NARROWING THE GAP…

The need for primary care providers for adults living with

SICKLE CELL DISEASE

Donell Ivy MD, MPH

Medical Officer
Division of Services for Children with Special Needs
Maternal and Child Health Bureau - HRSA

Treatment Demonstration Grant

- **Increase # of physicians caring for patients with SCD**
- **Increase # of physicians prescribing hydroxyurea**

Newborn Screening Grant

- **Locate patients - Community Health workers**
- **Get Connected – Over 6000 enrolled**

OBJECTIVES

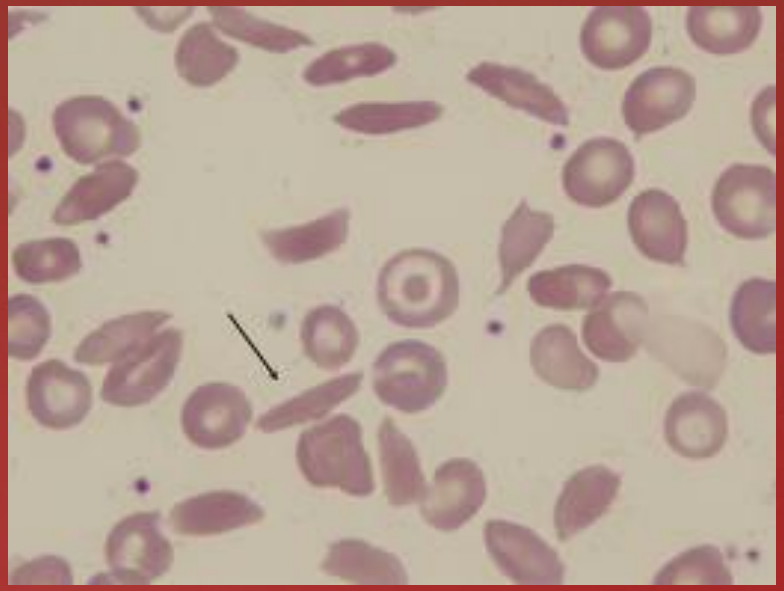
- Brief overview of your Organization
- History of Sickle Cell Disease
- Newborn Screening
- Challenges Patients face
- Resources you can provide
- Good News

YOUR MISSION

Brief Overview of Your Organization

History of SCD

James Herrick



”peculiar elongated and sickle shaped"
red blood cells in
"an intelligent negro of 20."

Internal Medicine 6 [1910]:517

Jeremy Irons

Chwechweechwe
(Ga)

Ahotutuo (Twi)

Nwiiwii (Faute)

Nuidudui (Ewe)

NEWBORN SCREENING

The New England
Journal of Medicine

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Volume 314 JUNE 19, 1986 Number 25

PROPHYLAXIS WITH ORAL PENICILLIN IN CHILDREN WITH SICKLE CELL ANEMIA

A Randomized Trial

MARILYN H. GASTON, M.D., JOEL I. VERTER, PH.D., GERALD WOODS, M.D., CHARLES PEGELOW, M.D., JOHN KELLEHER, M.D., GERALD PRESBURY, M.D., HAROLD ZARKOWSKY, M.D., ELLIOTT VICHINSKY, M.D., RATHI IYER, M.D., JEFFREY S. LOBEL, M.D., STEVEN DIAMOND, M.D., C. TATE HOLBROOK, M.D., FRANCES M. GILL, M.D., KIM RITCHEY, M.D., AND JOHN M. FALLETTA, M.D., FOR THE PROPHYLACTIC PENICILLIN STUDY GROUP

Gaston et.al.

PENICILLIN STUDY

	Penicillin	Placebo
Total #	105	110
Deaths	0	3
Infection	2	13

CHALLENGES

- Awareness – describe myths
- Young people don't know their trait status
- Only one disease modifying medication for 20 years (generic)
- No universal cure
- Pediatric vs adult care

(Insert slide of you walk)

MY PAIN
IS REAL!

Living Well with
SICKLE CELL
©

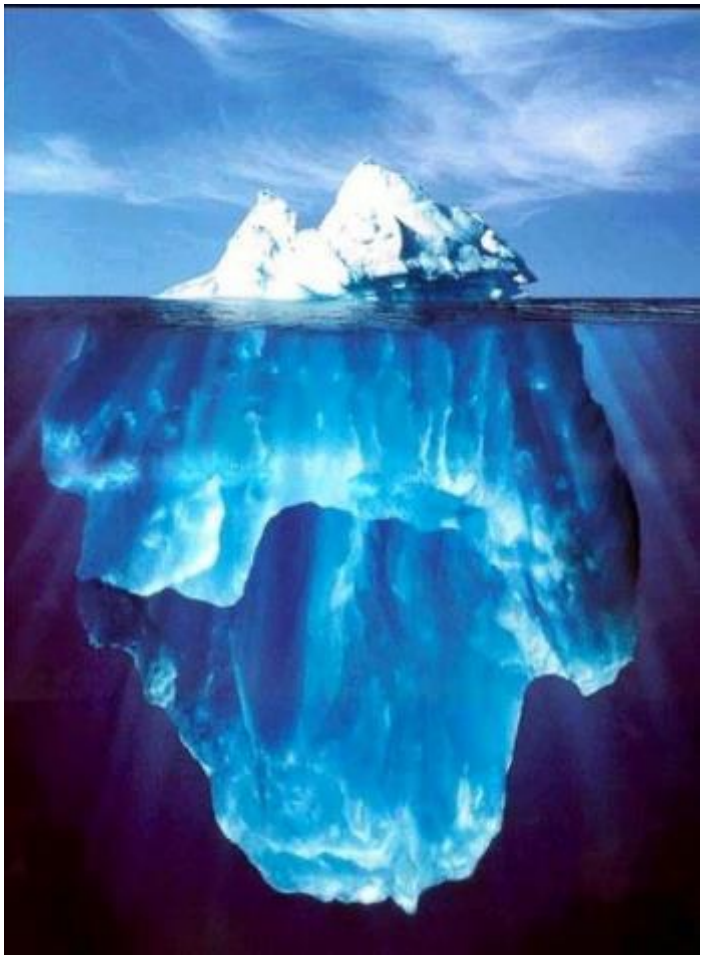
I'm Still Here!
Living with Sickle Cell Disease

