease. In addition to murine models of cancer, TTX-MC138 was successfully delivered and demonstrated preliminary efficacy in a case study of spontaneous feline mammary carcinoma.

Dudley further stated, “ODD status provides several potential benefits including seven years of marketing exclusivity if the designated candidate subsequently receives FDA marketing approval, tax credits for qualified R&D expenses, and an exemption from payment of the Prescription Drug User Fee Amendment (PDUFA) filing fee, a savings estimated to be more than $3 million.”

TransCode Therapeutics recently received approval for a first-in-human clinical trial with TTX-MC138 in patients with advanced solid cancers. In this clinical trial, up to 12 patients will be given a single dose of radiolabeled TTX-MC138 followed by noninvasive positron emission tomography-magnetic resonance imaging (PET-MRI). The trial is intended to quantify the amount of TTX-MC138 delivered to metastatic lesions and the pharmacokinetics of the therapeutic candidate in cancer patients. The trial could yield critical data regarding therapeutic dose, timing, and potential safety that could inform later stage clinical trials and further advance TTX-MC138 as a therapeutic candidate against pancreatic cancer and other advanced malignancies. This trial is not intended to demonstrate any therapeutic effect.

About TransCode Therapeutics

TransCode is an RNA oncology company created on the belief that cancer can be more effectively treated using RNA therapeutics. The Company has created a platform of drug candidates designed to target a variety of tumor types with the objective of significantly improving patient outcomes. The Company’s lead therapeutic candidate, TTX-MC138, is focused on treating metastatic cancer, which is believed to cause approximately 90% of all cancer deaths totaling over nine million per year worldwide. The Company believes that TTX-MC138 has the potential to dramatically improve clinical outcomes in a range of cancers, including breast, pancreatic, ovarian and colon cancer, glioblastomas and others. Two of the Company’s other drug candidates, TTX-siPDL1 and TTX-siLIN28B, focus on treating tumors by targeting PD-L1 and LIN28B, respectively. TransCode also has three cancer-agnostic programs: TTX-RIGA, an RNA–based agonist of the retinoic acid-inducible gene I designed to drive an immune response in the tumor microenvironment; TTX-CRISPR, a CRISPR/Cas9–based therapy platform for the repair or elimination of cancer-causing genes inside tumor cells; and TTX-mRNA, an mRNA-based platform for the development of cancer vaccines designed to activate cytotoxic immune responses against tumor cells.

Forward-Looking Statements

This release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including, without limitation, statements concerning the potential clinical benefits of TTX-MC138, statements concerning the potential benefits of Orphan Drug Designation, statements concerning the occurrence or expected timing and outcome of the Phase 0 clinical trial, including whether this trial will demonstrate proof-of-mechanism, statements concerning the occurrence or the timing and outcome of expected regulatory filings, statements concerning the effects any of our treatments may deliver in the future and whether they will successfully deliver certain outcomes where other treatments have failed, statements concerning preclinical studies and the outcomes of those studies, statements about what early trials may indicate about the effects of any of our drug candidates, statements about TransCode’s goals to advance its drug portfolio, and statements concerning TransCode’s development programs and its TTX technology platform generally. Of note, a Phase 0 clinical trial is an exploratory study, conducted under an exploratory Investigational New Drug (eIND) application. Exploratory IND studies usually involve very limited human exposure to a therapeutic candidate to evaluate mechanism of action in order to inform potential clinical evaluation in future clinical studies, but otherwise have no therapeutic intent. Any forward-looking statements in this press release are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: the risk associated with drug discovery and development; the risk that the results of our planned clinical trials will not be consistent with our pre-clinical studies or expectations; risks associated

with the timing and outcome of TransCode's planned regulatory submissions; risks associated with TransCode's planned clinical trials for its product candidates; risks associated with obtaining, maintaining and protecting intellectual property; risks associated with TransCode's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties; the risk of competition from other companies developing products for similar uses; risks associated with TransCode's financial condition and its need to obtain additional funding to support its business activities, including TransCode's ability to continue as a going concern; risks associated with TransCode's dependence on third parties; and risks associated with the COVID-19 coronavirus. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause TransCode's actual results to differ from those contained in or implied by the forward-looking statements, see the section entitled "Risk Factors" in TransCode's Annual Report on Form 10-K for the period ended December 31, 2021, as well as discussions of potential risks, uncertainties and other important factors in any subsequent TransCode filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release; TransCode undertakes no duty to update this information unless required by law.

For more information, please contact:

TransCode Therapeutics, Inc.
Alan Freidman, VP Investor Relations
alan.freidman@transcodetherapeutics.com