The IDF 2013 National Conference provided hundreds living with primary immunodeficiency diseases the opportunity to learn more about the disorders, to discover ways to manage their health, and to connect with others who understand. For rare disorders like primary immunodeficiency, many may not meet someone who has had similar experiences if not for an event like the IDF National Conference—it is the only national meeting in the U.S. specifically for the primary immunodeficiency community.

“It was really nice to have the person-to-person sessions as I was able to meet people of similar age,” said one patient, “We were able to stay connected throughout the weekend, and we plan to continue to stay in touch.”

At this year’s conference, more than 1,200 people of all ages gathered in Baltimore, MD June 27-29. Attendees traveled from all over the world, including 44 states, Guam, France, South Africa, the United Kingdom, Canada, Italy, Germany, Australia, Israel, Belgium and Argentina. It all began with IDF Advocacy Day on Thursday, June 27. Approximately 300 people traveled to Washington, D.C. to advocate for needs of our community—this was the largest delegation of the primary immunodeficiency community on Capitol Hill ever. They met with Members of Congress for personal meetings throughout the day. One patient said, “Advocacy Day was by far the best, most empowering event I have participated in since my diagnosis in 2006.” Read more about Advocacy Day on page 6. Later that day, everyone returned to Baltimore for the Welcome Reception officially kicking off the conference, featuring a performance of the national anthem and an Armed Forces Color Guard.

Over the next two days, healthcare and life management experts conducted nearly 40 presentations that provided vital information to patients and families. Speakers discussed advancements in diagnosis and treatments as well as topics related to living with primary immunodeficiency, like autoimmunity, allergies, mental health, parenting skills, employment, health insurance and more. “All of the speakers presented their topics in a manner which was easy to understand for non-clinicians,” said one attendee. “This was my first conference. I intend to make it the first of many.”

Children, tweens and teens participated in age-appropriate, educational activities during the IDF Youth Program. Parents were thankful for the experiences of their children. A mother of a patient explained, “The experience of meeting other children that have the same diagnosis as my son with CVID was priceless for him.” Another mother said, “Thank you for helping our children become better educated about their disease. You have made our lives easier and better because we have the knowledge and understanding of primary immunodeficiency.”

Continued on next page
In addition to learning and connecting with others, conference attendees took advantage of the IDF Exhibit Hall featuring services and products for the primary immunodeficiency community. They also witnessed the launch of several new IDF initiatives:

- **Redesigned Website** – Enhanced, mobile responsive IDF website with easier-to-use menus and additional content, including life stage sections: www.primaryimmune.org. Read more on page 8.
- **IDF eHealthRecord 2.0** – Recently redesigned online personal health record specifically for patients with PI: www.idfehealthrecord.org. Read more on page 9.
- **Clinical Focus Chronic Granulomatous Disease** – Authored by Drs. Jennifer Leiding, Harry Malech and Steve Holland. Read more on page 9.
- **New IDF Logo** – After more than 30 years, the IDF logo underwent a makeover.

At the end of each day, everyone had a chance to relax with special celebrations. Friday’s Stars and Zebra Stripes Gala included the THINK ZEBRA! Silent Auction, musical entertainment and an awards presentation. Family fun was had by all at Saturday’s IDF Night at the National Aquarium in Baltimore.

The IDF 2013 National Conference was a tremendous success, educating patients and families and bringing together so many members of the community. “This was the most amazing and positive experience,” said one patient, “I will be recommending this to anyone with primary immunodeficiency … You put together an experience that was so affirming for us.”

To see more photos of the IDF 2013 National Conference, go to http://www.flickr.com/photos/idfcommunity/sets
First Gene Therapy Patients Attend Conference

Ashanti DeSilva and Cindy Kisik were born with ADA-SCID, a type of Severe Combined Immune Deficiency (SCID) with mutations in a gene that encodes an enzyme called adenosine deaminase (ADA). On September 14, 1990, Ashanti, only 4 years old, underwent the first human gene therapy, and four months later 10-year-old Cindy’s identical treatment followed. IDF Medical Director R. Michael Blaese, MD was part of the team of physicians who pioneered gene therapy and worked with the girls and their families. Now adults, Ashanti and Cindy attended the IDF 2013 National Conference and enjoyed a reunion with Dr. Blaese.

Dr. Blaese said “It was wonderful to see ‘my girls’ happy and healthy nearly 23 years after helping pioneer the use of genes as medicines for serious inherited diseases. The contributions of the girls and their parents were critical to the initial steps in bringing this new technology to clinical practice which is now helping patients with a range of disorders beyond primary immunodeficiency including metabolic problems, cancer and blindness. During the coming decades gene therapy will become increasingly common thanks to their willingness to participate in the clinical trial that started it all.”