SICKLE CELL DISEASE
It's not JUST a BLACK THING

The Iceberg of SCD Pain

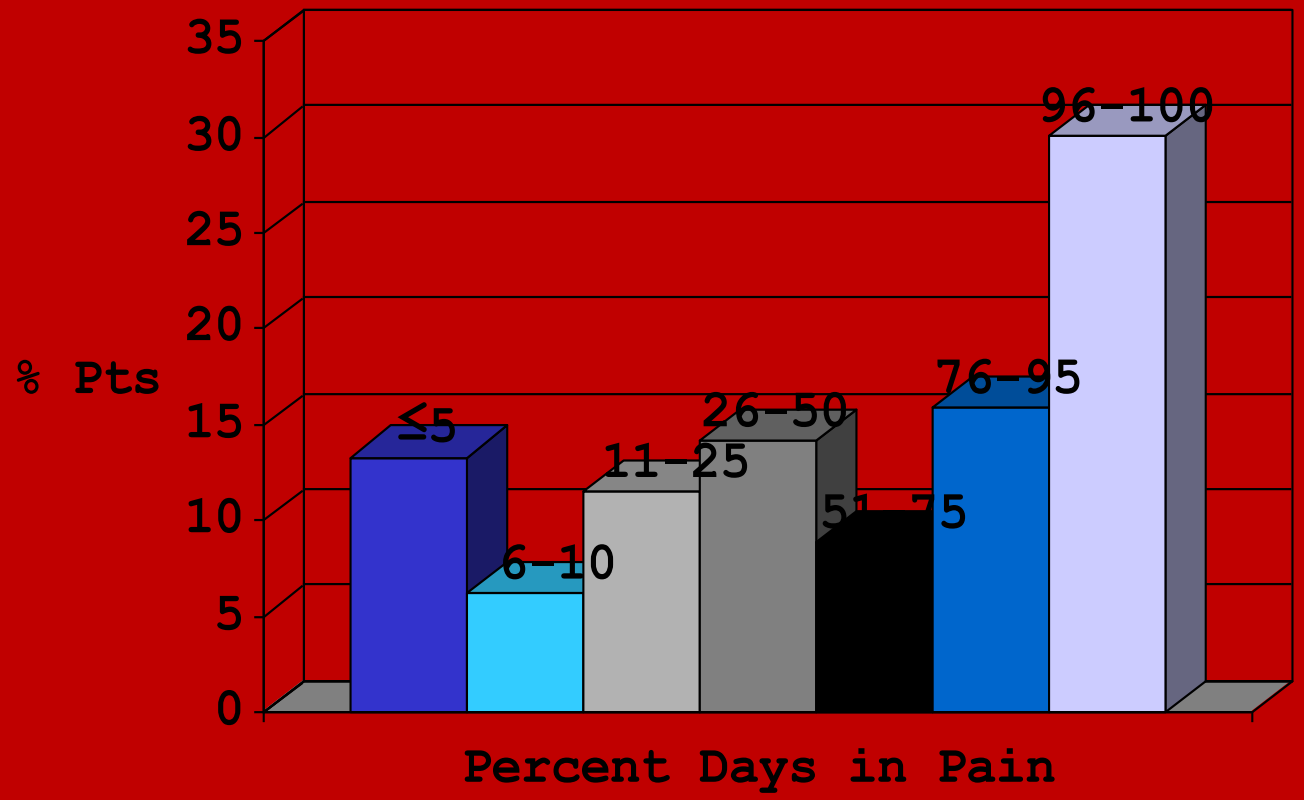
Wally Smith

- The largely “submerged” iceberg of pain in SCD is not seen by most doctors, but is managed outside of hospitals.
  - Most sickle cell pain, though severe, is not severe enough to be called “crisis” pain
  - Most crises are not associated with hospitalization



(Key point – when you see a patient in the ER you are seeing the tip of the iceburg)

Pain Frequency: Histogram, PiSCES



Key point: 30% of adult patients have pain all the time

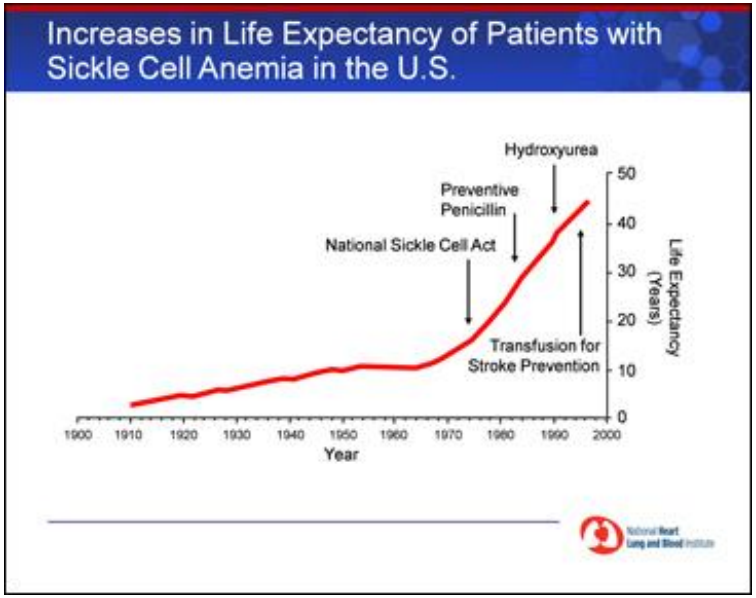
- 30% of subjects had pain nearly every day
- Only 13% of subjects almost never had pain

