

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT  
PURSUANT TO SECTION 13 OR 15(d)  
OF THE SECURITIES EXCHANGE ACT OF 1934**

Date of report (Date of earliest event reported): November 5, 2020

**ZIOPHARM Oncology, Inc.**

(Exact Name of Registrant as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-33038**  
(Commission  
File Number)

**84-1475642**  
(IRS Employer  
Identification No.)

**One First Avenue, Parris Building 34, Navy Yard Plaza**  
**Boston, Massachusetts**  
(Address of Principal Executive Offices)

**02129**  
(Zip Code)

**(617) 259-1970**  
(Registrant's telephone number, including area code)

**Not applicable**  
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425).
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12).
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b)).
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c)).

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	ZIOP	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act (17 CFR 230.405) or Rule 12b-2 of the Exchange Act (17 CFR 240.12b-2).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 2.02 Results of Operations and Financial Condition**

On November 5, 2020, Ziopharm Oncology, Inc., or the Company, issued a press release announcing its financial condition and results of operations for the three months ended September 30, 2020. A copy of the press release is furnished as Exhibit 99.1 and is incorporated herein by reference.

**Item 7.01 Regulation FD Disclosure**

On November 5, 2020, representatives of the Company presented slides with a business update. A copy of the presentation is furnished as Exhibit 99.2 and is incorporated herein by reference.

The information contained in Items 2.02 and 7.01, including Exhibits 99.1 and 99.2, are being "furnished" and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, or the Exchange Act, or otherwise subject to the liability of that section or Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended, or the Securities Act. The information contained in Items 2.02 and 7.01, including Exhibits 99.1 and 99.2, shall not be incorporated by reference into any registration statement or other document pursuant to the Securities Act or into any filing or other document pursuant to the Exchange Act, except as otherwise expressly stated in any such filing.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press Release of Ziopharm Oncology, Inc. dated November 5, 2020</a>
99.2	<a href="#">Presentation of Ziopharm Oncology, Inc. dated November 5, 2020</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Ziopharm Oncology, Inc.

Date: November 5, 2020

By: /s/ Satyavrat Shukla  
Name: Satyavrat Shukla  
Title: Executive Vice President and Chief Financial Officer



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

- 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 autologous 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 Designation 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, November 5, 2020** — 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<sup>+</sup> 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<sup>+</sup> 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 ([NCT0363647Z](#)) and with Libtayo as a phase 2 trial ([NCT04006112](#)) 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. EDT. 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](http://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](http://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.

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– 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	<u>20,321</u>	<u>13,448</u>
Loss from operations	(20,321)	(13,448)
Other income, net	6	203
Noncash inducement warrant expense	—	(60,751)
Net loss	<u>(20,315)</u>	<u>(73,996)</u>
Basic and diluted net loss per share	<u>\$ (0.10)</u>	<u>\$ (0.43)</u>
Weighted average common shares outstanding used to compute basic and diluted net loss per share	<u>212,837,367</u>	<u>170,613,712</u>

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

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

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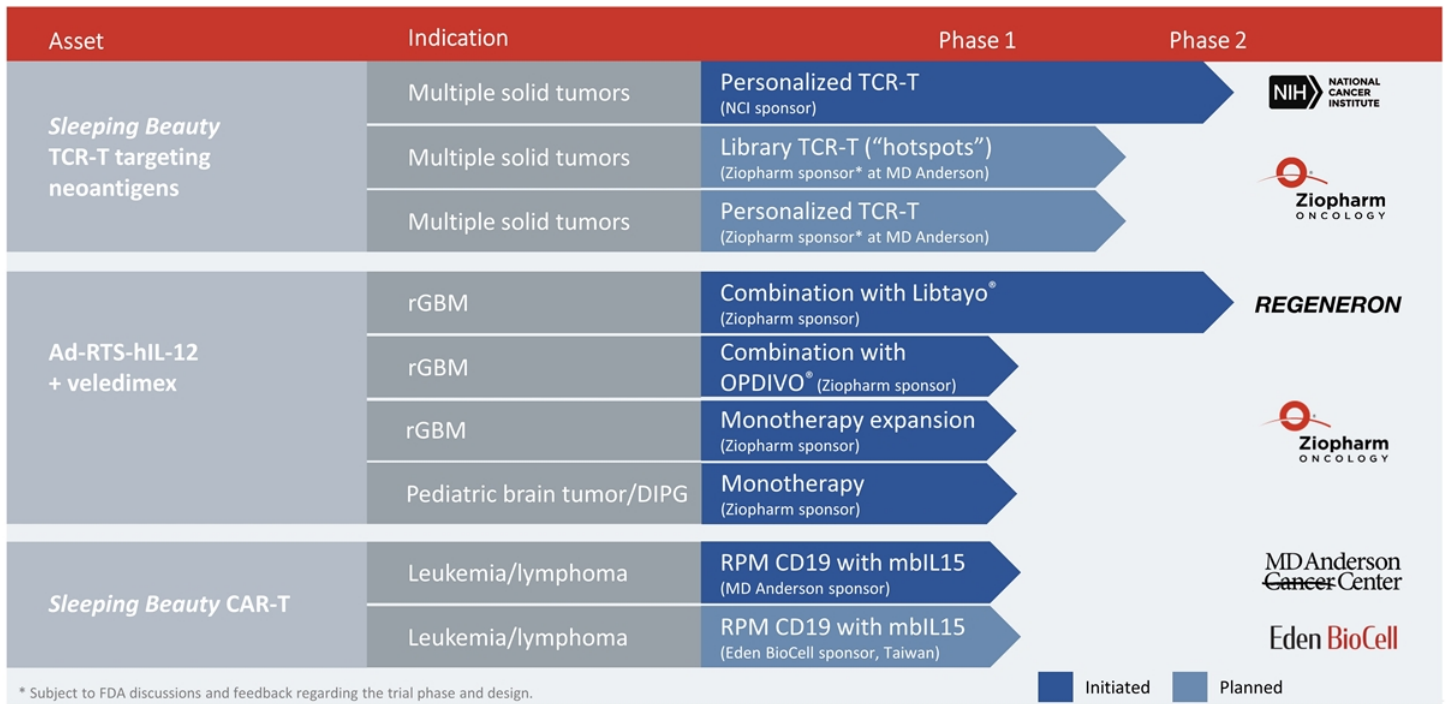
# **Third Quarter 2020 Financial Results and Update**

