PERSPECTIVES

NEWSLETTER OF THE NORTHERN CALIFORNIA THALASSEMIA CENTER - SPRING 2015

UCSF Benioff Children's Hospital Oakland

SPECIAL ISSUE: FOCUS ON BLOOD

Blood Donors–Partners for Life

by Kent Corley, Public Relations, Blood Centers of the Pacific

I've worked in blood banking now for almost 10 years, which, in comparison to many of my colleagues, is short. Blood banking seems to get into one's blood. Even those who leave the industry are often pulled back in, like I was several months ago.

When I came into the business in 2005 with Blood Bank of the Redwoods in Sonoma County, I was quickly humbled by the complexity of the operation. Make a blood donation, take it to a hospital, give it to a patient, save a life—not even close! Blood donors begin to get a sense of the intricacy of the process when they start filling out the donor health history questionnaire. The health history is just the first chapter of what becomes a very elaborate story: how pints of donated blood arrive in time for transfusions to trauma patients, thalassemia patients, and hundreds of other patients every day.

In Sonoma County, one of our ongoing challenges was collecting enough O-negative blood for the Trauma II center at Santa Rosa Memorial Hospital. Dr Brian Schmidt and his trauma team were and are still presented with a never-ending supply of patients, many needing transfusions before they can be typed. The response in those cases is to use O-negative blood, the universal blood type. My most vivid memory is from September 12, 2006. Jim Wilson, later dubbed "The Miracle Man," was involved in a motorcycle accident. After the REACH helicopter flew him to the hospital, he received 52 units of O-negative blood in the first two hours, and it took a total of 182 units of red blood cells, platelets and plasma to save his life. In the years following the accident, I was honored to get to know Jim and his wife, Lindy. He is alive because of the blood donors who gave blood in advance of his accident. The whole experience was, again, humbling, because all that blood needed to be ready on the hospital shelf for Jim, as well as all the other patients at the hospital that needed blood that night.

Now I'm with Blood Centers of the Pacific and we serve far more hospitals—over 45 from the base of Silicon Valley to the Oregon border. But even after being in blood banking for almost 10 years, just when I thought I'd seen and heard it all, a new need for blood transfusions becomes visible, like it did when I received a call from Laurice Levine, Senior Thalassemia Outreach Coordinator for the UCSF Benioff Children's Hospital Oakland. Blood Centers of the Pacific supplies all the blood that the hospital uses (some 10,000 units per year), including blood for the thalassemia patients. Ten years in the blood business and I had never heard of thalassemia before!

I had thought supplying blood for a trauma center was challenging, but as I began to ask questions about how we provide blood for patients at Children's Hospital Oakland, including thalassemia patients, the complexity just went off the charts. As I questioned

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The thalassemia program at UCSF Benioff Children's Hospital Oakland is now on Facebook and Twitter.

http://www.facebook.com/CHRCOTHAL

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Visit us at www.thalassemia.com
The website is always being updated. Please refer to it for new stories, resources, event calendar, and more.

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Blood Donors–Partners for Life

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our Hospital Services folks and people in our blood bank reference lab, it quickly became clear that the work that goes into preparing units of blood for Children's Hospital Oakland is highly customized and specialized. It's not as simple as preparing type specific blood. It was as if the people answering my questions were speaking another language. Just the discussion of all the work Blood Centers of the Pacific does with antigen specific units for the hospital was more than I could absorb.

Then I thought that by putting aside the trauma, oncology, cardiac, and neonatal cases that require blood transfusions at the hospital and just focusing on blood for thalassemia patients, it would be easier to get a handle on that process. Wrong again. It, too, is very complicated. What I did ultimately learn as my main takeaway was that unlike a trauma patient who may receive a transfusion and be done, many of the thalassemia patients will be receiving blood transfusions all of their lives. They are forever joined at the hip to blood donors, and this anonymous yet intimate relationship is made possible by a long line of health-care professionals first at the blood bank and then at the hospital. And the cycle must never end, which is why our bloodmobile regularly shows up at the hospital and why we ask ordinary people to roll up their sleeves every eight weeks. We collect blood most every day of the year all across Northern California at schools, community centers, churches, and businesses to keep the lifeline going.

Blood Centers of the Pacific is the link between blood donors and the blood transfusions that thalassemia patients depend on for a lifetime of treatment. If you are reading this newsletter, then you likely are a thalassemia patient or know a thalassemia patient and want to help. Here's a great idea—next time you see the bloodmobile, whether you are a patient, a family member, a friend, or even a doctor or a nurse, take a minute and just say thank you to a blood donor. A single thank you for that single blood donation may very well create a lifetime blood donor. And if there is one thing that thalassemia patients need, it is lifetime blood donors.

Blood Centers of the Pacific is a not-for-profit organization that provides volunteer blood donations to more than 45 hospitals throughout Northern California. It is the nation's oldest community-sponsored blood center, with locations throughout the region, from the base of Silicon Valley to the Oregon border. BCP is an affiliate of Blood Systems, the nation's second largest blood collection organization. For more information, visit www.bloodcenters.org.

A MULTITUDE OF THANKS TO RECENT DONORS

Rose and Don Arnaudo
AssetMark
Blue Bird Bio
David Botta
Bev and Tony Compagno
Pasquale and Julia Contestable
Pat and Louis Cordich
Robert and Joan Cowan
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Joe and Rose Digrado
Marlene Farber
Noreen and John Gillio
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Susan and Steve Injayan
Italian Catholic Federation
Rosalyn and Patty Jarrett
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Andy and Sid Pappani
Peet's Coffee, Temescal
Patti Phillips
Prudence Price
Terry Rosser
Roger and Kathy Santos
Roy and Christine Shackel
Spencer Family
Mr. and Mrs. Bruce Swarringim
Jackie Tribolet
Albert and Diana Vallorz

AssettMark: *Erich Elleson, Lisa Steffler, Naomi McKean, and Jim Croy*

We would like to acknowledge APO Pharma for sponsoring the following events:

- January 2014: **Thalassemia Patient/Family Conference**, Oakland, CA
- May 2014: **Evening Under the Stars benefiting thalassemia**, Oakland, CA
- September 2014: **Thalassemia Patient/Family Picnic**, Minneapolis, MN
- May 9, 2015: **Thalassemia Patient/Family Conference**, Seattle, WA

Thank you to Max Johnson and ApoPharma for enabling us to provide patient education, respite, and diversion to our families as an integral part of our global outreach program.

