

IDF ADVOCATE

THE NATIONAL NEWSLETTER OF THE IMMUNE DEFICIENCY FOUNDATION



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This Newsletter is sponsored by an educational grant from Talecris Biotherapeutics, Inc.



Marcia Boyle Named Chairperson and CEO

Marcia Boyle has returned to the IDF office to serve as IDF's Chief Executive Officer and Chair. As the co-founder of IDF in 1980, Marcia served as the Chair and CEO/President until 1995, as Chair until 2001, and has continued as a member of the Board of Trustees since that time. During her earlier tenure as President and CEO, Marcia developed many of the patient and medical programs for which IDF is known. She was also a co-founder of IPOPI, the International Patient Organization for Primary Immune Deficiency Diseases.

Prior to coming back to IDF on a full-time basis, Marcia held the positions of Director of



Marcia Boyle

Departmental Programs and Capital Projects and the Director of Principal Gifts for the Fund for Johns Hopkins Medicine, and has also served as Director of

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IDF Fights for Adequate Reimbursement for IVIG

On January 1, 2005, the Centers for Medicare and Medicaid Services (CMS) changed the reimbursement methodology for intravenous immune globulin (IVIG) provided by physicians in their offices, infusion suites and home care settings from average wholesale price (AWP) to average sales price (ASP). Payments to non-hospital providers initially decreased from the 2004 rate of \$66 per gram under Medicare to about \$40 per gram. Since providers could not purchase IVIG at \$40 per gram, many patients were sent to the hospitals to receive their infusions where IVIG is

being reimbursed at approximately \$80 per gram.

This is especially harmful for the primary immune deficiency (PIDD) community. Approximately 70 percent of patients require chronic replacement therapy consisting of IVIG infusions to protect individuals from frequent life-threatening infections and debilitating illnesses. This has forced Medicare patients to obtain their infusions in unfamiliar hospital settings and from unknown healthcare providers who may not be knowledge-

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A MESSAGE FROM THE EDITOR

I hope you find this issue of the *IDF Advocate* informative. As you will read, IDF has been very busy this Spring. IDF cosponsored an IGIV Workshop with the FDA, celebrated Primary Immune Deficiency Diseases Awareness Week and planned for our National Conference and many local education meetings. Most importantly, we have responded quickly to the needs of the community during the Medicare reimbursement crisis. I hope you will log on to our website and consider joining IDF's grassroots advocacy program to ensure our community has access to therapy and specialized health care. Together, our voices will be heard!

Jennifer Scharpf, M.P.H.

IDF ADVOCATE

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IDF and FDA Cosponsor Workshop: “Intravenous Immune Globulins in the 21st Century: Progress and Challenges in Efficacy, Safety, and Paths to Licensure.”

On April 13, 2005, The Center for Biologics Evaluation and Research, U.S. Food and Drug Administration (FDA) and the Immune Deficiency Foundation cosponsored a workshop addressing current issues and research priorities in IGIV safety and efficacy (effectiveness), current paradigms for IGIV licensure, and critical paths to licensure.

The workshop is one of a series of workshops that began in 1981. The last such workshop took place more than three years ago, and since then new manufacturing techniques, safety, efficacy, and surveillance issues have arisen. The workshop provided a forum for the preeminent academics in the field of primary immune deficiency diseases, FDA, National Institutes of Health and the Centers for Disease Control and Prevention and the IGIV industry to discuss these important issues.

Several important outcomes are expected to take place as a result of this important workshop.

- It is anticipated that a working group will be formed to advise on the development of studies to optimize IGIV efficacy in primary immune deficiency diseases.

Research questions include the following:

- Association of dose and trough levels with infections over time
- Optimization of treatment in patients with fixed lung disease
- Validation of surrogate markers for efficacy (specific antibody levels, markers of inflammation).

