Kymera Therapeutics Receives FDA Orphan Drug Designation for KT-253, a Novel, Highly Potent and Selective MDM2 Degrader for the Treatment of Acute Myeloid Leukemia

June 22, 2023

WATERTOWN, Mass., June 22, 2023 (GLOBE NEWSWIRE) -- Kymera Therapeutics, Inc. (NASDAQ: KYMR), a clinical-stage biopharmaceutical company advancing targeted protein degradation to deliver novel small molecule protein degrader medicines, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to KT-253 for the treatment of Acute Myeloid Leukemia (AML).

KT-253 is a highly potent and selective degrader that targets MDM2, the crucial regulator of the most common tumor suppressor, p53. p53 remains intact (wild type) in close to 50% of cancers, meaning that it retains its ability to modulate cancer cell growth. While small molecule inhibitors have been developed to stabilize and upregulate p53 expression, they have been found to induce a feedback loop that increases MDM2 protein levels, which can repress p53 and limit their efficacy. In preclinical studies, KT-253 has shown the ability to overcome the MDM2 feedback loop and rapidly induce cancer cell death, even with brief exposures. Given MDM2 overexpression and amplification is implicated in AML, KT-253 is being explored in this cancer, as well as other liquid and solid tumors.

“This orphan drug designation reinforces the potential of KT-253 to advance the treatment of AML by targeting MDM2, a protein that has been challenging to effectively drug with conventional medicines,” said Nello Mainolfi, Founder, President and CEO, Kymera Therapeutics. “We have a significant opportunity to deliver an important new medicine that acts on this common cancer mechanism, and we look forward to rapidly advancing KT-253 in AML and exploring its potential in other hematological and solid tumors.”

The Phase 1 study initiated in March 2023 will evaluate the safety, tolerability, pharmacokinetics/pharmacodynamics, and clinical activity of KT-253 in patients with relapsed or refractory high grade myeloid malignancies, including AML, acute lymphocytic leukemia (ALL), lymphoma and solid tumors. Patients in the KT-253 Phase 1a dose escalation study will receive IV doses of KT-253 administered once every 3 weeks. The open-label study is intended to identify the recommended Phase 2 dose for KT-253, and is comprised of two arms, with ascending doses of KT-253 in each arm. The first arm will consist of patients with lymphomas and advanced solid tumors and the second arm will consist of patients with high grade myeloid malignancies and ALL.

The FDA's Orphan Drug Designation program provides orphan status to drugs defined as those intended for the treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemptions and seven-year marketing exclusivity upon FDA approval.

About Acute Myeloid Leukemia (AML)
AML is a rapidly growing cancer of the blood and bone marrow characterized by a broad spectrum of cytogenetic and molecular abnormalities resulting in uncontrolled clonal expansion of myeloid blasts cells. In 2019, approximately 70,000 patients had been diagnosed with AML in the United States.

About Kymera Therapeutics
Kymera is a biopharmaceutical company pioneering the field of targeted protein degradation, a transformative approach to address disease targets and pathways inaccessible with conventional therapeutics. Kymera’s Pegasus platform is a powerful drug discovery engine, advancing novel small molecule programs designed to harness the body’s innate protein recycling machinery to degrade dysregulated, disease-causing proteins. With a focus on undrugged nodes in validated pathways, Kymera is advancing a pipeline of novel therapeutic candidates designed to address the most promising targets and provide patients with more effective treatments. Kymera’s initial programs target IRAK4, IRAKIMiD, and STAT3 within the IL-1R/TLR or JAK/STAT pathways, and the MDM2 oncoprotein, providing the opportunity to treat patients with a broad range of immune-inflammatory diseases, hematologic malignancies, and solid tumors.

Founded in 2016, Kymera is headquartered in Watertown, Mass. Kymera has been named a “Fierce 15” company by Fierce Biotech and has been recognized by both the Boston Globe and the Boston Business Journal as one of Boston’s top workplaces. For more information about our people, science, and pipeline, please visit www.kymeratx.com or follow us on Twitter or LinkedIn.

Cautionary Note Regarding Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, implied and express statements by Kymera Therapeutics regarding its: strategy, business plans and objectives for the IRAKIMiD, STAT3, and MDM2 degrader programs; plans and timelines for the preclinical and clinical development of its product candidates, including the therapeutic potential, clinical benefits and safety thereof; expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials. The words "may," "might," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "expect," "estimate," "seek," "predict," "future," "project," "potential," "continue," "target" and similar words or expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks associated with: the impact of COVID-19 on countries or regions in which we have operations or do business, as well as on the timing and anticipated results of our current and future preclinical studies and clinical trials, supply chain, strategy and future operations; the delay of any current and future preclinical studies or clinical trials or the development of Kymera Therapeutics' drug candidates; the risk that the results of current preclinical studies and clinical trials may not be predictive of future results in connection with current or future preclinical and clinical trials, including those for KT-474, KT-333, KT-413 and KT-253; Kymera Therapeutics' ability to successfully demonstrate the safety and efficacy of its drug candidates; the timing and outcome of the Kymera Therapeutics' planned interactions with regulatory authorities; obtaining, maintaining and protecting its intellectual property; and Kymera Therapeutics' relationships with its existing and future collaboration partners. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in the Quarterly Report on Form 10-Q for the quarter ended March 31, 2023 filed on May 4, 2023, as well as discussions of potential risks, uncertainties, and other important factors in Kymera Therapeutics' subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent Kymera Therapeutics' views only as of today and should not be relied upon as representing its views as of any subsequent date. Kymera Therapeutics explicitly disclaims any obligation to update any forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

Investor Contact:

Bruce Jacobs
Chief Financial Officer
investors@kymeratx.com
857-285-5300

Chris Brinzey
Managing Director, Westwicke
chris.brinzey@westwicke.com
339-970-2843

Media Contact:

Todd Cooper
Senior Vice President, Corporate Affairs
media@kymeratx.com
857-285-5300