Cincinnati Comprehensive Sickle Cell Center



www.cincinnatichildrens.org

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From the Director's Desk

Clinton H. Joiner, MD, PhD

One hundred years ago, a Chicago physician, Dr. James Herrick, published a scientific paper describing the case of a young dental student from Granada, West Indies, named Walter Clement Noel. The young man had come into the hospital with symptoms of fever, jaundice and cough and was diagnosed with pneumonia. His lab tests showed he had anemia and Dr. Herrick described the appearance of his blood under the microscope as showing "peculiar elongated and sickle shaped red blood corpuscles (cells)." Dr. Herrick was baffled by the case, "no duplicate of which I have ever seen described." But soon many people began to recognize the various symptoms of sickle cell disease, and its genetic nature became understood as well.

Mr. Noel was unusual for a person with sickle cell disease in 1910 - he lived to adulthood. For many years, sickle cell disease was fatal in childhood for most. This began to change in the late 1960's and early 1970's as clinical care became more organized and standards for treatment were developed. One important breakthrough was newborn screening and the use of prophylactic penicillin, which has reduced mortality among young children dramatically since the 1980's. Next came transcranial Doppler ultrasound (TCD) exams to identify risk for stroke. Since the 1990's, chronic transfusions for children at risk for stroke has greatly reduced mortality. During this time, hydroxyurea therapy was also developed and has further prolonged the lifespan of those with sickle cell disease. Now more than 95% of children with sickle cell disease grow to adulthood and the life expectancy of adults with sickle cell is steadily increasing.

The future looks even more promising. Improved ways of performing bone marrow transplantation are being developed that will make this curative treatment safer and more widely available. Here at Cincinnati Children's Hospital Medical Center, we are excited about developing gene transfer therapy, which uses the patient's own cells to produce a hemoglobin that does not sickle. We hope to open a clinical study testing the safety of this new treatment in the upcoming year. Although it's not clear how long it will take to develop a reliable cure for sickle cell disease, it's a good bet it won't be another hundred years.



Gene Therapy Survey

Enclosed in this newsletter is an optional survey about gene transfer therapy for sickle cell disease.

Doctors at Cincinnati Children's are exploring improved ways to treat genetic diseases, including sickle cell disease. One method currently being studied is called "gene transfer". The hope is that we will be able to begin human clinical trials using gene transfer sometime in the next one to two years.

At this time we are only attempting to gather information from potential subjects, not actually open a research study.

There are two parts to this process.

The first part will be mailing out and/or inviting sickle cell patients at CCHMC and UC to visit the sickle cell website and complete a patient questionnaire about their or their family member's sickle cell disease and their interest in participating in a focus group regarding sickle cell gene therapy.

The second part is having the interested participants participate in a focus group and answer some basic questions about whether or not they would be interested in participating in this research study in the future.

A Parent's Overview...

on the 4th Annual Sickle Cell Disease - Research and Educational Symposium & Grant Writing Institute and Annual National Sickle Cell Disease Scientific Meeting

Torie R. McKinney



On February 14-19, 2010, the 4th Annual Sickle Cell Disease – Research and Educational Symposium was held in Hollywood, Florida and the theme for this year was "Progress and Promise: Sickle Cell Disease at 100 years". What an appropriate theme since it has been 100 years since James Herrick, a Chicago physician, reported the first account of sickle cell anemia in medical literature. Dr. Herrick transcribed the symptoms of a 20-year-old black male student from the West Indies as being "shortness of breath, palpitations, and episodes of jaundice. He had an anemia." Dr. Herrick described the patient's blood smear as showing "thin, sickle-shaped and crescent-shaped red cells." These are many symptoms some patients and parents have experienced and know all too well.

