

# Sickle Selections

a quarterly newsletter from the University of Rochester Sickle Cell Program

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## Two Recent papers on Successful Treatments for Sickle Cell Anemia

### Hydroxyurea for Treatment of Severe Sickle Cell Anemia: A Pediatric Clinical Trial

Hydroxyurea (HU) enhances the synthesis of fetal hemoglobin (HbF) and can improve the clinical course of some adult patients with sickle cell anemia (SCA). In a randomized trial, the biologic effects and the clinical benefit of HU in children and young adults with severe SCA were studied. Twenty-five patients (median age, 9 years) were randomized to receive either HU (at the initial dosage of 20 mg/kg/d) or a placebo for 6 months and were then switched to the other arm for the next 6 months.

Of the 25 patients initially selected 22 were evaluable (median age 8 years).

Among these 22 patients significant increases in HbF and MCV occurred during the HU treatment period. The white blood cell and reticulocytes counts decreased significantly, but these changes were not clinically relevant.

Sixteen of the 22 patients (73%) experienced a complete disappearance of events requiring hospitalization. The number of days of hospitalization as well as the number of hospitalizations for patients on HU, as compared with those for the patients receiving placebo, were significantly reduced.

The authors report no clinically relevant toxicity associated with HU therapy and conclude that treatment with HU in children and young adults is feasible, well-tolerated, and improves the clinical course of Sickle Cell Anemia. Long term effects require further investigation.

*Ferster, A., Vermeylen, C., Cornu, G. et al Blood 88:1960, 1996*

### Bone Marrow Transplantation for Sickle Cell Disease

Twenty-two children less than 16 years of age who had symptomatic sickle disease received marrow allografts from HLA-identical siblings between September 1991 and April 1995. Twenty of the 22 patients survived. (Two died of central nervous system hemorrhage or graft-versus-host disease.)

Sixteen of the 20 surviving patients had stable engraftment of donor hematopoietic cells. In 3 patients the graft was rejected and sickle cell disease recurred; in a fourth patient a graft rejection was accompanied by marrow aplasia. One of the 16 patients with engraftment had a stable mixed chimerism.

Kaplan-Meier estimates of survival and event-free survival at 4 years were 91% and 73% respectively.

Among patients with a history of acute chest syndrome, lung function stabilized; among patients with prior central nervous system vasculopathy who had engraftment, stabilization of cerebrovascular disease was documented by magnetic resonance imaging.

*Walters, M.C., Patience, M. et al N Engl J Med 1996;335:369-76.*

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