TransCode Therapeutics Reports Third Quarter 2022 Results; Provides Business Update

November 14, 2022

BOSTON, Nov. 14, 2022 (GLOBE NEWSWIRE) -- TransCode Therapeutics, Inc. (NASDAQ: RNAZ), the RNA oncology company committed to more effectively treating cancer using RNA therapeutics, today reported financial results for the third quarter ended September 30, 2022, and recent business progress.

“We continued to advance our therapeutic programs during the third quarter,” said Michael Dudley, co-founder, president and CEO of TransCode. “We remain on track to achieve our planned milestones for 2H of 2022 which include submission of an exploratory Investigational New Drug Application (eIND) this year for our planned Phase 0 clinical trial in up to 12 cancer patients with advanced solid tumors. We continue to make strides in development of our therapeutic candidates in our pipeline, all of which utilize our proprietary TTX delivery platform. These include our immuno-oncology candidates, TTX-siPDL1 and TTX-RIGA, as well as TTX-mRNA, each of which has the potential to treat multiple solid tumor indications.”

“In addition to the progress towards filing our eIND for our First-in-Human (FIH) clinical study, we were granted orphan drug designation by the US Food and Drug Administration (FDA) for TTX-siPDL1 for pancreatic cancer, and we continued preclinical studies with other therapeutic candidates in a variety of tumor indications,” added Dudley.

TransCode co-founder and Chief Technology Officer, Dr. Zdravka Medarova, commented, “We have made substantial progress towards further advancement of our therapeutic candidates, TTX-MC138 and TTX-siPDL1, for the treatment of cancer, including pancreatic adenocarcinoma, which has a dismal prognosis and for which there are few therapeutic options. This focus on cancers associated with a poor prognosis and modestly successful conventional treatment options is driven by the broad applicability of our therapeutic candidates to multiple indications and reflects our philosophy that targeted RNA-based treatments could deliver much needed success where traditional cytotoxic approaches have failed. In addition to our progress on the research front, we have completed eIND-enabling studies to support our upcoming First-in-Human trial with our lead therapeutic candidate, TTX-MC138.”

Commenting on the current environment for biotech company stocks, Dudley noted, “The biotech sector has lost tremendous value starting in the second half of 2021 and continuing in 2022. Many public companies in the sector are trading at or below enterprise value. We firmly believe that our current stock price does not in any way reflect the value we have created since we completed our IPO in July 2021. We continue our R&D efforts to de-risk our therapeutic candidates with the goal of bringing to market cancer therapeutics that we believe could offer longer term survival benefits over current cancer therapeutics, and to build long term value for our shareholders.”

Recent Business Highlights

- In July 2022, TransCode announced expansion of its intellectual property portfolio through the filing of a provisional patent application (US 63/356,449) entitled *RADIOLABELED NANOPARTICLES AND TEMPLATE DIRECTED IMMUNOMODULATION FOR CANCER THERAPY* (the ‘449 application). TransCode expects to convert the ‘449 application into an international application in approximately one year.

- In August 2022, TransCode and The University of Texas MD Anderson Cancer Center (MD Anderson) announced a strategic alliance to advance TransCode’s pipeline of RNA-targeted oncology therapeutic and diagnostic candidates. Through the alliance, TransCode and MD Anderson scientists plan to collaborate on preclinical studies to further validate TransCode’s therapeutic and diagnostic candidates and to expand the reach of TransCode’s discovery engine. The results of these studies will inform future clinical trials with these agents, including trials to be led at MD Anderson.

- In September 2022, TransCode announced the appointment of Frank J. Slack, PhD, to its advisory board. Dr. Slack is the Shields Warren Mallinckrodt Professor, Department of Pathology, and Director of the Harvard Medical School Initiative for RNA Medicine (HIRM), Beth Israel Deaconess Medical Center. Directed by Dr. Slack, the HIRM Initiative seeks to harness the potential of RNA to revolutionize the way cancer and other diseases are treated and diagnosed. Having pioneered pivotal studies proving that microRNAs drive oncogenesis and represent critical targets for the treatment of cancer, his guidance is expected to prove highly valuable as TransCode enters the clinical phase of development.