*FDA Workshop
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Marcia Boyle continued from page 1

Development for the Wilmer Eye Institute and Director of Development, Neurology and Brain Sciences at Johns Hopkins Medicine. She has a B.A. from Skidmore College and an M.S. from Columbia University.

Marcia has been a passionate patient advocate for 25 years, after founding IDF in reaction to the diagnosis of her son, John, with x-linked agammaglobulinemia. Her hope for IDF is to greatly expand its ability to quickly react

to issues that affect patients and develop long-term solutions. This means reaching many more patients with primary immune deficiency diseases and the physicians who treat them, developing a strong advocacy program for issues that impact patient care, increased education for physicians to improve the rate of diagnosis and optimal care, access to education and tools for patients and families to manage their lives and health, and expanded research. ■

2005 IDF National Conference

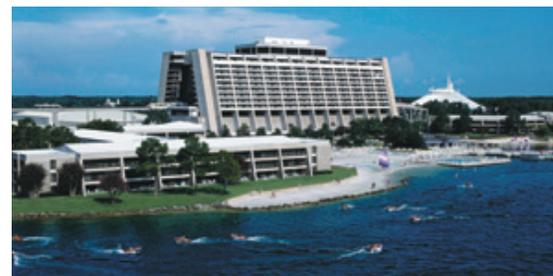
On June 23, 2005, more than 1,300 individuals will arrive in Orlando, Florida to attend the 3rd IDF National Conference. The national conference is dedicated to the memory of David Vetter and all individuals with primary immune deficiency diseases. This year celebrates IDF's 25th Anniversary and the "Legacy of Firsts" accomplished by the Foundation and the primary immune deficiency community over the past two and a half decades.

Conference attendees will learn about scientific advancements in the diagnosis and treatment of primary immune deficiency diseases from world-renowned immunologists and they will gain the skills needed to manage their health care. Attendees will also have the

opportunity to participate in a Legislative Advocacy workshop and attend symposia on IGIV reimbursement and newborn screening for primary immune deficiency diseases. IDF aims to unite this community around these important issues that will ensure access to care and earlier diagnosis for patients.

According to Jennifer Scharpf, IDF's Vice President of Patient Services, "IDF's goal is to empower patients and families attending the National Conference by providing education, information and the opportunity to gain support from their peers."

This conference is made possible through the generous support of our industry partners. IDF wishes to extend thanks to the following sponsors.



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Industry Update

Baxter Healthcare Corporation to Purchase Plasma from American Red Cross –American Red Cross Exits Plasma Therapeutics Business

Baxter Healthcare Corporation announced it will purchase plasma from the American Red Cross under a new long-term supply agreement effective July 1, 2005. Upon strategic review of its Plasma Services business, the Red Cross will exit the plasma therapeutics business. As a result, the parties will terminate their contract manufacturing agreement for the processing of plasma products. According to Joy Amundson, president of Baxter's Bioscience Business, "This new agreement per-

mits Baxter to continue to utilize its existing capacity for plasma processing and enhances our manufacturing flexibility and efficiency. Our goal is also to ensure there is no interruption of supply in the marketplace and we intend to offer our products and leading patient support and services programs to the patients and customers who today rely on Red Cross branded plasma therapies." *Excerpted from Baxter News Release, March 1, 2005*

Talecris Biotherapeutics Acquires Bayer Healthcare, LLC Biological Products Division

Talecris Biotherapeutics, Holdings Corp. acquired the contributed

assets of Bayer HealthCare, LLC, Biological Products Division's plasma business and launched its worldwide therapeutic proteins business, Talecris Biotherapeutics, Inc. Talecris is committed to growing and strengthening the previous Bayer plasma products it has acquired, including Gamunex. According to Talecris Executive Chairman, Larry Stern, "Talecris has a unique opportunity to expand and grow the long history of patient service and new product innovation, quality and safety we inherited from Bayer." *Excerpted from Talecris Biotherapeutics News Release, April 1, 2005*

USIDNET Reaches Critical Goals



The Primary Immunodeficiency Research Consortium, US Immunodeficiency Network (USIDNET), a research consortium, was established in October 2003 to address research issues for more than 120 genetically determined primary immune deficiency diseases. It is the intent of the Consortium to fund research, which will have important clinical implications for our community and to introduce some of the young, talented physicians and researchers to this field. The Immune Deficiency Foundation administers USIDNET.

