Ziopharm Oncology Reports Third Quarter 2020 Financial Results and Provides Corporate Update

November 5, 2020

– Company plans to file IND for Ziopharm TCR-T program in Q1 of next year for its Library "hotspot" trial –

– Eden BioCell on track for IND filing in Taiwan for autologus CAR-T clinical trial this year based on rapid personalized manufacturing; several patients dosed under compassionate use –

– Three abstracts accepted at Society for Neuro-Oncology, including first clinical data from phase 2 combination clinical trial with Regeneron's Libtayo® –

– Controlled IL-12 receives Rare Pediatric Disease Designantion for DIPG; all three clinical sites active in phase 1/2 pediatric brain tumor trial –

– Strengthens leadership with two new Directors; Populates Scientific Advisory Board; Names former Gilead Executive Adam Levy as EVP, Investor Relations and Corporate Communications –

BOSTON, Nov. 05, 2020 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. ("Ziopharm" or the "Company") (Nasdaq: ZIOP), today announced its financial results for the third quarter ended September 30, 2020 and provided a corporate update. The Company will host a conference call and webcast today at 4:30 pm ET.

"During the third quarter, we again made progress in all three programs and strengthened the Board of Directors, Scientific Advisory Board and Executive Team," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer. "Ziopharm is on track to file an IND for the first TCR-T trial early next year and the NCI is taking steps to begin the phase 2 *Sleeping Beauty* TCR-T trial under their direction. We are pleased by Eden BioCell's steps to submit an IND for the RPM CAR-T trial in Taiwan to infuse T cells the day after gene transfer and heartened by the initial reports we are receiving regarding the first patients dosed by Eden BioCell and partners under compassionate use. Later this month, we will share data from our Controlled IL-12 program at the 2020 Society for Neuro-Oncology Annual Meeting."

Recent Corporate Highlights

Sleeping Beauty TCR-T Program

- **Personalized and Library TCR-T Clinical Trials with MD Anderson Cancer Center.** During the third quarter, Ziopharm further expanded its library of T-cell receptors (TCRs) targeting shared neoantigens in hotspots for use with the *Sleeping Beauty* platform. The Company remains on track to file an Investigational New Drug (IND) application with the FDA in the first quarter of next year, seeking clearance to begin its TCR-T trial utilizing allogeneic TCRs from its library. The Company is working closely with MD Anderson, the initial site for this trial, which is expected to commence mid-2021 to treat patients with gynecologic, colorectal, pancreatic, non-small cell lung and cholangiocarcinoma cancers. The Company continues with its planning for a clinical trial evaluating its Personalized TCR-T platform, which will be initiated at MD Anderson after the Library TCR-T trial.

- **NCI Phase 2 Personalized TCR-T Trial.** This phase 2 study, under the direction of Steven Rosenberg, M.D., Ph.D., Chief of the Surgery Branch at the National Cancer Institute (NCI), and his team, is a first-in-human, non-viral TCR-T study that will enroll patients with a range of solid tumors. The NCI controls the timing of patient treatment for this trial, which is now estimated for next year. During the quarter, data regarding the engineering runs conducted by Ziopharm were successfully transferred to the NCI and the manufacturing process was authenticated by their team and is being validated in their GMP facility. Despite this, the NCI has recently informed Ziopharm that enrollment to the trial will additionally be delayed by regulatory requirements being implemented by the National Institute of Health as well as the impact of COVID-19. The ongoing pandemic led to the depletion of patients available to be treated on TCR-T trials at the NCI due to disease progression while the facility was largely shut down and has added time needed to accrue new patients. As previously mentioned, the NCI has been proactively screening patients for neoantigens and TCRs to render them eligible for the trial (NCT0402436).

