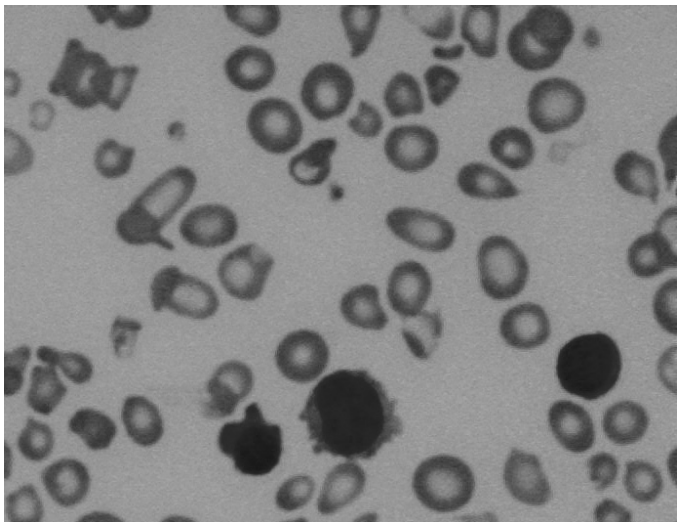
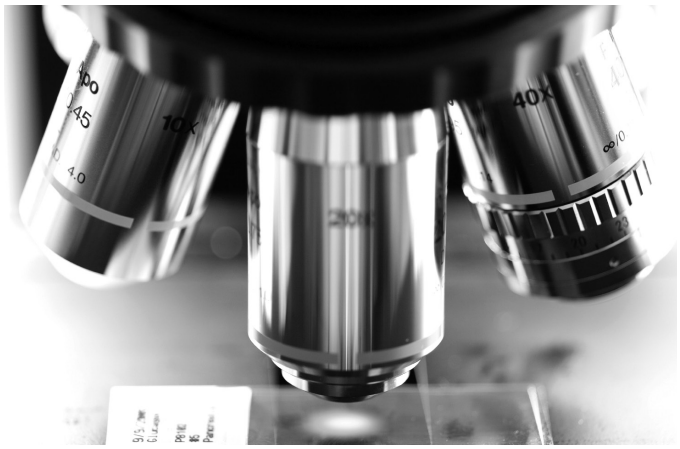
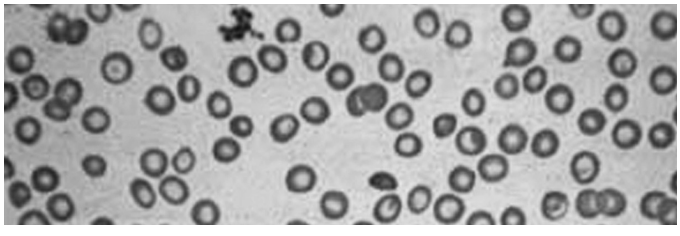
Thalassemia Research and Care: An Update

2012







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Dear patients, families, friends, and providers:

Along with patient care, medical research is a primary focus at the Comprehensive Thalassemia Center at Children's Hospital & Research Center Oakland. This special edition of the Perspectives newsletter focuses on research studies being conducted for thalassemia. This is the fourth Research and Care Update, and we hope that this issue will be a useful tool in keeping patients, families and the extended medical community abreast of current research, as well as in educating the community on thalassemia itself.

All of the studies mentioned here are optional. If you are interested in participating in a study or have questions, please talk to your medical care provider.

Thalassemia Research and Care: An Update
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I. Thalassemia Clinical Research Network (TCRN)

The Thalassemia Clinical Research Network (TCRN) was established and funded in 1999 by the National Heart, Lung and Blood Institute (NHLBI), part of the National Institutes of Health (NIH), to provide a national structure to conduct clinical studies in thalassemia. The TCRN was successfully funded by the NHLBI for two five-year cycles. Many of the TCRN studies that patients and families have participated in over the past 10 years have changed health-care practices in thalassemia. Those studies have also provided researchers with vast amounts of important data with which to move forward in thalassemia research. As of December 2011, approximately 20 publications in medical journals or abstracts have been the results of data obtained in studies funded by the TCRN.

