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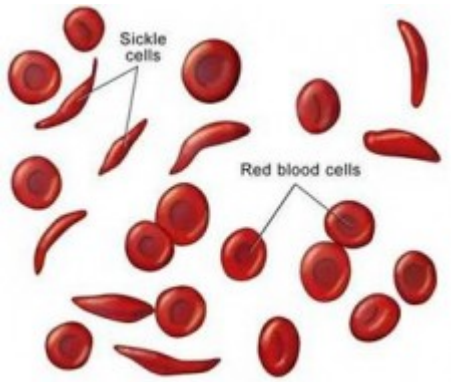
Bone marrow transplantation seems to help adults with sickle cell disease

By ACSH Staff — July 2, 2014



A small study conducted by the National Institutes of Health [1] shows bone marrow transplantation as an effective means to treat severe sickle cell anemia. The disease, which is prevalent among people of African descent, affects about 100,000 people (Afro-Americans, by and large) in the United States and millions across the world. Sickle cell anemia is a genetic condition that targets oxygen-carrying hemoglobin in red blood cells, producing abnormal crescent shaped or sickle cells. The rigidity of these cells in turn impedes normal blood flow, leading to health problems, such as anemia, severe episodes of pain, organ damage, and infection. With standard treatment today, sickler are able to live into their fifties, sometimes longer (until two decades ago, survival for SS patients beyond age 20 was unusual).

However in severe cases, adult patients often fail to respond to typical pharmacotherapy regimens, and succumb to a rapidly debilitating condition. It is this scenario that prompted senior author and senior investigator Dr. John Tisdale to employ bone marrow transplantation in adults with severe sickle cell disease.

Patients were not responding to the standard drug treatment called hydroxyurea. As such, Tisdale and his colleagues looked to a common procedure used for children with sickle cell disease. This involves exploiting chemotherapy and radiation to destroy host marrow and replacing it with healthy donor marrow. While it poses risk of toxicity in adults, bone marrow transplantation is an option for children because their bodies are better able to tolerate it. Unlike the technique used in children, researchers modified the procedure to only partially eradicate host bone marrow instead of entirely depleting it. In this way, less donor marrow is necessary and it can effectively outlive the patient s own sickle cell-derived marrow.

The study, published in the JAMA [2], included 30 patients, aged 16 to 65, who exhibited the homozygous SS (severe) sickle cell disease phenotype. As of October 25, 2013, successfully transplantation was evident among 26 (87%) patients. Moreover, 15 of the participants were able to come off immunosuppressant medication one year later. Authors of the study comment,

Engrafted patients continued to be disease free and without graft vs. host disease.

The success of this research paves the road for both clinicians and their adult patients with sickle cell disease in the future. Should standard treatment fall short, this simplified bone marrow transplantation is available as an option although retrieving compatible donor marrow presents a complication in the future because sibling marrow is required to carry out the technique. The genetic disposition of sickle cell disease lends to futile odds, as less than 1 in 4 patients have brothers or sisters that make a good match. As such, researchers are investigating whether other close relatives can serve as viable donor options.
