

## FDA approves new treatment option for patients with Thalassemia

The Cooley's Anemia Foundation (CAF), the only national non-profit dedicated solely to fighting the genetic blood disorder thalassemia, applauds a Food and Drug Administration (FDA) decision today to approve the new drug application for the oral chelator, Ferriprox. This action follows a 10-2 vote on September 14, 2011 by the Oncology Drugs Advisory Committee to recommend the approval of this drug.

"As the premier voice of the thalassemia community in the United States, we are thrilled that our patients will have the benefit of this drug which has proven beneficial to patients throughout the world," says CAF National President Anthony J. Viola. "The FDA clearly responded to the overwhelming need for this drug in our patient population and has provided those patients needing daily drug therapy to remove iron, an option that has improved cardiac health and prolonged life in thousands of patients over the past decade."

Ferriprox is an iron chelator, which is a drug that is used to help rid the body of excess iron, a serious and often fatal complication in thalassemia. Because individuals with the severe form of thalassemia are born with a life threatening anemia, they require lifelong blood transfusions as often as every two weeks. These transfusions overload the body with iron; if it is not removed, it settles in the organs, causing heart and



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liver failure, as well as numerous other complications.

For many years, the only FDA-approved chelator was Desferal, which must be administered by pumping the drug into the body for 8-12 hours, 5-7 nights per week. In 2005, the FDA approved Exjade, a chelator that is administered orally.

Ferriprox is also an oral chelator. In addition to being easier to administer than Desferal, Studies published in Europe demonstrate that use of Ferriprox has been shown to protect the heart from iron accumulation, a crucial concern for individuals with thalassemia.

"The major cause of death in our patient population is iron-related heart failure," says Viola. "And with a very significant percentage of our population unable to use either Desferal or Exjade, there is a vital need for another option; that option is Ferriprox."

"We have seen too many patients die too young," Viola concludes. "Our patients' lives depend upon having more treatment options available to them. The FDA addressed the unmet medical needs of these desperately ill patients and provided approval for a drug that will help them. Their decision has the opportunity to extend our patient's lives and significantly improve their quality of life."

Founded in 1954, the Cooley's Anemia Foundation ([www.cooleysanemia.org](http://www.cooleysanemia.org); (212-279-8090) is the only national non-profit organization dedicated solely to thalassemia. The Foundation's mission is advancing the treatment and cure for this fatal blood disease, enhancing the quality of life of patients and educating the medical profession, trait carriers and the public about Cooley's anemia/thalassemia major.



### FDA APPROVES FERRIPROX (DEFERIPRONE/L1)

The U.S. Food and Drug Administration recently approved Ferriprox (deferiprone/L1) to treat patients with iron overload due to blood transfusions in patients with thalassemia. Ferriprox represents the first new FDA-approved treatment for this disorder since 2005 when Exjade was approved. To read the press release and for more information please refer to: <http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm275814.htm>

Ferriprox is marketed by ApoPharma Inc. of Toronto.

## The Italian Catholic Federation and Thalassemia

The Italian Catholic Federation continues to amaze and inspire us with this year's contribution of \$77,000 to the Thalassemia (Cooley's Anemia) program at Children's Hospital Research Center Oakland. The ICF along with the Robert Kemp Trust Funds was above and beyond this year at the 81st Annual National Convention in Foster City.

As always we appreciate the ICF's love, hospitality, and for always welcoming us to their convention activities with open arms. It was an honor to be more involved this year by going to the Kick-off Luau—thank you Burlingame branch; by conducting a CHO hospital tour—we thank those of who came; and by participating in registration and workshops. We always love catching up with friends at the Saturday night banquet and the following festivities. We feel a deep sense of gratitude that goes beyond words.

The ICF donation is divided between research, patient care, and psychosocial programs for patients and families. Once again we were fortunate to be adopted as the ICF's National Charity for 2012—a

continued miracle for us year after year.

We would like to thank the ICF for their generosity and continued support of our program. The ICF members all touch the lives of countless people and make this world a much better place to live.

