



First Quarter 2020

Financial Results and Update

07 May 2020



Forward Looking Statements

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, 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 in its clinical trials, 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.

First Quarter Results and Update

Response to COVID-19:

- Ziopharm employees working remotely with exceptions such as in our laboratory; successfully advancing internal programs
- Ziopharm's CAR-T and TCR-T trials impacted by broad work restrictions at National Cancer Institute (NCI) and MD Anderson Cancer Center; Company instituted plans to minimize the impact
- Controlled IL-12 Program phase 2 trial actively enrolling patients; on track to complete enrollment in 1H20

Sleeping Beauty TCR-T Program

- Discussions underway with NCI and MD Anderson about plans to re-open
- Final engineering runs being completed in Ziopharm's Houston lab to facilitate patient TCR-T dosing at NCI once re-open
- Pre-IND reviewed and instructive feedback received from FDA related to IND process for Ziopharm-led TCR-T clinical trial
- Additional laboratory and infrastructure completed

Controlled IL-12 Program

- Enrollment in phase 2 trial of Controlled IL-12 in combination with Libtayo® continues; on track to complete enrollment in 1H20
- Company to present updated data on Controlled IL-12 monotherapy and combination with Opdivo® at ASCO 2020

Sleeping Beauty CAR-T Program

- Eden BioCell to file IND this year for clinical trial in Taiwan to assess autologous CD19-specific CAR-T produced using Rapid Personalized Manufacturing (RPM)
- Waiting on MD Anderson to undertake Site Initiation Visit (SIV) prior to commencing phase 1 *Sleeping Beauty* allogeneic CD19-specific CAR-T trial using RPM technology; MD Anderson alliance partners like Ziopharm expect to have priority on re-open

Operational

- Raised more than \$100 million in Q1 2020 prior to pandemic; providing more than \$171 million to fund operations into mid-2022

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

Selected Balance Sheet Data

Cash and cash equivalents as of 3/31/20
\$171.0 million

At MD Anderson from prepayment for programs to be conducted by the Company as of 3/31/20
\$18.0 million

Proceeds from financings in Q1 2020
\$101.7 million

Cash resources of approximately \$171 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 continued buildout of our expanded footprint on MD Anderson campus
- Allow for visibility into additional clinical milestones / data readouts in our three core programs

Anticipated 2020 Milestones

- Present additional data at ASCO of Controlled IL-12 in combination with OPDIVO® and as monotherapy
- Complete enrollment in phase 2 trial for Controlled IL-12 in combination with Libtayo®
- First patient dosing in NCI-led *Sleeping Beauty* TCR-T phase 2 trial targeting solid tumors
- Update guidance on TCR-T trial to be undertaken at MD Anderson Cancer Center
- Complete SIV and commence enrollment in phase 1 *Sleeping Beauty* allogeneic CD19-specific CAR-T RPM trial with membrane-bound IL-15 at MD Anderson
- File IND for autologous CD19-specific CAR-T RPM trial in Taiwan with Eden BioCell
- Recruit Chief Medical Officer; add business development and investor relations capabilities
- Expand Board of Directors with complementary expertise



Thank you...

07 May 2020