Ziopharm Oncology Reports Fourth Quarter and Full Year 2018 Financial Results and Provides Corporate Update

March 5, 2019

- Phase 1 Trial of Sleeping Beauty*-TCR-T cell therapy for patients with solid tumors to begin at National Cancer Institute in mid-2019 –*
- Third-generation Sleeping Beauty *CD19-specific CAR-T U.S. trial to begin in 2H2019 –*
- Eden BioCell to advance third-generation Sleeping Beauty *CAR-T for Greater China –*
- Third cohort enrolling patients in Controlled IL-12 combination trial with OPDIVO® for recurrent glioblastoma (rGBM) –
- Phase 2 trial of Controlled IL-12 in combination with Regeneron's Libtayo ® for patients with rGBM expected to open 2Q2019 –
- Company to host conference call today at 4:30 p.m. ET –

BOSTON, March 05, 2019 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. (Nasdaq:ZIOP) today announced its financial results for the fourth quarter and year ended December 31, 2018, and provided an update on the Company's recent activities.

"We are executing on our strategy to focus on solid tumors with our *Sleeping Beauty* TCR-T program and our Controlled IL-12 platform and advancing a solution that addresses the cost and complexity of CAR-T therapies," said Laurence Cooper, M.D., Ph.D., CEO of Ziopharm. "Under our collaboration at the National Cancer Institute (NCI), we expect to begin treating patients with solid tumors mid-year with the first non-viral, neoantigen-specific TCR-T cell therapy designed to attack the very mutations that cause cancer. In addition, we are advancing - in both the United States and greater China - our non-viral CAR-T therapy to solve the issues standing in the way of commercial success for approved CD19-specific CAR-T therapies. And, with maturing data showing a positive effect on overall survival for patients with recurrent glioblastoma, there is growing excitement for our Controlled IL-12 platform among treating physicians."

David Mauney, M.D., President of Ziopharm, added, "We have significant momentum following our transformational fourth quarter 2018 when we established a new license agreement that provides us with clinical development autonomy. We secured two new business development deals and strengthened our balance sheet by eliminating $157 million in preferred stock and raising $50 million in a private placement. Thus, we are now well positioned to achieve multiple milestones and to be in the clinic in 2019 with each of our pillar programs: TCR-T, CAR-T and Controlled IL-12."

Program Updates

Sleeping Beauty TCR-T Therapies

The Company is using its *Sleeping Beauty* platform to develop a personalized T-cell therapy targeting solid tumors with T-cell receptors or TCRs. Under a Cooperative Research and Development Agreement (CRADA), the NCI is expected to initiate a Phase 1 clinical trial to treat patients who may have one of a variety of solid tumors using the *Sleeping Beauty* platform to genetically modify T-cells to target patient-specific neoantigens.

- **Phase 1 trial for TCR-T cell therapy expected to begin in mid-2019**: This trial is on track to begin treating patients in mid-2019 under the direction of Steven A. Rosenberg, M.D., Ph.D., Chief of the Surgery Branch at the NCI.
- **CRADA extended**: Ziopharm announces that the Company and the NCI agreed to extend the CRADA governing this program to evaluate genetically modified T cells targeting solid tumors, which was established in January 2017. This agreement has been extended through January 2022.

Sleeping Beauty CAR-T Therapies

Ziopharm is advancing the *Sleeping Beauty* platform towards the very rapid manufacturing of genetically modified CAR^+ T cells, co-expressing membrane-bound interleukin-15, or mbIL15, with a safety switch, within two days after genetically modifying T cells from the patient. This work is being done **in collaboration with the University of Texas MD Anderson Cancer Center in the United States and will be done in Greater China through a joint venture, Eden BioCell.**

- **Third-generation Phase 1 trial for very rapid manufacturing of *Sleeping Beauty* CD19-specific CAR-T with mbIL15 expected to begin 2H2019:** The Company affirms guidance on beginning this trial and treating patients at MD Anderson Cancer Center in the second half of this year. Ziopharm announced in June 2018 that the FDA placed this investigator-led IND on clinical hold and requested additional information relating to chemistry, manufacturing and controls, specifically requesting that the product meet a minimum threshold for T-cell viability. The Company, in partnership with MD Anderson Cancer Center, has made progress toward achieving this threshold in manufacturing through improved engineering and cell processing.