Subsequent to the end of the third quarter, TransCode reported positive preclinical results with its immunotherapy candidate, TTX-siPDL1, in pancreatic adenocarcinoma. After two weekly treatments of study animals with TTX-siPDL1 combined with the standard-of-care chemotherapeutic, gemcitabine, tumor volumes were 25% of those in untreated animals. By the fifth week of treatment, 75% of animals treated with TTX-siPDL1 plus gemcitabine were still alive versus 25% of those treated with gemcitabine alone. Additionally, immune cell profiling of tumors from the treated animals indicated successful PD-L1 inhibition and immune cell activation, consistent with the known mechanism-of-action (MOA) of checkpoint inhibitors. Because TTX-siPDL1 incorporates a silencing RNA (siRNA) against PD-L1 as its functional component, it has the potential to trigger the degradation and/or translational repression of the PD-L1 messenger RNA (mRNA), preventing the tumor cell from expressing the PD-L1 antigen altogether.

TransCode also recently reported positive preclinical results with its lead candidate, TTX-MC138, in a murine model of pancreatic adenocarcinoma. 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 (40%) of animals treated with TTX-MC138 had complete regression of disease and long-term survival without

recurrence. TransCode believes that, combined, this study and the study with TTX-siPDL1 described above in pancreatic cancer models further demonstrate successful delivery of therapeutic candidates to genetic targets inside tumor cells with our proprietary TTX delivery system.

Planned Milestones

TransCode's goals to continue to advance its portfolio include:

- TTX-MC138
  - Submission to FDA of an eIND application for its First-in-Human Phase 0 clinical trial.
  - Completion of the FIH clinical trial intended to demonstrate quantifiable evidence of delivery of radiolabeled TTX-MC138 to metastatic lesions in advanced solid tumors; measure pharmacokinetics and biodistribution in vital organs and other tissues; potentially inform therapeutic dose levels for future trials based on microdose results; and validate delivery for the TTX pipeline more broadly, potentially opening-up additional relevant RNA targets that have been previously undruggable due to challenges with RNA delivery.
  - Completion of IND-enabling studies to support filing an IND application for a Phase I clinical trial of TTX-MC138.
- Completion of preclinical *in vivo* studies supporting the lead therapeutic candidate, TTX-MC138, in pancreatic cancer and glioblastoma multiforme, among others.
- Completion of preclinical *in vivo* studies supporting therapeutic candidates, TTX-siPDL1, TTX-RIGA, and TTX-siLIN28B.
- Continuation of discussions regarding potential partnerships.
- Filing for orphan drug designation for TTX-MC138 in additional tumor indications including pancreatic cancer.

Third Quarter 2022 Financial Highlights

- Cash was $8.8 million at September 30, 2022, compared to $20.8 million at December 31, 2021, which does not include up to approximately $1.8 million expected from the Small Business Innovation Research (SBIR) grant we were awarded in 2021 comprising approximately $928 thousand available for year two of the award, the current year, and up to $870 thousand related to year three of the award.
- Research and development expenses were $3.0 million in the third quarter of 2022, compared to $993 thousand in the third quarter of 2021.
- General and administrative expenses were $1.9 million in the third quarter of 2022, compared to $1.4 million in the third quarter of 2021.
- Operating loss for the three months ended September 30, 2022, was $4.95 million, compared to an operating loss of $2.36 million in the prior year period.

Financial Guidance

TransCode expects that its cash of $8.8 million at September 30, 2022, along with approximately $928 thousand of additional year-two funding from our SBIR grant award, is sufficient to fund planned operations through the first quarter 2023 but not for a full 12 months from the date of its financial statements.

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 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 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 occurrence or the timing and outcome of expected regulatory filings and clinical trials, including the filing of an eIND for the planned FIH study of TTX-MC138, statements concerning the occurrence or expected timing and outcome of this trial, including whether this trial will demonstrate proof-of-mechanism, 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 orphan drug designation and the possible benefits therefrom, 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, statements concerning our financial condition, financial results, and financial expectations including regarding a SBIR grant, statements concerning the achievement of related milestones and the award of funds thereunder, statements concerning the strategic alliance with MD Anderson Cancer Center and the results that may arise therefrom,

statements concerning TransCode's development programs and its TTX technology platform generally, and statements concerning patents and patent applications and the use of related technologies. 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 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