### Italian Catholic Federation Update

Dr. Vichinsky and Laurice and Matt Levine has the honor of attending the 82nd Annual Italian Catholic Federation Convention in Foster City, California. This special event, which was held September 1 to September 5, kicked off with a luau at the Burlingame Branch, followed by a tour of CHO and luncheon on Friday. Approximately 30 ICF members were able to experience firsthand all that CHO has to offer people with thalassemia: the Fifth Floor Hematology/BMT/Playroom, Radiology, the Family Resource Room, the Outpatient Center, and the Day Hospital. Nancy Sweeters, RN, gave a talk at the PCRC, and Franz Kuypers, PhD, gave a talk at CHORI about the research being done on thalassemia.

ICF members also visited the HEDCO building to see the ferritometer that they were so instrumental in raising funds for. Special thanks to Franz, Nancy, and Ken McKinney, who helped lead the tour.

On Saturday, Laurice spoke on the thalassemia program at CHO during two workshops run by ICF member Andy Pappani. The Saturday banquet was a time to celebrate the ICF and the amazing work that they do. On Sunday, the ICF adopted thalassemia (Cooley's anemia) as its national charity once again and donated \$77,000 (\$47,000 directly from the ICF and \$30,000 from a private trust—with gratitude to Christina Wagstaff and Nettie Descalso Del Nero).

We are grateful to the ICF President and First Lady, Jim and Janice Jones, and the ICF Members and Staff for their continued generosity and support. They are like family to us in the thalassemia community, and for their love, friendship, and prayers, we are forever grateful.

[www.ICF.org](http://www.ICF.org)



l-r, top-bottom: ICF Gilroy's Roger and Kathy Santos hosting the Mike Santos Memorial Motorcycle Run to benefit thalassemia; Donna Pfaff, Denise Campagna, Sal Campagna, and Richard Pfaff; Bob Osorio, Elaine Osorio, Lydia Norcia, Dom Norcia; ICF Branch 13, Martinez; ICF Grand President Jane Dianda; ICF Past Grand Presidents Jim Jones and Mike Rossi.

# A Lesson Remembered: Reflections of a Volunteer

by Zoë Oppenheim

When I was in seventh grade, a panel of successful women spoke to the girls at my middle school. Marian Diamond urged us to get our careers in order and our educations behind us prior to starting a family. Because I was interested in too many careers for my own good at that time, I realized I needed to focus on a particular path. I began imagining life as a 60-year-old student, still unable to choose between being a doctor, a spy, a teacher, or a firewoman. As Diamond continued to speak, my daydream unraveled into an epiphany: I had to decide what I was going to be when I grew up—before it was too late.

My quest to map out the rest of my life continued through high school. During the summer before my senior year at Berkeley High, I concluded that avid viewings of TV shows such as Grey's Anatomy and House were not enough to prove that I wanted to be a doctor. With good fortune, I landed a job as a volunteer at Children's Hospital Oakland (CHO). My bosses, Laurie Levine and Eve Alley, were kind enough to train me so that I could work with both of with them in thalassemia outreach and with kids in the intensive care unit and at the day hospital. By the end of the first week, I already felt as though I was an

integral part of the CHO community.

As my schedule changed from working with adults to working with children, I began to observe the subtle differences between the two worlds. Both parties deal with stress on a daily basis. Hospital administrators are constantly wrestling with the growing pile of debt that the suffering economy only worsens. Doctors struggle to find a balance between treating patients and processing endless paperwork. Parents fight to be with their sick children while also maintaining their jobs. And children battle diseases that are both physically and emotionally draining. As I continued on with my fly-on-the-wall position, I watched as people from all ages and backgrounds encountered the stresses of life.

What is the main difference between a sick child's world and the parents' world? I learned it is not the hardships, but rather how they deal with these hardships. While adults often suppress their more negative emotions, children voice them. Adults often struggle to find healthy ways to process hardships, while children almost always play out their feelings. This play therapy is essential for a child facing a chronic or life-threatening illness. The same approach, however, is seldom deemed appropriate for adults

facing all kinds of trials and tribulations.