The Boyle Scientific Achievement Award

At the IDF 2013 National Conference, Michael S. Hershfield, MD received The Boyle Scientific Achievement Award. Established by IDF in 1992, this award pays tribute to a member of the medical/scientific community who has extensively contributed toward improving the diagnosis and care of patients with primary immunodeficiency diseases.

Dr. Hershfield is a professor of medicine and biochemistry at Duke University Medical Center in Durham, NC. He has authored or co-authored nearly 100 research articles and case reports related to primary immunodeficiency diseases. Dr. Hershfield has also sparked collaboration with an international group of pediatric immunologists in efforts to reach a consensus on optimal therapy for patients with Severe Combined Immune Deficiency (SCID) and other specific types of primary immunodeficiency diseases.

“Dr. Buckley and Dr. Hershfield

“For more than two decades, Dr. Hershfield’s pioneering research efforts and generous donation of his expertise and laboratory testing of SCID blood samples have provided a vital global resource for diagnosing and treating ADA-deficient SCID and ruling it out in the others,” said Rebecca Buckley, MD, Professor of Pediatrics and Professor of Immunology at Duke University Medical Center and Chair of the IDF Medical Advisory Committee. “We are confident Dr. Hershfield’s dedication to primary immunodeficiency will inspire other physicians and researchers by showing how scientific achievement can directly impact patients and lead to a better quality of life for people with these diseases.”

IDF Achievement Award

Carol Ann Demaret, a long time IDF Board member, received the IDF Achievement Award at the IDF 2013 National Conference for her work on behalf of the primary immunodeficiency community. Carol Ann’s son, affectionately known as David, the Boy in the Bubble, was born with Severe Combined Immune Deficiency (SCID).

Carol Ann has been an active and devoted member of the IDF Board of Trustees since 1992. “She has never turned down a request to assist in our mission,” explains IDF President & Founder Marcia Boyle, “She has given testimony to Congress and in her state on a number of issues, ranging from SCID Newborn Screening, Medicare access to IVIG.” Whenever given the opportunity, she works to raise awareness of primary immunodeficiency. IDF has worked for many years on the Medicare IVIG Access Act, which was signed into law by the President on January 10, 2013. This law was the result of advocacy from the whole community, but Carol Ann holds a special place in its success. U.S. Representative Kevin Brady (R-TX) was our lead champion in Congress because of his constituent, Carol Ann. Representative Brady actually thanked her in his remarks on the floor of the U.S. House of Representatives on H.R. 1845, the Medicare IVIG Access Act, which was signed into law by the President on January 10, 2013.

To view the remarks go to: http://primaryimmune.org/medicare-ivig-access-act-introduced-on-the-u-s-house-of-representatives-floor/.

The first edition of the Handbook was published in 1987. Since then tens of thousands of copies have been distributed, and it has been translated in part or in whole into at least seven different languages. It is a trusted resource for patients, families and healthcare providers.

“The growth of this handbook over the years is remarkable,” explains R. Michael Blaese, MD, Executive Editor. The first edition was composed of nine chapters covering five primary immunodeficiency diseases. By the time the 4th edition was published in 2007, it contained 22 chapters and covered about 60 disorders. This new 5th edition expands the content of the Handbook by another 50% to 33 chapters with descriptions of nearly 100 different primary immunodeficiencies.

The Handbook includes an overview of the immune system and primary immunodeficiency diseases to provide a basic description of the components of the immune system and how its defects lead to disease.

There are 18 chapters covering the specific details of many types of individual primary immunodeficiency diseases themselves. There are additional chapters with general information relevant to the inheritance, laboratory diagnosis, general care and specific medical treatments as well as chapters on life management issues for patients of different ages.

Important new chapters have been added on subjects such as autoimmunity, allergies and infections – topics of critical interest to many in our community. There are also new chapters on stem cell and gene therapy, innate immune defects and an enlarged section on phagocytic cell disorders within the chronic granulomatous disease chapter.

Dr. Blaese says, “This expanded handbook can be used as a tool for patients and families, helping them understand the information that they receive from their providers and arming them with background information so that they can better communicate with their healthcare team.”

The extensive publication was developed by 44 contributors, including leading immunologists, healthcare providers, and life management experts. Editors were R. Michael Blaese, MD; Francisco A. Bonilla, MD, PhD, Boston Children’s Hospital; E. Richard Stiehm, MD, University of California Los Angeles; and M. Elizabeth Younger, CPNP, PhD, Johns Hopkins.