Being the mother of a 2.5 year old son with sickle cell disease and who has battled a host of complications; it was an honor to be chosen to attend this symposium. It allowed me to learn and collaborate with some of the world's leading healthcare experts, advocates, patients, and parents on sickle cell disease. Information was provided about several topics, including Leg Ulcers, Sickle Cell Trait, Public Health, Transition, Transplantation, along with an array of sessions and posters on basic science, clinical, psychosocial, and health services research. Provided below are key highlights from a few of the educational sessions (information provided below is from the 4th Annual Sickle Cell Disease Scientific Meeting abstracts):

- **Sickle Cell Trait –** Sickle cell trait is categorized as an individual who carries one sickle hemoglobin producing gene and one normal hemoglobin gene, resulting in the presence of sickle cell trait. The educational sessions provided information on identifying the gaps in public health, clinical health services, basic research, and community based outreach strategies for sickle cell trait. Since there have been recent unexpected deaths of athletes with sickle cell trait, the question has become, "should more be done" and "are there important medical implications and how should carriers be counseled?" In addition, the session addressed the National College Athletic Association (NCAA) controversy on potential public health policy implications of athlete screening and training and could there be "social consequences of testing for sickled cells in twentieth century America".
- Leg Ulcers (LU) Leg ulcers are ulceration of the skin and underlying tissue of the lower extremities. These sessions provided background on the severity, classifications, and management of leg ulcers in sickle cell disease. Research was shared on the discovery of genetic modifiers to identify patients at high-risk for this complication. Severities of leg ulcers are categorized based on size, depth, and duration, ranging from low to high severity. Based on severity, treatments can include: wet-to-dry dressings, blood transfusions, skin grafting, Unna boots, zinc sulphate, hyperbaric oxygen, arginine butyrate, topical herbal applications, and others. Since leg ulcers can often recur after minimal trauma, protective leggings with non-elastic lower extremity orthoses with ankle straps have been found to be an effective measure.
- Stem Cell Transplantation (SCT) This educational session and panel discussed the only cure for sickle cell disease. These SCT experts discussed the history of transplants for sickle cell disease (children and adults), and information about clinical trials, eligibility, limitations, conditioning regimens, complications/ risks before and after transplantation, and current statistics.

In 100 years, there have been many advances in sickle cell disease: from the first published case by Dr. Herrick in 1910 to the latest advances of better disease and pain management, hydroxyurea, and transplantation. However, the hopes from many patients and caregivers are to go beyond current paradigms of disease management/treatments to focus on more promising cures. As a result, many challenges still remain in the areas of medical treatment, research, education, and funding. However, I am assured these challenges will provide the biggest opportunities to shape the next 100 years within the Sickle Cell community.

Lastly, I would like to thank the following sponsors: Cincinnati Children's Hospital - Sickle Cell Comprehensive Center, Sickle Cell Disease Association of America (SCDAA) and the Health Resources and Services Administration (HRSA) organizations.

New Physicians At the Cincinnati Comprehensive Sickle Cell Center

The Cincinnati Comprehensive Sickle Cell Center is pleased to welcome and introduce two new physicians, both from Texas, who will soon be seeing families in clinic: Dr. Charles Quinn and Dr. Cristina Tarango.



Dr. Cristina Tarango is an Assistant Professor of Clinical Pediatrics at Cincinnati Children's Hospital Medical Center. She graduated from Stanford University School of Medicine and completed her pediatric internship and residency at Yale-New Haven Hospital. She then went on to the University of Texas Southwestern Medical Center at Dallas to complete her Pediatric Hematology/Oncology Fellowship.

Before attending medical school, Dr. Tarango, who is fluent in Spanish, taught English as a Second Language, was a tutor for a homeless program, and was an English and GED instructor in El Paso, Texas.

Dr. Tarango will be seeing patients in clinic on Mondays and Thursdays.

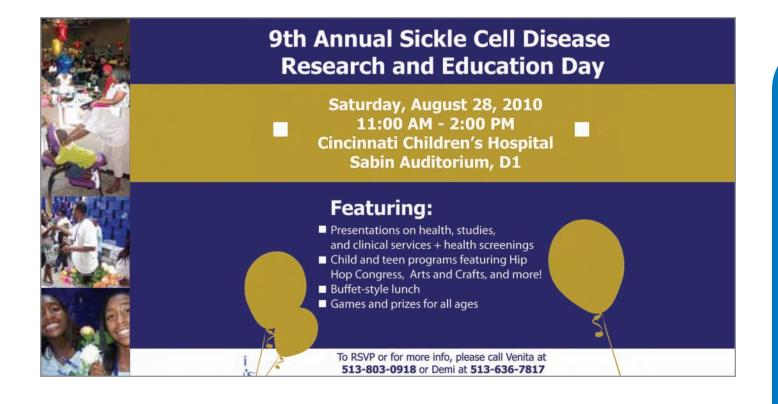


Dr. Charles Quinn is an Associate Professor at Cincinnati Children's Hospital Medical Center. He graduated from medical school and completed his residency and fellowship in Pediatric Hematology/ Oncology at University of Texas Southwestern Medical School.