As of June 2010, the TCRN was no longer funded by the NHLBI. Its funding structure has been replaced by new funding mechanisms within the NHLBI under the heading of hemoglobinopathies, including thalassemia and sickle cell anemia. At Children's Hospital & Research Center Oakland (Children's), we are dedicated to both thalassemia and sickle cell anemia research, and we are working with the NHLBI to secure funding to continue to move forward. In May 2012, we will be submitting a hemoglobinopathies grant that will encompass both sickle cell anemia and thalassemia.

Here at Children's, over the past 10 years of working with the TCRN, we have established collaborative relationships with other centers around California, in multiple other U.S. states, and in Canada to provide comprehensive clinical care and access to research studies to patients with thalassemia in as broad an area as possible. The satellites we have worked with include: Children's Hospital Central California in Madera; The University of California, San Francisco (UCSF); Children's Hospital Los Angeles; the Lucile Packard Children's Hospital at Stanford; Children's Hospital of Orange County; the University of California, Davis; and BC Children's Hospital in Vancouver. Our partnerships in thalassemia research with these institutions will continue in the future with the new funding that is being established through NHLBI.

One of the first successful projects from the TCRN group to secure funding from the NHLBI is for the Thalassemia Longitudinal Cohort Study. This study will continue to follow the patients who were enrolled for up to two more years.

Clinical research in thalassemia cannot be successful without participation from patients and families of patients with thalassemia. We thank all of you who have participated and those who will participate in the future for the time and effort that you have dedicated to further the cause of thalassemia research. By working together, we hope to establish new therapies to keep patients with thalassemia healthy throughout their lives.

There are multiple studies open for patients with thalassemia at Children's at this time. We have listed each below, with a brief description. If there are any studies that you would like further information about, please call Olivia Oliveros, at Children's, (510) 428-3885, ext. 4987, or ask your clinical care provider for a referral to our comprehensive thalassemia center.

II. Studies That Have Been Completed

(These are closed studies, not open for patient enrollment.)

» "Think Zinc" Study: Zinc and Bone Metabolism in Thalassemia

Sites that participated in this study were Children's; UCSF; Stanford University; Children's Hospital Central California; and Children's Hospital of Philadelphia (CHOP). The purpose of this study was to determine if zinc supplementation improves bone health in young patients with thalassemia. It is theorized that patients with thalassemia have osteoporosis (weak bones), in part due to zinc deficiency. Poor zinc status has been identified in patients with thalassemia, and zinc supplementation has been shown to improve growth, but its effects on bone health have never been studied.

An 18-month study was completed last year in 42 patients with thalassemia, aged 8 to 30, who also had low bone mass. A group of 38 controls of similar age and ethnicity (individuals without thalassemia) were also studied. Half of the patients with thalassemia received a capsule that contained 25 mg of zinc to be taken each day during the course of the study, while the other half received a placebo pill. The last subject measurement was completed in the end of September, and we are now actively analyzing the results.

If zinc supplementation is found to have a clinically important effect in patients with thalassemia, this simple, safe, noninvasive therapy could quickly become a part of standard thalassemia care and may improve overall health in children and adult patients with thalassemia.

We will have some preliminary findings at local and national meetings, and manuscripts are being prepared. This coming spring, we plan to send the results directly to those who participated in the study.

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Contributed by Ellen Fung, PhD, RD

» "Good Vibrations" Study: Improving Bone Health Through Vibration Therapy in Patients with Thalassemia

Dr. Ellen Fung and co-investigators have just recently completed this study, which was funded by the Cooley's Anemia Foundation.

Osteoporosis (weak bones) is a significant problem in patients with thalassemia that leads to risk of fracture and decreased quality of life. The most effective way to prevent osteoporosis is to build strong, dense bones during youth. Cardiovascular, weight-bearing, physical activity is known to strengthen bone; however, exercising regularly can be difficult for patients with thalassemia due to low energy levels or pre-existing cardiac problems. There are few noninvasive therapies to enhance bone health in patients. Over the last decade, vibration therapy has been used to enhance bone

health in astronauts, post-menopausal women, young women with fracture history, and children with cerebral palsy. The purpose of this study was to test the feasibility of introducing low-magnitude mechanical stimulation (vibration therapy) into the daily routine of patients with thalassemia. This simple therapy could be conducted in the home and did not increase strain on the heart.

