

## Phase II clinical trial for children and young adults examining stem cell transplantation in sarcoma patients

Pediatric and young adult sarcoma patients up to age 25 may be eligible for a Phase II clinical trial at [MD Anderson Children's Cancer Hospital](#) examining the side effects and response of receiving allogeneic stem cell transplantation for pediatric solid tumors. Study [2020-0496](#) is being evaluated in high-risk patients with refractory or recurrent disease. The principal investigator for the trial is [Kris Mahadeo, M.D., M.P.H.](#), associate professor of Pediatrics and section chief for the Pediatric Stem Cell Transplantation and Cellular Therapy (SCT-CT) Program.

Eligibility criteria include:

- Patients up to age 25
- Patients with the following solid tumors: Ewing's, peripheral primitive neuroectodermal tumor (PNET), malignant peripheral nerve sheath tumor, neurofibrosarcoma, rhabdosarcoma, neuroblastoma, and desmoplastic small round cell tumor (both new and recurrent)
- Patients must have chemo-responsive disease—defined as 30% or greater decrease in the tumor target lesions; patients with complete response will be eligible to participate
- Available hematopoietic donor: matched related donor bone marrow, matched related allogeneic umbilical cord blood, or matched unrelated allogeneic umbilical cord blood which meet specific testing standards
- Creatinine clearance or glomerular filtration rate (GFR)  $\geq 50$  ml/min/1.73m<sup>2</sup>, and not requiring dialysis

Exclusion criteria include:

- Lack of histocompatible suitable related donor/ graft source
- End-organ failure that precludes the ability to tolerate the transplant procedure, including conditioning regimen
- Renal failure requiring dialysis
- Congenital heart disease resulting in congestive heart failure
- Ventilatory failure: requires invasive mechanical ventilation
- Human immunodeficiency virus (HIV) infection
- Uncontrolled bacterial, viral, or fungal infections (currently taking medication yet clinical symptoms progress); stable, controlled disease with treatment is not an exclusion criteria
- A female who is pregnant, planning to become pregnant during the study, or is nursing a child

To find out if your patient is eligible for this trial or others at MD Anderson Children's Cancer Hospital, please email [PediatricCellTherapy@mdanderson.org](mailto:PediatricCellTherapy@mdanderson.org).

## Cytotoxic T cell lymphocytes (CTL) under study: We have options to address common infections in immune compromised pediatric patients

Immunotherapies that boost the body's own natural defenses have ushered in a new era of treatment to help attack cancer cells while distinguishing between healthy ones. The agents work alone or with chemotherapy and other modalities. While investigators keep working to identify new agents that safely unlock the immune system's anti-tumor potential, they are also assessing drugs they believe show promise in fighting life-threatening complications, which can occur in immunocompromised patients.

Pediatric and young adult patients at MD Anderson have access to a wide array of studies which target Epstein-Barr virus-associated lymphoproliferative disorders (including EBV PLTD), infections such as cytomegalovirus, BK virus, JC virus, SARS-CoV-2 and adenovirus.

These CTL trials are open to teens and young adults:

- Phase II study of HLA-matched allogeneic cytomegalovirus (CMV) specific CTL to treat CMV infection after hematopoietic stem cell transplantation (HSCT). [2013-0657](#)
- Phase II study assessing the effect of BK specific CTL lines generated by ex vivo expansion in patients with BK virus infection and JC virus infection. [2014-0279](#)
- Phase I study of the administration of off-the-shelf, expanded, HLA matched, third-party adenovirus specific T cells for therapy of adenovirus related disease and COVID-related pneumonia in immunocompromised patients. [2017-0350](#)
- Expanded access protocol for tabellecleucel for patients with Epstein-Barr virus-associated viremia or malignancies for whom there are no appropriate alternative therapies. [2019-0079](#)
- Phase III multi-center, open label trial of ATA129 for solid organ transplant subjects with Epstein-Barr Virus-associated post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE Study) [Atara 302]. [2017-0771](#)

The Children's Cancer Hospital has a [Pediatrics Clinical Trials](#) page. Visit for additional information about other CTL studies or stem cell transplantation protocols.

## Learn more about our other immune deficiency and regenerative medicine clinical trials

### Immune effector cells

- Phase I/II study of ex-vivo expanded allogeneic NK cells for the treatment of pediatric solid tumors. [2017-0085](#)
- Phase II clinical study to assess efficacy and safety of LN-145 (autologous centrally manufactured tumor infiltrating lymphocytes) across multiple tumor types. [2017-0672](#)
- Phase I/II study of anti-viral central memory CD8 veto cells in haploidentical hematopoietic stem cell transplantation. [2018-0221](#)

### Regenerative medicine

- Expanded access protocol: umbilical cord blood infusions for children with brain injuries. [2019-0481](#)

# Register now: Critical Care Complications of Pediatric Stem Cell Transplantation and Cellular Therapy Workshop in March

Registration is now open for the Critical Care Complications of Pediatric Stem Cell Transplantation and Cellular Therapy Workshop in March. Inpatient nurses, clinic nurses, doctors, and intensive care physicians who provide care to stem cell transplantation and cellular therapy pediatric and young adult patients will have an opportunity to learn and discuss evidence-based management of severe complications associated with these potentially high-risk treatments.

Participants can choose to attend the event on [March 24](#) or [March 26](#), and is limited to 50 participants per session to ensure a quality training experience. Two additional registration dates will be announced in April. The workshops will be broadcast from [The University of Texas MD Anderson Cancer Center](#). The online format presented at the Simulation Center will provide a mixed model experience consisting of a live didactic portion via videoconferencing and virtual simulation platform for patient cases.

Broadly, the sessions will cover:

- Acute Kidney Injury and Fluid Overload: Presenters are [Sangeeta Hingorani, M.D., M.P.H.](#), professor of pediatrics at the University of Washington School of Medicine and an attending physician in the Division of Nephrology at Seattle Children's Hospital; and [Theresa Mottes, M.S.N., A.P.R.N., C.P.N.P.-A.C., C.D.N.](#), acute care nurse practitioner in the Department of Nephrology at Ann & Robert H. Lurie Children's Hospital of Chicago.
- Sinusoidal Obstructive Syndrome: [Selim Corbacioglu, M.D.](#), professor of Pediatric Stem Cell Transplantation at the Children's Hospital of Regensburg and leading researcher in the field, will discuss evidence-based diagnosis and management of this condition.
- CAR T Complications: Recognizing clinical manifestations of cytokine release syndrome and immune effector cell associated syndrome (ICANS). Presenters are members of MD Anderson Children's Cancer Hospital's pediatric SCT-CT team. They are [Sarah Featherston, B.S.N., R.N. C.P.N., B.M.T.C.N.](#), lead transplantation coordinator; [Dristhi Ragoonanan, M.D.](#), advanced fellow focusing on pediatric SCT-CT, and [Kris Mahadeo, M.D., M.P.H.](#), associate professor of Pediatrics and SCT section chief, who are coauthors of a publication in press regarding joint academic society guidelines for the care of pediatric immune effector cell and cancer immunotherapy patients.
- Virtual Reality Simulation Learning Sessions: Virtual reality (VR) technology will provide an immersive experience to the learners for two clinical cases. Group debriefing and panel discussion will follow the VR simulation.
- Question and answers session on having difficult conversations with patients and families: Facilitated by [Karen Moody, M.D., M.S.](#), associate professor of Pediatrics, and director of the Children's Cancer Hospital's Supportive and Palliative Care Program. She also is the program director of the [Pediatric Palliative Care Hospice and Palliative Medicine Fellowship](#).

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