In the past two years the consortium has accomplished several major goals:

- Formed a 10 member Steering Committee
- Created a 54 member Advisory Panel
- Created a website - www.usidnet.org
- Contracted for the development of a new electronic disease registry
- Established a centralized repository for cell lines and DNA from patients with various primary immunodeficiency diseases
- Cosponsored a Summer School project allowing young investigators from 35 states and 17 countries the opportunity to learn new techniques and acquire clinical experience
- Reviewed research applications every 120 days for funding considerations
- Awarded nearly \$4 million to 16 researchers for a two-year period
- Awarded travel scholarships for attendance to national and international scientific meetings

The Consortium is funded through The National Institute of Allergy and Infectious Diseases (NIAID) and The National Institute of Child Health and Human Development (NICHD), which are components of the National Institutes of Health (NIH).

FDA Workshop continued from page 2

- The FDA and IDF plan to discuss patient registries and other methods that could facilitate monitoring for serious but uncommon adverse events resulting from IGIV therapy.
- The FDA hopes to generate an IGIV repository, with advice from the IDF Medical Advisory Committee. Testing over time could include

surveillance for specific antibody titer declines, or increases that may be protective. Research could also be conducted to determine whether products intermittently, or ever, achieve measurable titers to pathogens such as echoviruses, and mycoplasma which are problematic for certain patients.

- IDF will update the FDA on

issues of reimbursement and access to care.

IDF applauds the FDA for working with the Immune Deficiency Foundation and bringing together leaders in the field of primary immune deficiency diseases, public health agencies and industry to discuss important clinical and manufacturing issues for IGIV therapy. ■

BLUE JEANS for HEALTHY GENES

IDF promotes *Blue Jeans for Healthy Genes* fundraising and awareness program

The Blue Jeans for Healthy Genes program was initially launched during PIDD Awareness week in April 2004. This program enables you to help support IDF research and patient programs by coordinating a Blue Jeans for Healthy Genes Day at your workplace. This program provides the ideal opportunity to educate those around you about primary immune deficiency diseases while raising funds for these important endeavors. Blue Jeans for Healthy Genes Day is a special workday on which your colleagues make a \$5.00 donation or more to IDF in exchange for wearing blue jeans to work on the specified day. This program allows for IDF to receive 100% of all funds collected and will benefit the patient community through funding additional medical and patient services programs.

It's easy to get involved! Last year, ZLB Behring planned a Blue Jeans for Healthy Genes Day and their

employees were able to raise several thousand dollars for IDF. ZLB Behring continued their support of PIDD Awareness week by sponsoring another Blue Jeans Day on April 22, 2005.

If you are interested in helping IDF with this fundraising and awareness program, call Diana Gill at 1-800-296-4433 or email dg@primaryimmune.org to express interest in becoming a Blue Jeans for Healthy Genes Coordinator and request an informational packet. This packet includes information about IDF for you to present to your employer. It also contains the details of your responsibilities as a Blue Jeans for Healthy Genes Coordinator. Once you decide to participate and have permission from your employer, IDF will provide you with the materials necessary to promote the event and you can start signing up your coworkers!

Primary Immune Deficiency Diseases Awareness Week: April 17 - 23, 2005

Since 1994, thanks to local and federal advocacy efforts by members of the Immune Deficiency Foundation, the third week of April has been designated "Primary Immune Deficiency Diseases Awareness Week". The goal of this week is to increase awareness and education of the over 120 different primary immune deficiency diseases by reaching out to public and medical communities. This year, many activities took place to work toward this goal.