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05 November 2020

*This presentation 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 Company's hiring expectations and expected additions to its Board of Directors or Scientific Advisory Board, the progress, design 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 Company's expectations regarding the number of patients or the timelines for the commencement of patient dosing in its clinical trials, the Company's expectations regarding the timing of IND filings, the buildout and expansion of the Company's facilities, and the Company's expectations regarding the impact of the ongoing COVID-19 pandemic, including the expected duration of disruption and immediate and long-term impact and effect on its business and operations. 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 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.*

# Broad Pipeline of Oncology Innovation



# Third Quarter Results and Update

## *Response to COVID-19:*

- Ziopharm employees primarily work remotely with exceptions such as in our laboratory; safely and advancing programs; adapting to pandemic

## *Sleeping Beauty TCR-T Program:*

- Ziopharm on track to file IND for library hotspot trial in Q1 2021 through build out of team and infrastructure in Houston
- Further expanded library of TCRs targeting neoantigen hotspots through additional license of IP from NCI
- Completed technology transfer of Ziopharm's engineering runs to the NCI; NCI authenticated methodology and now validating in GMP facility
- Queue of eligible patients with solid tumors at NCI depleted during shutdown due to COVID-19; NCI is screening patients for neoantigens and TCRs to expand patient pool for TCR-T trial; Pace of adding patients is slower than before pandemic
- NCI guides that enrollment to Personalized TCR-T trial to begin next year

## *Controlled IL-12 Program:*

- Three abstracts accepted at Society for Neuro-Oncology Annual Meeting November 19-21, 2020
  - Initial observations from phase 1/2 pediatric trial for diffuse intrinsic pontine gliomas (DIPG)
  - Update from phase 1 combination trial of Controlled IL-12 with Opdivo®
  - First presentation of data from phase 2 trial of Controlled IL-12 in combination with Libtayo®
- All three clinical sites active and screening for potential DIPG patients

## *Sleeping Beauty CD19-specific CAR-T Program:*

- MD Anderson screening patients for phase 1 *Sleeping Beauty* allogeneic CAR-T trial using Rapid Personalized Manufacturing (RPM)
- Eden BioCell commenced filing IND for clinical trial in Taiwan to assess autologous CAR-T produced using RPM
- Eden BioCell and partners report dosing of several patients with relapsed CD19<sup>+</sup> malignancies under compassionate use, infusing autologous CAR-T the day after gene transfer per RPM

## *Board Refreshment and Operational:*

- Named Kevin Buchi and James Huang to Ziopharm Board of Directors
- Populated Scientific Advisory Board under Carl June, M.D., Chairman
- Recruited Dr. Adam Levy from Gilead as EVP, IR and Corporate Communications