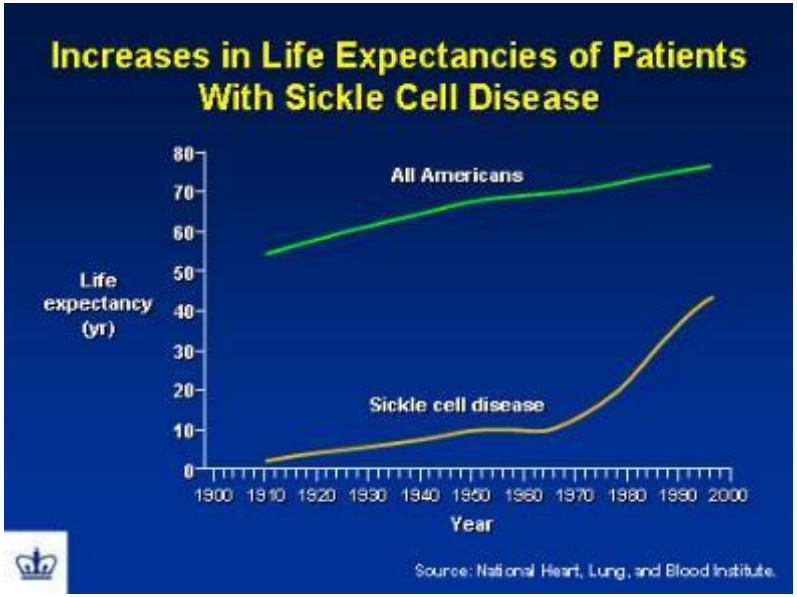
Wally Smith

Shirley Miller

I'm Still Here!
Living with Sickle Cell Disease

Manager - Adult sickle cell program
Carolinas Healthcare System





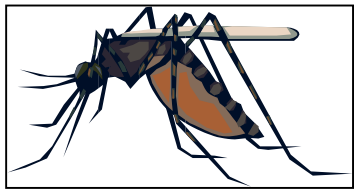
Quality of life!

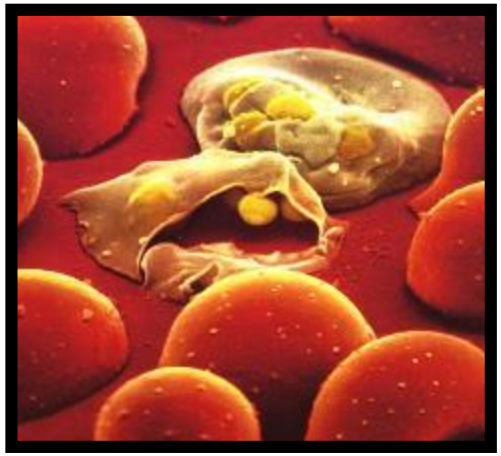
(With advances seen in the left hand slide we know patients are living longer an longer We are not just concerned about how long patients live but also about the quallity of their lives)