Twenty patients participated in this study (10 adults and 10 adolescents). Patients were asked to stand on a vibrating platform for 20 minutes per day, six days per week for six months. The last patient completed the study in November. Data from this study is currently in preparation for publication. Preliminary results were recently presented at the American Society for Bone & Mineral Research in San Diego, California, in September 2011. This coming spring, we plan to send the results directly to those who participated in the study.

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» Transfusion Quality of Life (TranQoL) Study

This study was developed at Children's Hospital of Eastern Ontario by Dr. Robert Klaassen and the thalassemia center at Children's was asked to be a participating site. Researchers in Canada met with patients who have thalassemia to discuss quality of life issues. From that information, the health-care professionals developed a questionnaire about what was important to the quality of life (QOL) of patients diagnosed with thalassemia. The questionnaire is called the TranQOL, which stands for Transfusion Quality of Life. The TranQOL questionnaire is made up of 37 questions for thalassemia patients and asks how thalassemia and transfusions affect patients' lives.

The purpose of the study was to take the TranQOL questionnaire and validate it (see if it worked) for the patients at other centers around Canada and the United States. The study enrolled 50 patients from children to adults and asked them to fill out the questionnaires three times: once before transfusion, then one week later, and finally just before their next transfusion. The data from this study is now being analyzed and will be published in the next year. Many thanks to all those at Children's who filled out the forms!

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» Pain in Thalassemia Study

Twelve sites participated in this study to assess pain in thalassemia: CHOP, Children's Hospital Boston, Children's Healthcare of Atlanta, Baylor College of Medicine, Weill Cornell Medical College, Children's Memorial Hospital, Children's, Children's Hospital of Los Angeles, Children's & Women's Health Centre of British Columbia, Toronto General Hospital, and The Hospital for Sick Children.

The purpose of this study was to assess the prevalence of pain in individuals who have thalassemia and to assess pain severity, location, and possible risk factors. Two hundred and fifty-two individuals with thalassemia, ages 12 to 71, participated in this study. Individuals were asked to complete the Brief Pain Inventory, a validated pain assessment questionnaire, at four times during a one-year period to assess pain. Participants answered questions about the frequency, severity, and location of their pain. They were also asked if pain interfered in their life activities and whether they utilized pain medications or other treatments. Data collection has been completed and results are currently being analyzed.

This is the first study to prospectively assess pain in patients with thalassemia. If study results suggest that pain is an emerging issue for thalassemia patients, data from this study could provide direction to research efforts in pain management strategies, as well as impetus for studies to identify possible causes of pain in thalassemia.

We have some preliminary findings that have been presented at local, national, and international meetings. A manuscript is currently being prepared.

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» Exjade Palatability Study

The purpose of this study was to take Exjade (deferasirox) mixed in soft foods or in a drink other than water, orange juice, or apple juice to see if taking it this way would change the stomach problems some people have with it while not changing how effective it is at clearing iron. In the study, people took Exjade with breakfast, with dinner, or without food.

Most subjects preferred to take Exjade mixed with a liquid. The most popular liquids chosen for mixing with crushed deferasirox tablets included orange juice, apple juice, milk, and water; other liquids included Gatorade, tea, vegetable juices, and other fruit juices.

Many subjects chose to take Exjade with a soft food, especially at breakfast. The most popular soft foods chosen for mixing included apple sauce, yogurt, pudding, and ice cream; other soft foods included oatmeal, cereal, nut butters, mashed potatoes, gelatin, sandwiches, noodles, tacos, pizza, rice with gravy, whipped cream, cottage cheese, and mashed banana.

Most GI symptoms were less frequent when subjects were taking Exjade with food or mixing in different liquids. There were no new adverse events and no significant changes in how well Exjade worked.

This was a short study (only four months) and included only 65 people, so there most likely will not be a formal change in instructions for how to take Exjade until there can be more studies about the effect of food.

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III. Studies Currently Being Conducted

(These studies have patients who are enrolled and/or are actively looking for new patients to enroll.)

» Combination Chelation Study: Desferal and Exjade

Exjade and Desferal are both licensed drugs for chelation therapy in thalassemia, but the safety of these drugs together has not yet been established. This study was originally opened in Fall 2007 as a pilot study funded only at Children's. In Fall 2011, we completed enrollment for the original pilot phase and extension of this study. The safety profile of the combination chelation was analyzed, and the first manuscript for this study was submitted to be reviewed by a medical journal in November 2011. We plan on the publication of this article in 2012.

