

IDF ADVOCATE

THE NATIONAL NEWSLETTER OF THE IMMUNE DEFICIENCY FOUNDATION



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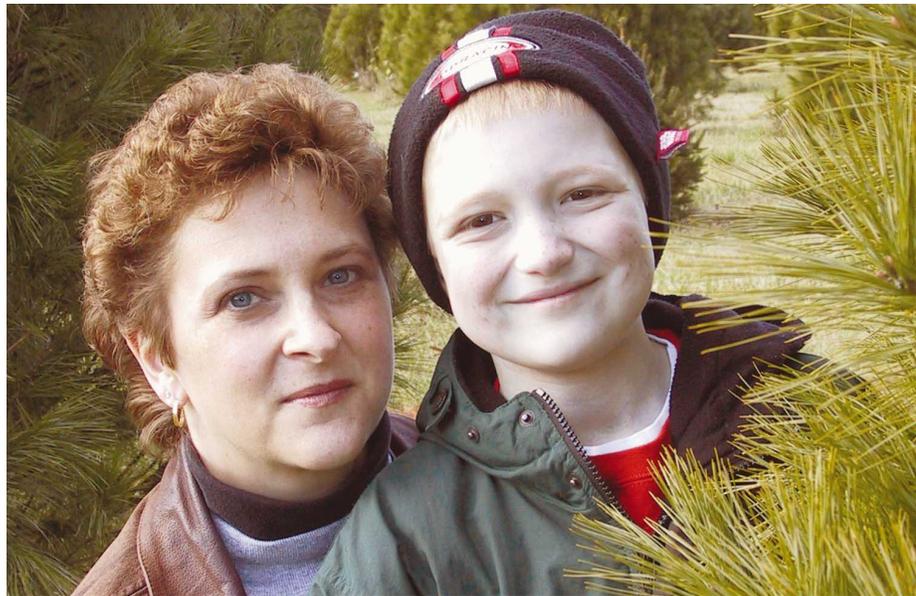
QSEAL program improves product safety



This newsletter is sponsored by an educational grant from Bayer Corporation



Early Diagnosis Saves Lives



Barb and Seth Ballard

NEW SERIES ON IDF CORE SERVICES

Starting in this issue of the *IDF Advocate*, we'll begin a series of articles focusing on IDF's Core Services Programs and how they improve the health and quality of life of people with primary immune deficiencies. Our main article discusses the benefits of early diagnosis and some of the problems experienced when diagnosis is delayed.

The dangers of delayed diagnosis

Eight-year-old Seth Ballard of Virginia has Severe Combined Immune Deficiency (SCID), the most severe of the primary immune deficiency diseases. Seth was not diagnosed until he was 10 and a half months old. As a result, he nearly died before his first year of life, contracting pneumonia and spending four months on a ventilator. In addition, a virus attacked his gastrointestinal tract, leaving him unable to eat and dependent on intravenous nutrition. He now uses a "G" tube that sends food directly to his stomach.

"Seth wasn't diagnosed at birth," recounts his mother, Barb Ballard, "and he became critically ill before anyone figured out what was wrong. He had sinus infections and other problems, but there's no family history of SCID and no one put it all together. Eventually an immunologist was brought in. He knew immediately what the problem was. We're very lucky."

A MESSAGE FROM THE EDITOR

Do you need more information on primary immune deficiency diseases? Are you looking for a sympathetic listener to share your concerns with? Either way, IDF can help. To request our educational literature for patients and family members, or for specific questions, call our toll-free IDF Hotline at 800-296-4433. Our professional staff will answer your questions about primary immune deficiencies and put you in touch with our network of volunteers. Remember—IDF is here to help!



IDF ADVOCATE

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EARLY DIAGNOSIS, CONTINUED FROM PAGE 1

SCID kills children if not diagnosed early and treated effectively. If treated late, many do not survive because of the amount of damage done to internal organs by infections prior to diagnosis. The cost is high, in lives lost, families crushed, and dollars spent.

