

- 
- 2 Hot Weather Health Tips
 - 3 The Believe Trial
 - 4 The Growing Number of Children in the United States With Thalassemia
 - 6 Provider Spotlight: Susan Carson
 - 6 Thal Anemia Foundation Inc.
 - 8 Cooley's Anemia Foundation, California Chapter Reopens

Upcoming Events

Thalassemia International Federation

**14th International Conference on Thalassaemia & Hemoglobinopathies
and 16th International Conference for Patients & Parents**

Nov. 17-19, 2017

Thessaloniki, Greece

For more information:

<http://thalassaemia.org.cy/>

Thalassemia Holiday Party

Friday, Dec. 1, 2017

7-9 p.m.

Stauffer Conference Room
Children's Hospital Los Angeles

RSVP:

krussell@chla.usc.edu or 323-361-3269



Health Tips for Hot Weather

Summer may be ending, but hot weather continues in southern California for a few more weeks.

- Stay out of the direct sun. We need sunlight to thrive and stay healthy because the sun provides essential vitamin D; however, staying out of direct sunlight, especially between the hours of 10 a.m. and 4 p.m., can lead to exposure to harmful ultraviolet rays. For more information about sun safety, go to: healthcommunities.com/sun-safety/overview-of-sun-safety.shtml
- Wear sunscreen of SPF 30 or higher, with both UVA and UVB protection. Beware! Even on cloudy days you can get burned.
- Drink plenty of fluids—water is the best choice. Avoid alcohol and caffeinated beverages (such as coffee and soda). Soda may taste good, but contains empty calories and has no nutritional value.
- Wear light-colored, lightweight clothing, a wide-brimmed hat and sunglasses to avoid harmful ray exposure.
- If you display signs of heat stroke (nausea, NOT sweating, altered mental state) seek medical attention immediately.
- Foodborne illness is more common during the summer. To learn more about food safety, go to: foodsafety.gov/keep/events/summervacations/index.html
- Swimming is a great way to beat the heat and a terrific form of exercise. For safety tips at the ocean, go to www.parks.ca.gov and look under the “Visit Parks” tab.
- Eat healthy, especially seasonal fruits and veggies. Check out your local farmer’s market for the freshest organic produce.
- Take care of your health when you travel by following these tips:
 - Get required shots/immunizations if traveling outside the country.
 - Have your doctor write a travel letter documenting your condition, medications you take, and medical supplies that you use and will be traveling with.
 - Make sure you have enough prescription medication to last the duration of your trip.
 - Adjust your transfusion schedule as needed. Plan ahead to assure you get the appointments you need.
 - Sign up for Medic-Alert at medicalalert.com
 - Be informed: Know what to do in case you get sick during your trip. For more information on obtaining medical care abroad, go to: wwwnc.cdc.gov/travel/page/getting-health-care-abroad
 - Share information about your trip with family and friends. Have a designated emergency contact at home and leave behind a copy of your passport, a copy of your prescriptions and contact information for your doctor.

For more travel tips, go to:
wwwnc.cdc.gov/travel/page/important-info

The Believe Trial

An Efficacy and Safety Study of Luspatercept (ACE-536) Versus Placebo in Adults Who Require Regular Red Blood Cell Transfusions Due to Beta (β) Thalassemia (BELIEVE)



The Believe trial was opened by Celgene in August 2016 at 73 sites around the world. Children’s Hospital Los Angeles is one of the sites under the direction of Principal Investigator Thomas Coates, MD, and was the first to open and start treating patients.

The hallmark of thalassemia is the body’s inability to make red blood cells effectively. Red blood cells are produced in our bone marrow from stem cells. They go through many phases as they “grow up” into the mature cells that circulate through our body, carrying vital oxygen and other nutrients. Patients with thalassemia try to make lots of red blood cells, but the cells never develop fully and die in the marrow. Side effects in non-transfused and under-transfused thalassemia patients are caused by this ineffective blood making (erythropoiesis). They include bone disease, thalassemic facies, spinal tumors, enlarged liver and spleens, and growth failure.