Currently, we are following the final five patients enrolled in this study for a second year. We expect to have the second year follow-up for this study completed by the end of 2012.

We want to thank all the patients who participated in this study to make it such a success!

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» Thalassemia Longitudinal Cohort (TLC) Study—Extension

The TLC Study has been very successful, enrolling over 400 patients with transfusion-dependent thalassemia to be followed over two to three years. This study is now closed to enrollment because the number of needed patients was achieved. At this time, the TLC is doing yearly follow-up visits with the patients who were enrolled previously. The TLC continues to correspond to the following eight areas identified by the TCRN Steering Committee as critical to thalassemia research (see below), and publications in these areas are currently being developed.

- Cardiac disease and pulmonary hypertension
- Iron measurement and chelation
- Hepatitis C and liver disease
- Growth and development, endocrine status, and fertility (including bone health and pregnancy)
- Nutrition status
- Strategies for enhancing fetal hemoglobin
- Psychosocial issues, including quality of life
- Measures of compliance with prescribed regimens

We would like to thank all of you who are currently participating in this important study.

» Fertility Study

Dr. Sylvia Titi Singer is conducting a small pilot study examining the effects of iron overload on the female and male reproductive systems. When this system is affected severely in women, they do not have their menstrual period on a regular basis and many do not have it at all. The condition can also make it hard for women to get pregnant when they want to, most likely due to the toxic effect of iron in the organ that produces hormones which allow a woman to become pregnant. The endocrine system in males is also affected by iron overload, which can affect the male reproductive hormones and the ability of males to father children.

The purpose of this study is to try to understand the relationship between iron overload, hormones, and fertility in teen and adult males and females with transfusion-dependent thalassemia. The study includes: labs and a pituitary MRI for both males and females. There is an optional sperm analysis done for males over 18 years. Any female or male patients over 12 years old with transfusion-dependent thalassemia are welcome. You will be given information about your fertility hormones, and if males participate in the sperm sample, they will be provided with further information about their fertility status.

The study is one to two visits only. You must be fasting, so morning visits are best. The visit at Children's Hospital & Research Center Oakland will last about 30 minutes. The sperm sample is done by the Reproductive Science Center in San Ramon and will require a special visit. We will reimburse for transportation costs to San Ramon. During the Children's visit, we will ask some basic health questions and questions about your chelation history.

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» Glutamine Study

The purpose of this research study is to find out how glutamine, an important amino acid found naturally in the body and some foods, affects people with sickle cell disease and thalassemia who have pulmonary hypertension (PHT). Participants must have sickle cell disease or thalassemia, and they must have PHT as indicated by test results. PHT can cause fatigue, dizziness, and shortness of breath, because the blood vessels that supply the lungs narrow, forcing the heart to work harder to push blood through. People with PHT tend to have more health problems (shortness of breath, pain crisis in sickle cell disease, pneumonia, and death) than those without PHT. Because of this increased risk, we want to find new treatments for PHT.

We believe that certain conditions present in those with sickle cell disease and thalassemia may contribute to developing PHT. In particular, hemolysis (the breaking apart of red blood cells, which causes anemia) may contribute to PHT. We think glutamine may decrease inflammation and hemolysis in the body. We will measure any changes by doing specialized blood tests and a

echocardiogram. By decreasing inflammation in the body, we may be able to prevent or treat PHT.

Glutamine is the most abundant naturally occurring amino acid in the body. It helps build other important proteins and provides fuel for some cells (especially cells of the immune system, the small intestine, and the kidneys). Glutamine has been shown to help in healing wounds and is sometimes used to treat tissue injuries from accidents, burns, or other trauma or after surgery. It is also sometimes given to babies born very small for their age to improve their nutrition and to help their intestines work properly. Glutamine can be found in many foods (meat, fish, dairy, beans, spinach, parsley, and cabbage). It is available as a nutritional supplement and is marketed to help build muscles. However, glutamine is currently not a standard of care for PHT for patients with sickle cell disease or thalassemia.