Fortunately, Seth Ballard is a fighter. He goes to school, participates in activities—but as soon as he gets sick, all of his mother's fears come back. "You never forget that terrible feeling of helplessness," she explains. To help others through the same challenges she and Seth have faced, Barb started an Internet group for parents of SCID children and participates in local IDF programs.

Delayed diagnosis exacted a high cost in the life of another person with a primary immune deficiency, Neil Ross of southern California. Neil has Common Variable Immune Deficiency (CVID), but was not diagnosed until the age of 42. Like Seth, he was diagnosed while undergoing a medical crisis, and then only because his doctor happened to attend a seminar in Europe at which CVID was discussed.

"It was in 1997," Neil explains. "I had been ill for decades—more than 200 cases of pneumonia, sinusitis so frequent that I needed surgery to remove scar tissue, accusations of hypochondria, strained family relations, a difficult life. After all this I developed an upper respiratory condition that lasted three and a half months, together with gastrointestinal problems. I was hospitalized, my electrolytes were off, I was dehydrated." By chance, Neil's doctor had recently heard about CVID, and realized that the description of the deficiency fit Neil's condition. It took a month to get a laboratory to perform the needed tests—the lab did not understand what to look for—but finally the diagnosis was confirmed and proper treatment begun.

"Finally having the mystery explained, and beginning to live a more normal life, leaves you feeling a strange mixture of joy, relief, and bit-

terness," Neil concludes. "You begin to experience health for the first time, and people no longer think you're crazy. I can only hope that others with my condition will find out earlier and escape what I went through."

Average delay in diagnosis: eight years.

All too often, the history of a person with a primary immune deficiency begins with months, years, and even decades spent trying to get a diagnosis, before appropriate treatment can begin. "From what we've seen at IDF, the average length of time between the onset of symptoms and an accurate diagnosis is about eight years," explains IDF Medical Director Jonathan Goldsmith, M.D. "During that time, a lot of bad things can happen to the patient: recurrent infections, loss of lung function (which is a permanent defect), and psychosocial problems such as loss of employment."

For children with SCID, like Seth Ballard, the risk can be much greater. "According to a new study from Duke on SCID patients," Dr. Goldsmith notes, "very early bone marrow transplantation, within the first 28 days of life, leads to 95% survival. If transplantation happens later, the survival rate is only about 65%."

IDF works to improve the chances that patients will be diagnosed early by educating medical professionals. Dr. Goldsmith relates, "When a patient comes to a primary care physician with an infection, say recurring sinusitis, the physician typically sees only an infection and treats it with antibiotics. We want to help the physician consider the possibility of an underlying primary immune deficiency, and give him or her the resources to pursue that possibility." IDF accomplishes this task through a number of educational programs and two important initiatives: the Consulting Immunologist Program and the LeBien Visiting Professor Program.



CONSULTING IMMUNOLOGIST PROGRAM
877-666-0866

FREE CONSULTATION/SECOND OPINION
Physicians may contact our faculty of expert clinical immunologists to receive a second opinion for patient-specific issues in the diagnosis, treatment, and management of Primary Immune Deficiency Diseases.



Immune Deficiency Foundation

The Consulting Immunologist Program is supported through an educational grant from the American Red Cross, Aventis Behring, Baxter Healthcare Corporation, Bayer Corporation, FFF Enterprises, Inc., and ZLB Bioplasma Inc.

Consulting Immunologists provide free consults to physicians

There's a team of experts who volunteer their valuable time to advise physicians around the world on the diagnosis and treatment of patients with primary immune deficiencies. They are members of IDF's Consulting Immunologist Program, and for physicians and their patients they provide an invaluable source of knowledge and experience. The program greatly improves the chances that patients will be diagnosed and treated earlier and thus lead healthier and more satisfying lives.

Created by IDF in 1998, the phone-based program has provided more than 330 consultations in the U.S., Canada, Asia, the Middle east, Europe, and South America. The great majority of calls come from allergists, with immunologists, pediatricians, internists, and infectious disease specialists as other major sources of requests.