As a volunteer at CHO, I played with kids through their boredom, loneliness, blood transfusions, and chemotherapy. Although I was far from being able to physically help these children while they dealt with their diseases, I was still able to play with them through the roller coaster of emotions they experienced. I wish I could say the same for the adults that I interacted with. Perhaps the adult world could learn a thing or two about emotional health from a children's perspective.

The truth is that the binary division of "adult" and "child" is a construct—just as age-specified healing is. By the end of my journey at CHO, I realized that learning this lesson was much more important than mapping out my life. Although guidelines can be useful, there is no blueprint that I can refer to for the rest of my time on this earth. The CHO community showed me that throughout our lives, we find ourselves, at one point or another, in need of healing. This fact is a result of the messy nature and uncertainty of life. We can't control the obstacles that come our way, but we can have a say in how we deal with them. Whether you are healthy or sick, rich or poor, 3 or 93, remember one of the first lessons we all learned: to play.



## Natural Disasters: Are You Prepared?

by Eve Alley, MA, CCLS



Throughout the last year, both the United States and nations abroad have been faced with many natural disasters. From earthquakes to fires, floods, and tornadoes, it feels like we have seen everything in the last several months. It is important for all of us to have a well-thought-out plan ready in the event

that we are faced with a sudden emergency or natural disaster. Everyone in your home should know what to do in an emergency. The following are suggestions on how to stay ready for such an event:

- Prepare simple, one-page emergency instructions, and update them as things change.
- Create a support network including relatives, friends, neighbors, and caregivers, and keep them informed about where to find health information, medical supplies, equipment, and important contact numbers.
- Inform the local emergency services, such as the fire department, about any special needs or conditions you or a family member has.
- Create an evacuation strategy, and practice it.
- Create a Family Emergency Kit that includes first aid, three weeks' supply of medications and medical supplies, batteries, flashlight, hand-cranked radio, canned food, blankets, etc.



## Farewell Catherine and Eve!

Dear Friends,

As many probably know, I left the Bay Area in July to reunite with my husband, whose training took him to San Diego. I am now working at Rady Children's Hospital San Diego in the hematology/oncology department, learning about the oncology side of the discipline. I wanted to thank all of you for entering my life and teaching me about living with thalassemia. I loved getting to know all of you over the course of my two years at CHRCO and will sincerely miss you. I feel so lucky to have worked with such knowledgeable and courageous individuals. I wish you the very best, and if you are in the San Diego area, please let me know—I would love to meet up with you. I hope we will cross paths again.

Catherine

catherine.gariepy@gmail.com



Dear Friends,

After four years as a Thalassemia Outreach Coordinator, it is hard to say good-bye. I am so grateful to have had the opportunity to work in the thalassemia program. I had many wonderful experiences and met the most amazing people along the way. I will never forget the friendships I have made, and I will continue to build on everything I have learned. I will always be a loyal fan of the thalassemia program at Children's Hospital & Research Center Oakland for all the incredible things it does for the thalassemia community. Thank you so much for giving me this opportunity and for sharing your lives with me. I wish all of you nothing but the best in the future, and I will be sure to come and say hello once in a while.

Take care!

Love,

Eve



## Welcome Marcela Weyhmiller!

Let's welcome Marcela Weyhmiller, the new Program Coordinator for the SQUID-ferritometer. A native of Oakland, Marcela is proud to be a part of the thalassemia team here at Children's Hospital Oakland (CHO).

SQUIDs are not new to Marcela. She used them to study the magnetic properties of iron oxide nanoparticles while she earned her PhD in Materials Science Engineering from the University of Washington. The transition from an engineering background to working with patients has been very rewarding. She

never imagined that she would be able to apply her expertise in magnetism directly to the care of the people in her community and beyond.

When Marcela is not at HEDCO, she also works on various other iron-overload related research at CHO. Away from work, she spends lost of time with her extended family in the Bay Area. Marcela is married to Sean, Chef de Cuisine at Bocanova, and is a mom to 3-year-old son Mathias and 18-month-old daughter Paloma.