About 30 people (15 people with sickle cell disease and 15 with thalassemia), ages four and older, will be in this study, which will last for eight weeks. We will also be looking for people that do not have thalassemia or sickle cell disease to be control subjects for results comparisons.

There is an optional pharmacokinetics (PK) part of the study. You do not have to be in the main study to do the PK portion. In the PK study, the patient is given one dose of arginine in the morning, and during that day, multiple blood draws are done from an IV and tested for the amount of arginine in the blood.

We hope that taking the glutamine will help improve the underlying causes of your PHT, but we cannot be certain that will happen. Information we get from this study may contribute to a better understanding of your disease and may be useful in selecting medicines for your future treatment.

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» Thalassemia Intermedia and Exjade Study

This is a clinical research study of patients with thalassemia intermedia who do not require regular blood transfusions and have too much iron in the body. The excess iron comes from the frequent breakdown of red blood cells and from increased absorption of iron from food. Too much iron damages organs in the body—particularly the liver and the heart. Since the body cannot eliminate iron on its own, a chelator is used to remove excess iron. There is no chelator approved for use by people who are not receiving regular blood transfusions. Deferasirox (Exjade) is an iron chelator that is approved by the FDA for people who have too much iron because of blood transfusions. This study is looking at the effectiveness and safety of chelation therapy via deferasirox in people with thalassemia intermedia.

In the first year of the study, Exjade was compared to a placebo ("sugar pill"). There were no unexpected, serious, adverse events, so a longer, open-label study where everyone gets deferasirox (no placebo) was started. This study will be completed in the summer of 2012. No new subjects are being enrolled.

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» New Oral Chelator Study

FBS0701 is a new oral iron chelator. It comes in a capsule and is taken once a day. Its safety and effectiveness in the treatment of iron overload resulting from frequent blood transfusions in humans is currently being studied. There is one ongoing study in adults that is closed to new subjects. A second study, comparing FBS0701 to another chelator, will be opening later this year. There is also a pediatric study that is open and enrolling now.

Participants in the current adult study, which began in August 2010, take FBS0701 once a day and come in for study visits every four weeks. Every three to six months, an MRI is done to check iron stores in the liver and heart. The adult study was originally designed for subjects to be on the study drug for six months. This was extended, first for another six months, and again for an additional 12 months. If no new risks are identified, the study will most likely continue for at least another year.

So far, subjects report few GI problems when using FBS0701. There have been no serious adverse events related to the study drug. Adverse events possibly related to FBS0701 include: headache, flatulence, a change in the color of urine, warmth in the stomach, blood in the urine, catching the common cold, a change in the sense of taste, and increases in liver enzymes, diarrhea, indigestion, and pain in the upper abdomen.

Original dose levels were 16 mg/kg or 32 mg/kg per day. Most subjects have had their doses increased over the course of the study. New safety information will allow for an increase to 60 mg/kg, if necessary.

The pediatric study is enrolling subjects in two age groups: 6 to 12 years, and 12 to 17 years old. The study looks at safety and how well FBS0701 works at getting rid of iron over 12 months. The study drug is taken on an empty stomach. Subjects must be getting regular transfusion or apheresis and have iron overload (for example, a high serum ferritin and increased liver iron). They must also be willing to stop all chelation therapies for about two weeks.

The study involves a screening visit for blood and urine sampling, a physical exam, an ECG, and an MRI. If subjects continue in the study, they will come to the Pediatric Clinical Research Center once a week for the first month, every two weeks for the second month, and then once a month for the rest of the year. At study visits, blood samples and urine samples will be taken and a brief physical exam will be done. Every three months, additional testing (ECG, MRI) will be done. Subjects will receive regular medical care and reimbursement for transportation and efforts related to study participation.

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» Innate Immunity Study

A strong immune system is necessary to fight infections in the body. It is possible that iron damages the immune system, which may lead to an increase in bacterial infections.

This study is being done to examine the effect of iron on the immune system. Thalassemia patients and matched healthy controls (people without thalassemia) will be asked to participate in the study.

The study only requires one visit to the clinic. Subjects will come to Children's between 8 a.m. and 10 a.m., where they will be asked questions about their medical history. They will also have height and weight measured and receive a full physical examination. Females over the age of 12 will be given a urine pregnancy test.