In Dallas, Dr. Quinn was the Director of the Sickle Cell Disease and Thalassemia Program at Children's Medical Center. Dr. Quinn has been involved in the National Institutes of Health Comprehensive Sickle Cell Centers Program at University of Texas Southwestern Medical Center, and has received multiple other grants to research sickle cell disease.

Dr. Quinn has studied clinical outcomes of children with sickle cell disease; pulmonary function in sickle cell disease; causes of stroke; causes of bone pain; and acute chest syndrome.

Dr. Quinn will be seeing patients in clinic on Fridays.



Camp Joy's Teen Leadership Retreat 2010

Lisa R. Leace, MSW, LSW

Transitioning from adolescence to adulthood can be a scary process for many teenagers. However, through the Teen Leadership Retreat, we found a way to add some fun back into the equation. The retreat was held April 16th-18th at Camp Joy. There were a total of 17 teenagers ranging in ages from 13-18. The sickle cell patient population made up more than half of the teenagers in attendance.





The teens were able to develop and build trust in their peers by working together to maneuver through Camp Joy's "Gemini" ropes course. This ropes course required everyone to work in pairs to problem solve and make decisions as a team. Staff working with these teens were impressed with their ability to work together and form great partnerships.

Another important aspect of the Teen Leadership Retreat was the lessons in life that each teen experienced through participating in a CEO (Career, Education, & Opportunities) program that allowed them a virtual tour of life. This unique program allowed the teens to imagine their life at the age of 27. They had the option of choosing marital status, number of children, career path, automobile, and the option to reside in a house or apartment.

After making those choices, they were given a checkbook with designated salaries to participate in the Reality Store. The Reality Store was made up of booths that contained various stations in life such as; insurance, child care, clothing, unplanned expenses, utilities, etc. They were expected to balance their checkbooks and account for each transaction as they made their way through the Reality Store. Many of the teens displayed a great deal of leadership as they worked with their peers to figure out ways to conserve resources and reduce monthly spending.

This program gave the teens an opportunity to experience the responsibilities that come with transition into adulthood. It also provided the opportunity to see the importance of working collaboratively with others to get from one phase of life to the next. Our hopes are that each year we will increase the number of teen retreat participants. This will allow us to develop and maintain a strong leadership program that is teen specific and provides a pathway for transitioning into adulthood.

Summer Camp 2010

Lisa R. Leace, MSW, LSW

It's that time again! Summer camp at Camp Joy is just around the corner. We know many of our families are awaiting this special event. This year offers even more excitement for the campers.

Camp will be held for two weeks this year. One week will be dedicated to children ages 7-12 years old, and the other week will be dedicated to teens ages 13-18 years old.

Extending camp over a two-week period allows us to bring a larger number of kids to camp, and it gives the 17 -18 year old campers the opportunity to come back and enjoy the fun and excitement that Camp Joy offers.

The schedule for summer camp 2010 is:

- Teen Camp, ages 13-18, will be held July 11th 16th.
- Kids Camp, ages 7-12, will be held July 18th 23rd.

Camp applications have been mailed out to families. If you did not receive an application and would like to attend camp, please contact Heather Binning at 636-9736. If you have additional questions or concerns regarding camp, please contact Karen Martin at 636-3791.

School Intervention Program



School may be out for the summer, but the School Intervention Program (SIP) is still available to help plan next school year's Individual Education Plan (IEP) and 504's for your child.

SIP will work with your child's school to help provide the classroom and medical accommodations necessary for your child to stay healthy at school, and be a successful student.

If you have questions about the School Intervention Program, contact 513-636-6741.

Summer Care Tips for Children with Sickle Cell Disease

Darice Morgan, RN, MSN, CNP Peggy Kaiser, RN, MSN, CPNP





Hydration

- Keep water bottles on hand at all times.
- Drink water as often as possible.