During the week of April 17 - 23, 2005, IDF volunteers and representatives from one of IDF's core sponsors, Talecris Biotherapeutics, Inc., partnered to increase

awareness of primary immune deficiency diseases. Displays were set up at medical centers across the United States to provide information on primary immune deficiency diseases. "It is so important that we continue to make patients, families and medical professionals more aware of these under-diagnosed diseases" said Linda Keegan, IDF Volunteer Local Coordinator, who participated in this local outreach effort in her home state of Wisconsin. Like other IDF volunteers, Ms. Keegan also worked with her state government to get a proclamation issued by the governor designating April 17 - 23, 2005, Primary Immune Deficiency Diseases Awareness Week.

IDF Spring Education Events

Spring 2005 was a busy time for Immune Deficiency Foundation patient education events around the country. Gail Nelson planned a local meeting in Baton Rouge, Louisiana on April 16th featuring Dr. Prem Menon with the Asthma, Allergy

and Immunology Center in Baton Rouge. He led a discussion on subcutaneous immune globulin.

The education continued with local meetings in Westminster, Colorado on May 14th and Minneapolis, Minnesota on May 21st.

Susan Rhodes coordinated the Colorado meeting, where Dr. Isaac Melamed, an immunologist with First Allergy and Asthma Center, gave a lecture on *The Interaction of the Immune System with other Organ Systems*. Barb Lindenbaum, head nurse in the infusion suite at National Jewish Medical Center discussed rapid infusion of IGIV and patient experiences. Kathy Antilla organized the annual Minnesota Family Conference Day on May 21st, which included discussions on educational

rights and living a full life with primary immune deficiency, as well as a separate youth program.

All local meetings are made possible by the generous sponsorship from all IDF Core Services Sponsors.

Operation Outreach meetings were held in Rye Brook, New York on April 30th and Birmingham, Alabama on May 7th. The Operation Outreach Program is sponsored by ZLB Behring as a way to bring educational programs and activities to communities where IDF has not held previous meetings. Dr. Charlotte Cunningham-Rundles and Dr. Lloyd Mayer from Mt. Sinai Medical Center gave informative overviews on primary immune deficiency diseases and gastrointestinal complications at the Rye Brook meeting. Rob Dash, Manager of Reimbursement Services for ZLB Behring, gave an excellent presentation on understanding health insurance and successfully working with insurance companies. At the Birmingham meeting, Dr. Prescott Atkinson and Dr. Harry Schroeder, both immunologists from the University of Alabama at Birmingham Medical Center, discussed the general aspects of primary immune deficiency diseases as well as the genetic aspects of these diseases. After the Birmingham meeting, the participants and their families enjoyed a beautiful afternoon at The Birmingham Zoo.

Please visit IDF's website or call 800-296-4433 to learn about IDF events in your community. ■

Philanthropy

IDF extends its gratitude to those who generously contribute to the organization, which enables the IDF to fulfill its mission of education, research and advocacy. For more information call 800-296-4433.

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able in the administration of IVIG. Additionally, sending primary immune deficient patients to the hospital for their IVIG infusions can increase patients' risk for infections.

In the Medicare Modernization Act (MMA), Congress exempted IVIG from competitive bidding, understanding that patients need access to all brands of IVIG. Additionally, Congress added a new site of service for primary immune deficient patients to receive IVIG and that was in the home care setting. However, with the new rates being implemented by CMS, patients are losing access to most products, as well as losing access to most sites of service. IVIG is a life-saving therapy for PIDD.

The Immune Deficiency Foundation (IDF) worked with CMS and key Members of Congress to increase the reimbursement for IVIG, and on January 14, 2005, the reimbursement rate was increased to \$56.72 per gram. This was helpful, but not enough. Providers continued to take a loss on both the purchase of IVIG, as well as the administration.