- **Eden BioCell to advance third-generation *Sleeping Beauty* CAR-T for Greater China**: In December 2018, Ziopharm in conjunction with TriArm Therapeutics announced Eden BioCell will be launched to develop and commercialize *Sleeping Beauty*-generated CD19-specific CAR-T therapies in the People's Republic of China (including Macau and Hong Kong), Taiwan and Korea. The teams have begun meeting to prepare for technology transfer and launch the new company. Ziopharm and TriArm each will own 50 percent of the joint venture. Eden BioCell will be funded with up to $35 million from TriArm, a privately-owned cell therapy company that was formed by Panacea Venture Healthcare, a fund co-founded and managed by James Huang, Managing Partner of Kleiner Perkins Caufield & Byers China. Ziopharm and TriArm expect to close on this joint venture in the first half of 2019. Ziopharm's CEO Laurence Cooper, and Panacea Venture Healthcare co-founder James Huang will serve on Eden BioCell's Board of Directors with each party sharing decision-making authority. Ziopharm looks forward to providing an update on clinical development plans for Eden BioCell later in the year.

Controlled IL-12

Ziopharm is developing its Controlled IL-12 platform, or Ad-RTS-hIL-12 plus veledimex, as a drug to control the production of human interleukin 12 (hIL-12) which activates the immune system to recruit cancer-fighting T cells into tumors. In the setting for the treatment of recurrent glioblastoma (rGBM), Ziopharm is advancing Ad-RTS-hIL-12 plus veledimex as a monotherapy and in combination with immune checkpoint inhibitors.

- **Enrollment completed in Phase 1 monotherapy expansion substudy:** Ziopharm announced that it rapidly completed enrollment and treated a total of 36 patients in less than six months in a substudy to expand a Phase 1 trial evaluating its Controlled IL-12 platform as a monotherapy for the treatment of rGBM. The trial was over enrolled by eleven patients more than the target goal of 25, which the Company attributes to enthusiasm stemming from encouraging survival and tumor biopsy data. Preliminary data from this substudy is expected to be presented in 2019.
- **Third cohort has begun in combination substudy with OPDIVO® (nivolumab):** Ziopharm today announced that it has completed two dosing cohorts in its Phase 1 substudy of adult patients with rGBM to evaluate a single dose of Ad-RTS-hIL-12 plus daily veledimex in combination with OPDIVO® (nivolumab), an immune checkpoint inhibitor targeting programmed death-1 (PD-1). The Company has begun the third cohort for this study to evaluate the safety and tolerability of this combination regimen, establish optimal dosing of veledimex and nivolumab, and measure overall patient survival. The Company expects to complete enrollment in the third cohort in the second quarter this year and looks forward to presenting preliminary data from this trial in 2019.
- **Phase 2 combination trial with Regeneron's Libtayo ® (cemiplimab-rwlc) expected to open 2Q2019**: The Company, in collaboration with Regeneron Pharmaceuticals, expects to open a Phase 2 trial to evaluate Ad-RTS-hIL-12 plus veledimex in combination with Regeneron's PD-1 antibody Libtayo ® (cemiplimab-rwlc) for treating patients with rGBM. The Company expects to enroll approximately 30 patients in this trial.
- **Phase 1 for pediatric tumors ongoing**: Ziopharm is enrolling pediatric patients in its Phase 1 trial of Ad-RTS-hIL-12 with veledimex for the treatment of brain tumors at multiple U.S. sites.

Corporate Update

In addition to the clinical collaboration with Regeneron and the execution of an agreement to launch Eden BioCell during the fourth quarter of last year, Ziopharm on October 9, 2018, announced that it entered into a new licensing agreement that replaced all existing agreements with Intrexon Corp. and its subsidiary Precigen, Inc. Under the new license agreement, Ziopharm has full developmental control and exclusivity utilizing *Sleeping Beauty* for TCRs for the treatment of cancer. The CRADA with the NCI related to *Sleeping Beauty*-generated T cells expressing TCRs to target neoantigens within solid tumors was transferred to Ziopharm and the Company will maintain this program. Ziopharm will build on its Controlled IL-12 platform with exclusive access to Precigen's RheoSwitch Therapeutic System ® gene switch with adenovirus for the treatment of cancer. Using the *Sleeping Beauty* system, Ziopharm will continue to exclusively advance its CD19-specific chimeric antigen receptor (CAR) program leveraging membrane-bound interleukin 15, while retaining rights to a second, unnamed CAR target. Ziopharm has sole oversight for the relationship with MD Anderson Cancer Center, the Company's initial development partner for the *Sleeping Beauty* platform.

As part of the new licensing agreement, Ziopharm successfully negotiated the complete elimination of preferred stock that had been issued to Intrexon that was valued at approximately $157 million at that time.

In December, David Mauney, M.D., was promoted to President. Dr. Mauney had joined the Company in September 2017 as Executive Vice President and Chief Business Officer.

