

Sickle Selections

a quarterly newsletter from the University of Rochester Sickle Cell Program

October 2001

Highlights from the National Sickle Cell Disease Program Conference of April 2001

Hydroxyurea

Hydroxyurea (HU) is a medication that has been found to reduce the incidence of vaso-occlusive crisis pain as well as chest crises in adults and children with sickle cell disease. Its efficacy has been linked to its ability to raise the fetal hemoglobin level and reduce the white cell count.

Further follow-up from the multi-center Phase I-II pediatric hydroxyurea trial (HUG-KIDS) was presented at the National Sickle Cell Disease Program Meeting. Growth characteristics of patients prior to the initiation of hydroxyurea (HU) therapy and during treatment with the maximum tolerated dose (MTD) were reported. Sixty eight children and adolescents (ages 5.2 to 16 y) had serial height, weight, and Tanner Stage measurements performed. Growth data from 1533 children from the Cooperative Study of Sickle Cell Disease (CSSCD) were used for comparison. Height and weight gain before HU therapy was not significantly different from that on treatment. When compared with those from the CSSCD, greater height and weight gains were noted in boys over most age periods. Ages of attainment of Tanner Stages II-IV were comparable to those reported by the CSSCD. In all, the study concluded that treatment for 1 year at MTD had no adverse effect on height and weight gain or pubertal development in school aged children with sickle cell anemia.

Dr. Ohene-Frempong et al reported results from experiments wherein infant mice were exposed to hydroxyurea for 5 days from day 3 of life (corresponding in brain development to a 6 mo old human infant) to day 8 (comparable to a human 2 y old). They demonstrated adverse effects on growth of the brain and other organs. Since the dose of 300 mg/kg/day was similar to a 30-40 mg/kg/day oral dose in humans, it was felt that the safety of HU in very young children with SCD could not be assumed until studies specifically designed to assess its effects on the growth of the brain and other organs have proven such safety. (Of note, a multi-center trial of HU in very young children is currently being organized and its safety is of some concern to these investigators.)

Strong Children's Hospital

is currently treating a number of children with sickle cell disease and intractable pain with HU. The youngest patient that we have treated to date is 10 years old. We have had generally favorable results with patients reporting far less frequent pain episodes and requiring fewer hospitalizations. We follow all patients closely using a protocol designed to identify drug toxicity.



*Please contact either
Patricia Lamarche PNP*

or

Dr. Norma Lerner at 275-2981

*if you would like to have
a patient evaluated
for this therapy.*



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Sickle
Program
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Regarding treatment contact:

*Norma Lerner MD, Pat Lamarche R.N., C.P.N.P.,
Denise Houston, Outreach Worker Department of Pediatrics 275-2981
Dr. Karen Kaplan, Department of Medicine 275-3761*

Regarding laboratory diagnosis, newborn screening and genetic counseling, contact:

*Peter Rowley MD, Sandra LaBella MS or Starlene Loader RN,
Division of Genetics 275-4602*