Thank you to the contributors for their work. Special thanks to Dr. Blaese, Dr. Bonilla, Dr. Stiehm and Dr. Younger for their tremendous efforts. To order a copy or download a PDF, go to http://primaryimmune.org/idfpublications/.

The IDF Patient & Family Handbook for Primary Immunodeficiency Diseases – 5th Edition has been made possible through a generous grant from Baxter Healthcare Corporation. Many thanks to Baxter who has supported all editions of the Handbook.

Bright Tote Bag Brings Family to IDF

Donna Decker, a longtime IDF volunteer, and her family headed home from the IDF 2013 National Conference, complete with her brand new, bright green and blue IDF tote bag filled with materials she collected at the conference. She rested the bag on top of her suitcase at the Detroit Airport when a man approached her and asked what “IDF” was. She explained, and the man proceeded to tell her that his 3-year-old daughter has a primary immunodeficiency disease and he and his wife did not know what to do.

Donna reached into her IDF bag and handed the father the new IDF Patient & Family Handbook and the children’s book Our Immune System. She told him to reach out to IDF and shared her contact information. As a parent of a child living with primary immunodeficiency, Donna understands how many questions a family may have. “I am so excited that he stopped me and that I was able to help him,” said Donna. The father thanked Donna for the material and said that the brightly colored bag caught his attention.

Attendees, like Kelly Clardy, Nancy Dalin and Lisa Miller (from left), carried their bright tote bags throughout the conference.
IDF Launches
Walk for Primary Immunodeficiency

Officially announced at the IDF 2013 National Conference, Walk for Primary Immunodeficiency is IDF’s new team-based fundraising campaign designed to support the thousands of individuals and families living with primary immunodeficiency (PI) across the U.S.

This new initiative is an incredibly exciting step for the primary immunodeficiency community. Not only will the funds raised allow IDF to increase its impact, but it also gives us an incredible opportunity to raise awareness about primary immunodeficiency.

There is no registration fee or required minimum to participate. Once you register, you will receive an online fundraising page, making it easy for you to raise funds for IDF and earn prizes. All funds raised will be used to support IDF’s mission of improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency diseases through advocacy, education, and research.

IDF will host Walk for Primary Immunodeficiency in the following cities this fall:

**Greater Philadelphia Walk**
**Sunday, September 29, 2013**
Penn’s Landing - Philadelphia, PA

**Greater Los Angeles Walk**
**Saturday, October 19, 2013**
El Dorado East Regional Park - Long Beach, CA

**Greater Chicago Walk**
**Sunday, October 27, 2013**
Cantigny Park - Wheaton, IL

If you do not live near one of those three sites, you can still participate in our Nationwide Virtual Walk, or you can organize a community walk in your hometown. Just e-mail us at walk@primaryimmune.org to learn more.

Help us spread the word so that people all over the U.S. join us to Walk for Primary Immunodeficiency!

Visit [www.walkforPI.org](http://www.walkforPI.org) to start your team today!

**IDF Board of Trustees**

Front row, from left: Mary Hurley; Carol Ann Demaret; Marcia Boyle; Barbara Ballard; Rebecca H. Buckley, MD, Medical Advisory Committee Chair; Amy Walsh. Back row: John Seymour, PhD, LMFT, Chair; Steve Fietek, Vice Chair; Terry Halper; Richard Low, Jr.; John Smith; Chuck Lage; John Boyle, PhD; Robert LeBien; Joel Buckberg, Secretary. Not pictured: Douglas R. Bell.
Approximately 300 people traveled to Washington, D.C. on June 27 to advocate for needs of those living with primary immunodeficiency—this was the largest delegation of the primary immunodeficiency community on Capitol Hill ever. The enthusiastic advocates boarded buses to make their way from Baltimore, the location of the IDF 2013 National Conference, to Capitol Hill. They represented 32 states, and throughout the day, they had 140 meetings with Members of Congress. During those meetings, the advocates thanked their representatives for their support of the Medicare IVIG Access Act, which passed unanimously last Congress; asked them to support appropriations language that would continue funding of the United States Immunodeficiency Network (USIDNET); and requested their support for the Patients’ Access to Treatments Act (HR 460), which is bipartisan legislation that will restrain high out-of-pocket costs for specialty medications like immunoglobulin.

Advocacy Day empowered members of the primary immunodeficiency community to advocate for issues important to them by making their voices heard by Members of Congress. Those who participated overwhelmingly enjoyed the day:

 ★ “We loved Advocacy Day; we felt like we had a voice that mattered.”
 ★ “Advocacy Day was an unforgettable experience for me, and I truly appreciate all the work it took for you to organize it. All in all it was a day I will never forget and I am grateful to have had the opportunity to represent IDF.”
 ★ “I heard many first timers talking about how empowered they felt after the meetings. I was thrilled to participate in such an awesome day.”