The consulting immunologists review callers' cases, suggest tests and treatments, and help with referrals to the closest available specialists. Physicians are uniformly pleased with the help they receive. A California physician notes that, "This

In over 20 years of guiding the Immune Deficiency Foundation, our Board of Trustees has learned that continuity is one of the most important aspects of the services we provide to people with primary immune deficiencies. Why? "Primary immune deficiencies are chronic conditions," explains IDF Founder Marcia Boyle. "The patient and his or her family need to know that our services will be available throughout the patient's life."

To ensure continuity, IDF identified the most essential of our programs—our "core services." These fall into three categories, as follows:

2002 CORE SERVICES

Services to Individuals and Families

- IDF Hotline
- Patient Advocacy
- IDF Local Volunteer Network
- Patient Notification System
- Scholarship Program

Public Policy

- Blood Safety and Availability
- Access to Quality Health Care
- Support for Publicly Funded Research

Medical and Scientific Programs

- Consulting Immunologist Program
- Fellowship Program
- IDF Research Grant Program
- Visiting Professor Program

In order to offer these Core Services to patients and medical professionals, IDF relies on the generosity of various organizations for program funding. To recognize the organizations that support the Core Services programs, in 2001 IDF created the **IDF Champions Circle of Support**. The Champions Circle consists of select organizations that support IDF at the highest level.

"The organizations in the Champions Circle make an enormous difference in our ability to plan and act on behalf of people with primary immune deficiencies," Marcia concludes. "With Core Services funding, we can do an even better job of improving patients' day-to-day health by promoting earlier diagnoses, greater awareness among physicians and insurance carriers, and a steady supply of medications. We can also work toward a more secure future by sponsoring new research, and advocating for public funding for that research. Champions Circle members help IDF provide long-term security for people with primary immune deficiencies."

2002 CHAMPIONS CIRCLE

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is a great service. Even board-certified allergists have few patients with these rare diseases. It is very helpful to be able to get this kind of expert opinion." "The program was very helpful," writes a Virginia pediatrician, "and I want to thank IDF for providing such a service." For more information on the Consulting Immunologist Program, call 1-877-666-0866 or e-mail tb@primaryimmune.org.

Visiting Professors: New knowledge and awareness for medical professionals

While the Consulting Immunologist Program helps individual physicians on patient cases, the LeBien

Visiting Professor Program helps entire medical facilities—both teaching hospitals and community health centers—by increasing their medical professionals' knowledge and awareness of primary immune deficiencies. This increases the likelihood that the next patient who comes to a physician with a deficiency will find a caregiver better prepared to diagnose and treat it.

Established in 1997, and named in honor of IDF Board Vice Chairman Robert LeBien and his family, the program offers educational grants to academic and community medical centers. Each grant pays for the travel and lodging expenses of an expert in primary immune deficiencies, who volunteers his or her time to visit a selected facility for one or two days. During a typical visit, the

visiting professor will speak to more than 100 medical professionals—through grand rounds, lectures, patient evaluations, and medical student conferences—concerning the diagnosis and treatment of primary immune deficiencies, current research in the field, and IDF's core services.

IDF awards grants to facilities on the basis of their stated need for information and their distance from experienced specialists. Approximately 230 facilities have applied for grants and 36 visits have been made. Tamara Brown, IDF Medical Programs Manager, states that, "We're extremely fortunate to have such a dedicated group of visiting professors and we hope to expand this important program in 2002."

The Consulting Immunologist and Visiting Professor Programs are supported through an educational grant from American Red Cross, Aventis Behring, Baxter Healthcare Corporation, Bayer Corporation, FFF Enterprises, Inc., and ZLB Bioplasma Inc.

