

NEWS RELEASE

ReCode Therapeutics Doses First Patient in Phase 1b Clinical Study of RCT2100 for the Treatment of Cystic Fibrosis

2024-09-26

- Phase 1b study will evaluate the safety and tolerability of multiple ascending doses of RCT2100 in people with cystic fibrosis –

MENLO PARK, Calif.--(BUSINESS WIRE)-- ReCode Therapeutics, a clinical-stage genetic medicines company using tissue-specific delivery to power the next wave of mRNA and gene correction therapeutics, today announced that the first patient has been dosed in a Phase 1b study evaluating RCT2100, an investigational inhaled mRNA therapy for people with cystic fibrosis (CF).

CF is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, impacting approximately 130,000 people worldwide. Although recent advancements in CFTR modulator therapies have improved outcomes for many people with CF, those with certain mutations remain underserved because these mutations prevent the production of functional CFTR protein, making current treatments ineffective.

"The initiation of our Phase 1b study of RCT2100 in cystic fibrosis marks a significant milestone for us and is a step closer to offering new hope to those who cannot benefit from existing CF therapies," said Shehnaaz Suliman, M.D., MBA, M.Phil., chief executive officer of ReCode Therapeutics. "We are immensely grateful for the ongoing support and partnership of the CF patient community and are focused on accelerating enrollment to bring this potential new treatment to those in need as quickly as possible."

Formulated using ReCode's proprietary Selective Organ Targeting (SORT) lipid nanoparticle (LNP) platform, RCT2100 is designed to deliver CFTR mRNA directly to target cells in the lungs, instructing them to produce a functional

version of the CFTR protein that is missing in some individuals with CF. By providing this CFTR mRNA, the therapy aims to address the root cause of CF, potentially restoring CFTR protein function in the lungs rather than just managing the symptoms.

ReCode recently received global regulatory authorization to proceed with the RCT2100 Phase 1b study in the United States (U.S.), United Kingdom (UK), and Europe. The study is currently enrolling at sites in the Netherlands, with future expansion planned for France, the U.S. and the UK. For more information, please visit [CF-Clinical-Studies.com](https://www.cf-clinical-studies.com).

About Cystic Fibrosis

Cystic fibrosis (CF) is a progressive genetic disease that causes persistent lung infections and respiratory failure. CF is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. An estimated 105,000 people have been diagnosed with CF across 94 countries, according to the Cystic Fibrosis Foundation. The absence or dysfunction of the CFTR protein results in a defect in airway hydration, which leads to excessive mucus buildup in the lungs. It also creates a mucociliary clearance defect, recurrent infections, inflammation, respiratory failure, and other complications. Despite advancements in CFTR modulator treatments, approximately 10% of the CF community have genetic mutations that do not benefit from these life-changing therapeutics.

About ReCode Therapeutics

ReCode Therapeutics is a clinical-stage genetic medicines company using precision delivery to power the next wave of mRNA and gene correction therapeutics. ReCode's proprietary Selective Organ Targeting (SORT) lipid nanoparticle (LNP) platform enables highly precise and targeted delivery of genetic medicines directly to the organs, tissues, and cells implicated in disease, enabling improved efficacy and potency. ReCode's lead programs include RCT2100 for the treatment of the 10% of cystic fibrosis patients who have genetic mutations in the CFTR gene that do not respond to currently approved CFTR modulators and RCT1100 for the treatment of primary ciliary dyskinesia caused by pathogenic mutations in the DNAI1 gene. RCT1100 and RCT2100 are inhaled disease-modifying mRNA-based therapies formulated using the SORT LNP delivery platform. ReCode is expanding its pipeline to develop potential therapies for other rare and common genetic diseases, including musculoskeletal, central nervous system, liver, and infectious disease indications.

ReCode has been recognized by the San Francisco Business Times and Silicon Valley Business Journal as a Best Place to Work. For more information, visit www.recodetx.com and follow us on [LinkedIn](#) and [Instagram](#).

Investor Contact:

Anne Marie Fields

Precision AQ
annemarie.fields@precisionaq.com
IR@recodeadx.com

Media Contacts:

Erica Jefferson
SVP, Corporate Affairs
ReCode Therapeutics
ejefferson@recodeadx.com
650-629-7965

Tara Cooper
The Grace Group
tara@gracegroup.us
650-303-7306

Source: ReCode Therapeutics