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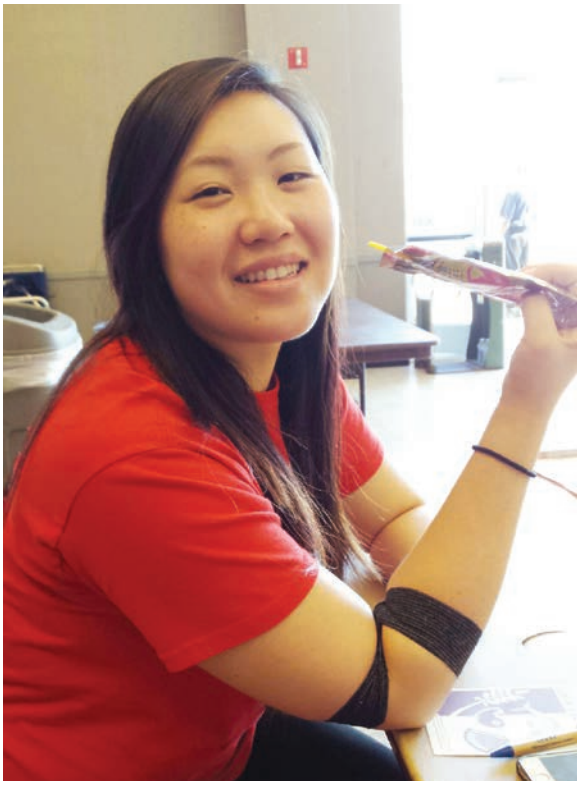
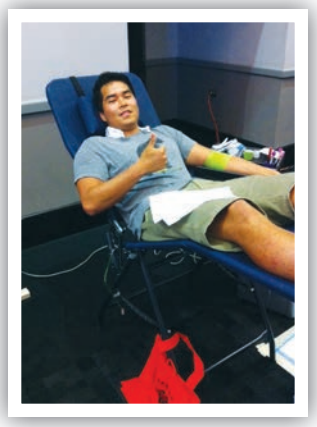
Donating Blood in Honor of Thalassemia

by Laurice Levine, MA, CCLS

People with beta-thalassemia major require approximately 34-40 units of blood per year to survive, as they are unable to produce working red blood cells. There are approximately 60 thalassemia patients at UCSF Benioff Children's Hospital Oakland who require blood transfusions every two to three weeks. This means that blood transfused into the thalassemia population at Children's alone totals approximately 2,040-2,400 units of blood a year. Each year, the lab at Children's uses 6,636 units of red cells, 1,196 units of platelets, and 876 units of fresh frozen plasma.



UCB Thal Intern Leslie Yeh donates blood at the Raider's Blood Drive



Jeff Nagata, Thal Team, donates blood at the Raider's Blood Drive

As a person with thalassemia, I firmly believe that we patients, as blood recipients, have a responsibility to replenish the blood supply. There are so many ways to help—plan a blood drive, volunteer at one, donate goodies or a raffle prize to thank blood donors, or recruit friends and family to donate blood every 56 days.

Many people have asked if blood can be donated specifically for thalassemia patients. Due to high costs and administrative issues at the blood banks, this is not feasible. In spite of this, the importance of donating blood cannot be emphasized enough. Blood is not only vital to the survival of people with thalassemia, but to countless others, as well.

Consider the following blood facts:

- Every two seconds in the United States, a person (or 6 million people each year) will have an accident, a heart or kidney transplant, a major surgery, or cancer treatments or suffer from an injury and will need blood.
- Over 20 percent of all blood used in Northern California has to be imported from outside the state because of insufficient community collections.
- On any given day, more than 40,000 units of red blood cells are needed.
- Ninety-five percent of the community will use a blood product in their lifetime; however, only 5 percent of the population actually donates blood.
- A single trauma victim can use 100 units of blood in a few hours; in a single day, a patient needing a liver transplant can require 200 units of blood products.
- Adult males have 12 pints of blood and females have 10 pints of blood.
- A person can donate one unit of blood every 56 days.
- After donating blood, the body's liquid volume is restored within 24 hours, and red cells are replaced within three to four weeks.

You can help by donating blood or planning a blood drive in honor of thalassemia. If you are interested, please contact:

Laurice Levine, MA, CCLS
Senior Thalassemia Outreach Coordinator
(510) 428-3885, ext. 5427
LLevine@mail.cho.org

HOW TO COORDINATE A SUCCESSFUL BLOOD DRIVE

by Laurice Levine, MA, CCLS

Blood drive coordinators play a vital role in collecting lifesaving blood for patients. Without people to plan blood drives, blood banks would not have as many opportunities to collect blood from donors.

Planning a blood drive is fun and easy—however, like planning any event, it takes time. Fortunately, a blood bank representative will help you every step of the way.

The following are helpful tips on planning your blood drive:

1. **Find a location.** A school, college, or church hall is ideal. If you do not have access to a big building, you can coordinate with a store, a business, or any location to have a blood mobile come instead.
2. **Find out the what blood bank serves your area.** For example, the American Red Cross and Blood Centers of the Pacific serve the Bay Area.
3. **Set a date** (three to six months ahead of time), and contact your local blood bank.
4. **Set a goal.** Your blood bank representative will help you decide how many units of blood you want to collect at your blood drive. Remember, you will need to schedule an additional number of donor appointments to assure that you reach your collection goal.
5. **Plan a theme for your blood drive.** A luau, Mardi Gras, rock and roll—let your creativity flow!
6. **Give away free items.** It is always nice to thank your blood donors with little gifts that you buy or get donated. The blood bank often also has giveaways to contribute. Setting up a raffle is also a fun way to add spice to your blood drive. Some ideas for giveaways:
   - Gift cards—get small denominations from Starbuck's, Jamba Juice or a local ice cream store.
   - Candy and treats.
   - Pens.
   - Logo items from your work or local business.
   - Canvas bags—a great prize item, and all your other giveaways can go inside.

