ZIOPHARM Oncology

# Positive Updated Results of Ad-RTS-hIL-12 Study in Brain Cancer Presented at SNO 2017

November 20, 2017

# 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 progress, timing and results of preclinical and clinical trials involving the Company's drug candidates, and the progress of the Company's research and development programs. All of such statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied by, the forward-looking statements. These risks and uncertainties include, but are not limited to: whether chimeric antigen receptor T cell ($CAR^+$ T) approaches, Ad-RTS-IL-12, TCR and NK cell-based therapies, or any of our other therapeutic candidates will advance further in the preclinical or clinical trials process and whether and when, if at all, they will receive final approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies and for which indications; whether chimeric antigen receptor T cell ($CAR^+$ T) approaches, Ad-RTS-IL-12, TCR and NK cell-based therapies, and our other therapeutic products will be successfully marketed if approved; the strength and enforceability of our intellectual property rights; competition from other pharmaceutical and biotechnology companies; and the other risk factors contained in our periodic and interim SEC reports filed from time to time with the Securities and Exchange Commission, including but not limited to, our Annual Report on Form 10-K for the fiscal year ended December 31, 2016 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, 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.*

# Agenda

- ZIOPHARM CEO Laurence Cooper, M.D., Ph.D.
  - Introduction
- Antonio Chiocca, M.D., Ph.D.
  - Harvey Cushing Professor of Neurosurgery at Harvard Medical School, Surgical Director at the Center for Neuro-oncology at Dana-Farber Cancer Institute, and Chairman of Neurosurgery and Co-Director of the Institute for the Neurosciences at Brigham and Women's Hospital
  - President of SNO, 2017
- Stewart Goldman, M.D.
  - Division Head Hematology-Oncology, Neuro-Oncology & Stem Cell Transplantation at Ann & Robert H. Lurie Children's Hospital in Chicago
- Brief Q&A

# Summary of Data Presented at SNO

**Apparent survival benefit:** median overall survival (mOS) of 12.5 months in all comers with recurrent glioblastoma (rGBM) withstands longer follow up, continues to best historical controls (as of October 18, 2017)

**Four lines of evidence supporting why patients with rGBM may be benefiting from Ad-RTS-hIL-12 + veledimex**

- **Biopsies show IL-12 makes glioblastoma "hot":** Three of three biopsies undertaken many weeks after completion of veledimex show sustained infiltration of activated killer $CD8^+$ T cells and upregulation of immune checkpoints
- **Emerging biomarker:** Ratio of killer $CD8^+$ to suppressor $FoxP3^+$ T cells in circulation correlates with mOS
- **Low-dose steroids:** Correlates to improved survival with 100% survival in cohort receiving lowest dose of steroids at mean follow up of 11.1 months
- **Anti-tumor effect:** MRI demonstrates that some tumors are shrinking