Team Progress Updates

SU2C–St. Baldrick's Foundation Pediatric Cancer Dream Team

"Immunogenomics to Create New Therapies for High-Risk Childhood Cancers"

New classes of therapeutics are needed to improve survival of children with cancer and decrease the life-altering physical, emotional, and financial costs of curative therapies. The team uses new technologies in the fields of cancer genomics, epigenetics (the study of mechanisms that alter gene expression), and proteomics (research into proteins and their functions) to discover and validate new targets for immunotherapy.

The team is building new antibodies, antibody-drug combinations, and CAR T cells to attack these targets. It is developing innovative new immunotherapies, discovering basic mechanisms of effectiveness (or lack thereof) in both antibody and cellular engineering, and devising novel methods to monitor clinical effectiveness and toxicity.

Team members have opened 25 clinical trials and treated 688 pediatric patients with cancers that have resisted treatment. They are demonstrating the potency of immunotherapy against acute lymphocytic leukemia (ALL) and defining mechanisms by which these cancer cells develop resistance. They have also made progress against childhood solid cancers, with many emerging therapeutics in clinics or scheduled to enter testing in the future.

In its work to date, the Pediatric Cancer Dream Team has:

- Launched 25 clinical trials and treated 688 pediatric patients with cancers that have resisted existing treatments.
- Pioneered work in immunotherapy, including chimeric antigen receptor T cell (CAR-T) therapy that has resulted in the previously unheard-of rate of complete response in more than 70 percent of children treated who have a type of acute lymphocytic leukemia (ALL).
- Made major contributions to the work that led to FDA approval of tisagenlecleucel, the first CAR T-cell therapy approved for ALL.
- Helped identify biomarkers predicting which patients receiving T-cell therapy will encounter severe Cytokine Release Syndrome (CRS), "cytokine storm," a life-threatening complication that sometimes occurs with the use of antibody therapies.
- Developed a standardized diagnosis and management plan for the management of cytokine storm, which is now widely used.
- Made progress against childhood solid cancers, with many emerging therapeutics against diseases such as neuroblastoma, glioblastoma, medulloblastoma, osteosarcoma, Ewing sarcoma and rhabdomyosarcoma just now entering the clinics, or scheduled to enter into Phase 1 testing over the next 1-3 years.

Generated 105 peer-reviewed publications and 16 patent applications.