   Ideas for raffle prizes are as follows:
   - Gift cards to movies (Regal, AMC); clothing stores (GAP, Old Navy, Macy's); retail/food (Target, Safeway, restaurants).
   - Themed gift baskets such as a spa kit; a snack basket; or an Italian-themed food basket.
7. **Promote the event.** About six weeks prior to the blood drive, start promoting the event. Your blood bank will provide flyers—hang them at the location of your event and in surrounding areas/stores, etc. Plan to distribute (in person or by mail) smaller flyers to friends, family, and your community. Promote your blood drive through email and social media such as Facebook and Twitter. Send weekly reminders starting four weeks prior to your blood drive.
8. **Recruit volunteers.** The blood bank phlebotomists and nurses will be responsible for collections, registration, intake, and the canteen (snack stand). You will need to recruit volunteers to greet people, run your raffle, and distribute prizes.
9. **Gather literature to give away the day of the drive.**
   - Give out literature on your organization.
   - If you are doing a blood drive in honor of thalassemia, give out literature on the disease.
10. **Schedule appointments.** While publicity is important, there is nothing like a personal touch—contact friends, family members, classmates, and fellow churchgoers and educate them on the importance of donating blood. The blood bank will also send emails to prior donors in their database. Appointments can be scheduled online.
11. **Inform yourself.** Learn about donor eligibility guidelines; information on the blood bank website; and the sign-up process. It is vital to be informed so that you can answer questions and/or direct people regarding where to find the information they need.
12. **Communicate with your blood bank representative.** Communicate regarding donor appointment sign-ups; the day's schedule; room setup, etc.
13. **Reconfirm appointments about one week prior to your blood drive.** This can be accomplished via a postcard, email, or phone call. Also remind donors to drink plenty of water and eat a good meal prior to donating.
14. **Be ready for the day of the blood drive.** Arrive when the blood bank staff arrives to greet them and help set up; assure that the room temperature is adequate; orient your volunteers; and organize your welcome and prize tables. Depending on the number of appointments and the population that is donating, you may get walk-in donors—make sure the lines of communication between you and your blood bank staff stay open to assure that the maximum number of units are collected.
15. **Have fun!** Show the donors your full appreciation, and enjoy the blood drive. At the end of the day, give yourself a pat on the back—your hard work, time, and creativity were instrumental in collecting blood donations and saving lives.

For more information or for assistance planning a blood drive, please contact your local blood bank or:

Laurice Levine
LLevine@mail.cho.org
(510) 428-3887, ext. 5427

DONATING BLOOD: WHAT TO EXPECT AND HOW TO PREPARE

by Laurice Levine, MA, CCLS

When you donate blood you will help save the life of up to three people! It is one of the easiest ways to contribute to your community and it is free. If you have never donated blood before you may be feeling a bit nervous. However, giving blood is safe and simple. This article will help you understand the process and hopefully allay any fears you may have.

You can donate at a community blood drive in your area; often schools, hospitals, and churches hold blood drives, or sign up to donate at a local blood collection center in your area. It is important to eat iron rich food in the days or weeks before donating blood; this is especially important for women who tend to have lower iron counts. It is also important to have a meal before donating blood and be very well hydrated—drink plenty of water the day before, day of, and after donating.

Here are the steps to donating blood at a blood drive or blood bank:

Step 1: Register

- Present your photo ID Card.
- Read information sheet about donating blood.

Step 2: Donor Health History and Mini Physical

- Provide basic demographic and health information.
- Answer questions about past and present health history (information is kept private).
- Undergo a mini-exam to determine if you are able to donate (temperature, blood pressure, heart rate). Give a blood sample (usually from the finger) to determine hemoglobin level.

Step 3: Drink Fluids

- Drink water or other beverage prior to your donation. The more hydrated you are, the faster your donation will be and the better you'll feel afterwards.

Step 4: Donate

- Sit in a comfortable donor chair.
- Once the needle is in place, it usually takes less than 10 minutes to draw a unit (about a pint) of blood. You'll squeeze a ball to keep the blood flowing while you relax and let the blood flow.
- Finish your donation, after which the needle will be removed and a bandage applied—a badge of courage!

Step 5: Visit the Canteen

- Sit, relax, and have a snack and drink at the donor canteen for a few minutes (California law states you'll need to stay for at least 15 minutes).
- All done! Enjoy your day. You're now a lifesaver!

Step 6: After Donating

- Avoid strenuous physical activity or heavy lifting for a few hours. If you feel light-headed, lie down until you feel better. Have everyone cater to you for the day and do something special for yourself—you deserve it!

The entire process usually takes less than one hour.
The perks of donating blood—along with the fact that you save three lives:

- You'll receive free wellness screenings, including blood pressure, cholesterol, and iron levels every time you donate.
- You'll learn your blood type.
- You can donate in honor of someone—usually the blood bank can give you a card to send to a friend or loved one—what a unique birthday gift that would be!

Resource:
www.bloodcenters.org

For more information contact:
Laurice Levine, MA, CCLS
(510) 428-3885, ext. 5427
Llevine@mail.cho.org

If You Can't Donate, Coordinate!

by Mary Tavormina Cook

Tom Cook

After so many years unable to donate blood myself because I have the thalassemia trait, I thought that I would run a blood drive instead. I had no prior experience coordinating a drive, so I had no frame of reference—other than it was requested that we set a goal of 25 units. Laurice Levine offered her help. I thought: How difficult could it be to get 25 folks to give blood?

It was a year in the making. Actually, the date was scheduled over a year prior. As it turned out, the first date was erroneously listed as Saturday when a Sunday had been requested, and the date could not be staffed; so we postponed the blood drive until one year later, on Sunday, October 19, 2014.

With her mother's permission, we chose to have the drive in memory of a young friend and parishioner, Molly Widner, who died of cancer in August 2014, just three months shy of her 18th birthday. Molly's mom, Kathy, joined me in spreading the word. We began promoting this event—by word of mouth, in the local newspapers, on social media—and we posted flyers around our church grounds and in the weekly bulletin.

At first, it looked like the ICF Branch #13 Blood Drive at St. Catherine of Siena Parish would produce only a handful of donors. In fact, the last blood drive held here only garnered eight donors. Sign-ups were slim under the Blood Centers of the Pacific online registration. Then within a day, the registrations doubled, then tripled, and on the morning of October 19, we were expecting 28 potential donors. Great, I thought. We will hopefully get at least 25 units and reach our goal.