IDF Opens Centers for Primary Immunodeficiencies at Duke and Hopkins



From left: Rebecca Buckley, M.D., Chief, Division of Allergy and Immunology, Duke Children's Hospital; Alison Arter, Sr. Vice President, Commercial Development, Bayer Corporation; Marcia Boyle, Founder, Immune Deficiency Foundation



From left: Howard Lederman, M.D., Professor of Pediatrics, Johns Hopkins School of Medicine; Jerry Winkelstein, M.D., Director, Division of Immunology, Department of Pediatrics, Johns Hopkins Hospital

The Immune Deficiency Foundation has initiated a pilot program aimed at developing Centers for Primary Immunodeficiencies across the country. The goal of this program is to develop IDF Centers at leading academic medical institutions which:

- Provide comprehensive and inte-

grated health care services to primary immune deficient patients

- Serve as a platform for multidisciplinary research
- Serve as a forum for public and professional education focused on primary immune deficiency diseases.

The first two Centers opened at Duke University Medical Center and Johns Hopkins Hospital, and will serve as the pilot sites in this exciting program. Thanks, in part, to an unrestricted educational grant from Bayer Corporation, the two IDF Centers opened in Fall 2001 and will provide a model for the development of future Centers. According to Tom Moran, President of IDF, "The IDF Centers will be a valuable asset to patients with primary immune deficiencies, providing patients with the consultative services of the world's leading immunologists, regardless of patients' ability to pay."

In addition to providing expert physician consultation to patients,

Connecticut Volunteers “Spin” to Support National Research Program

Driven by a desire to increase funding for scientific research in primary immune deficiencies, Evelyn Maselli and Terri Lowell, local IDF volunteers in Connecticut, organized the first “Spin for the Cure.” They found local sponsors to support the event, printed flyers and T-shirts, and spread the word on local radio stations to attract “spinners” to the World Gym of Orange, Connecticut on Saturday, October 13, 2001.

Spinning is a popular, new way to exercise by riding a stationary bicycle in a class led by an instructor. It is also a great way to raise money. Individuals signed up to participate in the “Spinathon” and gathered pledges on behalf of the Immune Deficiency Foundation. Overall, 36 people participated and raised over \$6,200, all of which was given to IDF’s Research Fund.

According to Jennifer Bass, IDF Director of Patient Services, “It’s exciting to see individuals from across the country join together to support the important programs of the Foundation.”

Evelyn Maselli was cheered on during her three hours of spinning by her five-year-old daughter Gabriella, who is



Evelyn Maselli (at left) and Terri Lowell “spinning” for the IDF Research Fund

affected by a primary immune deficiency disease. Congratulations and thank you to all the volunteers and spinners who helped make the Spinathon a success.

Regional Activities

The Immune Deficiency Foundation continues to offer peer support and local programming opportunities to individuals and families affected by primary immune deficiency diseases on a regional basis. A team of volunteers throughout the U.S. provides invaluable peer support, coordinates local education programs, and increases awareness of primary immune deficiency diseases in their communities. To learn more about IDF activities in your community, call the IDF office at 1-800-296-4433 or visit our Web site at www.primaryimmune.org.

IS IDF ACTIVE IN YOUR COMMUNITY?

This spring, local IDF activities are being planned in:

- Cleveland, Ohio
- Kansas City, Missouri
- Chino Hills, California
- Minneapolis/St. Paul, Minnesota
- Central Florida.

Specific dates and program agendas will be available on the IDF Web site and invitations will be mailed to individuals in each state. Additional programming for other areas of the country will be scheduled throughout this year...Keep an eye out for IDF in your community!

IDF OPENS CENTERS, CONTINUED FROM PAGE 4

the IDF Centers will support important research projects sponsored by IDF, such as the National Patient Registries and clinical trials for new therapies for primary immune deficiencies. At the Center opening at Duke University Medical Center in

Durham, NC on October 31, 2001, Alison Arter, Senior Vice President, Commercial Development, Bayer Biological Products Division, stated, “This grant, which enabled IDF to fund a Center dedicated to the treatment of primary immune deficiencies,

is part of our dedication to the treatment of these disorders. Bayer has a long-standing commitment to funding research and treatment that focuses on improving patient care and quality of life.”

