



Maze Therapeutics Announces FDA Orphan Drug Designation Granted to MZE001 for the Treatment of Pompe Disease

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SOUTH SAN FRANCISCO, CA., Aug. 31, 2022– Maze Therapeutics, a company translating genetic insights into new precision medicines, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to MZE001, the company's investigational product for the treatment of Pompe disease. MZE001 is an oral glycogen synthase (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen buildup through substrate reduction therapy (SRT).

MZE001 is currently being evaluated in a Phase 1, double-blind, placebo-controlled, single ascending dose and multiple ascending dose study to assess its safety, tolerability, pharmacokinetics and pharmacodynamics, and food effect in healthy volunteers. Following the Phase 1 trial readout, which is expected by the end of 2022, Maze plans to initiate a Phase 2 trial in Pompe disease patients in the first half of 2023.

"Pompe disease is a serious and often fatal disorder, and MZE001 has the potential to offer patients an oral, disease-modifying option that improves clinical outcomes and quality of life," said Jason Coloma, Ph.D., chief executive officer of Maze. "We are pleased to have received this designation from the FDA for MZE001, which underscores the need for new, innovative treatments for Pompe disease. MZE001 is a novel mechanism we believe could be beneficial both as a monotherapy for late-onset patients as well as complement to enzyme replacement therapies across the disease spectrum. Our Phase 1 trial in healthy volunteers is advancing well, and we look forward to sharing data generated from the study later this year."

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 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.

Orphan Drug Designation is granted by the FDA to drugs or biologics intended to treat a rare disease or condition, defined as one that affects fewer than 200,000 people in the U.S. Orphan Drug Designation provides certain financial incentives to support clinical development, and the potential for up to seven years of marketing exclusivity for the product for the designated orphan indication in the U.S. if the product is ultimately approved for its designated indication.

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 engine that combines human genetic data, functional genomic tools and data science technology to map novel connections between known genes and their influence on susceptibility to disease, timing of onset and rate of 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](#).

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