Laurice asked Mint Bhetraratana from the Thalassemia Outreach Department to join us in Martinez, and she arrived with raffle basket prizes and tote bags filled with giveaway goodies. She was instrumental in assisting donors in completing paperwork and answering health questions that stumped some of us. Kathy Widner joined us with a raffle basket, as well as poster boards of pictures of her daughter, whom the drive honored.

The bloodmobile staff was busier than they expected to be. One after another, the donors came, filled out paperwork, answered preparatory interview questions, and remained in the "bus," as it is known, for about 45 minutes from deposit to recovery. Men, women, and youths kept arriving. Some were turned away because of low body weight, others due to age (without parental permission), and still others who didn't meet requirements. As an added surprise to donors, each received a t-shirt with both the San Francisco Giants and Blood Centers logo.

All in all, 48 potential donors were registered, and 33 units of lifesaving blood were collected. The bloodmobile staff was exhausted. One phlebotomist remarked that it had been the most blood she had ever seen collected in one bus. There could have been two mobiles dispatched to this location. Andrea Casson, the Blood Centers of the Pacific Senior Account Representative, stated that the blood drive was a huge success and broke all kinds of records; she is eager to set another date.

You, too, can have a successful blood drive! It was all possible because of teamwork and a cause that we all believe in. Many thanks to the St. Catherine parishioners and all those who came out to support the drive and donate, the bloodmobile staff, Kathy Widner, Mint Bhetraratana, Laurice Levine, Tom Cook, and Andrea Casson.

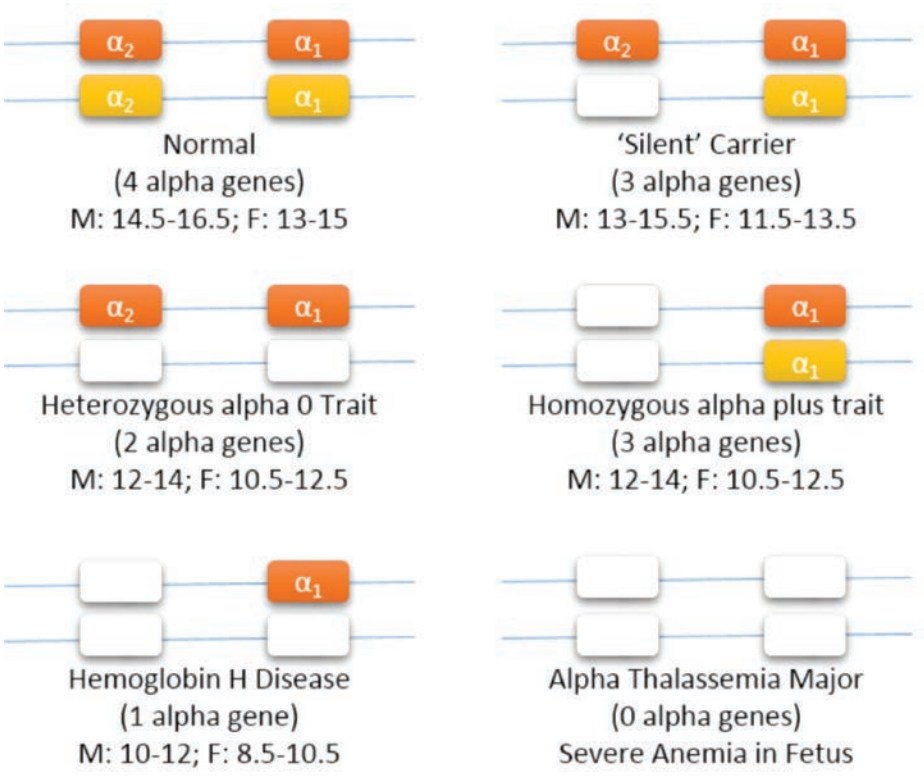
Focus on Alpha Thalassemia

A series to mark the establishment of the Multidisciplinary Center for Intrauterine Therapy in Alpha Thalassemia Major: a collaboration between UCSF Benioff Children's Hospital Oakland and the UCSF Fetal Treatment Center.

by Ash Lal, MD

What Is Alpha Thalassemia?

Thalassemia is caused by the inability of red blood cells to synthesize hemoglobin. Every hemoglobin molecule is formed by four globin proteins (tetramers), of which two are alpha-like globins (alpha or zeta) and two are beta-like globins (beta, gamma, delta, or epsilon). Tetramers of various combinations of these globin proteins produce hemoglobins suited to different stages of life. The most common hemoglobins are adult hemoglobin, or HbA (alpha-beta), and fetal hemoglobin (alpha-gamma). Thalassemias are named after the particular globin proteins that have their synthesis affected by gene mutations. For example, in beta thalassemia, mutations in beta globin gene cause deficiency of beta globin. Similarly, there is deficiency of alpha globin due to nonfunctioning alpha globin genes in alpha thalassemia.



Genetic basis of alpha thalassemia: Two alpha genes (α2 and α1) are located on each chromosome 16 (red and yellow chromosomes are inherited from different parents). White boxes indicate nonfunctioning alpha genes. The usual hemoglobin range in adults (males and females) is shown for each genotype.

There is one beta gene on each chromosome 11 (one inherited from the mother and one from the father). Absence of one beta gene causes beta thalassemia trait, while absence of both genes causes beta thalassemia major. As shown in the figure, alpha genes are different, since there are two copies (called alpha 1 and alpha 2) on each chromosome 16. Most alpha thalassemia is caused by large deletions, and only some cases are the result of point mutations in alpha genes. These genetic factors are responsible for the tremendous variability in the clinical symptoms seen in alpha thalassemia. If one out of four alpha genes is absent, the hemoglobin level and size of red blood cells are slightly reduced, but there is overlap with the range in the general population. This condition is called alpha thalassemia silent carrier or heterozygous alpha plus (α^+). Next, the absence of two out of four alpha genes causes a definite decrease in hemoglobin level and red blood cell size which can be detected with routine blood tests. This condition is called alpha thalassemia trait (homozygous α^+ trait if the missing genes are on opposite chromosomes or heterozygous α^0 trait if both missing genes are on the same chromosome). The anemia, which is mild and causes no symptoms, can be mistaken for iron deficiency anemia or beta thalassemia trait. DNA testing is needed to make a diagnosis of silent carrier and alpha thalassemia trait. The more severe forms of alpha thalassemia are caused by the absence of either three or all four alpha genes. When three alpha globin genes are missing, patients have hemoglobin H (HbH) disease. This is associated with a moderate anemia. The common type of HbH disease does not require transfusions, but individuals with variants of HbH—particularly HbH Constant Spring—may need occasional transfusions. Detailed DNA analysis is essential in all of these patients, as it can predict the clinical course of the disease. HbH disease can be diagnosed during newborn screening.

