Nkarta Receives U.S. FDA Orphan Drug Designation for NKX101 for Treatment of Patients with AML

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SOUTH SAN FRANCISCO, Calif., Dec. 16, 2021 (GLOBE NEWSWIRE) -- Nkarta, Inc. (Nasdaq: NKTX), a clinical-stage biopharmaceutical company developing engineered natural killer (NK) cell therapies to treat cancer, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to NKX101 for treatment of acute myeloid leukemia (AML).

NKX101 is a novel investigational NK cell therapy, engineered to augment the innate anti-tumor biology of NKG2D. NKG2D is an activating receptor found on naturally occurring NK cells that triggers the targeted killing of stressed and cancerous cells.

AML is a blood cancer that disrupts the production of normal blood cells in the bone marrow. In patients with AML, the five-year survival rate is 26%. While frontline therapy induces remission, most patients will relapse within 3 years. No standard of care is currently available for patients with relapsed/refractory (r/r) AML. These patients may be treated with various chemotherapeutic approaches, all of which have poor results. Clinical trials have resulted in complete response rates of 12% to 18% and a 3 to 9 month median overall survival in this challenging population.

“This orphan drug designation acknowledges the urgent need for new treatment options for patients with AML,” said Kanya Rajangam, MD, PhD, Chief Medical Officer of Nkarta. “At Nkarta, we are committed to advancing our NK cell therapy platform to develop ground-breaking treatment options for cancer, and we look forward to working with the leukemia community and the FDA to deliver the unique benefits of off-the-shelf cell therapy to AML patients.”

NKX101 is currently being studied in a first-in-human Phase 1 clinical trial in adults with r/r AML or myelodysplastic syndrome (MDS). As previously announced, Nkarta expects to announce initial data from the NKX101 clinical trial in the first half of 2022.

The FDA grants ODD 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. ODD may qualify the company developing 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 NKX101
NKX101 is an investigational, off-the-shelf cancer immunotherapy that uses natural killer (NK) cells derived from the peripheral blood of healthy donors and engineered with membrane-bound IL-15 and a chimeric antigen receptor (CAR) targeting NKG2D ligands on tumor cells. NKG2D, a key activating receptor found on naturally occurring NK cells, induces a cell-killing immune response through the detection of stress ligands that are widely expressed on cancer cells. By engineering NKX101 with the proprietary NKG2D-based CAR, the ability of NK cells to recognize and kill tumor cells in pre-clinical models is increased significantly compared to non-engineered NK cells. The addition of membrane-bound IL15, a proprietary version of a cytokine for activating NK cell growth, has been shown in pre-clinical models to enhance the proliferation, persistence and sustained activity of NK cells. To learn more about the NKX101 clinical trial in adults with AML or MDS, please visit ClinicalTrials.gov.

About Nkarta
Nkarta is a clinical-stage biotechnology company advancing the development of allogeneic, off-the-shelf natural killer (NK) cell therapies for cancer patients. By combining its cell expansion and cryopreservation platform with proprietary cell engineering technologies and CRISPR-based genome engineering capabilities, Nkarta is building a pipeline of future cell therapies engineered for deep anti-tumor activity and intended for broad access in the outpatient treatment setting. For more information, please visit the company’s website at www.nkartatx.com.

Cautionary Note on Forward-Looking Statements
Statements contained in this press release regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Words such as "anticipates," "believes," "expects," "intends," “plans,” “potential,” "projects,” “would” and "future" or similar expressions are intended to identify forward-looking statements. Examples of these forward-looking statements include, but are not limited to, statements concerning Nkarta’s expectations regarding any or all of the following: the anti-tumor activity, benefits, future success, and accessibility of Nkarta’s NK cell therapy candidates, including NKX101 for the treatment of AML; the potential development incentives due to receiving ODD; the timing of initial NKX101 clinical trial data; and Nkarta’s ability to build and advance its platform and a pipeline of cell therapies. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties include, among others: Nkarta’s limited operating history and historical losses; Nkarta’s lack of any products approved for sale and its ability to achieve profitability; Nkarta’s ability to raise additional funding to complete the development and any commercialization of its product candidates; Nkarta’s dependence on the success of its co-lead product candidates, NKX101 and NKX019; that Nkarta may be delayed in initiating, enrolling or completing any clinical trials; competition from third parties that are developing products for similar uses; Nkarta’s ability to obtain, maintain and protect its intellectual property; Nkarta’s dependence on third parties in connection with manufacturing, clinical trials and pre-clinical studies; the complexity of the manufacturing process for CAR NK cell therapies; and risks relating to the impact on our business of the COVID-19 pandemic or similar public health crises.

These and other risks are described more fully in Nkarta’s filings with the Securities and Exchange Commission (“SEC”), including the “Risk Factors” section of Nkarta’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, filed with the SEC on November 10, 2021, and Nkarta’s other documents subsequently filed with or furnished to the SEC. All forward-looking statements contained in this press release speak only as of the date on which they were made. Except to the extent required by law, Nkarta undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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