

Thalassemia 101

What is thalassemia?

Thalassemia is the name of a group of genetic blood disorders.

How does thalassemia affect the human body?

Thalassemia results in an imbalance in the production of hemoglobin, the oxygen-carrying component of blood.

How many types of thalassemia exist?

People whose hemoglobin does not produce enough alpha protein have *alpha thalassemia*. People whose hemoglobin does not produce enough beta protein have *beta thalassemia*. Within each type, there are three classifications: thalassemia minor (or trait), thalassemia intermedia, and thalassemia major (disease).

What are the symptoms of thalassemia?

Depending on the type and severity of thalassemia, symptoms can range from a debilitating and life-threatening anemia to no obvious symptoms whatsoever. A person with thalassemia minor will experience no significant health problems, except a possible mild anemia which *cannot* be corrected with iron supplements. Thalassemia intermedia is an intermediate form of disease that requires regular care by a doctor. Thalassemia major is a serious disease that requires regular blood transfusions and extensive medical care.

Who does thalassemia affect?

Thalassemia is most common in people of Southeast Asian (Vietnamese, Laotian, Thai, Singaporean, Filipino, Cambodian, Malaysian, Burmese, and Indonesian), Chinese, East Indian, African, Middle Eastern, Greek, and Italian descent. ■

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

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

Address change requested

Perspectives Newsletter is produced by the thalassemia outreach coordinator, Toutu Vongphrachanh, and the Communications department at Children's Hospital & Research Center at Oakland. For questions regarding the newsletter or for more information on thalassemia, call 510-428-3885 ext. 4398, or visit www.thalassemia.com.



Perspectives

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More Asian American Donors Needed in the Bay Area

By Angela Woon
Blood Centers of the Pacific

More than 60 percent of Bay Area residents are eligible to donate blood, yet less than 4 percent actually do. Unfortunately, this number is even lower among Asian Americans. And with new diseases like SARS and West Nile virus emerging, more blood donors are being excluded from giving the gift of life.

One's blood type is inherited the same way as one's eye or hair color. As a result, many blood phenotypes are predominantly found in specific racial or ethnic groups. Although most people think of blood as being type A, B, or O and either Rh positive or negative, there are actually many other minor blood types determined by the presence of other proteins on red blood cells. Varying phenotypes can cause transfusion reactions in chronically transfused patients, who are constantly exposed to other people's minor blood types.

For patients who develop these reactions, "phenotypically matched" blood, which often comes from donors of the same racial or ethnic background, is selected and transfused.

Only 7 percent of Blood Centers of the Pacific's (BCP) blood donors are Asian American, despite the fact that 31 percent of San Francisco residents are Asian American.

In contrast, diseases such as thalassemia, a blood disorder treated with lifelong transfusions, and hepatitis B are more prevalent in the Asian American community. The racial disparity between donor and recipient can make it difficult to find compatible blood for some Bay Area Asian American patients.

Last year, BCP received a generous research grant from the Blood Systems Foundation, which enabled the use of focus groups and surveys to understand why so few Asian Americans donate blood. An advertising campaign featuring themes found in the Asian American community was launched in an effort to boost donor numbers. Family unity and respect for elders emerged as important themes, and for many, the reminder that anyone, anywhere can need blood was a strong influencer. This year, BCP is focusing on increasing the number of blood drives in the Asian

CONTINUED ON P. 2



BENCH^{TO}BEDSIDE THALASSEMIA RESEARCH NEWS

Treatment of Hepatitis C in Thalassemia Patients on Clinical Trial

By Paul Harmatz, MD and Ellen Butensky

The Thalassemia Clinical Trials Network (TCRN) has initiated a clinical trial designed to evaluate the safety of PEG interferon alfa and ribavirin for the treatment of hepatitis C in patients with thalassemia.

Chronic hepatitis C infection affects about 3 million persons in the United States. Patients with

CONTINUED ON P. 5

Iron-Binding Drug Development Reaches a Milestone

By Vanya Rainova

At Children's Hospital & Research Center at Oakland providers treat approximately 200 children who have thalassemia and another 300 children who suffer from sickle cell anemia. Many of these children receive blood transfusions to manage their diseases. Regular blood transfusions inevitably lead to build-up of iron in the body. In order to avoid the toxic and

CONTINUED ON P. 5

Empowered by TAG By Cheryl Mar

In April, I attended—for the fourth time in a row—the Thalassaemia Action Group (TAG) Annual Conference. Each year, the conference features key speakers from the medical community who discuss issues of interest to thalassaemia patients and care providers. During topical workshops, participants can speak with physicians and other medical professionals. The conference is also a great opportunity to meet other patients and family members, and to share information and experiences. In the context of this year's 17th annual event, I considered, once again, how pivotal my involvement with TAG has been in my life.