Focus on Alpha Thalassemia

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Alpha Thalassemia Major

The most severe form of alpha thalassemia is alpha thalassemia major or Bart's hydrops fetalis. This disease occurs when all four alpha genes are missing. During fetal life, the main hemoglobin is fetal hemoglobin (HbF), which is made up of alpha-gamma globin tetramers. Even earlier hemoglobins are called embryonic hemoglobins, which consist of combinations of alpha or zeta globins with epsilon or gamma globins. The importance of alpha globin during fetal life is what makes alpha thalassemia major different from beta thalassemia major. The synthesis of fetal hemoglobin (alpha-gamma) is unaffected in beta thalassemia major, so that babies are born with normal hemoglobin levels but develop anemia after several months. In alpha thalassemia, fetal hemoglobin cannot be synthesized; therefore, anemia starts before the baby is born. This profound anemia has severe effects on the development of the fetus, and alpha thalassemia major is fatal during fetal life or soon after birth. The term hydrops refers to the consequences of anemia, which causes massive enlargement of liver and spleen and, eventually, heart failure. Survival is only possible if blood transfusions are given to the fetus (intrauterine transfusions) and then continued after birth throughout life. There is also an increased risk of maternal complications when the fetus has hydrops.

Alpha thalassemia major is more common in individuals of Southeast Asian and Chinese origin. It can be successfully treated provided the pregnancy is anticipated or the diagnosis is made early with ultrasound and genetic testing of the fetus. Several patients have been cured with bone marrow transplants after birth.

The establishment of the Multidisciplinary Center for Intrauterine Therapy in Alpha Thalassemia Major brings together expertise in diagnostic, prenatal, intrauterine, perinatal, and hematological management for this complex disorder. The center is a collaboration between the UCSF Benioff Children's Hospital Oakland Thalassemia Program and the UCSF Fetal Treatment Center. More information is available at our website: www.thalassemia.com/services-intrauterine-therapy.aspx#gsc.tab=0.

For more information about our program, treatment, and consultations, please contact:
Elliott Vichinsky, MD
(510) 428-3651 or
EVichinsky@mail.cho.org

Northern California Comprehensive Thalassemia Center:
(510) 428-3347

Tippi MacKenzie, MD
tippi.mackenzie@ucsfmedctr.org
UCSF Fetal Treatment Center:
(800) RX-FETUS

Stephanie Welty, CPNP,
Thalassemia Clinical Program

Welcome, Stephanie Welty

Stephanie is a licensed Pediatric Nurse Practitioner who joined the Thalassemia Team in August 2014. She earned her Master's Degree in Nursing at Columbia University. After completing her MSN she worked in primary care for low income families. Stephanie helps coordinate clinics for non-transfused and transfusion dependent patients and manages their care. Her major interest is in improving the care of patients with Thalassemia and helping them manage their own health. Stephanie has a two-year-old Australian Cattle Dog. She enjoys spending time outdoors with him, especially hiking around the Bay Area.

INTERVIEW WITH INSUB MONMEESIN:

My Experience with Gene Therapy

by Mint Bhetraratana

Imsub Monmeesin is a 22-year-old student from Thailand. She has beta-thalassemia and recently concluded her participation in Bluebird Bio's Northstar Study at UCSF Benioff Children's Hospital Oakland. This experimental gene therapy involves inserting a functional human βeta-globin gene in the patient's stem cells ex vivo—meaning that the cells are altered outside the human body—and then reinserting these modified stem cells into the patient's bloodstream. This process is also known as autologous stem cell transplantation (www.bluebirdbio.com). I was able to sit down with Imsub—or "Ae," as she is known to her friends—to talk to her about her journey. The following is an excerpt from an interview translated from Thai.

Imsub on the last day at the hospital: (r-l) Imsub's mother, Imsub, Cyrus Bascon and Marci Moriarty (from the BMT Team)

MB: Hi, Ae, let's start with the basics. How would you describe your home?
IM: I have a one-story home. I have a big family and I live in the country where we raise pigs.

MB: What do you study in Thailand, and what are your career goals?
IM: I am in my second year studying pharmacy at Srinakharinwirot University. When I finish school, I would like to become a pharmacist. But I would also like to own my own café and sell pastries.

MB: How old were you when you were diagnosed with beta-thalassemia?
IM: I was diagnosed with thalassemia at two years old, because I was sick for a long time. I went to the doctor and was treated at the Ramathibodi Hospital from then until now. Also, my sister was tested for the disease, and we found out she also has thalassemia. We both were treated.

MB: What was one of the challenges you faced growing up with beta-thalassemia?
IM: Oh, well, I don't remember much from when I was a child, but what I do remember is that I had to see the doctor every month to get blood transfusions and take my medications.

MB: Can you describe what your treatment is like in Thailand?
IM: In addition to getting blood transfusions and medications, I also have to get lab work done as the doctor orders. For instance, I have to get MRIs and other tests done that the doctor recommends to check my overall health. When I was younger, we looked into the possibility of getting matched for a bone marrow transplant. There was no match in my community, so we sent my blood work to Taiwan, but there was still no match.

MB: Do you know a lot of people who have thalassemia in Thailand?
IM: I do not know of anyone else close who has thalassemia [other than my sister], but I became friends with the other thalassemia patients I met at the hospital.

MB: Why did you want to participate in the Bluebird Bio Northstar study? Was it a hard decision to make?
IM: In the beginning, it was hard. At first, I didn't want to [participate in the study], because I didn't want to miss school and fall behind in my studies. At that time, there wasn't a lot of information [about the study], because it was new, so I wasn't sure if it would be safe or what to expect. It was a hard decision. But my parents wanted me to do it, and after speaking to a lot of doctors and getting more information about this therapy, I decided to do it, because we thought this could be a possible cure.

MB: Had you been to the United States before? When did you first arrive and who did you come with?
IM: This is the first time I have been to the United States. I came to the States in April 2014; this is also my first time outside of Thailand! I came with my mother. When I arrived in the United

Patient Perspective: My Experience with Gene Therapy

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States, I thought my English wasn't that good, but I wanted to visit this country.

