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## **A Virus, a Gene and a Pill Used to Harness the Immune System to Fight Brain Tumor in Children**

ZiOPHARM Oncology is pleased to [share a story from the Ann & Robert H. Lurie Children's Hospital in Chicago](#) on the Ad-RTS-hIL-12 plus veledimex clinical trial for pediatric patients with brain cancer. As we announced last week, **Stewart Goldman, M.D., Division Head Hematology-Oncology, Neuro-Oncology & Stem Cell Transplantation at Lurie Children's dosed the first patient in this pediatric trial.**

**First patient on a new Phase 1 pediatric brain tumor study injected with a common cold virus bioengineered to deliver a gene that mobilizes the immune system; a pill is used to control the targeted immune response**

The first patient in a new Phase 1 gene therapy trial for pediatric brain tumors underwent a leading-edge procedure at Ann & Robert H. Lurie Children's Hospital of Chicago. During surgery to remove the brain tumor, the patient was injected with an adenovirus, a common cold virus, at the tumor site. The virus was bioengineered not to cause illness but rather deliver a gene that produces human interleukin 12 (hIL-12), a powerful protein to jumpstart the immune system to kill remaining tumor cells. For the next 14 days, the patient is given a pill – veledimex – to activate the gene and control the immune response, so that the inflammation fights the tumor without overwhelming the rest of the body.

“Using the immune system to fight cancer is one of the most exciting new directions in cancer research,” said [Stewart Goldman, MD](#), Principal Investigator at Lurie Children's, Division Head of [Hematology, Oncology, Neuro-Oncology and Stem Cell Transplantation](#), and Professor of Pediatrics at Northwestern University Feinberg School of Medicine. “What is most challenging is regulating the immune response we unleash, and that is what we are doing in this study. The pill dose acts like a thermostat with which we can adjust the intensity of the patient's immune response.”

This is the first gene therapy clinical trial of this type for pediatric brain tumors. It will initially include patients with recurrent or progressive glioblastoma multiforme (rGBM) in the cortex of the brain. At a later date, children with diffuse intrinsic pontine glioma (DIPG), an incurable tumor located in the brain stem, will be added.

“Studies of this approach in adults with rGBM are showing promising results,” said Goldman. “We are hopeful that this will offer a viable treatment to children with gliomas who currently do not have any curative options.”

In addition to Lurie Children's, the Phase 1 study will be conducted at Dana-Farber Cancer Institute in Boston and the University of California, San Francisco. It is sponsored by ZiOPHARM Oncology, Inc. (Nasdaq:ZiOP), a Boston-based biopharmaceutical company focused on developing new gene and cell-based immunotherapies for cancer. Lurie Children's cancer program is part of Robert H. Lurie Comprehensive Cancer Center of Northwestern University.

Research at Ann & Robert H. Lurie Children's Hospital of Chicago is conducted through the Stanley Manne Children's Research Institute. The Manne Research Institute is focused on improving child health, transforming pediatric medicine and ensuring healthier futures through the relentless pursuit of knowledge. Lurie Children's is ranked as one of the nation's top children's hospitals in the U.S. News & World Report. It is the pediatric training ground for Northwestern University Feinberg School of Medicine. Last year, the hospital served more than 208,000 children from 50 states and 58 countries.