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Progress toward curing HIV with cord blood transplantation

In 2007 a patient who was infected with HIV and who also had acute myelogenous leukemia received a hematopoietic cell transplant using stem cells from an adult donor who had a homozygous CCR5 mutation (CCR5-/-). Persons with this mutation are known to be resistant to infection by HIV and, indeed, the patient was cured of his HIV as well as leukemia. This patient remains the only person to have been cured of HIV. This cure has not been repeated because the CCR5-/- mutation is very unusual and transplants using stem cells from adults require a very close HLA match between donor and patient. Cord blood transplants require significantly less stringent HLA matching, and thus it is more feasible to transplant HIV-infected patients with CCR5-/- donor cells from cord blood. Combined haploidentical and cord blood (haplo/cord) transplantation eliminates two problems associated with cord blood transplants, i.e., prolonged time to engraftment and cell dose needs. Inventories of hundreds of cryopreserved CCR5-/- cord blood units have been developed and are now available for transplantation, and one such transplant was performed in 2017. Further, an estimated 800,000 cryopreserved cord blood units exist worldwide which indicates that transplantation with CCR5-/- cord blood units is feasible for large numbers of patients. Cure of HIV is very important because even patients who are adequately treated with antiretroviral drugs for HIV are not protected from serious adverse effects of long-standing HIV infection, including the life-long stigma associated with the infection. Cure by transplantation is also economically beneficial because the estimated lifetime cost for persons who become infected with HIV at age 35 is ~\$326,500. A rigidly held opinion by many is that patients with HIV should not be transplanted with intent to cure the infection unless they also have an underlying indication for a transplant such as leukemia. However, even in this antiretroviral era, thousands of patients die of HIV annually, and it is our opinion that the serious adverse effects of long-standing HIV are greater than the adverse effects of haplo/cord transplants of young HIV-infected patients who have no co-morbidities. The time has come to accelerate research on this topic of tremendous public health potential including transplantation of CCR5-defective cells, especially those derived from cord blood.

Biography

Petz Lawrence serves as Chief Medical Officer of StemCyte, Inc. He serves as a Co-Medical Director of StemCyte International Cord Blood Center (US) of StemCyte, Inc. He is a founder and organizer of the annual International Symposium on Cord Blood Transplantation. He served as a Director of Transfusion Medicine and Professor of Pathology and Laboratory Medicine of UCLA Medical Center. He served as Section Head of Hematology and Director of the Department of Clinical and Experimental Immunology of City of Hope National Medical Center. He served as Chairman of Advisory Board of StemCyte, Inc. He has received almost all the highest honors in transfusion medicine, including the Emily Cooley Award and Morten Grove-Rasmussen Memorial Award from the American Association of Blood Banks, the Transfusion Medicine Academic Award from National Heart, Lung and Blood Institute, the Owen Thomas Award from the California Blood Bank Society. He received Tibor Greenwalt Memorial Award and Lectureship for 2006. He is a Diplomate in Internal Medicine and subspecialty board Diplomate in Hematology.

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