## The Registry and Surveillance System in Hemoglobinopathies (RuSH): Thalassemia as a public health issue in California

by Susan Paulukonis, California RuSH

Although we have a general idea of the number of transfusion-dependent individuals with thalassemia major in the United States, numbers cited are generally estimates based on information from large clinics and blood centers and may not include all patients. Public health officials do not know for certain how many people are affected by thalassemia disorders (whether transfusion-dependent or other forms of thalassemia) at the state or national level. Also unknown are the average age of those with thalassemia, current life expectancy, what proportion of those affected are receiving care that meets current standards, long-term outcomes for those who chelate or do not chelate, and a host of other questions important to patients, families, and clinicians, as well as those who set public health policy for programs impacting this population.

The Registry and Surveillance System in Hemoglobinopathies (RuSH) project, sponsored by the National Heart, Lung, and Blood Institute and coordinated by the Centers for Disease Control and Prevention, is a two-year cooperative agreement among seven states (California, Florida, Georgia, Michigan, New York, North Carolina, and Pennsylvania). Its objectives are to gather data at the state level to answer as many of the above questions as possible. In California, the state presumed to have the largest population of people affected by thalassemias, this work is being done in the Genetic Disease Screening Program (CDC), a part of the state's Department of Public Health. Data (including newborn screening

case data, hospital and emergency room discharge data, birth and death certificate data, Medi-Cal data, California Children's Services data, and Genetically Handicapped Persons Program data) have been requested from a number of public agencies and two large clinics: Children's Hospital & Research Center Oakland (CHRCO) and Children's Hospital Los Angeles (CHLA). We are able to link individuals across these data sets, so that we do not count the same person twice if he or she is recorded in both clinic data and Medi-Cal claims data. These linked data from multiple sources allow the most complete and detailed look yet at the California thalassemia patient population.

The CDC will collect de-identified data for the years 2004 to 2008 from all RuSH states. This data will be used to estimate the disorder prevalence and incidence across the United States and to begin to answer some of the important public health questions regarding thalassemia on a national level. Results from this initial RuSH effort will form the baseline data for evaluation of the Healthy People 2020 Goals in blood disorders, an important addition to the Healthy

People public policy agenda set every decade. Additionally, within each state, a larger pool of data is available to answer state-specific questions. Within California, we plan to use the data to determine patient demographics, outcomes, access to care, and geography, as well as trends in these areas.

In addition to collecting and analyzing public health data on thalassemia and

sickle cell disease, the RuSH project is supporting outreach and education efforts at the state level. The project's partners at CHRCO and CHLA have been actively involved in offering education about thalassemia disorders and information about resources to affected communities and those at risk. In Oakland, a series of stakeholder meetings with patients and families impacted by thalassemia have taken place, and staff members have taken the feedback given to them in these meetings and used it to craft a message about RuSH and about the disorders for the general public. They are also currently conducting educational sessions with groups that provide health services to the Asian communities in Northern California. In Los Angeles, CHLA staff members have offered information and resources at health fairs and other public events, and they have begun working directly with Los Angeles-area consulate staff from countries heavily impacted by thalassemia to provide information and increase awareness of the disorder in those communities.

Future steps in public health surveillance in sickle cell disease and thalassemia are likely to include continued collection and analysis of these data, as well as an active patient registry, similar to those in place for other chronic diseases. Patients would be asked to participate, and if they agreed, would be monitored over a long period for health status, treatments, and quality of life. Data such as these, collected over time to monitor for trends and changes, will be instrumental in assuring that all patients receive the best quality care, that insurance plans cover necessary treatments, and that quality of life is the best possible for those impacted by thalassemia.

**Public health officials do not know for certain how many people are affected by thalassemia disorders at the state or national level. The Registry and Surveillance System in Hemoglobinopathies (RuSH) project tries to answer this and many other questions about the U.S. thalassemia population.**

# Thalassemia Program Donation Guidelines

Due to hospital policy, limited storage space, fire hazard, and the prevention of infectious disease, the Thalassemia Outreach Program at Children's Hospital Oakland has a set of donation guidelines.