MB: What other places did you visit in the country?
IM: I went to San Francisco and visited Pier 39. [She also visited Oakland, Los Angeles, and Arizona.]

MB: What was your favorite place?
IM: [Imsub turns to ask her mom this question.] My mom liked many places! I liked San Francisco and L.A. the most.

MB: Can you explain the timeline of the gene therapy process?
IM: For the first two weeks, I had to get physical exams and other medical tests. Then my stem cells were collected over four days. My stem cells were then sent to the lab, which took about five to six weeks. Then they had to collect my stem cells for a second time, so it took longer than expected. After my stem cells were modified, I had to stay in the hospital for 40 days to get them transplanted. Overall, I will have stayed in the United States for almost nine months. [She went back to Thailand at the end of December.] I thought it would only take five to six months.

MB: Overall, was the gene therapy what you expected?
IM: Yes, it was what I was hoping for. All of my lab work is okay now. I'm not sure what to expect in the long run, but I believe I will get better.
MB: Were there any challenges with the gene therapy?
IM: I am uncertain because this was an experimental treatment, and the results are not yet known.

MB: What does completing gene therapy mean to you?
IM: I hope to be cured of thalassemia, which means that I wouldn't have to get monthly transfusions or take medications. If this treatment is successful, I will be cured, and others will be, as well!

MB: Are you cured of beta-thalassemia? What are your next steps?
IM: At this point, I am not 100 percent sure that I am cured because it has only been two months since the end of the treatment. I will need continuous follow-up. Having needed blood transfusions every month—to now not need them for these past two months, I feel like it's so far so good. When I go back to Thailand, I will have to get a monthly checkup, and see if everything is okay.

MB: Is there anyone that you'd like to thank who's helped you on your thalassemia journey?
IM: I would like to thank everyone! I would like to thank all of the bone marrow transplant team, the thalassemia team, the doctors, and all of the staff who helped me at the hospital. I would like to thank Dr. Lau, Dr. Vinchinsky, and Mint, too. [All laugh.] I would also like to thank my Thai interpreters, Joy and Benjawan.

MB: Please finish this sentence. One thing that I want people to know about me is…
IM: Wow, that's a hard question. I think…even though I have thalassemia, I will not give up; I will keep on fighting.

MB: How about this? One thing that I want people to know about thalassemia is…
IM: Some people may see thalassemia as a hardship, but I think it helps me know how to take better care of myself. I hope in the future, [this therapy] will provide a cure, and that everyone will take care of themselves and be well in their treatment. My mom also says that before you decide to have a family, you should get tested to see if your child will have thalassemia.

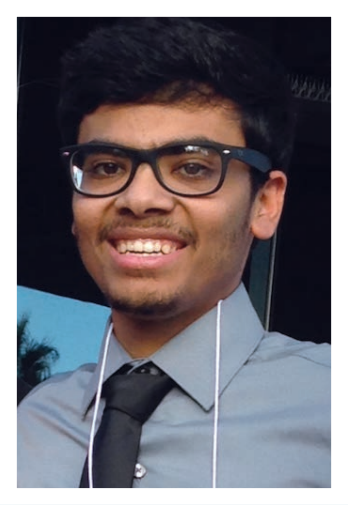
MB: What is the first thing that you will do when you go back to Thailand?
IM: I will hug my dad!

Imsub and Mint Bhetraratana, friend and UCB thalassemia outreach intern.

MB: Will you come back to visit the United States?
IM: I will definitely come back to visit the United States, but not for treatment—for vacation, because there are many places I want to see!

MB: Thank you for your time, Ae.
IM: Thank you.

Mint Bhetraratana has been a Thalassemia Outreach intern since June 2013 after graduating from UC Berkeley with a B.A. in Public Health and Integrative Biology. She also works as an advocate coordinator with Health Leads and as a medical and dental assistant with a local nonprofit called Kerry's Kids. Mint has enjoyed getting to know Ae over this past year and loves educating the community on thalassemia at health fairs and blood drives.



Siddant Talwar

PATIENT PERSPECTIVE

Thalassemia: The Passion to Help Others

by Siddant Talwar

At the time of my diagnosis with thalassemia, I am sure that the fact of it was a hard pill to swallow for my family. Today, after my whole life's worth of time, that perspective has shifted.

The journey has been an incredible one for me, full of many emotions and ups and downs, but ultimately, it has taught me to feel thankful and fortunate for all that I am blessed to have. My family and I moved from India 14 years back in the hopes of better treatment for me. Having spent the majority of my life in America, I have embraced the lifestyle while making sure that I retained my cultural values and traditions. Over the years, I made great friends in high school and in college (currently in am a junior at the University of Arizona). However, it is the support system I have had for the past 14 years from family, school teachers/professors, and most importantly, the nursing and doctoring staff, that have been so inspiring for me. The love and care with which I was treated made every poke from the transfusions and chelation medicines seem so insignificant. The constant encouragement and compassion left me understanding that the same type of committed care I received is one thing that would inspire anyone as it did for me.

I slowly recognized that I wanted to make an impact on people's lives and became more inclined toward the field of medicine to fulfill my aim. But with time, it grew apparent that there were things that could be and needed to be done before I could help patients directly with their care. Upon moving to America, my family settled down in Phoenix, Arizona, where, at the time, there were basically two or three thalassemia families residing. Today, we have somewhere between 20 and 30 families. This increase has been quite eye opening for everyone in my family and served to motivate us to try to help decrease the spread of thalassemia. In response, we started to attend local community events to raise awareness of thalassemia with the help of Children's Hospital Oakland, as well as Phoenix Children's Hospital. Attending these events slowly transitioned into us opening our local nonprofit, Thal Anemia Foundation, based in Phoenix.

The nonprofit holds a conference annually where we invite doctors specializing in the field of thalassemia to share their indispensable knowledge with the families of Phoenix. Our aim is to promote as much knowledge as possible in regard to thalassemia with the intention of reducing the spread of the disorder. Although the nonprofit is in its beginning stages, we hope to grow the organization on a broader level and perhaps even reach patients internationally.

Living with thalassemia has been a humbling experience. Beyond the hardships, it provides a different perspective and perhaps a deeper respect for life. I am very blessed to be in the position that I am in today and strive to help others like myself to reach the same place in life.