Temperature changes

- Avoid excessive heat and cold.
- Prepare for sudden changes in temperature:
 - Take a sweater with you (for example in restaurants).
 - Have rain coat/umbrella available.

Activities

- Swimming
 - Dry off as soon as you get out of the pool.
 - Limit swimming to one hour at a time.
 - No swimming in temperatures less than 80 degrees.
- **Exercise and sports**
 - Make sure your coach knows you have sickle cell disease.
 - Rest often and stay hydrated.

- Carry medical travel letter in case of emergency.
- Airline travel
- Pack your carry-on bag with warm clothes and socks.
- Ask for a blanket when you get onboard.
- Buy bottled water or bring an empty water bottle and fill it at the water fountain prior to boarding the plane.
- - Plan for frequent stops for bathroom breaks.
- Out of the country travel
 - Make appointment with Comprehensive Sickle Cell Center at least 6 weeks before travel for necessary shots, anti-malarial prescriptions (if needed), and prescription refills.

Frequently Asked Questions About Clinical Research

Clinton Joiner, MD, PhD

Great strides have been made in the treatment of sickle cell disease over the last 30 years. This is the result of hardwork by researchers and dedicated participation by patient heroes. To continue to find better treatments for sickle cell disease, we must continue to do clinical research. This means that patients and parents must continue to participate in clinical studies.

What Are Clinical Studies?

Clinical studies can be very different. There are observational studies, and clinical trials.

Observational studies measure something about a patient. It is often how the test changes with time, or how it is associated with other conditions. An example is enrolling children with a sickle cell disease to do a certain blood test and watching them for several months to see if the test predicted which children got sick.

Clinical trials usually compare a new treatment (drug, medical device or procedure) to the best available treatment for that condition. Researchers can only do a clinical trial if it is truly unknown whether "new" treatment is better than the "usual" treatment.

How Do Clinical Trials Work?

In clinical trials, patients are randomly assigned to either receive the "new" treatment being tested, or the "usual" treatment already being used in clinics. Patients randomly assigned to get the "usual" treatment often are given a placebo. A placebo is a harmless and ineffective sugar pill, so that neither the patient nor the doctor knows what treatment they are getting. However the researchers leading the trial know what treatment the patient is on.

How Do Parents Know A Clinical Trial Is Safe?

A rigorous process is in place to review all clinical studies to make sure that they are ethical and do not put patients at risk for harm. Most clinical trials are designed by a committee of doctors and scientists. It can take several years to develop a safe clinical trial.

All studies are reviewed by impartial review committees and a Data Safety Monitoring Board (DSMB). Each hospital or clinic where the trial will be conducted also requires approval by an Institutional Review Board (IRB). Cincinnati Children's Hospital Medical Center has an IRB that must approve every clinical trial done at the hospital to make sure it is safe for patients to participate.

As clinical trial research is done, reports on how patients are doing on the study are given to the DSMB regularly to monitor the safety of the study. As soon as it is determined that new treatment is better or worse than the "usual" treatment, the trial is stopped and the better treatment is used in the clinic for all patients. This insures that all patients are being offered the best possible treatments.

Would My Child Be Part Of A Clinical Trial Without My Permission?

No. No one can enroll your child on a research study without your written consent. The research staff should explain the trial to you, answer your questions and give you a consent form that explains the trial in detail. You should also be told what treatment is available if you do not participate in the trial.

What If I Choose Not To Participate In A Study?

If you choose not to participate, your child will still get the standard treatment available, although you will not be able to receive the experimental research. No research will begin until you sign the consent form.

Is Personal Information Kept Confidential?

Researchers must safeguard your privacy during a study. Any information about you or your child that is sent to a studycoordinating center or DSMB, will be coded. This means that it will not include your name, your child's name or any other identifying data. If the results of the study are published in a medical journal, your personal information will not be released.

How Should Parents Evaluate A Clinical Study?

