



Maze Therapeutics Announces Phase 1 Trial Initiation Evaluating MZE001 as a Potential Oral Treatment for Pompe Disease

February 17, 2022

SOUTH SAN FRANCISCO, CA., Feb. 17, 2022– Maze Therapeutics, a company translating genetic insights into new precision medicines, today announced the initiation of dosing in the company's Phase 1 clinical trial of MZE001 in healthy volunteers. MZE001, an oral glycogen synthase (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen buildup, is being evaluated for the potential oral treatment of patients with late-onset Pompe disease.

"The initiation of this study is a significant milestone for both Maze and the Pompe community as we advance into the clinic with a potentially disease-modifying treatment for patients," said Sarah Noonberg, M.D., Ph.D., chief medical officer of Maze. "This rapid transition to a clinical-stage company represents the dedication by our team and underscores the broad potential of our Compass platform to accelerate the drug discovery and development process. This study brings us a step closer in our mission to harness the power of genetic insights and translate that into precision medicines for patients with serious diseases."

Pompe disease is a rare, inherited disorder caused by mutations in the gene coding for acid alpha-glucosidase (GAA), which lead to the buildup of glycogen in skeletal muscle, respiratory muscle and cardiac muscle tissues resulting in progressive weakness and respiratory compromise. In preclinical disease models, treatment with MZE001 has demonstrated potent and selective inhibition of GYS1, leading to reduced accumulation of glycogen through a substrate reduction approach. Importantly, treatment in multiple preclinical species was generally well-tolerated with no on- or off-target toxicity observed. Maze recently [presented](#) preclinical data supporting the advancement of MZE001 at the 18th Annual WORLD Symposium.

The Phase 1 trial is a double-blind, placebo-controlled, single ascending dose and multiple ascending

dose study to assess the safety, tolerability, pharmacokinetics and pharmacodynamics, and food effect of MZE001 in healthy volunteers. In addition, target engagement pharmacodynamic biomarkers will be evaluated to provide dose-dependent proof-of-mechanism data.

About Maze Therapeutics

Maze Therapeutics is a biopharmaceutical company applying advanced data science methods in tandem with a robust suite of research and development capabilities to advance a pipeline of novel precision medicines for patients with genetically defined diseases. Maze has developed the Maze Compass™ platform, a proprietary, purpose-built platform that combines human genetic data, functional genomic tools and data science technology to map novel connections between known genes and their influence on susceptibility, timing of onset and rate of disease progression. Using Compass, Maze is building a broad portfolio of wholly owned and partnered programs. Maze is based in South San Francisco. For more information, please visit mazetx.com, or follow us on [LinkedIn](#) and [Twitter](#).

Contacts:

Jillian Connell, Maze Therapeutics

jconnell@mazetx.com

650.850.5080

Media:

Katie Engleman, 1AB

katie@1abmedia.com