Sleeping Beauty CAR-T Program

- **Eden BioCell CAR-T Study.** The Company's joint venture partner, Eden BioCell, has commenced filing of an IND for a

clinical trial in Taiwan to assess patient-derived (autologous) CD19-specific membrane bound IL-15 (mbIL15) CAR-T cells, produced using our Rapid Personalized Manufacturing (RPM) platform. The team expects the filing to be complete before year end, as planned. In addition, Eden BioCell and partners have dosed several patients with relapsed $CD19^+$ malignancies under compassionate use, infusing autologous CAR-T the day after gene transfer per RPM. They report initial data showing the presence of infused T cells, measured weeks after infusion, in peripheral blood and bone marrow. Preliminary observations appear to indicate that mbIL15 supports the manufacturing of CAR-T under RPM which can be safely infused without unexpected toxicities. Additional follow-up is underway in Asia.

- **Ziopharm CAR-T Study.** The Company's clinical collaborators at MD Anderson are actively screening and evaluating patients for enrollment in the phase 1 clinical trial infusing donor-derived (allogeneic) CD19-specific CAR-T therapies produced using RPM. Up to 24 patients with $CD19^+$ leukemias and lymphomas who have relapsed after allogeneic bone marrow transplantation will be enrolled in this investigator-initiated trial (NCT03579888).

Controlled IL-12 Program

- **SNO 2020.** Three abstracts detailing clinical data and observations of Controlled IL-12 have been accepted for presentation at the upcoming 2020 Society for Neuro-Oncology (SNO) virtual annual meeting next month. Updates to be presented on Controlled IL-12 in DIPG (NCT03330197) and combination studies of Controlled IL-12 with Opdivo as a phase 1 trial (NCT03636477) and with Libtayo as a phase 2 trial (NCT04006119) in recurrent glioblastoma (rGBM).

- **Pediatric Trial.** In the Company's phase 1/2 study of Controlled IL-12 for the treatment of pediatric brain tumors, all three clinical sites are now active. The trial is designed to evaluate the safety and tolerability of a single intratumoral injection of Ad-RTS-hIL-12 with up to 14 days of oral veledimex in children with diffuse intrinsic pontine glioma (DIPG). Up to 12 patients with DIPG may be enrolled in phase 1 of the study, which is being conducted at: Lurie Children's Hospital, the Dana-Farber Cancer Institute and the University of California in San Francisco. The FDA recently granted a Rare Pediatric Disease Designation to Controlled IL-12 for the investigational treatment of DIPG.

Operational

- **Expanded Board of Directors.** During the third quarter, Ziopharm announced the appointment of two new members of the Company's Board of Directors. Biotech entrepreneur James Huang, Managing Partner at Kleiner Perkins Caufield & Byers China, was added to the Board in July, and in September, Kevin Buchi, biotech industry veteran and former CEO of Cephalon joined the Board. Directors will continue to actively review the Board membership, in coordination with a retained national search firm, to ensure the skills and experience of directors support the progress and future prospects of the business.

- **Scientific Advisory Board.** Following the appointment of immunotherapy pioneer, Carl June, M.D., as Chairman of Ziopharm's Scientific Advisory Board (SAB), the Company announced population of the SAB in September with the addition of Adi Barzel, Ph.D., Gavin Dunn, M.D., Ph.D., Matthew Porteus, M.D., Ph.D., and Kole Roybal, Ph.D. The SAB will provide strategic counsel to guide the efficient development of Ziopharm's innovative technologies and pipeline of immunotherapies.

- **Executive Leadership.** Ziopharm is pleased to welcome former Gilead executive Adam Levy, Ph.D., M.B.A., as EVP, Investor Relations and Corporate Communications. Most recently, Dr. Levy was Executive Director and Head, Corporate Strategy and Investor Relations for Gilead Sciences. Previously, Dr. Levy was VP, Corporate Strategy for Alexion and Executive Director, Corporate Strategy for Bristol-Myers Squibb. He had prior leadership positions with Novartis and McKinsey & Company. Adam holds a Ph.D. in Molecular Biology from the University of Illinois and an MBA in Finance and Strategy from Northwestern University Kellogg School of Management.

Third Quarter 2020 Financial Results

- Research and development expenses were $14.0 million for the third quarter of 2020, compared to $8.6 million for the third quarter of 2019, primarily reflecting increased manufacturing activity and headcount.

- General and administrative expenses were $6.4 million for the third quarter of 2020, compared to $4.8 million for the third quarter of 2019. The increase in general and administrative expenses for the third quarter of 2020 is primarily due to increased headcount, legal costs associated with its expanded patent portfolio and facility costs.

