



Ziopharm
ONCOLOGY

Fourth Quarter and Full Year 2019 Results Call

02 March 2020

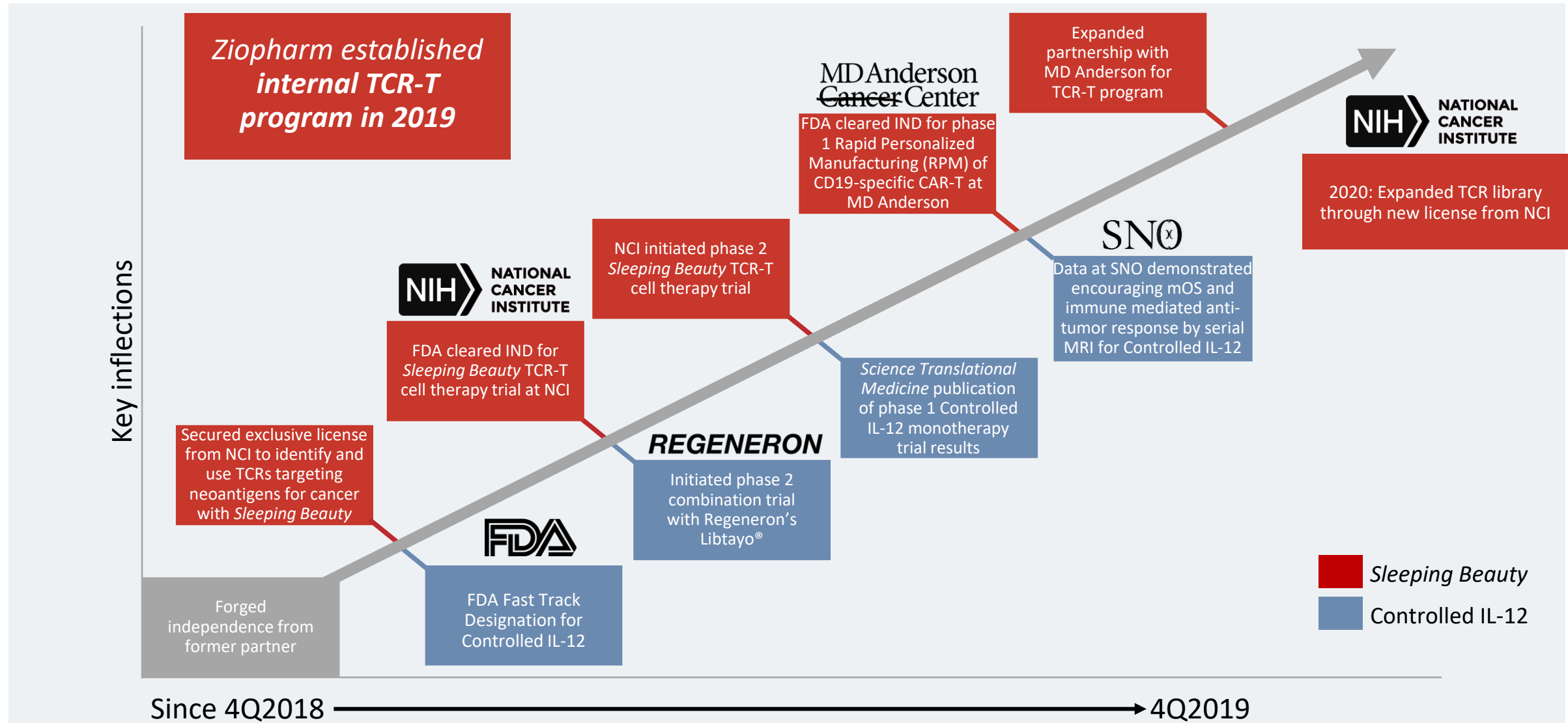


Forward Looking Statements

This presentation contains certain forward-looking information about Ziopharm Oncology, Inc. that is intended to be covered by the safe harbor for "forward-looking statements" provided by 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, and the progress and timing of the development of Ziopharm's research and development programs, including the timing for the initiation and completion of its clinical trials. Although Ziopharm's management team believes 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 for the year ended December 31, 2019 filed by Ziopharm 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 of the presentation, and we do 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.



