Mahzi Therapeutics Announces FDA Rare Pediatric Disease Designation for MZ-1866 Investigational Therapy for Pitt Hopkins Syndrome

MZ-1866 Phase 1/2 UNITE Study is now more than 50% enrolled

SOUTH SAN FRANCISCO, Calif., Aug. 25, 2026 /PRNewswire/ -- Mahzi Therapeutics Inc., a clinical-stage biotechnology company developing precision therapies for neurogenetic disorders, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) to MZ-1866, Mahzi's investigational gene therapy for the treatment of Pitt Hopkins syndrome. The designation was granted by the FDA's Office of Orphan Products Development and Office of Pediatric Therapeutics.

The FDA grants RPDD to therapies intended to treat serious or life-threatening diseases that primarily affect children from birth to 18 years of age. Upon approval of a qualifying marketing application, drugs with RPDD may be eligible for a Priority Review Voucher (PRV). A PRV can be used to obtain priority review of a subsequent marketing application or sold to another sponsor. Recently disclosed PRV sales have ranged from $150-$205 million.

"Receiving Rare Pediatric Disease Designation is a major milestone for the MZ-1866 program and an important recognition of the critical unmet need for Pitt Hopkins patients," said Yael Weiss, M.D., Ph.D., Chief Executive Officer of Mahzi. "We are grateful for the continuing support of the Pitt Hopkins community and the Pitt Hopkins Research Foundation in advancing the MZ-1866 program and the Phase 1/2 UNITE study."

"For families living with Pitt Hopkins syndrome, the absence of any approved treatment has meant managing symptoms with no way to address the underlying cause or to change the course of this disease," added Alex Fay, M.D., Ph.D., Principal Investigator for the MZ-1866 Phase 1/2 study at UCSF Benioff Children's Hospitals. "The MZ-1866 Phase 1/2 trial has now enrolled more than 50% of the planned participants. Meeting this important milestone ahead of schedule reflects both the urgency families feel and the strength of the scientific rationale behind gene replacement for TCF4 deficiency."

The MZ-1866 Phase 1/2 study has enrolled 7 of 12 planned participants and enrollment is projected to be completed by the end of this year.

MZ-1866 was developed in collaboration with the Muotri Lab and licensed from the University of California San Diego. The Phase 1/2 study was made possible by funding from the California Institute for Regenerative Medicine (CIRM), a state of California Agency that funds regenerative medicine, stem cell, and gene therapy research (Grant Numbers TRAN1-13997 and CLIN2-19119).

About the Phase 1/2 UNITE Study
The Phase 1/2 UNITE clinical trial is an open-label study evaluating a single administration of MZ-1866 in participants with genetically confirmed Pitt Hopkins syndrome. The study is designed to enroll 12 participants across 4 sites in the United States and Israel and to evaluate a single dose of MZ-1866 delivered via intracerebroventricular administration. In addition to its primary safety objectives, the trial will evaluate developmental, communication, cognitive, and motor function exploratory endpoints.

Additional information on the Phase 1/2 UNITE study can be found at: https://clinicaltrials.gov/study/NCT07135050

About MZ-1866
MZ-1866 is a novel AAV9-TCF4 gene replacement therapy that aims to address the underlying disease biology of Pitt Hopkins syndrome by providing functional copies of the *TCF4* gene. In addition to Rare Pediatric Disease Designation, MZ-1866 has previously been granted Orphan Drug Designation and Fast Track Designation by the FDA.

About Mahzi Therapeutics
Mahzi Therapeutics is a clinical-stage biotechnology company developing precision therapies for neurogenetic disorders in partnership with patient organizations and academic collaborators. For more information, visit www.mahzi.com.

About the California Institute for Regenerative Medicine (CIRM)

The California Institute for Regenerative Medicine (CIRM) is a state agency created by California voters to accelerate stem cell and gene therapies for people with unmet medical needs. Since 2004, Californians have entrusted CIRM with $8.5 billion to accelerate promising discoveries through clinical trials, train a regenerative medicine workforce, strengthen the state's biotechnology economy, and

expand access to transformative treatments. Today, CIRM is pioneering new models of therapy development and accelerating medical breakthroughs that change lives — in California and around the world. For more information, visit www.cirm.ca.gov.

About the Pitt Hopkins Research Foundation

The Pitt Hopkins Research Foundation (PHRF) is a nonprofit organization dedicated to accelerating research and advancing treatments for Pitt Hopkins syndrome. Working in close partnership with leading scientists, clinicians, and families worldwide, PHRF funds innovative research, supports natural history and clinical studies, and builds the infrastructure needed to translate laboratory discoveries into clinical practice. Through strategic investment, collaborative leadership, and an unwavering commitment to the community it serves, PHRF is driving progress toward meaningful therapies and an eventual cure for Pitt Hopkins syndrome. For more information, visit www.pitthopkins.org.

Forward-Looking Statements
This press release contains forward-looking statements regarding the Company's clinical development programs and future operations. These statements are subject to inherent risks and uncertainties and results may differ materially. The Company assumes no obligation to update these statements except as required by law.

Investor & Media Contact: Aaron Olsen, info@mahzi.com