Transfusions serve not just to increase hemoglobin and make patients feel better, they also suppress the bone marrow; red cell production slows down as the body detects that hemoglobin levels are high

enough. This is a goal of transfusions. Due to the side effects of transfusion—including iron overload—researchers through the years have looked for other ways to increase hemoglobin levels in thalassemia patients. Other drugs such as hydroxyurea have been tried. Hydroxyurea raised hemoglobin in some patients, but did nothing to correct their overactive blood-making drives, so those patients still had the same problems as before.

Luspatercept is designed to help the late-stage development of the red blood cells, which will hopefully raise hemoglobin by making erythropoiesis more effective. It’s a complicated mechanism, but when it works, it helps the body make red blood cells that grow up completely rather than stalling in the marrow. As mature cells make their way into the bloodstream, the marrow senses it can slow down—which is what we want it to do. Preliminary studies done in Europe had very promising results. Some patients who responded were able to come off transfusions and some were able to decrease their amount of blood, or increase the interval between the transfusions.

The BELIEVE study is a Phase III study. Its primary goal is to determine whether Luspatercept works or not. The protocol is looking at this by comparing the study drug to a placebo. One of every 3 patients is randomized to receive the placebo. Neither the investigators nor the patients know what they are getting. The medication syringes look exactly the same, but some patients are getting Luspatercept and some are getting saline water. “Blinding” the study helps all of us remain objective, and is standard research practice.

Patients will be treated for 48 weeks to see if their transfusion requirements are reduced. Investigators will also be looking at iron levels, bone density, quality of life and other measurements. Once they have all the data in, they will analyze it to see if the medication worked. If it did, they will use the information to apply to the U.S. Food and Drug Administration (FDA) for approval. This process will take many years.

The study has proven to be very popular and enrollment advanced very quickly. Due to FDA rules, the study was only available to adults. Though some sites may still be open, we closed enrollment earlier this year. We are very excited to be part of this study and look forward to seeing the results.

Feel free to ask us more about this study and all the new research going on with thalassemia patients when you come for your clinic visit! 

For more information about clinical trials, visit the Cooley’s Anemia Foundation page on current studies.

<http://www.thalassemia.org/learn-about-thalassemia/clinical-trials/>

The Growing Number of Children in the United States With Thalassemia

by Liberty Joy

Did you know that the number of people who have thalassemia in the United States has been on the rise over the past few years? The reason for this growth might be surprising. Dozens of children diagnosed with thalassemia are being adopted by American families annually. These children are adopted primarily from China; however, there are a few who come from India and Vietnam as well. Most of these children have beta thalassemia major, and their adoption has given them a future that they otherwise would likely not have known.



In China, parents must pay up front for any medical care that their child receives. This means that if they have a child with an illness or a chronic condition, they must walk into the hospital with money in hand for their children to receive the medical care they need. For a family that has a child with thalassemia, this can become burdensome quite quickly, especially for the working class or for migrant workers. Families simply cannot pay for the ongoing lab work and transfusions. Many of these families face the unthinkable, a most difficult choice between two options that both result in a huge loss. They can keep their children and be unable to provide them with the necessary medical care and watch them die, or they can abandon their children and hope that the government will provide the foundlings with the medical care they need to survive.

Our son's story began with a brave family in China making the impossible decision to leave him in a hospital corridor at what was estimated to be 3 months of age. He was severely jaundiced, had a hemoglobin level lower than 4, and was in very poor shape. He was sent to live in the local orphanage, where he received the minimum medical care he needed to survive. The orphanage officials began to prepare his paperwork to make him eligible to be adopted internationally. Blood donations and blood transfusions remain taboo in Chinese society, which has led to blood shortages nationwide. In fact, there is very little blood available in the southern provinces of China. Our little guy was in a very poor orphanage in a small city in southern China. He only received a blood transfusion when his hemoglobin level fell

below 6, which was generally every eight to 10 weeks. The lowest hemoglobin he had (that we know of) was right before his first birthday, when it was 2.3.

Our son, Daniel, remained in China until he was 16 months old, when we finally completed mountains of paperwork and hours of education, paid all of our fees, and received approval from both governments to complete the adoption in the summer of 2013. Daniel is one of over 150 children with thalassemia who have been adopted and who now live and receive care in the United States. Daniel has been home for almost four years, is very healthy, and receives excellent care under physician Thomas Coates, MD, and nurse practitioner Susan Carson, RN, MSN, CPNP, at Children's Hospital Los Angeles.



they have low growth and developmental delays. The only chance at life these children have is to be adopted by families in the United States, Canada or Europe. Currently, there are many children with thalassemia in China—mostly boys—who wait for families to choose to adopt them.

