



Team Progress Updates

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

“Immunogenomics to Create New Therapies for High-Risk Childhood Cancers”



Medicine needs new classes of therapeutics to improve survival of children with cancer and decrease the potentially life-altering physical, emotional, and financial costs of current 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 (tumor-seeking killer 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 previously opened more than 20 clinical trials and treated nearly 700 pediatric patients with cancers that have resisted treatment. Their work has demonstrated the potency of immunotherapy against acute lymphocytic leukemia (ALL), and the scientists have also made progress against childhood solid cancers.

In its work to date, the St. Baldrick's–SU2C Pediatric Cancer Dream Team has:

January 2019

- Scientists on the team have prioritized ten drug targets or strategies which either have ongoing clinical trials or plans to open a trial within the next 1-2 years.
- The team has two active trials of treatments that targets both CD19 and CD22 in patients with relapsed B-ALL, including testing a tandem bivalent CAR.
 - They have treated four children and seven adults and continue to dose escalate. All four children with B-ALL experienced complete remission at the first dose levels and remain alive. One of the children has regressed, however.

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- Begun multiple new trials targeting B-cell acute lymphoblastic leukemia, aimed at diminishing patient relapse following CD19-CAR T cell therapy and increasing CD19-CAR T cell persistence. The team is also continuing to develop newer and potentially more effective CAR T cell therapeutics.





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- Demonstrated that a CAR T cell targeting HER2 is safe, even following chemotherapy, and that it shows clinical activity in pediatric sarcoma and glioblastoma patients.
- Found a promising target (GD2) in DIPG, an incurable pediatric brain cancer. Researchers have developed CAR T cells aimed at this molecule that show potent activity in DIPG, and they are working on translating this therapy for neuroblastoma and osteosarcoma.
- Implemented, in partnership with a team supported by an SU2C Catalyst grant, a clinical trial for patients with DNA repair mutations in pediatric cancers.
- Focused on 19 prioritized immunotherapeutic targets and developed multiple industry partnerships to move them into trials.