CALLING FOR SCHOLARSHIP APPLICATIONS

IDF is pleased to announce the annual IDF Scholarship Campaign for the 2002-2003 academic year. Since 1983, IDF has awarded over 400 scholarships to primary immune deficient individuals pursuing post-secondary education. This includes college undergraduate students and those attending community colleges and technical schools. Applicants must meet the following criteria to receive an award:

- Prior diagnosis with a primary immune deficiency disease
- Current enrollment in a post-secondary education program
- Financial need

If you or someone you know is interested in the IDF Scholarship Campaign, please call IDF at 1-800-296-4433 or e-mail tb@primaryimmune.org to request an application. The deadline date for scholarship submissions is March 31, 2002.

The IDF Scholarship Campaign is supported through an educational grant from American Red Cross, Aventis Behring, Baxter Healthcare Corporation, Bayer Corporation, FFF Enterprises, Inc., and ZLB Bioplasma Inc.



Present at the QSEAL ceremony in Washington, D.C. were (from left): Edward Colton, Alpha; Miriam O'Day, Alpha-1 Foundation; Harro Walger, Biotest; Gunnar Reimann, Bayer; Congresswoman Solis; Jan Bult, PPTA; Jan Hamilton, Hemophilia Federation of America; Thomas Glanzmann, Baxter; Marcia Boyle, Immune Deficiency Foundation; and Ruedi Waeger, Aventis Behring.

IDF Joins Congress in Congratulating the Plasma Protein Industry

The first five companies to be certified through the Plasma Protein Therapeutic Association's (PPTA) Quality Standards of Excellence, Assurance and Leadership Program (QSEAL) were recognized on November 7, 2001 at a Capitol Hill ceremony in Washington, D.C. Congresswoman Hilda L. Solis (D-CA) presented the awards to the CEOs of Alpha Therapeutic Corporation, Aventis Behring, Baxter BioScience, Biotest Pharma, and Bayer Corporation. Marcia Boyle, IDF Founder, represented the Immune Deficiency Foundation in thanking the companies for their commitment to quality and safety.

The QSEAL program acknowledges companies that demonstrate adherence to the four voluntary standards endorsed by PPTA in 1996. In order for a manufacturer of plasma protein therapeutics to be certified as a QSEAL manufacturer, its facilities must be audited to show that they comply with the following:

- Source plasma must come from qualified donors
- Plasma must be sourced from collection centers in compliance with the viral marker standards for specific viruses
- Incoming source plasma must be tested for specific viruses by nucleic acid amplification technology
- Incoming source plasma must undergo a 60-day inventory hold.

By meeting the QSEAL standards, companies ensure the highest level of quality and safety for plasma protein therapies, including immunoglobulin therapy.

IDF Gives Back to the Community

In response to the terrorist attacks on September 11, 2001, millions of Americans have provided help in many ways, opening their homes to those left without shelter, preparing meals for those in need, donating blood, and contributing money. The Immune Deficiency Foundation established a Disaster Relief fund to help support the families of fallen firefighters in New York City. 100% of the donations will benefit the Widow's and Children's Fund of the Uniformed Firefighter's Association of New York. To date, IDF friends and families have contributed almost \$2,000 to this worthwhile cause.

Money Raised at "Dress Down Day"

Every month, the Bristol Board of Education in Bristol, Connecticut holds a "Dress Down Day," in which employees can donate \$5.00 for a particular cause. Last year, Kathryn Middleton worked with the Assistant Superintendent of Schools to name IDF as the fundraiser's October beneficiary.

The phenomenal group of teachers, administrators, clerical, technical, and building personnel from the Bristol Board of Education raised \$2,500, in the name of Kathryn's son, Kevin Middleton. Kevin is nine years old, and despite his diagnosis of Common Variable Immunodeficiency, lives a happy and productive life.

The Immune Deficiency Foundation thanks Kathryn for her hard work in coordinating this fundraising event, and thanks the Bristol Board of Education for their contribution to the Foundation.

