



Maze Therapeutics Announces Completion of Phase 1 First-in-Human Trial Evaluating MZE001 as a Potential Oral Treatment for Pompe Disease

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MZE001 was Well-Tolerated and Reduced Glycogen Accumulation in Blood Cells in Healthy Volunteers, Supporting Advancement into Phase 2 Clinical Trial

SOUTH SAN FRANCISCO, CA., Dec. 15, 2022 – Maze Therapeutics, a company translating genetic insights into new precision medicines, today announced the completion of its first-in-human clinical trial designed to evaluate the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD) and food effect 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 Pompe disease.

The double-blind, placebo-controlled, single and multiple ascending dose study enrolled 112 participants. Topline data demonstrated that administration of MZE001 was well-tolerated at single and multiple doses, with a PK profile supporting twice-daily dosing. Importantly, at exposures predicted to be clinically relevant, MZE001 reduced blood cell glycogen, a translatable biomarker for muscle glycogen, in line with preclinical observations. Based on these findings, Maze plans to initiate a Phase 2 clinical trial of MZE001 in 2023. Full results of the MZE001 Phase 1 trial will be presented at the 19th annual WORLD Symposium, being held from February 22 – 26, 2023.

“Through insights generated from our genetically driven Compass Platform, we’ve designed MZE001 to inhibit GYS1 and reduce skeletal and respiratory muscle glycogen synthesis and its subsequent accumulation in patients with Pompe disease,” said Harold Bernstein, M.D., Ph.D., president, research and development and chief medical officer of Maze. “Accumulation in these important muscle groups has proven particularly resistant to treatment with intravenous enzyme replacement therapy, the current standard of care. These topline results give us confidence that GYS1 may be safely inhibited, supporting MZE001’s advancement as the potential first oral therapy, alone or in combination with enzyme replacement, to treat Pompe disease and possibly other glycogen storage disorders. We look forward to carrying this program forward into a Phase 2 clinical trial in patients with Pompe disease who need improved treatment options for their disease.”

About Pompe Disease

Pompe disease is a rare, inherited disorder caused by mutations in the gene coding for acid alphaglucohydrolase (GAA), which lead to the buildup of glycogen in skeletal muscle, respiratory muscle and cardiac muscle tissues resulting in progressive weakness and respiratory compromise.

About MZE001

MZE001 is an oral glycogen synthase (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen buildup. MZE001 is currently being evaluated for the potential oral treatment of patients with Pompe disease and potentially other glycogen storage disorders.

About Maze Therapeutics

Maze Therapeutics is a clinical-stage 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 www.mazetx.com, or follow us on [LinkedIn](#) and [Twitter](#).

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