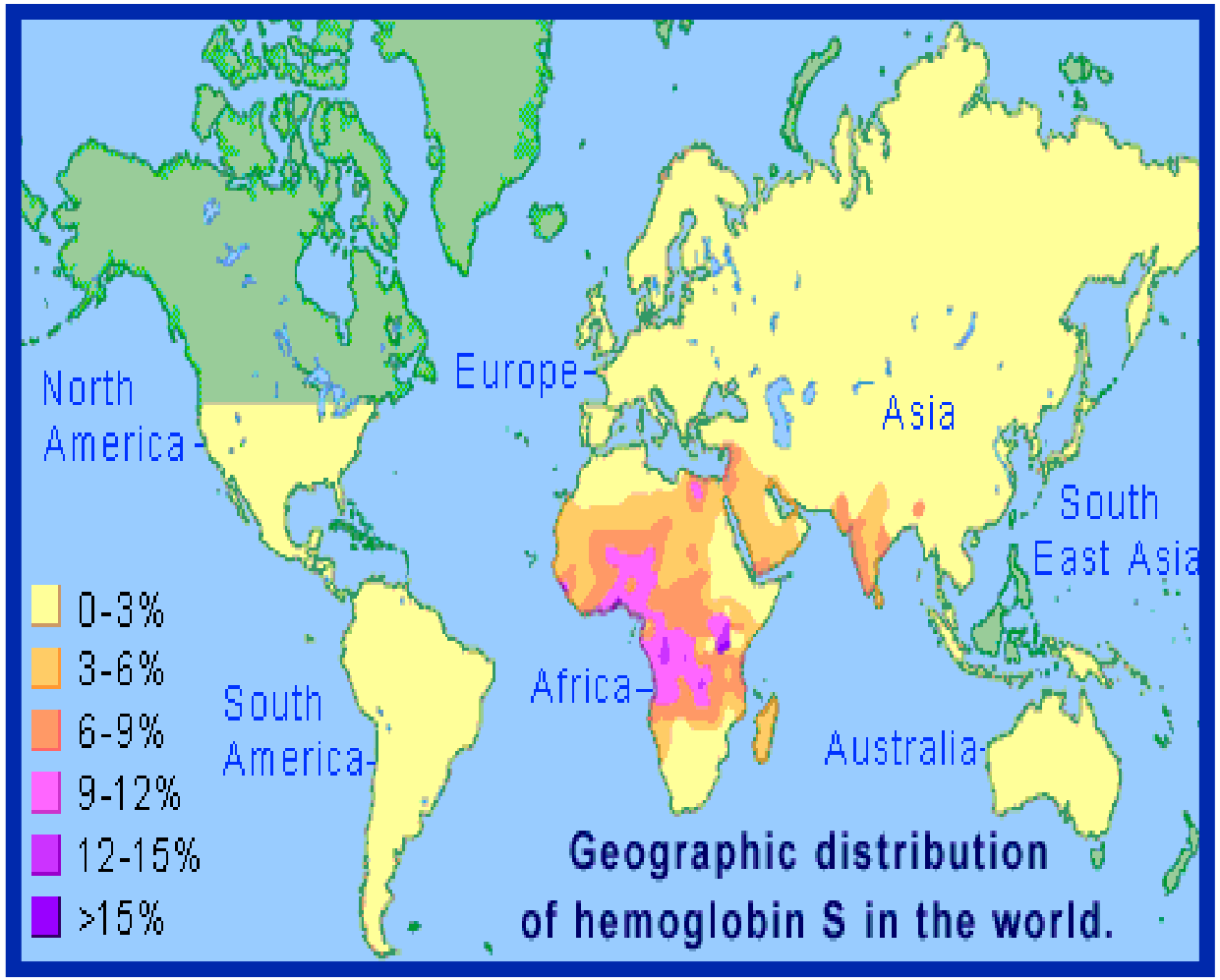
SICKLE CELL DISEASE

It's not JUST a BLACK THING

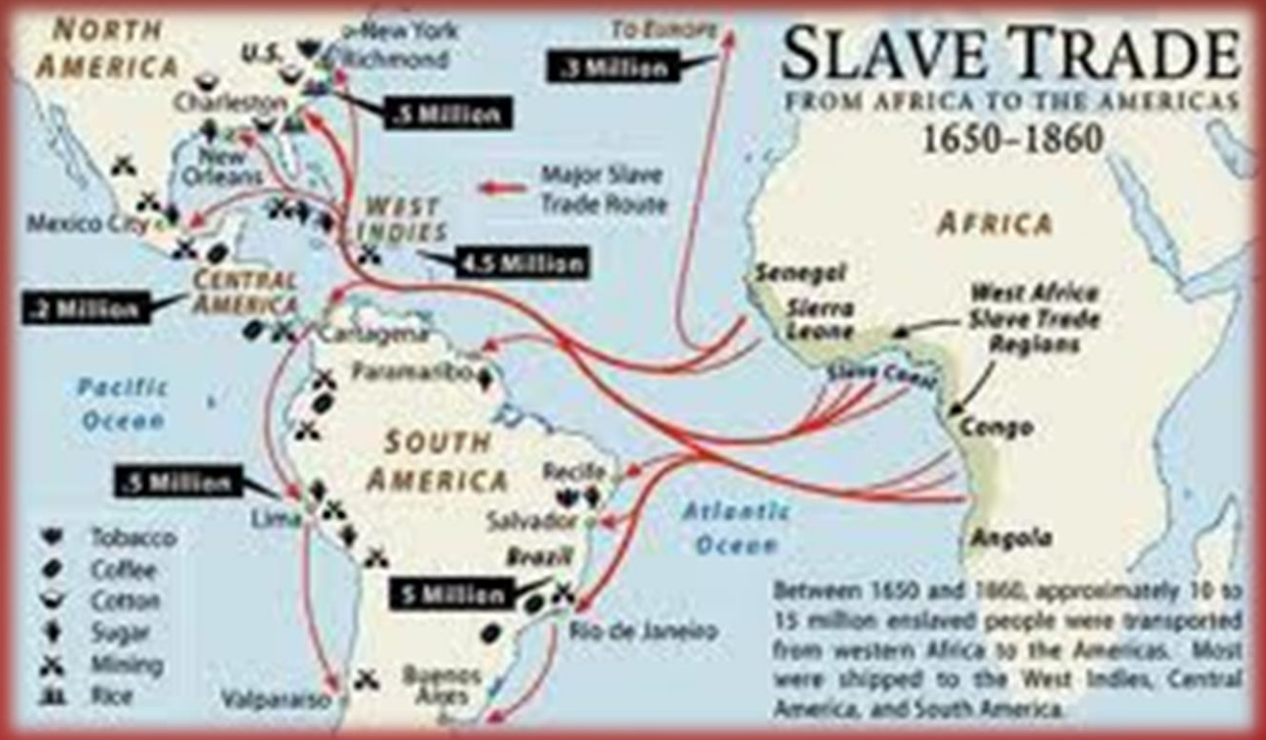
(Insert picture of
One of your patients
of course with their
Permission)






North America
Europe
Asia
South East Asia
Africa
South America
Australia
0-3%
3-6%
6-9%
9-12%
12-15%
>15%
Geographic distribution
of hemoglobin S in the world.

MALARIA

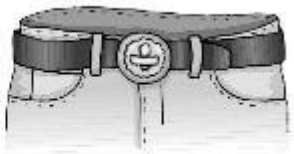

SLAVE TRADE
FROM AFRICA TO THE AMERICAS
1650–1860
NORTH AMERICA
U.S.
New York
Richmond
Charleston
New Orleans
Mexico City
.5 Million
Major Slave Trade Route
WEST INDIES
4.5 Million
CENTRAL AMERICA
.2 Million
To Europe
.3 Million
AFRICA
Senegal
Sierra Leone
West Africa Slave Trade Regions
Slave Coast
Congo
Angola
Cartagena
Paramaribo
Pacific Ocean
SOUTH AMERICA
.5 Million
Lima
Recife
Salvador
Brazil
5 Million
Rio de Janeiro
Buenos Aires
Valparaiso
Atlantic Ocean
Tobacco
Coffee
Cotton
Sugar
Mining
Rice
Between 1650 and 1860, approximately 10 to 15 million enslaved people were transported from western Africa to the Americas. Most were shipped to the West Indies, Central America, and South America.

CHALLENGES

- Awareness
- Young people don't know their trait status
- Only one disease modifying medication (generic)
- No universal cure
- Pediatric vs adult care

Do you have…
SICKLE CELL TRAIT?

…it's in your
"GENES"



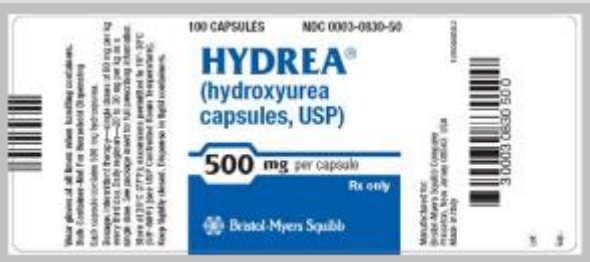
(The newborn screening result comes as a shock to them because one or both of the parent did not know yhey had SCT)

CHALLENGES

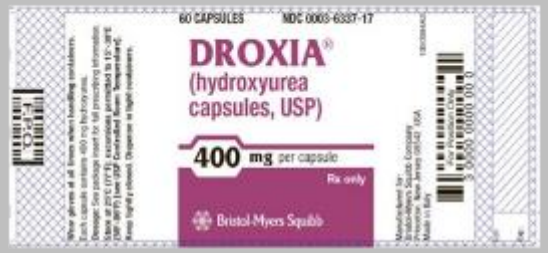
- Awareness
- Young people don't know their trait status
- For 20 years only one disease modifying medication (generic)
- No universal cure
- Pediatric vs adult care

