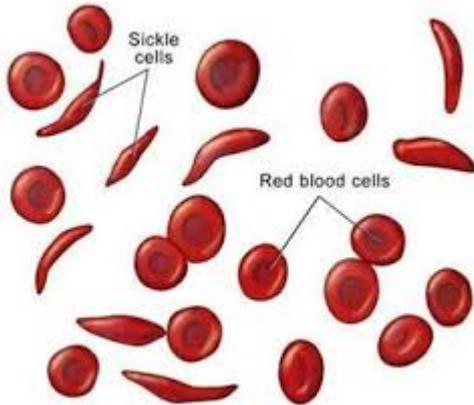


Groundbreaking Sickle Cell Study Uses Stem Cells with Adults

By Gil Ross — September 17, 2015



Sickle-cell disease, or SCD, is a hereditary (genetic) aberrancy among Africans and people of African descent. About one in 500 Afro-Americans have the condition. Normal hemoglobin, which is essential for oxygen transport via the red blood cells, is coded for by 2 hemoglobin-A genes, so normals are AA.

credit (left): oncofertility.northwestern.edu

But a new, small study from the University of Illinois reports on the amazing success among a small group of SCD adults with only minimal chemotherapy- and radio-therapy preparation for stem-cell transplantation.

First, some background. One sickle gene confers the genotype SA; people with this "sickle trait" often live quite normal lives, except for problems that sometimes occur when they are in a low-oxygen situations (airplane travel, mountain climbing) and under cardiovascular stress.

On the other hand, SCD (or sickle-cell anemia), with the double-dose genotype SS, is a devastating disease, characterized by frequent painful episodes of arterial occlusions due to the "sickling" (clumping) of SS-containing hemoglobin. Treatment with transfusions, painkillers and urea helps ameliorate the painful "sickle crises" but life expectancy is generally markedly foreshortened.

With good modern care most SCD patients now live until about 50, but they are often quite debilitated long before that. Previously, children have been, in effect, cured via chemotherapy- and radiation-induced destruction of their own immune system and bone marrow/blood forming cells, followed by bone marrow or stem-cell transplantation.

A group of transplantation researchers based at the University of Illinois/Chicago, led by Damiano Rondelli, MD,

Director of the Stem Cell Transplantation Program, have apparently cured 12 adult patients of sickle cell disease using a unique procedure for stem cell transplantation from healthy, tissue-matched siblings.

The [groundbreaking study](#), ^[1] published online in the journal *Biology of Blood & Marrow Transplantation*, is entitled "Non-Myeloablative Stem Cell Transplant with Alemtuzumab/Low Dose Irradiation to Cure and Improve the Quality of Life of Adults with Sickle Cell Disease."

Say what? People-friendly translation: the authors transplanted stem cells from 13 tissue-type matched sibling donors into recipients with SCD, aged 17-40. These adult patients received only a brief course of immunosuppressive drugs just before the transplant procedure, avoiding the onerous and complication-ridden immune-system destruction given to children better able to tolerate it. They also received a very low dose of total-body irradiation at the same time.

All 13 patients tolerated the procedure well and those who continued to take immune-suppressing drugs for at least one year thereafter eliminated the abnormal SS blood cells and were found to have only normal AA-containing hemoglobin in their red blood cells. One patient who failed to take the year-long medications did relapse to SS cells and symptoms of SCD recurred.

As the main author, Dr. Rondelli, [told](#) ^[2] *Medical Xpress* ^[2]: "Now, with this chemotherapy-free transplant, we are curing adults with sickle cell disease, and we see that their quality of life improves vastly within just one month of the transplant. They are able to go back to school, go back to work, and can experience life without pain. Our data provide more support that this therapy is safe and effective and prevents patients from living shortened lives, condemned to pain and progressive complications."

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[1] <http://www.ncbi.nlm.nih.gov/pubmed/26348889>

[2] <http://medicalxpress.com/news/2015-09-ui-health-validates-sickle-cell.html>