Let me share three of the many faces with you:

Evan: <http://www.nohandsbutours.com/2017/02/24/waiting-child-highlight-evan>

Owen: <http://www.nohandsbutours.com/2017/04/24/urgent-medical-need-owen>

Shaw: <http://www.nohandsbutours.com/2017/04/22/waiting-for-you-tommy> 

We returned to China to adopt two more children in 2014 and 2016, the youngest of whom we named Ava and who also has beta thalassemia major. She was frail and very small upon her adoption but is now thriving and growing like crazy under the expert care at CHLA.

We are so fortunate to have these children in our family. As an added bonus, we feel like our family has been immensely blessed by becoming part of the amazing thalassemia community in the United States.

In China, the average life expectancy for orphaned children with thalassemia major is about 10 to 15 years. They receive substandard whole blood transfusions, most do not receive any chelation, and



For more information about the adoption of a child with thalassemia from China, please feel free to contact Liberty Joy at chadandliberty@gmail.com. You can also visit these sources of information for families considering adopting a child with thalassemia:

choosingthalassemia.blogspot.com | nohandsbutours.com/types/blood-conditions

nohandsbutours.com/category/thalassemia

rainbowkids.com/adoption-stories/top-ten-questions-families-ask-about-thalassemia-831



Provider Spotlight

Susan Carson, MSN, RN, CPNP
Nurse Practitioner

Susan Carson, MSN, RN, CPNP, is a widely published, internationally known expert in thalassemia and iron overload. Carson was a primary driver in establishing the Iron Overload Clinic at CHLA, the first of its kind in the nation to assess and treat non-hematology patients who have transfusion-related iron overload. As nurse practitioner for the PASSAGES Program, she consults on thalassemia and iron overload therapy for patients of all ages, as well as physicians outside of CHLA. Carson sees many young adults and adolescents for annual comprehensive care assessments along with her hematologists, and then assists with case management in between appointments. As a researcher, she was one of three founders of the Phenomenon of Pain in Thalassemia study, part of the National Institutes of Health-funded Thalassemia Research Network. In her latest project, Carson is the principal investigator for a pilot study of a smartphone app aimed at promoting medication adherence and knowledge in patients ages 13 to 21. Her co-investigators include Anne Nord, BSN, RN, CCRP, nurse navigator for the PASSAGES transition program. A graduate of the University of Toronto, Carson began working with hematology patients in 1996 as the thalassemia nurse coordinator at the Hospital for Sick Children in Toronto. She completed her master's degree at the University of California, Los Angeles (UCLA) in 2001 and began working as a nurse practitioner in benign hematology with a focus on thalassemia, sickle cell disease, aplastic anemia and iron overload. Since 2003, Carson has been an assistant clinical professor in the UCLA School of Nursing, lecturing to both pediatric and family nurse practitioner students on the diagnosis and management of common hematological disorders.



*CAF-Cali meet and greet,
Rady Children's Hospital*

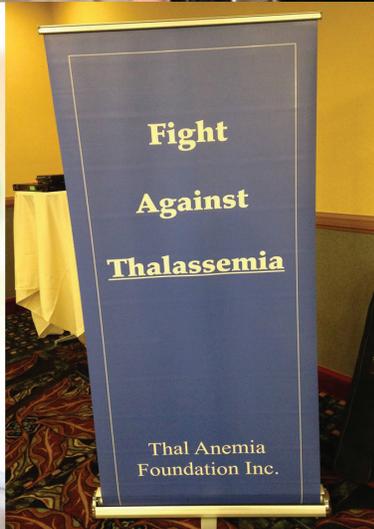
Thal Anemia Foundation Inc.

Thal Anemia Foundation is a family-owned nonprofit organization that was founded in Phoenix, Arizona, in 2009. At the time, the number of thalassemia patients was growing, and the lack of a collective thalassemia group prompted my family and me to take action to have an organized patient community.