## If you would like to make a donation, please adhere to the following guidelines:

- All toys must be new and unused
- Stuffed animals must be new, clean and not off the store shelf for more than a month
- Books must be new or in very good condition
- Used DVD's are acceptable; used VHS video tapes are not.

## Gift Cards

Gift cards are great for ages 10 and older, especially our teens and adults. We also are able to purchase the many supplies, arts, crafts and other wares needed to put on a party/event. You can designate if you would like your gift card to be used as a gift, as party expenses, or wherever needed.

Gift cards to: Target, Best Buy, Game Stop, Safeway (for party supplies), Albertsons (for party supplies), Gap, Old Navy, Banana Republic, Claire's, See's Candy, Barnes and Noble, Big 5, Sports Authority, Regal and AMC Movie Theaters, Costco, Starbucks, Jamba Juice. Denominations can be \$5 to higher. Usually \$10 and \$20 are preferable.

## Suggested donation items:

- Infant Toys: mobiles, musical toys, rattles, teethingers
- Books for all ages
- Board Games for all ages: Life, Monopoly, Clue, Sorry, Trouble, Candyland, Chess/Checkers, Uno, Chutes and Ladders, Simon, Trivial Pursuit, Jenga, Taboo, Hi Ho
- Cheerio, perfection.
- New Dolls: Barbies, baby dolls
- Hand held video games
- CD Players
- Sports equipment
- IPOD Minis
- Nintendo DS
- Make up kits and nail polish
- Cute women's socks, mens socks, children's socks
- Teen clothes – appropriate logos ie Nike wear; Puma
- Fleece blankets
- Disney attire
- Art supplies: pens, crayons, coloring books, paper, glitter pens
- PlayDoh and modeling clay
- Toy cars, match box cars
- Lego sets
- Action figures

If you would like to make a donation please contact: Laurice Levine at 510-428-3885, ext. 5427 or LLevine@mail.cho.org.

Thank you in advance for your generosity and support!

## Thank you to recent donors for your generosity and support

Charlie Abela  
Rose and Don Arnaudo  
Marlene Farber  
Fr. Marcos Ghali  
Maria and Bill Greco  
Italian Catholic Federation  
Janice and Jim Jones  
Robert Kemp Trust -  
Christina Wagstaff  
Marcie and Mike Rossi  
Joanna Van Blaricom

## Thank you to our volunteers for their hours of hard work and time:

Navpreet Dhillon  
Shani Litwin  
Sushrita Neogi  
Zoe Oppenheim  
Ivanna Pincilotti  
Vijay Prakash  
Siddhant Talwar





## P E R S P E C T I V E S

Northern California Thalassemia Center  
Children's Hospital & Research Center Oakland  
747 52nd Street, Oakland, CA 94609

Address change requested

**THE THALASSEMIA TEAM AT CHILDREN'S HOSPITAL & RESEARCH  
CENTER OAKLAND WOULD LIKE TO WISH YOU ALL HAPPY HOLIDAYS  
AND HAPPY NEW YEAR.**

## UPCOMING EVENTS

### November

Nov 2, 2011: ICF Br. 4 Dinner, San Jose, CA.  
Nov. 5, 2011: ICF Banquet, San Jose, CA.  
Nov. 9, 2011: Lion's Club Presentation and Lunch, Los Gatos, CA.  
Nov. 18, 2011: ICF Br. 438 Dinner and Meeting, Roseville, CA.

### December

Dec. 3, 2011: ICF Holiday Party and Thalassemia Presentation, Arcadia, CA.  
Dec. 4, 2011: ICF Br. 367 Holiday Party and Thalassemia Presentation, Los Vegas, NV.  
Dec. 8, 2011: Thalassemia Holiday Party, Oakland, CA.  
Dec. 9-13, 2011: 53rd American Society of Hematology (ASH) Annual Meeting and Exposition, San Diego, CA.

### January 2012

Jan. 3, 2012: ICF Meeting, Capitola, CA.

### February 2012

Feb. 14, 2012: ICF Br. 52 Valentine's Day Fundraiser for Thalassemia, Healdsburg, CA.