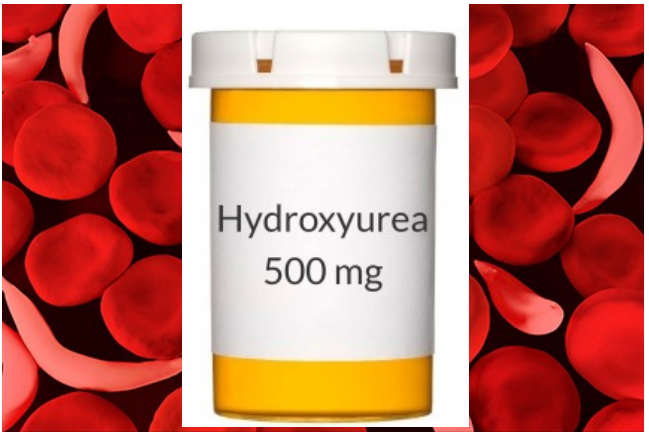
HYDROXYUREA AND SICKLE CELL DISEASE

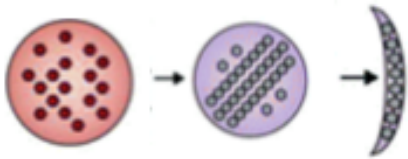


SCU
SICKLE CELL UNIT

THE PROBLEM

Persons with sickle cell disease (SCD) make abnormal sickle (banana) shaped red blood cells. Sickle red cells die off quickly in the circulation (they live only ~ 10-20 days). They are also stiff and sticky, tending to clump together and block the flow of blood . This causes anaemia ("weak blood") and other problems such as bone pain, acute chest (like a pneumonia) and stroke.

Red cells contain haemoglobin (Hb), a protein, which carries oxygen around the body. Normal red cells contain Hb A. Sickle red cells contain Hb S. Inside the red cell, Hb S can form strands/chains, This makes the red cell change shape from a round, soft cell to a sickle, rigid cell.



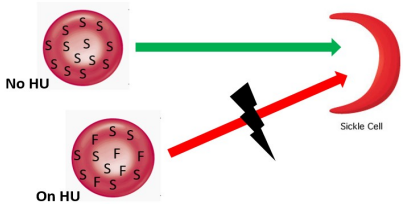
FETAL HAEMGLOBIN

Inside the womb, the fetus makes fetal haemoglobin (Hb F) which carries oxygen around. After birth, the body stops making Hb F and begins making Hb S or Hb A depending on whether you have SCD or not.

It has been shown that inside the red cell, Hb F can stop Hb S from forming strands / chains. This prevents the red cell from changing shape. The red cells remain round and soft and the blood stays healthier.

HYDROXYUREA

Hydroxyurea (HU) is a drug used to change how SCD affects a person. One the ways it works is by helping the body make more Hb F again.

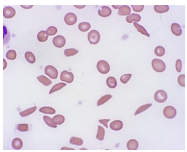


Imagine

Salt crystals (Hb S) in a shaker exposed to air will clump inside and will not pass through the shaker holes.

Adding a few rice grains (Hb F) to the shaker will prevent the salt (Hb S) from clumping and allow it to pass through the shaker holes

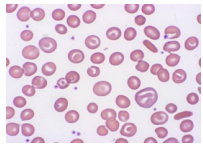
Before HU



Lots of sickle cells

Few red cells (anaemia)

After HU



Less sickle cells

More red cells (less anaemia)

Who can take HU?

Adults and children with SCD can take HU. Some of the common problems that HU is given for include:

1. Recurrent bone pain crisis
2. Recurrent acute chest syndrome
3. Stroke in children with SCD

How is HU given?

HU comes as a tablet or capsule. It can be made into a medicine (liquid) for children. It is taken once daily by mouth. It only works if taken regularly.

Common Side Effects

1. Fall in white cell and platelet count which may increase risk of infection or bleeding—stopping the drug for a short period will reverse this
2. Nausea and tummy discomfort—taking the drug at night may help this
3. Thinning of hair
4. Nail or skin pigment changes

At this time there is no increased risk for cancer or of fertility concerns when taking HU

Studies have shown that HU is <u>safe</u> for use in persons with SCD <u>providing</u> that persons who take HU receive <u>regular</u> medical care to monitor for side effects of the drug.

Benefits of HU

Studies worldwide have shown that HU can help persons with SCD in many ways. These include:

1. Fewer pain events
2. Fewer episodes of acute chest syndrome
3. Less need for transfusions and hospitalizations

In Jamaica, studies have also shown that

1. HU helps prevent recurrent stroke in children who have had a first stroke
2. HU can decrease the risk for children with SCD who are at high risk for stroke (based on Transcranial Doppler US)

Challenges to HU use

1. Frequent doctor visits—once per month at the start until the best dose of HU is found for you , after which visits occur every 3 months
2. Costs attached to blood tests—before starting to determine baseline and for monitoring while on HU. Getting your local hospital or health centre to assist with getting bloods done may help

Ensure that you or your child has a NHF card which can help with the cost of the drug

Living with SCD can be challenging. HU can help improve your quality of life—fewer sick days, less time off work / school—more time to lead a "regular" life

Discuss with your doctor if HU may benefit you or your child