Treadwell Announces Ocifisertib, a First-in-Class PLK4 Inhibitor, has Received Orphan Designation from U.S. FDA for the Treatment of Acute Myeloid Leukemia

Description

TORONTO & SAN FRANCISCO–(BUSINESS WIRE)–Treadwell Therapeutics, a privately held clinical-stage biotechnology company pioneering and advancing novel first-in-class medicines for unmet needs in cancer, today announced the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ocifisertib (CFI-400945), a first-in-class, investigational PLK4 inhibitor for the treatment of acute myeloid leukemia (AML). Ocifisertib is currently being evaluated in a Phase 1b/2 study in adults with relapsed/refractory AML following standard of care therapy.

Orphan drug designation is granted by the FDA to drugs intended for treatment, prevention or diagnosis of a rare disease or condition that affects fewer than 200,000 people in the U.S. at the time of designation. Under the FDA's Orphan Drug Act, orphan drug status provides incentives, grants, tax credits and waiver of certain administrative fees as well as seven years of market exclusivity following marketing authorization.

Treadwell also announced today the appointment of Brenda Marczi, as SVP and Head of Regulatory Affairs. Brenda comes to Treadwell with over 30 years of regulatory experience in the pharmaceutical and biotechnology industries. She most recently served as Senior Vice President, Regulatory Affairs at Tracon Pharmaceuticals, Inc., where she oversaw their regulatory function and led all of their strategies and filing activities in the US and UK. Brenda holds a Master of Science in Pharmaceutical Sciences from Rutgers University, a PharmD from the University of Maryland, and an MBA from Wharton, University of Pennsylvania.

"The FDA's decision to grant orphan drug designation, along with the previous FDA Fast Track designation for ocifisertib underscores Treadwell's dedication to addressing this patient population with few treatment options. Patients with relapsed and/or refractory AML, in particular *TP53* mutated disease suffer poor overall survival and represents a high unmet clinical need," said Roger Sidhu, M.D., Acting CEO of Treadwell. "We look forward to advancing ocifisertib in partnership with investigators, regulators, patients and their families for those with limited treatment options in tough to treat AML. In addition, we are thrilled to have Brenda join us to lead Regulatory Affairs at Treadwell. She is a seasoned strategic leader with a broad experience in leading regulatory strategy from early development through approval. Her leadership will be invaluable to Treadwell as we advance ocifisertib into potentially pivotal studies in 2025."

About Treadwell Therapeutics

Treadwell Therapeutics is a clinical-stage oncology company developing novel medicines to address unmet needs in patients with cancer. The Company's robust, internally developed clinical pipeline includes CFI-400945 (PLK4 inhibitor), CFI-402257 (TTK/Mps1 inhibitor) and CFI-402411 (HPK1 inhibitor). Treadwell also has a broad pre-clinical pipeline with multiple biologic and next generation TCR based autologous cell therapy programs. For more information, please visit www.treadwelltx.com.

Contact

ir@treadwelltx.com

Date Created
February 20, 2024