GIFTS IN MEMORY OF...

Dinah Abrams	Jesse Green
Joyce Barranco	Edna Caple Eric Marder Scholarship Fund
Fred Brandewie	Eric Metzger
Mace Crawford	Christian Michael
Brandon Michael Dahley	Jeffery Smith
Irene Fanta	Michael J. Reker
Ann Flower-DePaolo	Wanita M. Quелlette
Jimmy Fox, Jr.	Sarah Salas
Rachel Frosini	Casey Todd

GIFTS IN HONOR OF...

Robert Alan	Kevin McMahon
Mark Austin	Kevin Middleton
Mary Bloomquist	Gail Nelson
Cameron Bryan-David	Jacob Dean Porche
Cusick	GiGi Reedy
Taylor Dahley	Richard Rembecki, M.D.
Bonnie Doak	Bernie & Shelly Shablott
Amanda Flood	Thomas Ryan Smith
Ronald Furbay Jr.	Matthew Staive
Henry Gewurz, M.D.	John Cody Tadlock
Richard Herscher, M.D.	Stacy & Dave Wertheimer
Mike & Judy Jones	Jerry Winkelstein, M.D.
Eileen Lackey	Sheryl Vrieze
Elisa Lange, M.D.	
Mark Vincent Lonergan	
Gabriella Masselli	

Medical Professionals—Look for IDF at the 58th AAAAI Annual Meeting

IDF invites physicians, nurses and allied health professionals to attend two symposiums at the 58th Annual meeting of the American Academy of Allergy, Asthma, and Immunology this March in New York City. The Academy has identified experts in the field of immunology to lead these informative sessions on the diagnosis and management of primary immune deficiency diseases.

"Primary Immune Deficiency for the Health Care Provider"

Session 3002, Sunday, March 3, 2002; 6:30 AM – 8:00 AM

Sponsored by an educational grant from Aventis Behring.

Moderators: Francisco Bonilla, M.D., Ph.D.
Kathleen Conby-Ellis, RN, Ph.D, MHA, CPN

- Overview of Primary Immune Deficiency
- Genetics of Primary Immune Deficiency
- Resource Integration for the Patient with Primary Immune Deficiency

"Dilemmas in Diagnosis and Management of Antibody Deficiency: Ask the Expert"

Session 5703, Tuesday, March 5, 2002; 2:00 PM – 4:00 PM

Sponsored by an educational grant from ZLB Bioplasma Inc.

Moderators: Melvin Berger, M.D., Ph.D, FAAAAI
Ramsay Fuleihan, M.D.

- Diagnosis of Common Variable and Specific Antibody Deficiencies
- Administration of IVIG and Treatment of Adverse Effects

In addition to sponsoring the educational sessions, IDF will be exhibiting at the meeting. Please stop by the IDF Booth (#380) to learn more about IDF and receive free professional and patient education materials on primary immune deficiency diseases.

IDF ADVOCATE

IDF NATIONAL CONFERENCE

Save the Date: June 19-21, 2003

IDF is excited to announce our second IDF National Conference in 2003! Mark your calendars to come to Baltimore, Maryland from June 19 to 21, 2003. The meeting will bring together over one thousand individuals affected by primary immune deficiency diseases. In addition to providing a wealth of information about specific diagnoses, scientific advances in treatment and therapies, and innovations in disease management, there will be many opportunities to interact with others affected by primary immune deficiency and a youth program for children.

Registration materials for the 2003 IDF National Conference will be mailed in Fall 2002. All of the conference information can also be obtained from our Web site at www.primaryimmune.org. If you have any questions about the conference, please contact IDF at 1-800-296-4433. We look forward to seeing you next year!

Address service requested



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The Champions Circle consists of organizations committed to supporting IDF at the highest level. The Circle provides vital resources to fulfill the vision, mission, and core services of the Foundation.

- American Red Cross
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CHAMPIONS CIRCLE OF SUPPORT



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