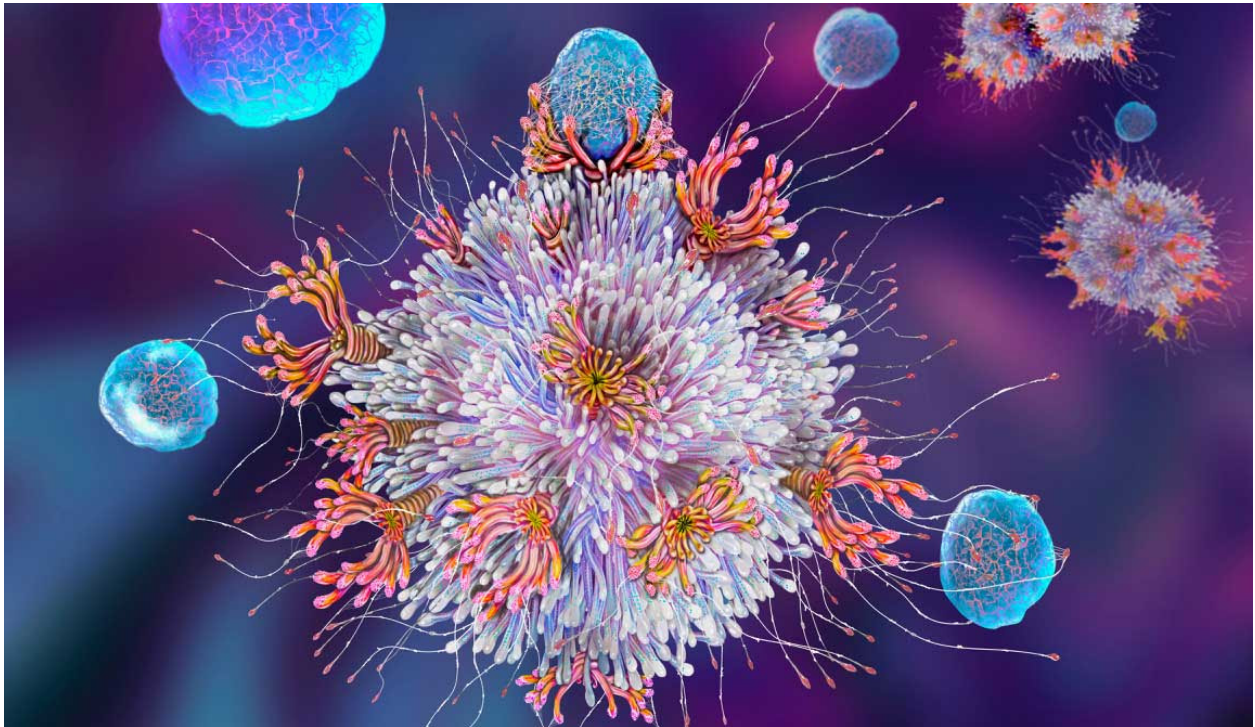




Cancer and Blood Disease Institute

Winter 2021



Modified CAR T-Cells Show Promise in Neuroblastoma

A team led by Shahab Asgharzadeh, MD, and Babak Moghimi, MD, developed a modified version of CAR-T that shows promise in neuroblastoma—sparing healthy brain tissue and effectively killing cancer cells. The preclinical study, published in Nature Communications, took advantage of the gating properties of SynNotch CAR T-cells.

[See how it works](#)



New Findings on Cisplatin Hearing Loss Risk

Etan Orgel, MD, MS, led a multicenter study on hearing loss in over 1,400 youth treated with cisplatin. The study—published in *The Lancet Child & Adolescent Health*—identified clear at-risk populations and found that *how* cisplatin is given, not just total dose, influences risk.

[Learn more](#)



Cell Therapy Program Expands

The Transplantation and Cellular Therapy Section, led by Michael Pulsipher, MD, has added four faculty investigators since 2019—including a director of our new joint cell therapy program with the Keck School of Medicine of USC. The team is developing new ways to strengthen CAR-T therapy in pediatric cancers.

[See who joined us](#)



Factors Affecting Survival in Rhabdomyosarcoma

Leo Mascarenhas, MD, led a Children's Oncology Group study looking into the clinical factors influencing survival in children with localized FOXO1 fusion-positive rhabdomyosarcoma. Patients older than 10, with large primary tumors, had the poorest prognosis.

[See the findings](#)



First Results From Hemophilia Trial

Guy Young, MD, and a group of international colleagues reported the first results from a phase 3 clinical trial of nonacog beta pegol (N9-GP) in previously untreated patients with hemophilia B. Inhibitor incidence was 6.1%, with 68% of patients experiencing zero bleeds.

[Read more](#)

Upcoming Events

- American Association for Cancer Research
April 9-14, 2021
- American Society of Pediatric Hematology/Oncology
April 20-23, 2021
- American Society of Clinical Oncology
June 4-8, 2021

Division Chief: Alan S. Wayne, MD
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