



TransCode Therapeutics Announces First Patient Dosed in Third Cohort with Lead Candidate in Phase 1 Clinical Trial

January 14, 2025

Follows Safety Review Committee (SRC) approval based on safety data from patients in Cohorts 1 and 2

- No significant safety or dose limiting toxicities reported in Cohorts 1 and 2
- PK data from Cohorts 1 and 2 consistent with preclinical and Phase 0 trial results

BOSTON, Jan. 14, 2025 /PRNewswire/ -- TransCode Therapeutics, Inc. (NASDAQ: RNAZ), the RNA oncology company committed to more effectively treating cancer using RNA therapeutics, today announced that the first patient in Cohort 3 of its Phase 1 clinical trial has been dosed with TTX-MC138, its lead candidate. The Safety Review Committee monitoring the clinical trial unanimously approved opening of the third cohort based on its favorable review of Cohort 1 and 2 safety and pharmacokinetic (PK) data. Additional Cohort 3 patients have been scheduled. The dose administered to patients in the third cohort is approximately double the dose administered to those in the second cohort.



Several patients in the first and second cohort remain on study for continued treatment, receiving additional doses of TTX-MC138. No significant safety or dose limiting toxicities have been reported. Analyses of PK data and pharmacodynamic (PD) activity from Cohorts 1 and 2 is ongoing. To date, the analyses suggest that TTX-MC138 demonstrates a PK/PD profile consistent with preclinical results and results from TransCode's previous Phase 0 clinical trial. Specifically, results from Cohort 1 confirmed the Phase 0 observation that TTX-MC138 shows evidence of pharmacodynamic activity in the presence of high baseline expression of miR-10b, reaching a 66% inhibition at 24 hours after infusion, similar to that seen in the Phase 0 trial. Additionally, TTX-MC138 activity increased with the escalated dose administered in Cohort 2 and was consistent at subsequent administrations of TTX-MC138, suggesting a favorable pharmacokinetic profile.

"An SRC is a group of clinicians and other experts that oversee patient safety during a clinical trial. The SRC determines whether and how a study should proceed, including dose escalation and de-escalation decisions in accordance with the study design. Enrollment into this study continues based on the SRC's cumulative safety data review. We are very pleased with the commitment from our clinical sites which may enable quick completion of the third cohort," commented Sue Duggan, TransCode's Senior Vice President of Operations.

About TTX-MC138

TTX-MC138 is a first-in-class therapeutic candidate designed to inhibit microRNA-10b, or miR-10b, a microRNA widely believed to be critical to the emergence and progression of many metastatic cancers. TransCode's 2023 Phase 0 clinical trial produced evidence of delivery of a radiolabeled version of TTX-MC138 to metastatic lesions and pharmacodynamic activity, even at a microdose of the drug candidate, suggesting a broad therapeutic window for TTX-MC138.

About the Trial

TransCode's Phase 1 clinical trial is a multicenter, open-label, dose-escalation and dose-expansion study designed to generate critical data to support evaluation of the safety and tolerability of TTX-MC138 in patients with a variety of metastatic solid cancers. While not an endpoint, the trial may provide early evidence of clinical activity of TTX-MC138. The trial comprises an initial dose-escalation stage followed by a dose-expansion stage. The primary objective of the dose-escalation stage is to evaluate the safety and tolerability of escalating dose levels of TTX-MC138. In the dose-expansion stage, the safety, tolerability and anti-tumor activity of TTX-MC138 will be further evaluated in certain tumor types selected based on preliminary results from the dose-escalation phase.

Further information is available at www.clinicaltrials.gov NCT Identifier: (NCT06260774).

About TransCode Therapeutics

TransCode is a clinical-stage oncology company focused on treating metastatic disease. The company is committed to defeating cancer through the intelligent design and effective delivery of RNA therapeutics based on its proprietary TTX nanoparticle platform. The company's lead therapeutic candidate, TTX-MC138, is focused on treating metastatic tumors which overexpress microRNA-10b, a unique, well-documented biomarker of metastasis. In addition, TransCode has a portfolio of other first-in-class RNA therapeutic candidates designed to overcome the challenges of RNA delivery and thus unlock therapeutic access to a variety of novel genetic targets that could be relevant to treating a variety of cancers.

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 timing, conduct and results of the Phase 1 clinical trial, statements about microRNAs and their involvement in cancer, and statements concerning the therapeutic potential of TransCode's TTX-MC138 and other therapeutic candidates. 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 risks associated with drug discovery and development; the risk that the results of clinical trials will not be consistent with TransCode's preclinical studies or expectations or with results from previous clinical trials; risks associated with the conduct of clinical trials; 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 the timing and outcome of TransCode's planned regulatory submissions; 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; risks of competition from other companies developing products for similar uses; risks associated with TransCode's dependence on third parties; and risks associated with geopolitical events and pandemics, including the COVID-19 coronavirus and military actions. 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, 2023, 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 this release; TransCode undertakes no duty to update this information unless required by law.

 View original content to download multimedia:<https://www.prnewswire.com/news-releases/transcode-therapeutics-announces-first-patient-dosed-in-third-cohort-with-lead-candidate-in-phase-1-clinical-trial-302350016.html>

SOURCE TransCode Therapeutics, Inc.

For more information, please contact: TransCode Therapeutics, Inc. , Tania Montgomery-Hammon, VP of Business Development , tania.montgomery@transcodetherapeutics.com