In the initial few years, and even prior to the launch of the nonprofit, my parents and I would participate in local Indian cultural events in Phoenix and Oakland, California, to organize a stall to advocate for thalassemia awareness and prevention. The next step was to bring knowledge and current developments in treatment



Siddhant Talwar



Sue Carson, NP

to families. In 2012, we successfully organized and held our first thalassemia educational conference. As part of the event, we invited a variety of thalassemia specialists from around the world to share their expertise in thalassemia research and care. We held a similar conference in 2014 and another in April of this year. In addition to thalassemia education conferences, we also hold annual family support-group events where the thalassemia families of Phoenix can come together to share their experiences, as well as any questions or concerns. These sessions typically are less formal and serve as a way to have open communication between families. Families are able to

share knowledge and brainstorm ways of approaching treatment.

We aim to continue organizing the support-group meetings and education conferences and attend more cultural outreach events. We look forward to continuing our service to the community of patients in Phoenix and hope to reach communities beyond Arizona. Our goal is to contribute positively to the well-being of thalassemia patients and families as we hopefully grow closer to finding a solution to this illness.

For any questions about the Thal Anemia Foundation or suggestions, please feel free to contact us at thalanemia@gmail.com. 

Please visit our website at thalanemia.org.

Thal Anemia Foundation Inc.

Vijay Talwar, Director and Co-founder,
Thal Anemia Foundation

Jyoti Talwar, Officer and Co-founder,
Thal Anemia Foundation

Siddhant Talwar, Officer and Co-founder,
Thal Anemia Foundation

Siddhant Talwar is a beta thalassemia major patient who resides in Arizona. He moved to Phoenix, Arizona, from India with his family back in 2000 in hopes of receiving better medical treatment. Siddhant finished his undergraduate studies in 2016 at the University of Arizona in Tucson, where he will be starting medical school later this year.

Cooley's Anemia Foundation California Chapter Reopens

By Maria Saradpon

Many of you already know about the Cooley's Anemia Foundation (CAF) and have come to rely on it for one reason or another. CAF is an amazing, supportive resource for thalassemia families. One of the many benefits to a relationship with CAF is getting to connect with fellow patients and families, as well as the best thalassemia doctors in the country. As thalassemia is so rare, it's nearly impossible to find fellow patients on our own. CAF brings the thalassemia community together, providing a much-needed support network to rely on. The only challenge for us in California is that the CAF national office and most regional chapters are on the East Coast.

We're proud to announce that CAF's sole West Coast chapter has been reactivated and is in full swing. CAF California is based in San Diego and is run by parents Maria Saradpon and Erica Martinez. Erica and Maria met at Rady Children's Hospital, where their daughters, Ella (9) and Kamila (3), receive treatment. Both girls are transfused every two to three weeks. CAF California's primary function is to fundraise for CAF National, but it also strives to connect fellow thalassemia patients and families. The chapter held its first official gathering, a meet and greet for local thalassemia families, at Rady Children's Ronald McDonald House on April 1, 2017. Its most recent event was a World Thalassemia Day celebration on May 6—a blood drive and fundraiser with a focus on raising awareness for thalassemia. Over 70 units of blood were collected, and so far, over \$4,000 was raised. The chapter



First-ever CAF-Cali meet and greet, Rady Children's Hospital

Patients Kamila Saradpon (left) and Ella Martinez braving arm pokes together like the brave warriors they are!



has decided to extend its fundraising efforts to the end of May in hopes of raising much more.

To connect with CAF California, please "friend" us on Facebook at [facebook.com/CAFCalifornia](https://www.facebook.com/CAFCalifornia), or email us at thalpalsofsandiego@gmail.com. You can also call or text chapter President Maria Saradpon at 619-739-0455. CAF California will hold a gathering every first Saturday of the month, and you're always welcome! Please connect with us and introduce yourself to stay in the loop. ✨



CAF California Vice President Erica Martinez and President Maria Saradpon at the World Thalassemia Day Celebration earlier this year.

Keep in touch!

If you would like to get thalassemia news and information via email, please write to krussell@chla.usc.edu. Thanks!

This newsletter is made possible through the kind support of the Italian Catholic Federation.

For more information visit ICF.org.