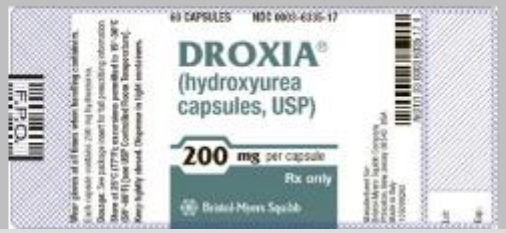
Hydroxyurea

Hydrea 500 mg

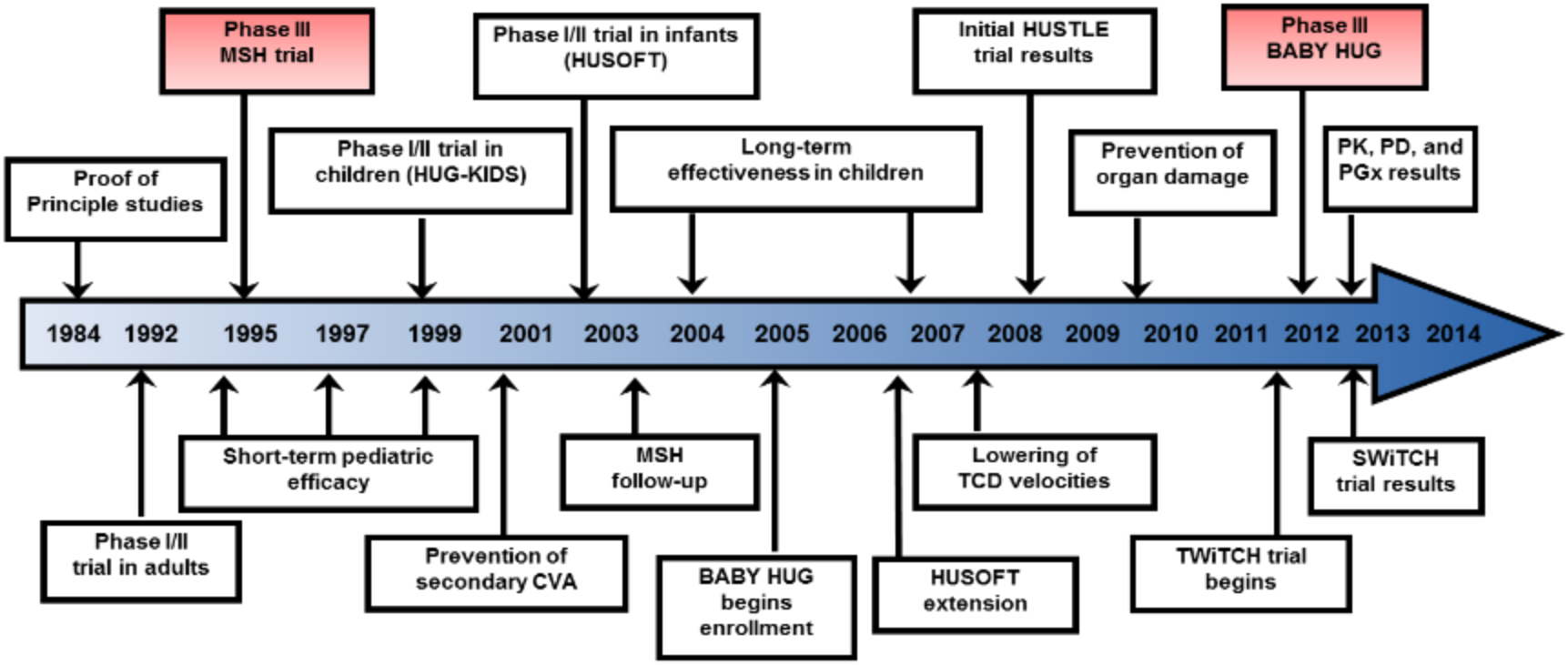


Droxia 200/300/400 mg





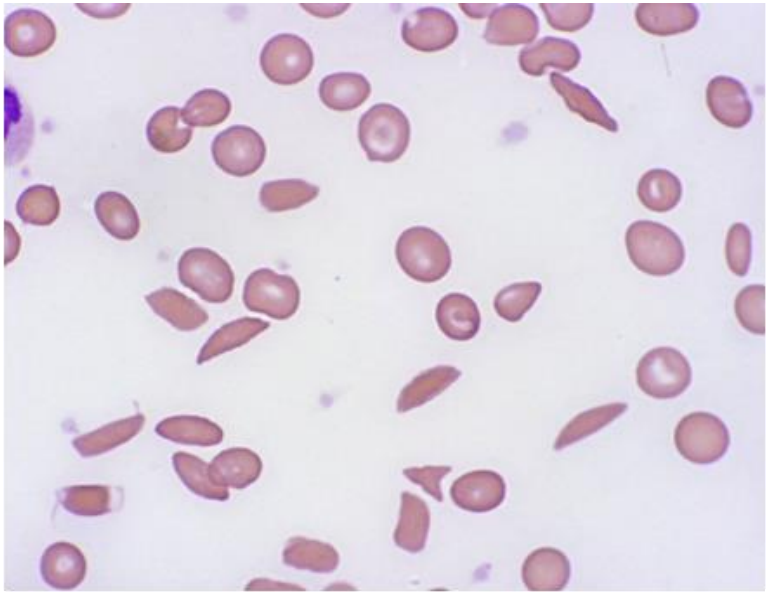
Hydroxyurea: 30 Years of Research



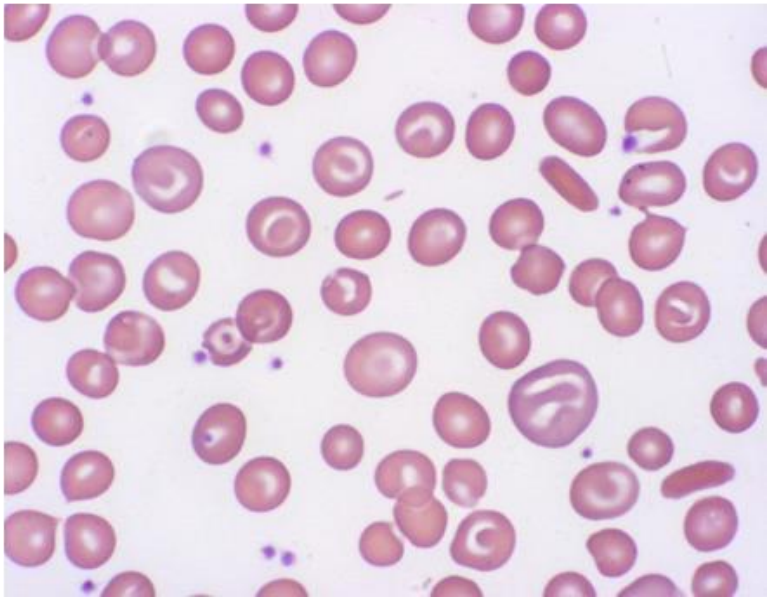
Patients are afraid to take it so describe
All of the research that went into getting
FDA approval including animal studies

Russell Ware MD

Before Hydroxyurea



Taking Hydroxyurea



Hydroxyurea

(hi-drok-see-yoo-ree-uh)

A Treatment Choice for Patients with Sickle Cell Disease

Is hydroxyurea safe?