## ***First Clinical Program – Evaluating Ziopharm's Existing TCR Library in Multiple Cancers***

- ***Ziopharm's TCR Library Clinical Trial***
  - ***Timing:*** On track to file IND in Q1 2021; begin dosing patients in mid-2021
  - ***Indications:*** gynecologic, colorectal, pancreatic, non-small cell lung cancer and cholangiocarcinoma
    - Casting a wide net to bolster enrollment
  - ***TCRs:*** Initiate with TCRs from existing library targeting prevalent mutations and HLA; Additional TCRs from library will be added to trial as it progresses
  - ***Ongoing activities:*** Several workstreams with MD Anderson, including identifying potential populations, intended to streamline program
    - *Unique relationship with MD Anderson facilitates approach*
- ***Attributes of Ziopharm's Existing Library***
  - Existing library includes 30+ TCRs, capable of treating 19 unique mutation/HLA combinations
  - Initial TCRs target subsets of common mutations in KRAS and TP53 hotspot genes
    - Mutation targets are pan-cancer and considered drivers of tumor biology
    - KRAS
      - Dominant oncogenic mutations of KRAS are single amino acid substitutions present in multiple cancers
      - Small molecules address only one KRAS target (G12C); We are building a library that targets multiple genetic changes to KRAS
        - Data confirm targeting KRAS can result in anti-cancer responses; Bodes well for our TCR-T library that includes TCRs against multiple KRAS mutations
    - TP53
      - Considered among the most mutated gene in human cancers
      - Functions as a transcription factor to regulate cell division and stabilize the genome
  - TCRs in library have broad HLA representation



## ***Second Clinical Program – Evaluating Personalized TCR Approach in Multiple Cancers***

### Overview:

- Personalized approach evaluates therapies using patient-derived TCRs targeting neoantigens specific to each patient
  - “TCRs from the patient, for the patient”
- Treatment: Each patient may receive TCRs with multiple specificities per infusion
  - Takes advantage of scalable *Sleeping Beauty* manufacturing approach to create commercial solution
- Timing: Commence after the initiation of TCR-T Library trial
- Indications: Multiple cancers
- Process improvements: Building on methodologies from the NCI's Personalized TCR-T trial; Commercializing the approach by reducing overall time to treatment
- Library expansion: Personalized TCR-T trial may serve to feed expansion of TCRs in library

- Building TCR Library
  - Library in-licensed from NCI; Approach validated by multiple publications and in-house team
    - Already sufficient to begin clinical trial at MD Anderson
  - Ziopharm has multiple avenues for expanding the number of TCRs in library; Will continue growing the library in future
- Manufacturing
  - Will initially outsource most of manufacturing for clinical trials
  - Ziopharm expeditiously building pilot clinical product unit (CPU) to provide manufacturing backup to outsourced model
    - Intended to ensure we can meet enrollment goals for MDACC trial
    - Using small portion of existing footprint on MD Anderson; expects to be ready for cGMP manufacturing in 2H:2021
    - Modest investment to ensure clinical product supply
  - Evaluating potential expansion in Houston for full cGMP facility; buildout of this space will be determined based on clinical progress
    - Personalized therapies require manufacturing control
- Process Improvements
  - *Sleeping Beauty* non-viral DNA plasmid approach is key differentiator; Taking advantage of scalable solution
- Bioinformatics Expertise
  - Built a robust bioinformatics team that supports neoantigen identification and prioritization
- TCR Identification
  - Built immunology team that supports TCR identification and prioritization
- Next Gen Therapies
  - Team engaged on foundational science for next generation programs intended to address tumor micro-environment and enhance T-cell functions
  - Clinical programs for next gen programs will follow our core two TCR-T clinical trials (library/personalized); Details provided as science progresses



Current resources fund operations into mid-2022; allows visibility into key clinical readouts

**Selected Balance Sheet Data**

Cash and cash equivalents as of 9/30/20  
**\$135.5 million**

At MD Anderson from prepayment for programs to be conducted by the Company as of 9/30/20  
**\$11.4 million**

Cash resources of approximately \$135.5 million, plus pre-paid balance at MD Anderson, is sufficient to:

- Fund planned operations and execute our strategy into mid-2022; recruit key personnel to support growth
- Continue the expansion of our proprietary TCR library to support our TCR hotspot mutations trial
- Support potential broadening of Controlled IL-12 Program
- Proceed with incremental buildout of our expanded footprint on MD Anderson campus
- Allow for visibility into additional clinical milestones / data readouts in our three core programs

## Anticipated Milestones

- Present additional clinical data for Controlled IL-12 program at SNO 2020
- Advance enrollment and dosing of DIPG patients in pediatric trial for Controlled IL-12 at multiple sites
- Commence patient dosing in allogeneic RPM CD19-specific CAR-T study; Initiated at MD Anderson
- Complete IND filing for autologous RPM CD19-specific CAR-T trial in Taiwan with Eden BioCell this year
- IND in early 2021 for Ziopharm's TCR-T Library trial to be undertaken at MD Anderson; Treat patients mid-2021
- Dose first patient in NCI-led *Sleeping Beauty* TCR-T phase 2 trial targeting solid tumors
- Host virtual R&D Day with team and guest speakers in Q1 2021
- Continue review of potential additions to Board of Directors; Recruitment of key executive leadership



# Thank you...

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05 November 2020