Researchers to develop next generation immunotherapy for children with deadly solid tumors

April 3 2013

Recently, research using adoptive T-cell immunotherapy in blood cancers have shown success, most notably in the case of a seven-year-old girl whose leukemia went into remission using altered T-cells and a disabled HIV virus. Now, two of the pediatric cancer scientists involved in the T-cell/HIV study will develop a new experimental cancer immunotherapy treatment option for children with high-risk solid tumors based on the same novel approach that uses a patient's own T-cells to attack tumor cells.

Researchers at the Children's Hospital of Philadelphia, the University of Pennsylvania, and the Primary Children's Medical Center of Salt Lake City received a $550,000 collaborative grant to test next generation T-cell immunotherapy strategies in children with neuroblastoma, a solid tumor with poor prognosis that is responsible for 15% of all childhood cancer deaths. The awardees were selected within a rigorous peer-review process through a new initiative called ACT FAST (Adoptive Cell Therapy For Adolescent/pediatric Solid Tumor), which is spearheaded by Solving Kids' Cancer. ACT FAST aims to fast-track promising research into a clinical trial within one year of the award through a collaborative team of researchers.

"Only a handful of cancer centers in the world have the capabilities and the infrastructure to implement adoptive cell therapy," said Scott Kennedy, the Executive Director of Solving Kids' Cancer. "Our goal in

bringing together these researchers and institutions was to harness their collective power in bringing faster cures to kids with neuroblastoma."

The National Cancer Institute (NCI) and the Society for the Immunotherapy of Cancer (SITC) provided strategic counsel and expert review of the grant award, which was jointly funded by Solving Kids' Cancer, the Pierce Phillips Charity, and the Catherine Elizabeth Blair Memorial Foundation.

The clinical trial will be the first to use transiently modified T-cells in children with neuroblastoma and is estimated to open before the end of the year. Stephan Grupp, M.D., Ph.D., Professor of Pediatrics at the Children's Hospital of Philadelphia, will direct the trial in collaboration with Michael Pulsipher, M.D., Professor of Pediatrics at the Primary Children's Medical Center/University of Utah, and Carl June, M.D., the Director of Translational Research at the Abramson Cancer Center at the University of Pennsylvania, who led the altered T-cell/HIV study.

Today, children with relapsed high-risk neuroblastoma have little or no options to cure their disease through traditional methods, including chemotherapy, surgery and radiation. Early clinical studies utilizing cancer immunotherapies such as modified T-cells have demonstrated initial success and promising potential as more effective treatment options in some blood cancers, including chronic myelogenous leukemia in adults and acute lymphoblastic leukemia in children.

"The ACT FAST initiative aims to bring the newest engineered T cell therapies to pediatric solid tumors. The ACT FAST approach is rigorous, highly focused and intensely translational. By collaborating with pediatric physician-scientists across institutions through ACT FAST, we will not only be able to advance research into engineered T-cell treatments, but just as importantly, offer cutting-edge cancer immunotherapy clinical trial options rapidly to children whose cancer

recurs or resists current treatment," said Dr. Grupp.

Provided by Solving Kids' Cancer