After I had an adverse reaction to desferal, my care providers considered alternative treatments, including L1 (deferiprone). To allow physicians to determine the best option, I had to undergo two tests to evaluate the iron content of my liver and heart. These tests, respectively called the SQUID (Superconducting Quantum Interference Device) and T2*, are new in the United States. They are not FDA-approved and are being used on an investigational basis. Information about the equipment and procedures was scarce; educational material had just begun making its way to the pages of outreach newsletters.

However, I was already familiar with the tests. During TAG conferences, I was able to hear what it was like to undergo these procedures, how the technology worked, and what the risks and benefits associated

PATIENT PERSPECTIVE

with each test were. During the 2002 conference, I attended Dr. Beatrix Wonke's presentation, which included visual images the MRI produced, so I was able to see what doctors look at and how they determine the results. Dr. Wonke also discussed her experience with placing patients on L1, including the drug's possible side effects, benefits, and effectiveness when used alone or in combination with desferal. She presented specific cases, focusing on the improvements of her patients ferritin levels and organ function. Having access to this information helped me throughout the entire process. I did undergo the SQUID and T2. I have also started taking L1.

Sometimes it's easy to become a passive patient who only receives whatever information happens to come her way. However, I learned first-hand how beneficial it is to actively keep oneself up-to-date on research and clinical trials. Undergoing "experimental" tests would have been a much more confusing and overwhelming experience, had I not had the tools to make an informed decision. I did not acquiesce just because the doctor said so; I agreed to testing and treatment changes because I felt confident it was the right and safe thing to do.

This experience made me realize the value of TAG. It motivated me to get involved. I am proud to say that I have joined the TAG board with the hope of benefiting other patients. ■

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Asian American Donors Needed

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American community.

A nonprofit, community-based organization with 10 centers, BCP needs to collect 500 pints of blood each day to meet the needs of area patients. BCP supplies blood and blood components to 40 hospitals in Northern California.

Eligible donors must be at least 17 years old, weigh at least 110 pounds, and must not be at risk for HIV or hepatitis.

If you would like to schedule an appointment to donate blood, please call 1-888-393-GIVE or visit our website at www.bloodcenters.org. To schedule a blood drive at your business or place of worship, please call Helen Lau-Takenami at 415-749-6634 or email her at Hlau-Takenami@bloodcenters.org. ■



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Treatment of Hepatitis C - CONTINUED FROM P.1

hepatitis C are at higher risk for developing severe liver disease, cirrhosis, and hepatocellular carcinoma (a cancer that arises from hepatocytes, the major cell type of the liver). Patients with thalassemia major receiving frequent blood transfusions before effective screening tests became available in the early 1990's were at high risk of acquiring HCV.

Therapy for hepatitis C has improved greatly in the 1990s. Interferon alfa, first approved in 1995 for treatment of hepatitis C, eliminated the viral infection in 15 to 20 percent of patients. The addition of ribavirin in 1998 improved the cure rate to 40 to 45 percent. In the last year, two brands of long-acting interferon alfa (also known as PEG interferon-alfa) have been tested alone and with ribavirin. When given alone, PEG interferon alfa equaled the efficiency of the combination of standard interferon alfa and ribavirin (40 to 45 percent). When combined with ribavirin, PEG interferon achieved viral cure in approximately 60

to 65 percent of patients. Although now approved by the FDA for use in hepatitis C, the combination has not been tested in patients with thalassemia or other hemoglobinopathies. Two small studies using standard interferon alfa and ribavirin have shown the combination to be as effective as in patients without thalassemia and there were no significant complications related to the ribavirin medication, although increased transfusions were required. In this clinical trial, the Thalassemia Clinical Trials Network will examine the safety and effectiveness of PEG interferon alfa and ribavirin in a large group of patients with thalassemia.

Sixty patients will be in this study in the United States, Canada, and Great Britain, approximately 10 of them treated at Children's Hospital & Research Center at Oakland. Patients will receive PEG interferon alfa and ribavirin for one year. They will be followed closely to see how well the medications are working and to watch for side effects. ■

Iron-binding drug - CONTINUED FROM P.1

potentially life-threatening effects of iron accumulation, patients must undergo treatment with an iron-binding drug. Currently, such chelation therapy requires slow infusion of a drug over a period of eight hours per day, at least five days a week. But a new iron-binding drug may make therapy easier and less time-consuming for all children who need iron chelation, especially those who suffer from beta thalassemia (Cooley's anemia) and sickle cell anemia.

Biomedical Frontiers, Inc. (BMF), of Minneapolis, announced completion of a Phase 1b clinical trial in thalassemia patients with BMF's injectable iron-binding drug, 40SD02. 40SD02 is longer-acting and can be administered only once or twice a week. Therefore, specialists anticipate it will become a preferred method of treatment for patients who need chelation therapy.

The trial was conducted at Children's Hospital & Research Center at Oakland and at the

Weill Medical College of Cornell University in New York City. Twelve beta thalassemia patients received intravenous injections of 40SD02 over one hour. Three groups of four patients received 150, 300, or 600 mg/kg. Excretion of iron was followed for seven days. The drug was well tolerated and, at the highest dose level, iron excretion equivalent to four to five days of conventional chelation therapy was achieved.