Participants will also be given a blood test. Thalassemia patients will have about 4 teaspoons of blood drawn, either from the IV for their regular transfusion or from their pre-transfusion blood draw the day before (but *not after* transfusion). Subjects with thalassemia will be asked to stop chelation for 72 hours before the blood test. Subjects without thalassemia will have about 4 teaspoons of blood drawn from a needle in the arm.

All participants must fast (except for water and medications) for 10 to 12 hours before their blood is drawn in the morning. We will provide breakfast after patients have their blood drawn.

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» Centers for Disease Control (CDC) Study

This national study was set up by the Centers for Disease Control (CDC) to monitor the safety of the nation's blood supply. The CDC is collecting blood samples from people with thalassemia to investigate the diseases that people can potentially get from blood. This study is enrolling patients of all ages and requires a once-yearly blood sample (about 2 teaspoons) and a questionnaire about your (or your child's) health. The blood sample will be tested to see if you (or your child) have been exposed to hepatitis A, B, or C or HIV. You can get the results of these tests from your health-care provider. The information gained from this study will be used to help thalassemia specialty centers improve patient care.

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» Multi-Center Study of Iron Overload (MCSIO) Study

This is the latest Multi-Center Study of Iron Overload (MCSIO) project. For over 10 years, Children's has been collaborating with other clinics around the world to understand the differences in how iron overload affects people with thalassemia, sickle cell disease, and diamond blackfan anemia.

There are two parts to the current MCSIO project: the pilot study and the survey study. The pilot study is nearly complete. That study involves getting an MRI to look at the amount of iron in the liver, heart, and pituitary gland (a small part of the brain that helps regulate the body's balance of hormones) and having blood samples taken just before and one week after a transfusion. This study was done to find the best ways of measuring iron in the body.

The survey study is still enrolling. Subjects who consent to be in the study will be asked questions about their medical history, chelator use, and transfusions. Subjects also will allow study staff to review their medical records for this information. No extra visits, no testing, and no treatments are part of this study. The purpose of this project is to gather data from people who have had regular transfusions for 10 to 20 years, starting before the age of 10. This information will help design a larger study comparing how iron moves through cells in the body and where it gets stored.

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» Mitochondrial Function in Disorder of Iron (MitoIron)/Functional Significance of Mitochondrial DNA Abnormalities in Thalassemia Major Study

Mitochondria are the energy-producing parts of our cells. Pre-clinical studies have revealed the marked susceptibility of their function to excess iron. Hence, the preservation of mitochondrial function should be a goal of iron chelation therapy.

Using new techniques, we can monitor mitochondrial health in peripheral blood samples. We are studying if the mtDNA defects in thalassemia major are linked to the degree of iron overload and oxidative stress. We will also evaluate if mitochondrial defects are related to the ability of immune cells to work properly. This will help us to develop a functional assessment of iron chelation therapy, which is currently based entirely on the measurement of tissue iron burden. The study will enroll 20 individuals with thalassemia who are on chronic transfusions. Participation entails a one-time fasting blood sample.

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» Physical Activity Assessment Study

This is a study of physical activity patterns in patients with thalassemia. It is a sub-study of the "Good Vibrations" study and funded through a grant from the Cooley's Anemia Foundation.

The study will be done at Children's. A total of 30 patients

with thalassemia will be enrolled, in addition to 30 control subjects (people without thalassemia). Patients need to be more than 10 years of age and not pregnant. Patients will be asked to come to the HEDCO Health Science Center. They will be asked to complete a 10-minute questionnaire on exercise habits and have their height and weight measured. Patients will also be asked to wear an actigraph (a small electronic device attached to a belt around the hip) for the weeks prior to and after transfusion. Non-transfused subjects wear the actigraph for one week only. Participants will be provided a $20 Target gift card for their contributions.

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» Nutrition and Thalassemia Study

We have found that some patients with thalassemia have low levels of nutrients in their blood, possibly related to marginal nutritional status. These deficiencies may be caused by an increased requirement for the nutrient in patients with thalassemia or related to poor dietary intake. The relationship between dietary intake and circulation levels has not been explored in patients with thalassemia.