- Net loss for the third quarter of 2020, was $20.3 million, or $(0.10) per share, compared to a net loss of $74.0 million, or $(0.43) per share, for the third quarter of 2019 (which reflected a $60.8 million, $(0.36) per share, non-cash charge for an inducement warrant).

- Cash and cash equivalents, as of September 30, 2020 were $135.5 million.

- Additionally, a prepayment of approximately $11.4 million remains for work to be conducted by the Company at MD Anderson under the Company's research and development agreements.

Conference Call and Webcast
Ziopharm will host a conference call and webcast for the investment community today, November 5, 2020, at 4:30 p.m. ET. The conference call can be accessed by dialing 1-800-920-9723 (U.S. and Canada) or 1-212-231-2932 (international). The passcode for the conference call is 21971110. To access the live webcast or the subsequent archived recording, click here or visit the "Investors" section of the Ziopharm website at www.ziopharm.com. The webcast will be recorded and available for replay on the company's website for two weeks.

About Ziopharm Oncology, Inc.
Ziopharm is developing non-viral and cytokine-driven cell and gene therapies that weaponize the body's immune system to treat the millions of people globally diagnosed with a solid tumor each year. With its multiplatform approach, Ziopharm is at the forefront of immuno-oncology with a goal to treat any type of solid tumor. Ziopharm's pipeline is built for commercially scalable, cost effective T-cell receptor T-cell therapies based on its non-viral *Sleeping Beauty* gene transfer platform, a precisely controlled IL-12 gene therapy, and rapidly manufactured *Sleeping Beauty*-enabled CD19-specific CAR-T program. The Company has clinical and strategic partnerships with the National Cancer Institute, The University of Texas MD Anderson Cancer Center and others. For more information, please visit www.ziopharm.com.

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 Company's business and strategic plans and the progress, design and timing of the Company's research and development programs, including the anticipated dates for the clearance of the IND for its TCR-T clinical trial and the submission of the IND by Eden BioCell, timing for the treatment of patients in the NCI's clinical trial, enrollment expectations for its CAR-T and DIPG clinical trials, and the timing for the data readouts for its Controlled IL-12 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 NCI's ability to complete the requirements prior to treating patients, 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 receiving 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. In addition, the extent to which the COVID-19 pandemic impacts the Company's business, clinical development and regulatory efforts and the value of its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements, and the effectiveness of actions taken globally to contain and treat the disease. The global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic could have a material adverse effect on the Company's business, financial condition, results of operations and growth prospects. 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.

Investor Relations Contacts:
Ziopharm Oncology:
Chris Taylor
VP, Investor Relations and Corporate Communications
T: 617.502.1881
E: ctaylor@ziopharm.com

LifeSci Advisors:
Mike Moyer
Managing Director
T: 617.308.4306
E: mmoyer@lifesciadvisors.com

Media Relations Contact:
LifeSci Communications:
Patrick Bursey
T: 646.876.4932
E: pbursey@lifescicomms.com

– Financial Tables Follow –

ZIOPHARM Oncology, Inc.
Statements of Operations
(in thousands except share and per share data)
(unaudited)

	Three Months Ended September 30, (unaudited)	
	2020	2019
Operating expenses:		
Research and development	$ 13,968	$ 8,641
General and administrative	6,353	4,807
Total operating expenses	20,321	13,448
Loss from operations	(20,321)	(13,448)
Other income, net	6	203
Noncash inducement warrant expense	-	(60,751)
Net loss	(20,315)	(73,996)
Basic and diluted net loss per share	$ (0.10)	$ (0.43)
Weighted average common shares outstanding used to compute basic and diluted net loss per share	212,837,367	170,613,712

ZIOPHARM Oncology, Inc.
Balance Sheet Data
(in thousands)
(unaudited)

	September 30, 2020	December 31, 2019
Cash and cash equivalents	135,471	79,741
Working capital	135,750	92,966
Total assets	165,894	109,114
Total stockholders' equity	144,918	95,010

Source: ZIOPHARM Oncology Inc