Paul Harmatz, MD, the principal investigator at Children's Hospital & Research Center at Oakland, states: "The observation that nearly a week's worth of iron balance can be achieved with a single, one-hour, intravenous infusion of 40SD02 is a significant development. If such excretion patterns and lack of toxicity can be maintained in the next round of trials, this novel treatment would represent a major improvement in quality of life and will likely improve compliance with chelation therapy for these patients." ■

* * OUTREACH * *

☆ CALENDAR OF EVENTS

August

11-14: Desferal Camp, Oakland

23-24: 15th Annual Chinatown Street Fest, Oakland

24: ICF Council Meeting, Santa Rosa

September

13: Provider Meeting For more information, please contact Laurice Compagno at 510-528-8617

October

3: Headstart Annual Family Event and Resource Fair, Sacramento

4: Prevention 2003 Health Fair, Sacramento

15: Birth & Beyond Health and Literacy Fair, Sacramento

16: Family Forum
For more information, please contact Toutu Vongphrachanh, Thalassemia Outreach Coordinator at 510-428-3885 ext. 4398 or Jean-Marie Knudsen, Thalassemia Social Worker at 510-428-3268.

18: YMCA Cheadle Family Community Health Fair, Stockton

December

12: Annual Thalassemia Holiday Party

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☆ YOUR RESOURCES

Children's Hospital & Research Center at Oakland:

www.childrenshospitaloakland.org

CHRCO Thalassemia:

www.thalassemia.com

CHRCO Cord Blood Program:

website: www.siblingcordblood.org

Cooley's Anemia Foundation and the Thalassemia Action Group (TAG):

1-800-522-7222

www.cooleysanemia.org

Thalassemia International Federation e-mail:

thalassaemia@cytanet.com.cy

United Kingdom Thalassemia Society:

www.ukts.org

Thalassemia Association of Hong Kong:

www.thalassemia.org.hk

National Marrow Donor Program:

1-800-MARROW-2

www.marrow.org

Blood Centers of the Pacific:

1-888-393-GIVE

www.bloodcenters.org

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☆ INCENTIVE AWARDS FOR STUDENTS WITH THALASSEMIA

Deadline: December 31, 2003

The Cooley's Anemia Foundation sponsors incentive awards to students with thalassemia intermedia or major (not trait carriers). Applicants must be enrolled in an undergraduate or graduate education program or in a technical certification program. High school students are not eligible.

The application process includes writing a 250-word essay. For applications or information, please contact Jean-Marie Knudsen, Thalassemia Social Worker at 510-428-3268, or Cooley's Anemia Foundation at 800-522-7222.

* * OUTREACH * *

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★ OUTREACH UPDATES

4th Annual Blood Drive in Honor of International Thalassemia Day

Monday, May 5th marked the 4th Annual Blood Drive for International Thalassemia Day. The theme was Cinco de Mayo, complete with fiesta decorations, a piñata, Chiclets, and a Cinco de Mayo Raffle. The American Red Cross collected 40 units of lifesaving blood. Our heartfelt gratitude goes out to all Children's employees and friends who donated blood. We appreciate their time and generosity. We would thank the following supporters for making this blood drive a success:

- Mrs. and Mr. Nguyen—sponsors
- American Red Cross
- Chevy's
- Claremont Hotel
- High Tech Burrito
- Party America
- Signature Theaters
- Speed Oil Change Center # 2 and Lucia Espresso
- Starbucks
- Volunteers – Tony and Bev Compagno, Susan Winner, and Huythong Nguyen



Tony Compagno gives blood.

The California Chapter Finds Success at Sea World

The California Chapter of the Cooley's Anemia Foundation hosted the 2003 Thalassemia Conference on June 28 in San Diego. Three physicians presented. Thomas Coates, MD, of the Children's Center for Cancer and Blood Disorders spoke about comprehensive care. Fernando Tricta, MD, of Apotex, Inc., gave a presentation on the oral chelator L1. Finally, Elliott Vichinsky, MD, of Children's Hospital & Research Center at Oakland presented new therapies available to thalassemia patients today. After the

informative presentations, the group of approximately 100 people broke up into two smaller groups: parents and patients. Parents of thalassemia patients shared their experiences with one another and asked questions concerning the care of their children. Patients used the time to discuss their personal experiences.

For more information about the conference and the California Chapter, please call: 800.601.2821, send an email to: ca_chapter@hotmail.com, or write to: California Chapter – CAF; 2629 Foothill Blvd. #319; La Crescenta, CA 91214

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★ INTERESTED IN HAVING A WORKSHOP OR LEARNING MORE ABOUT THALASSEMIA?

Contact:
Outreach
510-428-3885 ext. 4398
Nursing/Medical
510-428-3347



Cynthia Romanov, Susan Foxall, and Chela Rios Meñoz at the blood drive.