You can be assured that a clinical study has undergone appropriate review and is being conducted by reputable researchers, who will monitor the study properly if:

It is sponsored by the National Institutes of Health (NIH) or another federal agency or a major pharmaceutical drug company;

It is being conducted by a hospital with an Institutional Review Board. (All Cincinnati Comprehensive Sickle Cell Center studies must be approved by the Cincinnati Children's Hospital Medical Center IRB):

AND

Your doctor approves of your participating in the study. If your doctor is not the researcher doing the study, be sure to ask your doctor about participating in the study. You may want to take the consent form to your doctor for their opinion before agreeing to participate in the study;

You are asked to sign a consent form that explains the study before enrolling in the study.

Do your part to help improve treatment for sickle cell disease by participating in clinical research. You can find information on clinical studies of sickle cell disease at ClinicalTrails.gov (http://www.clinicaltrials.gov/ct2/results?term=sickle). The site lists studies that are registered with the National Institutes of Health.

Welcome to the Center



The Cincinnati Comprehensive Sickle Cell Center is pleased to welcome **Deidre Haygood** to the team as the Newborn Screening Coordinator.

Deidra has several years experience working with families through the Hamilton County Department of Job and Family Services. She has worked as a Children's Services Intake Investigator, an Adult Protective Services Worker, and a Child Care Consumer Specialist. She also has experience working as a United Way of Greater Cincinnati After-Hours 211 Associate.

As the Newborn Screening Coordinator, Deidra will be responsible for following up on all of the babies identified with sickle cell trait in southwestern Ohio. This includes providing trait counseling to the parents of the newborn. Through a grant from the Ohio Department of Health for sickle cell newborn screening follow-up, the Center follows-up on several hundred babies with sickle cell trait each year.

Zoo Day - April 24, 2010



Mark Your Calendar!

Sickle Cell Parent Support Group Canceled

Canceled until further notice. For more information, please contact: Lisa Leace at (513) 636-1747 or Cheryl Blair at (513) 636-8315.

July 11 – 16 Teen Camp (ages 13-18 years-old) July 18 – 23 Kids Camp (ages 7-12 years-old)

Kids Camp will be held at Camp Joy Outdoor Educational Center for children ages 7-12 years old with sickle cell disease. If you have questions or concerns, please contact Karen Martin at 513-636-3791.

July 24, 2010

Save the Date for the Gospel Music Workshop of America Fundraiser Event. This fundraiser will benefit the Cincinnati Comprehensive Sickle Cell Center and the Sickle Cell Affected Families of Greater Cincinnati. Watch your mailboxes for more information.

August 20 - 21, 2010

The Midwest Black Family Reunion will be held at Saylor Park along the Ohio River. The Cincinnati Comprehensive Sickle Cell Center will be providing free sickle cell trait testing at the event.

August 28, 2010

The 9th Annual Sickle Cell Disease Research and Education Day will be held at Cincinnati Children's Hospital Sabin Auditorium from 11:00 am – 2:00 pm. For more information, please contact Venita Robinson at 513-803-0918 or Demi Bruck at 513-636-7817.

September 2010

September is nationally recognized as Sickle Cell Awareness Month. Help increase awareness about sickle cell disease and sickle cell trait in your community.

September 17 – 18, 2010

Sickle Cell Sabbath will be observed at faith-based organizations throughout the state during this weekend as part of the Ohio Department of Health Regional Sickle Cell Services Program. Churches or other faith-based organizations in the Cincinnati area that would like to participate can contact Lisa Shook at 513-636-7541 or lisa.shook@cchmc.org for more information.

September 21-24, 2010

The Sickle Cell Disease Association of America (SCDAA) Annual Convention – "Giving New Voice to Sickle Cell Disease – 100 Years of Recognition" will be held in Washington DC at the Gaylord National Resort and Convention Center. For more information, please check their website at www.scdaaconvention.com or contact the SCDAA at 1-800-421-8453.

This newsletter is made possible by a grant from the Ohio Department of Health. Center Talk is published twice yearly by the Cincinnati Comprehensive Sickle Cell Center at Cincinnati Children's Hospital Medical Center. It informs consumers of our education/counseling, community outreach and treatment services, individuals and families affected by a hemoglobinopathy, and health professionals that care for persons affected by sickle cell disease and other hemoglobinopathies. Our goal is to provide a forum to communicate news and other items of interest in regards to sickle cell disease and other hemoglobinopathies. Feedback, ideas, and news can be directed to; Lisa Shook, 513-636-7541, lisa.shook@cchmc.org