Please feel free to contact Thal Anemia Foundation at thalanemia@gmail.com with any questions, concerns, or suggestions.

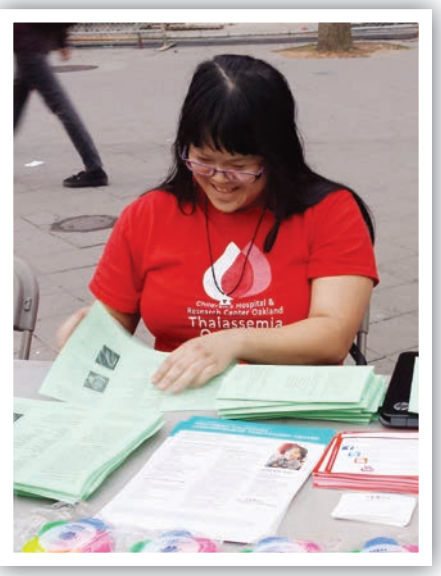
Outreach Highlights

Find out what has been going on for patients, families, and the community

UC BERKELEY THALASSEMIA INTERNSHIP

Still going strong and educating the community about thalassemia

Caroline Levan raises awareness of thalassemia at the Federation of Indo Americans in Fremont, CA.



Christine Dinh passes out literature and recruits students for the UCB DeCal Class "Thal at Cal—Invisible No More."

DeCal Students (l-r) Mohith Subbarao, Chibuzo Nwokocha, Beatrice Popescu and UCB Intern Christine Dinh

UC BERKELEY BLOOD DRIVE

31 people showed up to the Thal at Cal DeCal Thanksgiving Blood Drive, on November 25 ready to 'roll up their sleeves.' With 9 people deferred (unable to donate), 22 individuals were able to give the gift of life. We appreciate the support of all participants! When blood is broken down to its components (red cells, platelets, plasma) each unit collected has the potential to touch three different lives. This means that the generosity of blood donors may be the difference between life and death for 66 Bay Area patients. That is 66 people's lives that will be saved through your efforts!

Blood Drop with UCB Interns Christine Dinh and Caroline Levan

Special Thanks to the Blood Drive Coordinators:
Edith Yuan
Leslie Yeh
Christine Dinh
Caroline Levan
and the DeCal Team

Visit us at www.thalassemia.com. The website is always being updated. Please refer to it for new stories, resources, event calendar, and more.

ITALIAN CATHOLIC FEDERATION HIGHLIGHTS

Throughout the year Laurice Levine attends ICF events with the goal of educating members about thalassemia, the ICF's national charity. After 15 years of these visits, it continues to be a pleasure catching up with old friends and meeting new ones.

ICF Branch 28, Gilroy, Executive Christmas Party

(left to right) Frank and Louise Segreto, Kathy Santos, Pat Cordich and Roger Santos (Branch President).

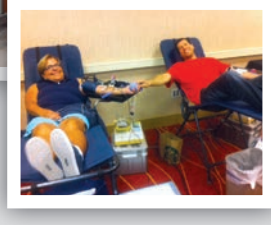
ICF Branch 28 members Roger and Kathy Santos make a donation to the thalassemia program. Their annual fundraiser, "The Mark Santos Motorcycle Run" in memory of their beloved son, raised over $3,000.

ICF Branch 374, La Canada Flintridge, Monthly Meeting

ICF Member Penny Baisley and Laurice Levine.

Mark G. Mannarelli, current President of ICF Branch 374, Laurice Levine, and Al Restivo, immediate past president of 374 and current Los Angeles Regional Vice President of the ICF Los Angeles Archdiocese District Council.

ICF Convention 2014, Oakland, CA

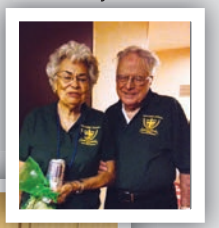


ICF members (and cousins) Pat Cordich, from Gilroy,and Danny Centurioni, from Las Vegas, give the gift of life.

ICF Members tour UCSF Benioff Children's Hospital Oakland.

ICF Member BG Zasoski donates to save a life!

ICF Members Stella and Leroy Taddei.



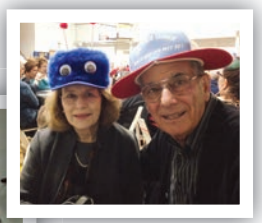
Michelle Feldman, Theresa and Tad Shaw, and Fabiola DiPaolo enjoy food, friendship and fun at the convention banquet.

Central Council Member Rose Jarrett donates blood.

ICF Branch 14, Crocket, Toy Donation

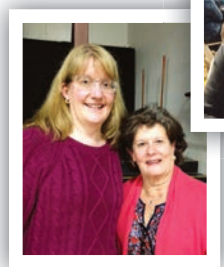
Johnny Valentini, Donna Hoffman (Branch President), and David Botta.

ICF Branch 52, Healdsburg, "Crazy Hat" Polenta Dinner



Jackie and Dante Corsetting don their crazy hats

ICF Central Council Member Lauren Kilcullen and Branch President Nancy Seppi strike a pose.



Thank you to the ICF for their genuine hospitality, friendship, and generosity.

Upcoming events

Dates subject to change and events are added frequently.

For the most current updates, please refer to our website, www.thalassemia.com/news-events.aspx.

APRIL 2015

Saturday, Apr. 18, 9:30 a.m. – 4:45 p.m.
Thalassemia Support Foundation Conference, Children's Hospital Los Angeles, CA

For more information: www.helpthals.org

For a direct link: www.helpthals.org/blog/2015/03/01/6th-thalassemia-support-foundation-conference/

Tuesday, Apr. 21
UC Berkeley DeCal Panel Presentation: Parent's Perspective, Berkeley, CA

Saturday, Apr. 25
5K Walk/Run to Benefit Thalassemia, UC Berkeley, CA

Saturday, April 25
UCB Thalassemia Intern Team Spring Dinner, Berkeley, CA

MAY 2015

Thursday, May 7, 9 a.m. – 2 p.m.
14th Annual Blood Drive in Honor of World Thalassemia Day, UCSF Benioff Children's Hospital Oakland, Outpatient Center, Oakland, CA

Friday, May 8
World Thalassemia Day

Saturday, May 9
Patient and Family Mini-Conference, Seattle, WA

Saturday, May 9
Root in the Boot Walkathon Benefiting Thalassemia, Lafayette, CA

Sunday, May 31
Mark Santos 7th Annual Motorcycle Run Benefiting Thalassemia (ICF Branch 28), Gilroy, CA

JUNE 2015

Sunday, Jun. 14
World Blood Donor Day

Safe blood supplies are a scarce commodity—especially in developing countries. World Blood Donor Day is observed by all member states of the World Health Organization and is an occasion to raise awareness of the problem and thank donors worldwide. It is held annually on June 14 which is the birthday of Karl Landsteiner who won the Nobel prize for his discovery of the ABO blood group system.