The study will be done at Children's. A total of 50 patients with thalassemia who are scheduled for a comprehensive clinical evaluation will be enrolled. Patients need to be more than five years of age and not pregnant. At the time of their comprehensive evaluation, patients will be asked to complete a 30-minute food frequency questionnaire, answer questions regarding personal medical history, and have their height and weight measured. They will also be asked to complete a 3-day dietary food record before their clinic visit.

There are *no* extra blood draws, examinations, or procedures as part of this study. Subjects will receive information about *their* usual dietary intake of some nutrients and be provided with a $10 Target gift card for study participation.

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IV. Update on the SQUID Program

Since 2002, the Superconducting QUantum Interference Device (SQUID) has been an integral part of thalassemia care at Children's. The SQUID allows the thalassemia team to monitor iron concentration in the livers of patients and gives them a reliable tool to help adjust medication in order to avoid serious complications. Recently, some insurance companies have realized the importance of the SQUID in thalassemia care and have started covering the cost of the procedure. We have been following many thalassemia patients with the SQUID, and their yearly visits have given us a good history of their liver iron concentrations. Over the last decade, the SQUID biosusceptometer at Children's has performed over 2,000 measurements on over 800 local and international patients at risk for iron overload.

Children's is home to the only SQUID (biosusceptometer or Ferritometer® manufactured by Tristan Technologies) in the United States that serves a clinical population. The SQUID measurement of liver iron concentration (LIC) offers vital information for the management of thalassemia. LIC measurement is used, along with serum ferritin, to monitor total body iron stores. However, ferritin values are dynamic and can change rapidly depending on inflammation, intensive chelation/phlebotomy, or need for transfusion. LIC provides a more steady-state indication of total body iron. Unless it is diagnosed early and properly managed, iron overload can lead to serious complications, including cirrhosis of the liver, heart disease, osteoporosis, and ultimately, multiple organ failure and death at an early age.

Liver biopsy used to be considered the "gold standard" for assessing iron levels. However, biopsy is a painful, invasive procedure that often requires general anesthesia. In contrast, SQUID biosusceptometry is a pain-free, noninvasive alternative with the same level of accuracy as a biopsy. SQUID biosusceptometry is performed annually or biannually with minimal risk or discomfort to patients, and its availability has largely reduced the need for liver biopsies.

For more information, or to schedule a SQUID, please contact:
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V. Update on Bone Densitometry (DXA)

Bone health is assessed by a Dual Energy X-ray Absorptiometry (DXA) examination. The DXA test is ordered by a doctor, and the results provide information about the density of a patient's bones in comparison to individuals of the same age and gender who do not have thalassemia.

Patients with thalassemia frequently have low bone mass, and many adults experience significant bone pain and have increased risk of fracture. These complications are not limited to patients with thalassemia major but are also observed in non-transfused thalassemia intermedia, e-beta thalassemia, and hemoglobin H patients.

The most effective way to prevent low bone mass is to build strong, dense bones during youth. A combination of disease, endocrine issues, and nutritional factors likely contribute to the etiology of osteoporosis in patients with thalassemia. To help build healthy bones, it is important to do the following:

- Consume a diet rich in calcium.
- Have vitamin D levels checked.
- Participate in non-contact, weight-bearing physical activity,

as tolerated.
- Get a measurement of your bone density every one to two years (DXA scan).
- Have an endocrine evaluation as indicated by your doctor.

If you already have low bone mass, there are treatments that can improve and/or stop the condition from progressing. It is important to discuss this with your health-care provider.

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Contributed by Ellen Fung, PhD, RD

VI. Overview of Upcoming Studies

» Expanded Fertility Study

Dr. Sylvia Titi Singer is continuing to seek funding to expand the fertility study in both males and females with thalassemia. This is a topic that patients have expressed interest in which we hope to support.

» International Combination Chelation Study

Based on the data from the Combination Chelation Study, Dr. Ash Lal is planning to submit a proposal to the NIH for a large international study in combination chelation.

VII. List of Published Articles About Thalassemia

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If you are interested in having someone from the thalassemia research team at Children's Hospital & Research Center Oakland contact you about current or future research studies, please complete this form. We will not give out your contact information to any other group.

Name _____

Street Address _____

City/State/Zip _____

Phone _____

Email _____

Date of birth _____

Where you usually get care for your thalassemia _____

To protect your privacy, please return this form in an envelope to:

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