What are the possible side effects?

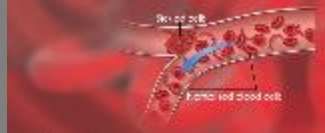
Does hydroxyurea cause cancer?

Is hydroxyurea safe during pregnancy?

Sickle Cell Disease Association of America
Michigan Chapter, Inc.

This information is for you if you are considering the choice to start or continue taking hydroxyurea.

What is hydroxyurea?



How does hydroxyurea work?

How has hydroxyurea helped people with sickle cell disease?

Here are the red blood cells of a patient before and after taking hydroxyurea.

Will I see the effects of taking hydroxyurea right away?

Is hydroxyurea a pain medication?

How long will I have to take hydroxyurea?

Is hydroxyurea a cure for sickle cell disease?

Does hydroxyurea work for everyone?

CHALLENGES

- Awareness
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- For 20 years only one disease modifying medication (generic)
- No universal cure
- Pediatric vs adult care
- Good News

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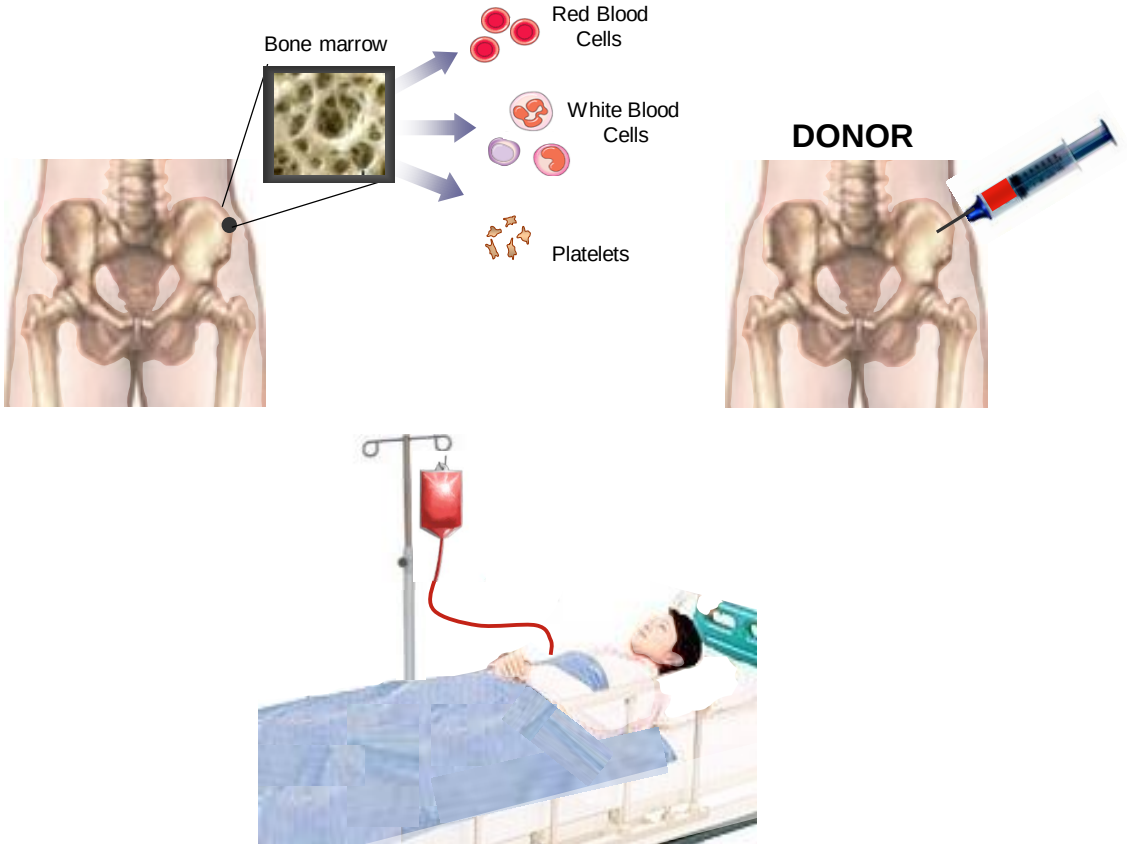


BONE MARROW TRANSPLANT (STEM CELL TRANSPLANT)

(Describe limitations of BMT)

CHALLENGES

- Awareness
- Young people don't know their trait status
- Only one disease modifying medication (generic)
- No universal cure
- Pediatric vs adult care – Transition

(There is a peak in mortality at the time of transition)

Good News

CLINICAL TRIALS

Cell adhesion

- Rivapensil – GMI 1070 (RESET)– interfere with adhesion to selectin
- Sel G2 – interfere with adhesion to selectin
- Poloxamer 188 (EPIC) – stabilizes damaged membranes making less sticky

Inflammatory pathways

- NKTT120 – iNKT cell inhibitor
- Regadenoson – iNKT inhibitor

Upregulation of hemoglobin F

- HQK-1001 – induce fetal hemoglobin
- panibinostat

Hemoglobin polymerization and sickling

- AES-103 – binds hemoglobin to prevent polymerization
- GBT440 – increased oxygen affinity

Nutrition

- Omega 3 Fatty Acid (SCOT Trial)

Platelet activation

- DOVE – (Prasugrel) – interferes with platelet response
- Hestia - Ticagrelor –Phase II

Celebrate the Launch of Endari
FDA Approved
On July 7, 2017 the U.S. Food and Drug Administration approved Endari®
Emmaus
Endari® (L-glutamine oral powder) for patients age five years and older with Sickle Cell Disease to reduce severe complications associated with the blood disorder.

FDA
BREAKTHROUGH THERAPY DESIGNATION
Global Blood Therapeutics' Voxelotor for sickle cell anemia
BIOPHARMAGLOBAL.COM

Genetic cure for SCD in the next 5 years!

Dr. Edward Benz Jr
Executive Director

CURE SICKLE CELL.

