Mattel Children's Hospital UCLA

Pediatric Blood and Marrow Transplant Program offers new therapies for cancer and blood disorders

Blood and marrow transplantation (BMT), also called stem-cell transplantation, can be a lifesaving treatment option for children with cancers and blood disorders, including leukemia, lymphomas, neuroblastomas and sickle cell disease, as well as disorders of metabolism and the immune system.

BMT replaces stem cells — blood-cell precursors found in hematopoietic (blood-cell-forming) bone marrow — injured or destroyed by disease, chemotherapy or radiation treatment. After entering the bloodstream via IV catheter, the stem cells make their way to the bone marrow. In a process called engraftment, they begin to produce new blood cells, restoring the patient's hematopoietic and immune system.

UCLA was one of the first centers worldwide to successfully perform a pediatric stem-cell transplant. The UCLA Pediatric Blood and Marrow Transplant Program was formally established in 1973.

To date, nearly 1,000 children from around the globe — many with the most complex, rare or severe types of cancers and blood disorders — have had stem-cell transplants performed at UCLA. Success rates and donor-match rates meet or exceed the national averages despite the increased complexity of cases.

Patients benefit from expanded donor pool

"Children without traditional marrow donors can increasingly find transplant donors today thanks to alternative donor transplants," says Theodore B. Moore, MD, director of the Pediatric Blood and Marrow Transplant Program and division chief of Pediatric Hematology/Oncology. "Advances in drug therapy that prevent the patient from rejecting donated marrow have made possible haploidentical bone-marrow transplants — using half-matched cells from a first-degree relative. And because an infant's immune system is immature, umbilical-cord blood transplantation requires less precise recipient tissue matching.

"The outcomes associated with these alternative donors for the treatment of hematologic malignancies and non-malignant hematologic disorders have reached the point where they are nearly the same as transplants from closely matched sibling donors," says Dr. Moore. "Today, we rarely have to turn away someone who needs a transplant for lack of a donor. Most importantly, we offer the chance for a cure to many children who previously did not have one."

A full spectrum of transplant options

The transplanted stem cells may be harvested from the patient's own body (autologous) or from a related or unrelated (allogeneic) donor matched by genetic markers or human leukocyte antigens (HLAs). These transplants can be collected from the bone marrow or umbilical-cord blood or mobilized from circulating blood.

UCLA has been a pioneer in many alternative transplant protocols including the use of haploidentical bone-marrow transplantation (hBMT). hBMT is a transplant method in which a half-matched, first-degree relative can donate stem cells if a matched related or unrelated donor cannot be found.

UCLA physicians can now identify an appropriate donor for almost every patient.

Developing new therapies

UCLA physician-scientists continue to break new ground in finding ways to improve survival rates and decrease complications in children undergoing BMT treatment.

UCLA has been a leader in alternative stem-cell therapies, umbilical-blood transplantation and immune-based therapies. As one of the original participants in the Cord Blood Transplantation Study (COBLT) funded by the National Heart, Lung and Blood Institute of the National Institutes of Health, UCLA helped pioneer the use of unrelated marrow donors for children who lacked a family match.

Ongoing research includes gene-therapy techniques in which bone-marrow stem cells produce immune cells that will seek out and destroy leukemia or lymphoma cells and two new clinical research studies that involve the use of haplotypic (DNA-related) donor hematopoietic stem cells for severe sickle-cell disease and the use of placental stem cells to augment engraftment of placental cord-blood cells.

Unique clinical care collaboration

Pediatric Blood and Marrow Transplant Program doctors, nurses, physician assistants and transplant coordinators are dedicated to the care of children undergoing BMT. The BMT team also includes social workers, psychologists, nutritionists and patient educators working together to provide the best care available to patients and their families in a comfortable, supportive environment.

A unique aspect of the UCLA program is the seamless integration of our distinct Pediatric Blood and Marrow and Adult Hematologic Malignancy/Stem Cell Transplantation programs into a collaborative group composed of more than 15 nationally and internationally recognized BMT specialists.

The physicians meet weekly to discuss clinical care and research initiatives, evaluating each patient's progress and prognosis.

Dedicated to exceptional and innovative patient care, research and education for children requiring blood or bone-marrow transplantation, the Mattel Children's Hospital UCLA program is one of the largest and longest-running in the world.

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