Sunday, Jun. 14, 10 a.m. – 3 p.m.
Oakland Chinatown Lion's Club 28th Annual Health Fair, Oakland, CA

Wednesday, Jun. 24
Thalassemia Presentation at the Kiwanis Club, La Canada Flintridge, CA

JULY 2015

Thursday, Jul. 9
Adoption Comprehensive Clinic, Oakland, CA

Friday, Jul. 17 – Saturday, Aug. 22
The Painted Turtle Camp Thalassemia/Hemophilia Week, Los Angeles, CA

For more information: www.thepaintedturtle.org

Visit us at www.thalassemia.com. The website is always being updated. Please refer to it for new stories, resources, event calendar, and more.

AUGUST 2015

Italian Catholic Federation 88th Annual Convention, Oakland, CA

Thursday, Aug. 27 – Sunday, Aug. 30
www.ICF.org

Friday, Aug. 28 – Sunday, Aug. 30
Outreach and Education Table with Literature

Friday, Aug. 28
"Live to Give" Blood Drive
Sponsored by UCSF Benioff Children's Hospital Oakland

Saturday, Aug. 29, 7 p.m.
Banquet

Sunday, Aug. 30, 9 a.m. – 12 p.m.
Presentation on Thalassemia, Research Updates, and Outreach

SEPTEMBER 2015

Friday, Sept. 25 – Sunday, Sept. 27
Feast of San Gennaro Italian Festival, Los Angeles, CA

OCTOBER 2015

Sunday, Oct. 18 – Thursday, Oct. 22
Tenth Cooley's Anemia Symposium, Presented by the Cooley's Anemia Foundation and the New York Academy of Sciences, Loews Chicago O'Hare Hotel, Chicago, IL

For more information:
www.nyas.org/Events/Detail.aspx?cid=244781db-3089-4f17-8add-f16c73d821fb

DECEMBER 2015

Saturday, Dec. 5 – Tuesday, Dec. 8
57th American Society of Hematology (ASH) Annual Meeting & Exposition, Orlando, FL

For more information: www.hematology.org/Annual-Meeting/

Date and details TBA
Annual Thalassemia Holiday Party, Oakland, CA

7TH ANNUAL
MARK SANTOS MEMORIAL
PASTA & POKER
MOTORCYCLE RUN
PROCEEDS BENEFIT COOLEY'S ANEMIA
MAY 30, 2015

$25.00 SINGLE RIDER
-- $30.00 DAY OF EVENT
$40.00 RIDER & PASSENGER
-- $45.00 DAY OF EVENT
($15.00 NON-RIDERS)

1195 BUENA VISTA AVE. GILROY, CA
SIGN-INS 10:00 TO 11:30

PRE-REGISTER BY MAY 23 CALL, EMAIL, OR MAIL YOUR RESERVATION.
ROGER OR KATHY SANTOS 408-842-8547, RKSANTOS@RAZZOLINK.COM,
MAIL: 1195 BUENA VISTA AVE. GILROY, CA, 95020

PLEASE JOIN US FOR A LEISURELY RIDE IN THE SCENIC HILLS OF GILROY, THEN BACK FOR A FABULOUS LUNCH. INCLUDES MEAL, POKER HAND, RAFFLE AND LOTS OF FUN AND RELAXATION.

SPONSORED BY: ICF BR. 28 MAKE CHECKS TO: ICF BR. 28

JULY 17 – AUGUST 22, 2015

The Painted Turtle Camp

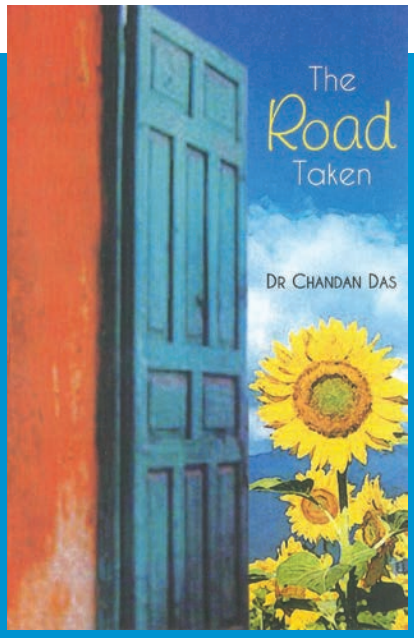
Now accepting online applications for campers for Thalassemia/Hemophilia Week

www.thepaintedturtle.org

Camper Admissions Office at
(661) 724-1768, ext. 203 or ext. 202

PERSPECTIVES
Northern California Thalassemia Center
UCSF Benioff Children's Hospital Oakland
747 52nd Street, Oakland, CA 94609

Address change requested



Chandan Das who was featured in the *Perspectives* newsletter Winter 2014 issue, recently published a book, The Road Taken, which can be ordered at: varuna@lifepositive.net. We would like to thank Chandan for sharing his amazing life story as a person living with thalassemia and raising awareness of thalassemia across the globe.

New literature available from the UCSF Benioff Children's Hospital Oakland Thalassemia Outreach Program

- Updated program brochure
- Thal disease and trait
- Thalassemia among the chinese population
- Social media postcard

Visit us at www.thalassemia.com

The website is always being updated. Please refer to it for new stories, resources, event calendar, and more.

Scan this with a QR reader app on your smartphone for the new mobile version of the Thalassemia Standard of Care Guidelines.

Perspectives is produced by Senior Thalassemia Outreach Coordinator Laurice Levine, MA, CCLS, at UCSF Benioff Children's Hospital Oakland. For questions regarding the newsletter or for more information on thalassemia, call (510) 428-3885, ext. 5427, or visit www.thalassemia.com. If you no longer wish to receive *Perspectives*, please email Laurice at llevine@mail.cho.org.