Fourth-Quarter 2018 Financial Results

- Net income (loss) applicable to the common shareholders for the fourth quarter of 2018 was $194.5 million, or $1.29 per share, basic and diluted, compared to a net loss of $18.3 million, or $(0.13) per share, basic and diluted, for the fourth quarter of 2017. The increased income attributable to common shareholders resulted primarily from the forfeiture and return of all of the Company's Series 1 preferred stock held by Intrexon Corporation and the relinquishment of Ziopharm's obligations under the Ares Trading Agreement.
- Research and development expenses were $8.2 million for the fourth quarter of 2018, compared to $11.2 million for the fourth quarter of 2017. The decrease in research and development expenses for the three months ended Dec. 31, 2018 is primarily due to decreased preclinical activity related to our cell and gene therapy programs.
- General and administrative expenses were $4.6 million for the fourth quarter of 2018, compared to $3.9 million for the

fourth quarter of 2017. The increase in general and administrative expenses for the three months ended Dec. 31, 2018 is primarily due to contracted outside service costs.

Full Year 2018 Financial Results

- Net income (loss) applicable to the common shareholders for the year ended December 31, 2018 was $137.2 million, or $0.96 per share, basic and diluted, compared to a net loss applicable to the common shareholders of $73.3 million, or $(0.53) per share, basic and diluted, for the year ended December 31, 2017. The increased income attributable to common shareholders resulted primarily from the forfeiture and return of all of the Company's Series 1 preferred stock held by Intrexon Corporation and the relinquishment of Ziopharm's obligations under the Ares Trading Agreement.
- Research and development expenses were $34.1 million for the year ended December 31, 2018, compared to $45.1 million for the year ended December 31, 2017. The decrease in research and development expenses for the year ended December 31, 2018, is primarily due to decreased preclinical activity related to our cell and gene therapy programs.
- General and administrative expenses were $19.9 million for the year ended December 31, 2018, compared to $14.8 million for the Year ended December 31, 2017. The increase in general and administrative expenses for the year ended December 31, 2018, is primarily due to contracted outside service costs.

The Company ended the year with unrestricted cash resources of approximately $61.7 million.

In addition, a prepayment of approximately $27.8 million remains for programs to be conducted by the Company at MD Anderson Cancer Center under the current Research and Development Agreement.

The Company believes its current resources will be sufficient to fund its currently planned operations into the second quarter of 2020.

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

	Three Months Ended December 31, (unaudited)		Year Ended December 31, (audited)	
	2018	**2017**	**2018**	**2017**
Collaboration revenue	$ -	$ 1,597	$ 146	$ 6,389
Operating expenses:				
Research and development	8,199	11,181	34,134	45,084
General and administrative	4,563	3,852	19,918	14,798
Total operating expenses	12,762	15,033	54,052	59,882
Loss from operations	(12,762)	(13,436)	(53,906)	(53,493)
Other income (expense), net	168	166	631	465
Change in fair value of derivative liabilities	113	(3)	158	(1,295)
Net loss	(12,481)	(13,273)	(53,117)	(54,323)
Preferred stock dividends	(342)	(4,999)	(16,998)	(18,938)
Settlement of a related party relationship	207,361	-	207,361	-
Net Income (loss) applicable to common stockholders	$ 194,538	$ (18,272)	$ 137,246	$ (73,261)
Net income (loss) per share - basic	$ 1.29	$ (0.13)	$ 0.96	$ (0.53)
Net income (loss) per share - diluted	$ 1.29	$ (0.13)	$ 0.96	$ (0.53)
Weighted average common shares outstanding used to compute basic net income (loss) per share	150,893,470	140,644,238	143,508,674	136,938,264
Weighted average common shares outstanding used to compute diluted net income (loss) per share	151,094,956	140,644,238	143,710,160	136,938,264

ZIOPHARM Oncology, Inc.

Balance Sheet Data
(in thousands)
(unaudited)

	December 31, 2018	December 31, 2017
Cash and cash equivalents	61,729	70,946
Working capital	74,802	69,927
Total assets	95,051	105,606
Total stockholders' equity (deficit)	85,564	(96,806)

Conference Call and Slide Webcast

The call can be accessed by dialing 1-844-309-0618 (U.S. and Canada) or 1-661-378-9465 (international). The passcode for the conference call is 3091306. To access the slides and live webcast or the subsequent archived recording, visit the "Investors & Media" 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 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 (TCRs) to target specific antigens in solid tumors and chimeric antigen receptors (CARs) to target CD19 in blood cancers with the Company's very rapid (3 rd generation) T-cell manufacturing process. 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 Company's business and strategic plans, the availability of cash resources, the progress and timing of the Company's research and development programs, including the anticipated dates for the initiation, completion and readouts of its clinical trials, the expected timing and closing of the joint venture with TriArm and the future funding of Eden BioCell by TriArm. 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 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 Annual Report on Form 10-K 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.

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