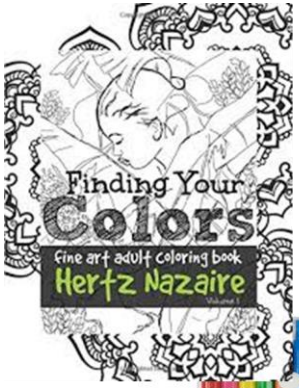
Gene Therapy & Gene Editing

NIH National Institutes of Health

LAMAR
ACADEMY

Walk With The 3bears

ST. VINCENT HOSPITAL


Finding Your
Colors
fine art adult coloring book
Hertz Nazaire

4000
3500
3000
2500
2000
1500
1000
500
Airlife

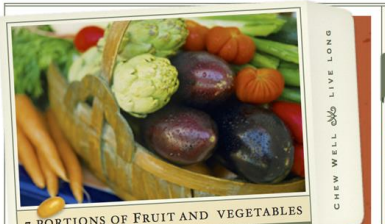
PHARMASOM

DR. ROWLEY'S

REVISED

3-4-4 NUTRITIONAL METHOD OF EATING

For more information, contact Cayenne Wellness Center at www.cayennewellnes.org :: 818 377 5120



Research shows that a diet consisting of whole foods which are plant-based, nutrient dense, and low-fat will prevent and often reverse most of the degenerative diseases that are killing us, and becoming more and more expensive to treat through drugs and surgery.

WHAT IS 3-4-4?

Three-four-four (3-4-4) stands for breakfast - lunch - and dinner. Three (3) items to eat for breakfast, four (4) items to eat for lunch, and another four (4) items to eat for dinner. It is an eating plan that is based on obtaining all the vitamins, minerals, other nutrients and phytochemicals necessary for optimum health.

In order to achieve a diet that encompasses all of the above, 3-4-4 was created so that one would not have to think about what particular food provides vitamin K and what particular food source would one obtain potassium. By eating the 3-4-4 way, you are guaranteed that each day, you have covered the gamut of all the food groups, vitamins and minerals.

How Can I Get Started on 3-4-4?

It's easy, really. 3-4-4 requires no special foods and has no hard to follow recipes. It requires eating 'real food'.

Breakfast: dried fruit, fresh fruit and grain.

Lunch: fresh fruit, green vegetable, orange vegetable and protein/ grain.

Dinner: Same as lunch

3-4-4 HAS MANY HEALTH BENEFITS

Benefits

Sickle Cell Disease

If you eat complex carbohydrates versus simple carbs, if you eat regular meals throughout the day, eat protein (but not too much) and healthy fats, you will do just fine.

3-4-4 and Sickle Cell

3-4-4 helps those with sickle cell disease. 3-4-4 is based on an eating plan rich in complex carbohydrates, protein and healthy fats (like olive oil). With 3-4-4, one eats three meals a day plus two snacks.

What You Need To Know About Sickle Cell and Diet

Research shows that children with SCD have decreased height and weight. Increased calories and protein needs, and deficiencies in zinc, folic acid, and vitamins A, C and E may factors.

1. **What to eat.** Vitamin C (kiwi and oranges), Vitamin E (mustard greens and sunflower seeds), Folic Acid (romaine lettuce, spinach, asparagus, black beans), Vitamin D (more sun exposure, salmon), Vitamin A (carrots, yams, sweet potatoes), spinach, kale), Magnesium (spinach, mustard greens, pumpkin seeds), Phosphorous (almonds, lentils), Zinc (spinach, broccoli, asparagus), Protein (beans, salmon, chicken), Fiber (cauliflower, broccoli, raspberries), Calcium (spinach, mustard greens, kale, oranges) and B12 (salmon).

ABOUT REFLEXOLOGY

Reflexology is the application of pressure to areas on the feet, hands and ears. Reflexology is generally relaxing and may be an effective way to alleviate stress.

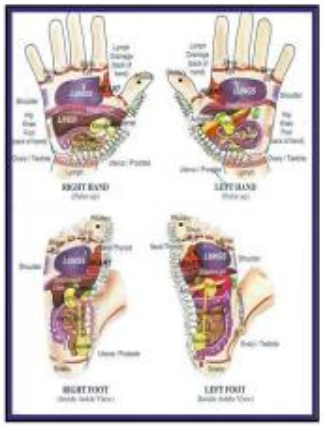
The theory behind reflexology is that these areas correspond to organs and systems of the body. Proponents believe that pressure applied to these areas affects the organs and benefits the person's health.

Reflexologists use foot charts to guide them as they apply pressure to specific areas. Sometimes these practitioners use items, such as rubber balls, rubber bands and sticks of wood, to assist in their work. Practitioners of reflexology include chiropractors, physical therapists and massage therapists, among others.

Several studies funded by the National Cancer Institute and the National Institutes of Health indicate that reflexology may reduce pain and psychological symptoms, such as anxiety and depression, and enhance relaxation and sleep. Studies also show that reflexology may have benefits in palliative care of people with cancer.

Reflexologists claim that reflexology also can treat a wide variety of medical conditions, such as asthma, diabetes and cancer. However, scientific evidence is lacking to support these claims.

Reflexology is generally considered safe, although very vigorous pressure may cause discomfort for some people.



MyMedSchedule Mobile

By MedActionPlan.com

- Print schedules that are easy to create, read, and update
- Keep track of all your medications
- Bring your pill schedule to all your medical and dental appointments
- Graph results to track progress
- Quick and easy data entry
- Receive reminders to take your medications by text or email
- Refill reminders help you reorder prescriptions before they run out
- Works with MedActionPlan.com if your provider has it.

Scan

Mango Health – Medication Manager

By Mango Health

Mango Health makes it fun and easy to manage your medications and supplements in order to reach your health goals. We help you take care of you.

- Reliable medication reminders
- Drug interaction warnings
- Points and rewards
- Daily Health Diary

Scan

MedCoach Medication Reminder

By GreatCall, Inc.

- Personal medication schedule
- Pill reminders and alerts when it's time to take your medications
- Log the pills you took
- Connect to your pharmacy to refill prescriptions
- Create a list of your medications for your doctor or

Scan

MediSafe Meds & Pill Reminder

By MediSafe Project

- Visual and super easy to handle medication reminder.
- Synchronizes the family's medicine pillboxes to 1 place.
- Reminds you to take medication on time, even if your device is asleep.
- Choose your medication reminder sound to make MediSafe a comfortable addition to your day.
- Rx refill reminds you when you need to restock.

