



TransCode Therapeutics Reports Positive Preclinical Results with its Lead Candidate, TTX-MC138, in Pancreatic Adenocarcinoma

October 26, 2022

Study provides additional support for further evaluation of TransCode's lead therapeutic candidate, TTX-MC138, in clinical trials

BOSTON, Oct. 26, 2022 (GLOBE NEWSWIRE) -- **TransCode Therapeutics, Inc.** (Nasdaq: RNAZ), an RNA oncology company committed to more effectively treating cancer using RNA therapeutics, today reported Positive Preclinical Results with its Lead Candidate, TTX-MC138, in Pancreatic Adenocarcinoma.

TransCode Therapeutics evaluated the bioactivity of its lead therapeutic candidate, TTX-MC138, applied as monotherapy, in a murine model of pancreatic adenocarcinoma. In the study, mice bearing human pancreatic tumors implanted in their pancreas were treated once weekly for 10 weeks with TTX-MC138. The drug demonstrated a pharmacodynamic response by successfully inhibiting its target, microRNA-10b (miR-10b), as measured by qRT-PCR, a commonly used assay for measuring gene expression. Serum miR-10b, a possible surrogate biomarker of therapeutic efficacy, was down-regulated by TTX-MC138 in this study. Forty percent of animals treated with TTX-MC138 had complete responses, defined as complete regression of disease and long-term survival without recurrence. Additional studies to better define the optimal dose and therapeutic window in pancreatic cancer are ongoing.

These new findings potentially expand the therapeutic relevance of TTX-MC138 to also include pancreatic adenocarcinoma. "TTX-MC138 has already been shown to induce durable regression of metastatic breast cancer in murine models, so with an outlook toward evaluating other solid tumor types that express miR-10b, our next step was to test the applicability of our therapeutic strategy in a pancreatic cancer model," commented [Zdravka Medarova, PhD](#), co-founder and CTO of TransCode. "As with prior studies, we believe the data from this pancreatic cancer study further support advancement of TTX-MC138 into the clinic. These preclinical data increase our confidence that TTX-MC138 has the potential to become a novel first-in-class therapeutic approach against advanced solid tumors that could ultimately improve clinical outcomes in human patients."

"Demonstrating successful treatment using our first-in-class RNA-targeted therapeutic candidate in a variety of solid tumor models is an important step in the preclinical development process and, we believe, further de-risks our approach," added [Michael Dudley](#), co-founder, president and CEO of TransCode. "As we have previously indicated, we are on track to submit an exploratory Investigational New Drug Application (eIND) this year for our planned Phase 0 first-in-human (FIH) clinical trial with TTX-MC138 in cancer patients with advanced solid tumors."

TTX-MC138 targets microRNA-10b, believed to drive metastatic disease. TTX-MC138 has been validated preclinically in multiple indications and has previously been shown to induce durable regression of metastatic disease in murine models of disseminated breast cancer.

About TransCode Therapeutics

TransCode is an RNA oncology company created on the belief that cancer can be 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 produce regression without recurrence 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 1, or RIG-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 results of a preclinical study of TTX-MC138 in pancreatic adenocarcinoma and other tumor types or expected clinical results of TransCode's therapeutic candidates, statements concerning the results of RNA research, statements concerning the potential for treating cancer with RNA therapeutics, statements concerning the timing and outcome of expected regulatory filings and clinical trials, including the filing of an eIND for the planned first-in-human study of TTX-MC138, statements concerning the timing and outcome of this study, including whether this study will demonstrate proof-of-mechanism, and statements concerning TransCode's development programs and 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 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 year 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.

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