Tvardi Therapeutics' TTI-101 Receives Orphan Drug Designation for Hepatocellular Carcinoma

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Houston, TX – April 21, 2022 – Tvardi Therapeutics, Inc. ("Tvardi"), a privately held, clinical-stage biopharmaceutical company focused on the development of STAT3 inhibitors, today announced that its lead product, TTI-101, has been granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the treatment of hepatocellular carcinoma (HCC).

The FDA has the authority to grant ODD to any drug or biological product which shows promise in treating rare diseases or conditions found in fewer than 200,000 people in the U.S. HCC is one such rare disease Tvardi is targeting using TTI-101. HCC is the most common form of liver cancer; however, treatment options for the disease are limited and overall prognosis for survival is poor, with a 5-year survival rate of 18%.

TTI-101 is an orally delivered, small molecule, direct inhibitor of STAT3. STAT3 is a key regulatory protein which plays a critical role in the pathogenesis of HCC by initiating tumorigenesis as well as promoting an immunosuppressive tumor microenvironment.

"We are pleased to receive Orphan Drug Designation for TTI-101 in HCC from the FDA," said Imran Alibhai, PhD, CEO of Tvardi. "This designation builds on the compelling safety and efficacy we have seen in last-line HCC patients in our ongoing Phase 1 trial. We look forward to the impending initiation of our Phase 2 trial in HCC to test TTI-101 as monotherapy as well as in combination."

The ODD will allow Tvardi to receive partial tax credits for qualified HCC clinical trials, benefit from exemptions from user fees for new drug applications and qualify the TTI-101 program for seven years of market exclusivity following FDA approval of TTI-101 as a treatment of HCC.

About Tvardi Therapeutics
Tvardi is a privately held, clinical-stage biopharmaceutical company developing small molecule inhibitors of STAT3, a key regulatory protein positioned at the intersection of many signaling pathways integral to the survival and immune evasion of cancer cells. STAT3 also plays a central role in the pathogenesis of many inflammatory and fibrotic diseases. To learn more, please visit https://tvarditherapeutics.com/.

About TTI-101
Early clinical studies have shown that the company's lead product, TTI-101, is well tolerated and has clinical activity across a broad range of tumors. TTI-101 is currently under investigation in a Phase 1 single agent multicenter clinical trial in patients with advanced solid tumors. For more information on the ongoing clinical trial of TTI-101, please visit clinicaltrials.gov.

Contact
Tvardi Investor Relations
Sara Manning
ir@tvardi.com