Scan

Modi & Loiselle, 2016, Cincinnati Children's Hospital Medical Center©

VOICE Crisis Alert

VOICE Crisis Alert™

- A crisis tracking and communicating app
- VOICE stands for "Vaso-Occlusive Ischemic Crisis Event"
- Includes a pain tracker, a pain history chart, and the VOICE My Crisis feature, which allows SCD patients or caregivers to communicate pain/ crisis events to pre-entered contacts of their choice (family members, caregivers, healthcare providers, or friends)
- All data is kept on the device and will need to be reentered if the user changes devices

Sickle-O-Scope

- Daily diary to track illness and disease symptoms
- Users can evaluate their pain and fatigue levels over time
- Sharing data with a doctor is possible. Users can - on their own initiative - send data collected by the application to their attending physician.
- All data is kept on the device and will need to be reentered if the user changes devices

SickleWell

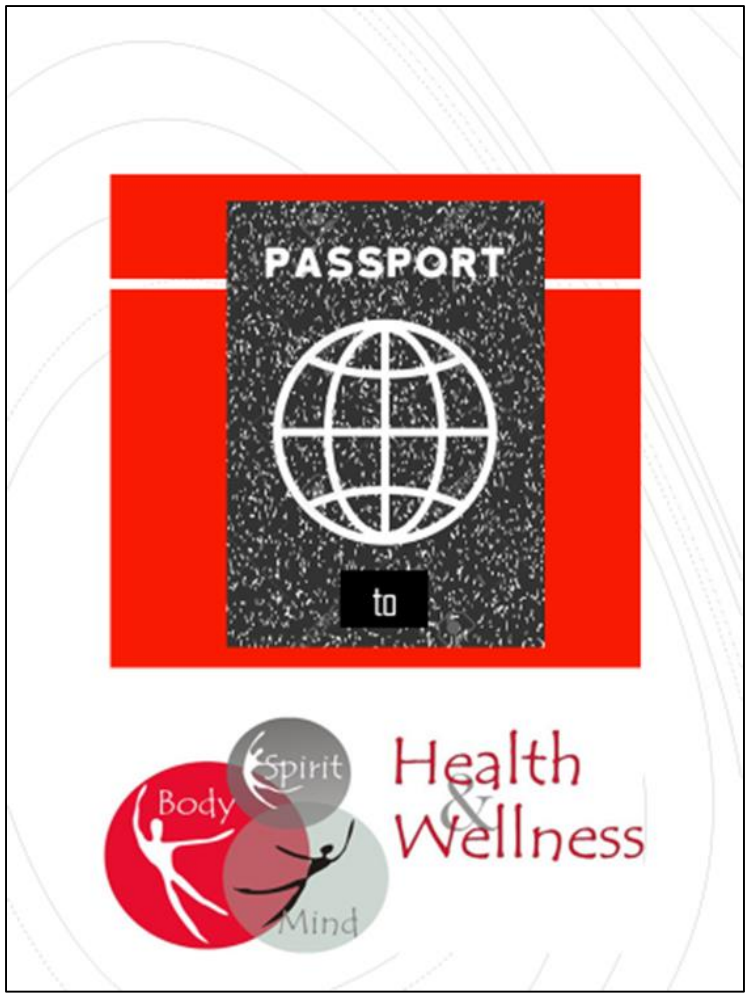
- Users can manage their disease using alerts, reminders, and education modules
- Available on iTunes store

Sickle Cell Anemia

- Contains information on sickle cell disease
- Causes
- Treatments
- Prevention

Sickle Cell Services

- Developed by The Sickle Cell Disease Association of America, Inc. (SCDAA) the app provides members of the SCD community access to a database of medical and support services.
- Also provides national news, live message feed from SCDAA, a messaging board and photo submission functionality



Type of sickle cell ____ SS ____ SC ____Sbeta thal zero ____ Sbeta thal plus

Other _____

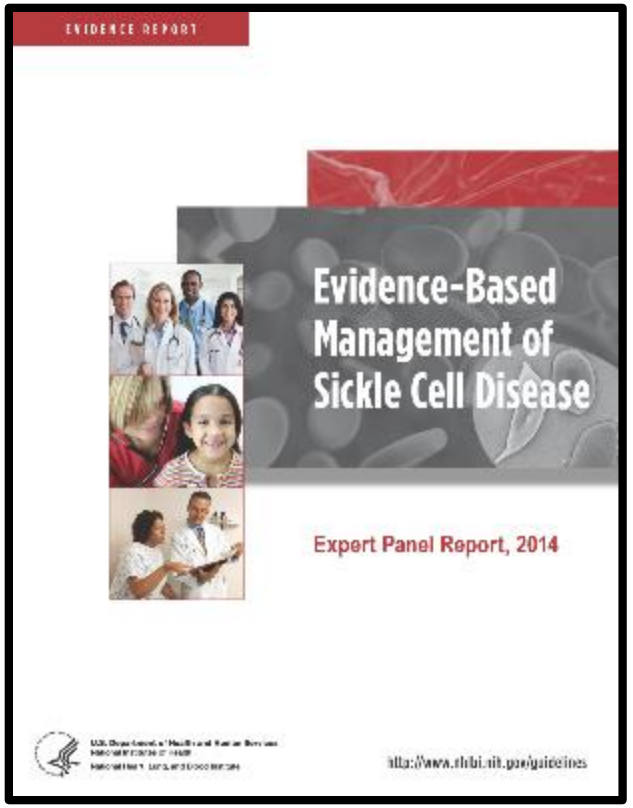
Usual Hemoglobin level _____

Blood Type _____

Allergies:

- Medications
- Hospitalizations
- Transfusion Hx
- Surgical Hx
- Diagnostic Studies
- Medical Appointments

NHLBI Guidelines



(Share link for guidelines and email
Address to get ASH guidelines
ASH will send you some for free
To give away as samples)

Lessons Learned

- Efforts to schedule presentations are labor intense
- The information is well received once you get your foot in the door
- Follow-up is key to ensuring patient access
- Engagement of other HCPs is important
  (eg, nurse practitioners and physician assistants)
- GBT can be helpful by helping you target the centers in your area that have the highest population of sickle cell patients

Current Practice:
Managing Complications

Managing wellness:
Keeping Patients Well

Gary Gibson
Julie Cantor