Strategic Milestones Achieved in One Year Since Independence



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

Selected Balance Sheet Data

Cash, equivalents and short-term investments as of 12/31/19

\$79.7 million

At MD Anderson from prepayment for programs to be conducted by the Company as of 12/31/19

\$20.3 million

Proceeds from financings subsequent to the close 2019

~\$98 million

Cash resources of approximately \$177 million (including recent financing proceeds), plus pre-paid balance at MD Anderson, will be sufficient to:

- Fund planned operations and execute our strategy into mid-2022
- Accelerate expansion of TCR library to support our hotspot TCR trial
- Support potential broadening of Controlled IL-12 Program
- Allow for visibility into additional clinical milestones / data readouts in our three core programs
- Buildout of our expanded footprint on MD Anderson campus, including cGMP facility



Company Vision: Offer Treatment to all Patients with Solid Tumors

How Ziopharm will capitalize on solid tumors as the main theme:

1

Infuse T cells to attack and kill cancer cells

2

Use TCRs targeting neoantigens present in multiple solid tumor types

3

Genetically modify T cells from peripheral blood

4

Harness cytokines to engage and control the immune response

5

Utilize non-viral manufacturing based on DNA plasmids from *Sleeping Beauty*



First-in-Human Phase 2 *Sleeping Beauty* TCR-T Trial at NCI

NCI Surgery Branch and Dr. Steven Rosenberg

are world experts in identifying neoantigens and TCRs, and Ziopharm is a proven leader in *Sleeping Beauty*

A Phase 2 Study Using the Administration of Autologous T-Cells Engineered Using the Sleeping Beauty Transposon/Transposase System to Express T-Cell Receptors Reactive Against Mutated Neoantigens in Patients With Metastatic Cancer

Enrollment:

- Patients with solid tumors including:
 - gastrointestinal
 - genitourinary
 - ovarian
 - breast
 - non-small cell lung cancers
 - glioblastoma

Endpoints:

- Primary: tumor response rate
- Secondary: safety and tolerability

NIH U.S. National Library of Medicine

[ClinicalTrials.gov](https://clinicaltrials.gov)

NCI PROTOCOL ID INVESTIGATOR
NCI-19-C-0143 Steven A. Rosenberg, M.D., Ph.D.

Providing a solution to cost and complexity of commercial CAR-T today

IND cleared for phase 1 trial: Evaluate allogeneic CD19-specific CAR-T

- Infuse as soon as day after gene transfer
- Validate technology, potential commercial opportunity
- Cleared phase 1 IND; expect to treat first patient in H1 2020
- Patients with CD19⁺ leukemias and lymphomas who have relapsed after allogeneic bone marrow transplantation
- Trial to be conducted at MD Anderson

Ziopharm & Eden BioCell pursuing autologous CD19-specific CAR-T

- US: planned phase 1 clinical trial at MD Anderson
- Greater China: Eden BioCell plans to file IND for phase 1 trial in Taiwan this year
 - 50-50 joint venture; up to \$35 million funding committed from TriArm Therapeutics

Eden BioCell



2020 Near-Term Clinical Milestones Driving Value

1H 2020

Phase 2

Patient dosing in NCI-led *Sleeping Beauty* TCR-T trial targeting solid tumors

Phase 1

Enrollment in *Sleeping Beauty* CD19-specific CAR-T RPM trial with membrane-bound IL-15 at MD Anderson

Phase 2

Complete enrollment and initial data readout for *Controlled IL-12* in combination with Libtayo[®]

Phase 1

Data readout of *Controlled IL-12* in combination with OPDIVO[®]

Phase 1

Data readout from *Controlled IL-12* as monotherapy in expanded cohort