Ziopharm Oncology Reports on Status of Investigational New Drug Application for Phase 1 Trial to Evaluate CD19-targeted CAR T Therapy

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BOSTON, June 18, 2018 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. (Nasdaq:ZIOP) today announced the U.S. Food and Drug Administration (FDA) placed on clinical hold a Phase 1 trial to evaluate CD19-specific CAR-T therapies manufactured under point-of-care and requested additional information in support of the investigational new drug (IND) application for the trial.

Ziopharm, Precigen, Inc., a wholly-owned subsidiary of Intrexon Corporation (NYSE:XON) and The University of Texas MD Anderson Cancer Center, the IND sponsor, are seeking to conduct a clinical trial to evaluate CAR^+ T cells manufactured with *Sleeping Beauty* technology as an investigational treatment for patients with relapsed or refractory, $CD19^+$ leukemias and lymphomas. CAR^+ T cells very-rapidly manufactured with the *Sleeping Beauty* platform for this third-generation trial are designed to co-express CD19-specific chimeric antigen receptor, or CAR, membrane-bound interleukin 15 and a safety switch. The FDA has requested additional information relative to Chemistry, Manufacturing and Controls. Ziopharm and its partners will address the FDA's requests, and the initiation of this trial may be delayed.

"We know what is needed to address the hold issues and are looking forward to responding to the agency in a timely manner," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "We are undertaking cutting-edge science and are on the verge of a paradigm shift based on our approach to very-rapidly manufacture CD19-specific T cells within two days using our non-viral approach to CAR-T therapy based on the *Sleeping Beauty* platform."

Ziopharm believes this feedback from the FDA does not affect timelines for the Company's planned trial at the National Cancer Institute using the *Sleeping Beauty* platform to target solid tumors infusing TCR-modified T cells. The second-generation clinical trial evaluating *Sleeping Beauty*-manufactured CD19-specific CAR-T cells continues enrolling and infusing patients at MD Anderson.

About Ziopharm Oncology, Inc.
Ziopharm Oncology is a Boston-based biotechnology company focused on the development of next-generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer. In partnership with Precigen Inc., a wholly-owned subsidiary of Intrexon Corporation (NYSE:XON), Ziopharm is focused on the development of two platform technologies designed to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of multiple cancer types: Controlled IL-12 and *Sleeping Beauty* for genetically modifying T cells. The Company's lead asset, Ad-RTS-hIL-12 plus veledimex, has demonstrated in clinical trials the potential to control interleukin-12, leading to an infiltration of T cells that fight brain cancer. The Company also is advancing therapies using *Sleeping Beauty*, a non-viral approach to genetically modify chimeric antigen receptor (CAR^+) and T-cell receptor (TCR^+) T cells, which target specific antigens in blood cancers and neoantigens in solid tumors. *Sleeping Beauty* uses the Company's so-called point-of-care technology, a shortened manufacturing process which potentially can be developed as a decentralized manufacturing process based in hospitals. These programs are being advanced in collaboration with Precigen and with MD Anderson Cancer Center, the National Cancer Institute and Merck KGaA, Darmstadt, Germany.

Forward-Looking Disclaimer
This press release contains certain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts, and in some cases can be identified by terms such as "may," "will," "could," "expects," "plans," "anticipates," and "believes." These statements include, but are not limited to, statements regarding the Company's business and strategic plans, the progress and timing of the development of the Company's research and development programs, including the timing for initiating its first in-human trial using its very rapid manufacturing process and other clinical trials using the *Sleeping Beauty* technology. All such statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied by, the forward-looking statements. These risks and uncertainties include, but are not limited to: the Company's ability to address FDA's clinical hold concerns, the FDA's review of our other *Sleeping Beauty* programs, changes in the Company's financial condition and cash needs, funding or other strategic opportunities that become available to the Company, the Company's ability to finance its operations and business initiatives and obtain funding for such activities; whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, or any of other product candidates will advance further in the preclinical research or clinical trial process and whether and when, if at all, they will receive final approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies and for which indications; whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, and the Company's other therapeutic products it develops will be successfully marketed if approved; the strength and enforceability of the Company's intellectual property rights; competition from other pharmaceutical and biotechnology companies; as well as other risk factors contained in the Company's periodic and interim reports filed from time to time with the Securities and Exchange Commission, including but not limited to, the risks and uncertainties set forth in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2017 and subsequent reports that the Company may file with the Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, and the Company does not undertake any obligation to revise and disseminate forward-looking statements to reflect events or circumstances after the date hereof, or to reflect the occurrence of or non-occurrence of any events.

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