



Ziopharm Oncology Announces FDA Clearance of IND for Rapid Personalized Manufacture of CD19-specific CAR-T

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- Phase 1 clinical trial of CAR-T produced and infused in two days or less from gene transfer using Ziopharm's *Sleeping Beauty* platform –
- Rapid personalized manufacture of CD19-specific CAR-T for investigation in unaddressed patient population with relapsed leukemias and lymphoma after bone marrow transplantation –

BOSTON, Oct. 01, 2019 (GLOBE NEWSWIRE) -- [Ziopharm Oncology, Inc.](#) ("Ziopharm" or "the Company") (Nasdaq:ZIOP), today announced that the U.S. Food and Drug Administration (FDA) has cleared an investigational new drug application (IND) for a phase 1 clinical trial to evaluate CD19-specific CAR-T, produced using a process termed rapid personalized manufacture (RPM), as an investigational treatment for patients with relapsed CD19⁺ leukemias and lymphomas.

The IND clearance builds upon the Company's experience with two prior generations of immunotherapy trials using the *Sleeping Beauty* platform, which it believes is the most clinically-advanced non-viral approach to the genetic modification of T cells. With this third-generation trial, DNA from the *Sleeping Beauty* system is stably inserted into the genome of resting T cells to co-express a chimeric antigen receptor (CAR), membrane-bound IL-15 (mIL15) and a safety switch, which is designed to reduce cost, simplify production, and preserve the therapeutic potential of the T cells.

"There are currently no effective treatment options for patients who relapse soon after allogeneic bone marrow transplantation (BMT), as evidenced by their low rate of remission and poor long-term survival. This trial expands the range of patients with CD19-expressing malignancies that can be treated using the RPM technology," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "We believe RPM is the fastest approach to manufacturing and releasing CD19-specific CAR-T, as T cells from the blood stream are genetically reprogramed with the *Sleeping Beauty* system and then infused within two days of gene transfer. Existing commercial T-cell products using viral-based manufacturing are costly, time consuming to make and complex to deliver. We are now positioned to not only address those issues, but also to treat a patient group that remains underserved by existing therapies."

Up to 24 patients will be enrolled to evaluate infusion of donor-derived RPM CAR-T in patients with CD19⁺ leukemias and lymphomas who have relapsed after allogeneic BMT. This study will be conducted at The University of Texas MD Anderson Cancer Center under an investigator-initiated trial expected to begin later this year.

Research reveals three-year survival for adults with CD19⁺ acute lymphoblastic leukemia after allogeneic BMT ranges from 30% to 65%.¹ For patients with other CD19⁺ cancers, allogeneic BMT can provide a three-year survival rates between 30% to 75%.¹ Few patients experience a durable remission who relapse in the months following allogeneic BMT, regardless of the treatment modality, with some having a median survival of only 2 to 3 months.²

About Ziopharm Oncology, Inc.

Ziopharm Oncology is an immuno-oncology company focused on developing end-to-end cost-effective solutions using its non-viral *Sleeping Beauty* platform for TCR and CAR T-cell therapies and immune-stimulating gene therapy with Controlled interleukin 12 (IL-12). The *Sleeping Beauty* platform genetically modifies T cells with DNA plasmids to express T-cell receptors to target neoantigens inside and outside hotspots for solid tumors and CAR to target CD19 for blood cancers using the Company's rapid personalized manufacturing to produce and release CAR-T within two days of gene transfer. The *Sleeping Beauty* platform is being advanced in collaboration with the National Cancer Institute, The University of Texas MD Anderson Cancer Center and Eden BioCell. The Company also is developing its Controlled IL-12 platform, or Ad-RTS-hIL-12 plus veledimex, as monotherapy and in combination with immune checkpoint inhibitors to treat brain cancer, including in collaboration with Regeneron Pharmaceuticals.

Forward-Looking Statements Disclaimer

This press release contains forward-looking statements as defined in 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 progress and timing of the Company's research and development programs, including the anticipated dates for the initiation of its clinical trials and the Company's expectations regarding the number of patients in its clinical trials. Although Ziopharm's management team believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Ziopharm, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, changes in our operating plans that may impact our cash expenditures, the uncertainties inherent in research and development, future clinical data and analysis, including whether any of Ziopharm's product candidates will advance further in the preclinical research or clinical trial process, including maintaining clearance from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies to conduct clinical trials and whether and when, if at all, they will receive final approval from the U.S. FDA or equivalent foreign regulatory agencies and for which

indication; the strength and enforceability of Ziopharm's intellectual property rights; competition from other pharmaceutical and biotechnology companies as well as risk factors discussed or identified in the public filings with the Securities and Exchange Commission made by Ziopharm, including those risks and uncertainties listed in Ziopharm's Quarterly Report on Form 10-Q filed by Ziopharm with the Securities and Exchange Commission. We are providing this information as of the date of this press release, and Ziopharm does not undertake any obligation to update or revise the information contained in this press release whether as a result of new information, future events or any other reason.

References:

¹ D'Souza A, Fretham C. Current Uses and Outcomes of Hematopoietic Cell Transplantation (HCT): CIBMTR Summary Slides, 2018. Available at <https://www.cibmtr.org>

² Keil F, Prinz E, Kalhs P, *et al.* Treatment of leukemic relapse after allogeneic stem cell transplantation with cytoreductive chemotherapy and/or immunotherapy or second transplants. *Leukemia* 2001; 15:355-361.

Ziopharm Oncology Contact:

Chris Taylor
VP, Investor Relations and Corporate Communications
617-502-1881
ctaylor@ziopharm.com



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