To address these issues, more than 100 providers attended an IVIG Reimbursement Roundtable breakfast hosted by IDF at the American Academy of Allergy, Asthma and Immunology's meeting in San Antonio on March 19, 2005. The Roundtable provided information on the new reimbursement formula under Medicare, on the changes that can occur to improve reimbursement, about the different rates in different sites of care, and how to work with CMS and Congress to reform the new average sale price formula.

As a result of the Reimbursement Roundtable, the Primary Immuno-deficiency Committee of the AAAAI unanimously voted to classify IVIG as a biologic response modifier therapy on March 21, 2005, which if approved by the AMA RUC Committee could lead to more adequate reimbursement for physicians administering IVIG. Starting April 1, 2005, CMS implemented new rules requiring all providers to bill Medicare using one of the two new "Q" codes that separately define lyophilized and non-lyophilized (liquid) IVIG products. The new codes and prices are as follows: Q9941, IVIG lyophilized, 1 gram, \$38.74 and Q9943, IVIG non-lyophilized, 1 gram, \$56.22. The lyophilized category includes the following brands: Carimune NF, Gammagard S/D, Gammar-PI.V., Panglobulin, and Polygam S/D. The non-lyophilized category includes: Flebogamma, Gamunex, and Octagam.

On May 16, 2005, the U.S. Department of Health and Human Services Advisory Committee on Blood Safety and Availability held a hearing on IVIG reimbursement and supply issues. IDF, the Plasma Protein Therapeutics Association (PPTA), FFF Enterprises, and CMS testified before the Committee on the current Medicare reimbursement crisis. The Committee found that there is a worsening crisis in the availability of and access to IVIG products that is affecting and placing patients' lives at risk; changes in reimbursement of IVIG products under the MMA since January 2005 have resulted in shortfalls in the reimbursement of IVIG products and their adminis-

ACTION ALERT!

Please join in IDF's efforts to ensure that all patients have access to all brands of IVIG in all sites of care by participating in our new on-line grassroots advocacy program. The on-line program is due to go live on June 23, 2005. Visit IDF's website at www.primaryimmune.org to view this new advocacy tool. Also, please visit the IVIG Reimbursement section on the website for Medicare alerts, background information and media articles.

tration; and immediate interventions are needed to protect patients' lives and health. Therefore, the Committee unanimously approved the recommendation to urge U.S. Secretary of Health and Human Services Secretary Mike Leavitt to declare a public health emergency so as to enable CMS to apply alternative mechanisms for determination of the reimbursement schedule for IVIG products, and to assist CMS in identifying effective short and long term solution to problems of unavailability of and access to IVIG products in all settings.

IDF realizes that the current Medicare rates are unacceptable and IVIG cannot be purchased at these rates. We are committed to working with CMS to find a short-term solution and Congress to **find a longer-term resolution for the reimbursement system for IVIG**. IDF advocates that physicians and patients should decide what site of care is best based on clinical appropriateness and individual circumstances, and **reimbursement should never dictate where a patient receives an IVIG infusion.** ■

IDF ADVOCATE

Spring Calendar of Events

April 16, 2005	Louisiana Local Education Meeting Tau Center of Our Lady of the Lake Medical Center Baton Rouge, LA
April 30, 2005	New York Operation Outreach Meeting Doral Arrowwood Conference Resort, Rye Brook, NY
May 7, 2005	Alabama Operation Outreach Meeting The Birmingham Zoo, Birmingham, AL
May 14, 2005	Colorado Local Education Meeting Double Tree Hotel - Denver North, Westminster, CO
May 21, 2005	Minnesota Family Conference Day Minneapolis Marriott City Center, Minneapolis, MN
June 23-25, 2005	IDF 3rd National Conference Disney's Contemporary Resort, Lake Buena Vista, FL
July 15-17, 2005	Visiting Professor Program, Mark Ballow, M.D., California Society of Allergy, Asthma and Immunology Monterey, CA

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