Thank you to all those who helped make history this IDF Advocacy Day!

IDF Advocacy Day was supported by charitable donations from Baxter Healthcare Corporation, CSL Behring, Grifols, and IgG America / ASD Healthcare / US Bioservices

If you would like to get involved with IDF’s advocacy efforts, visit the IDF Advocacy Center: http://primaryimmune.org/idf-advocacy-center.
Medicare IVIG Access Act Celebration
A Night to Recognize Those Who Made it Possible

April 25, 2013 marked a night of celebration for the primary immunodeficiency community. IDF welcomed stakeholders to Washington, DC for a reception to celebrate the Medicare IVIG Access Act becoming law and recognize the commitment of the community who worked for years to see this feat accomplished. IDF was thrilled to welcome U.S. Representatives Kevin Brady and Doris Matsui, who were IDF’s champions in Congress, to the celebration and present each of them with a special zebra award to commemorate their dedication to the primary immunodeficiency community.

Marcia Boyle Named Inaugural Recipient of PSI’s President Award

Marcia Boyle, IDF President & Founder, was awarded the inaugural President’s Award from the Patient Services, Inc. (PSI) for her leadership in the patient advocacy community.

U.S. Representative Doris Matsui (D-CA), a dedicated advocate for the patient community, presented the award to Marcia at a reception in Washington, DC on Wednesday, April 24, 2013. PSI’s President Award recognizes a leader in the patient advocacy community who embodies the strength, wisdom, and vision of PSI’s Founder and President, Dana A. Kuhn, PhD. Marcia exemplifies these qualities and has consistently devoted her time, energy, and passion to the service of patients in the rare disease community, specifically those living with primary immunodeficiency diseases.

Marcia founded IDF in 1980 when her son was diagnosed with X-Linked Agammaglobulinemia, a type of primary immunodeficiency disease. She organized a small group of families and physicians that agreed there was an urgent need for a strong national patient organization dedicated to individuals affected by these diseases.

Through her leadership, this network has since grown exponentially into an internationally recognized organization that provides vital services and resources to patients with primary immunodeficiency diseases, their families and healthcare providers treating the diseases.

“I am honored to be the inaugural recipient of such a meaningful award,” explains Marcia, “It is of the utmost importance to continue to bring a voice to the rare disease community.”

IDF is proud to support the first International Plasma Awareness Week (IPAW), October 13-20.

This is a joint initiative of the Plasma Protein Therapeutics Association (PPTA) and its member companies.

As PPTA celebrates the generosity of plasma donors and works to raise awareness for the rare diseases during IPAW, we encourage everyone to thank plasma donors who help to ensure a higher quality of life for patients around the world.

For more information about IPAW, visit www.pptaglobal.org.
IDF Website Redesigned
www.primaryimmune.org

Re-organization, New Menus, Now Mobile Responsive

Visit www.primaryimmune.org today to see IDF’s newly redesigned website. The site boasts an enhanced menu system, patient life stage sections, easy-to-navigate video pages, and more. Most importantly, it is now mobile responsive. We found that more than 40% of people were accessing our site with smartphones and tablets, so we redesigned it to make it mobile responsive. So whether you are on a desktop computer, laptop, tablet or smartphone, the new design conforms to your device.

Visit www.primaryimmune.org today to see all that the IDF website has to offer.

The IDF website is supported by a charitable donation from Grifols.

Free Accredited Course

IDF is proud to offer the IDF Online Continuing Education Course, developed as an initiative of the IDF Nurse Advisory Committee. This free, accredited course enhances the knowledge of the nurse clinician by providing an update on primary immunodeficiency diseases, immunoglobulin therapies and the nurse’s role with these therapies. Any nurse who is involved with administration and management of immunoglobulin therapy or the disease states where immunoglobulin is used would find this program very informative and applicable to practice.

You can experience the convenience of online continuing education while earning five free CE credit hours!

Video Series Available in French, German and Spanish

IDF also offers a free, non-credit video series translated into French, German and Spanish, based on the IDF Online Continuing Education Course.

Scan the QR codes below or visit: http://primaryimmune.org/healthcare-professionals/.

New IDF Logo

You may have noticed that the IDF logo looks a little different. While redesigning the website, we decided to re-do the IDF logo to give IDF a fresh, updated presence on the web and in print.

The IDF Online Continuing Education Course and the IDF Video Series for Nurses in French, German and Spanish were made possible by a grant from CSL Behring.
CGD is a genetically determined disease characterized by an inability of the body’s phagocytic cells to make hydrogen peroxide and other oxidants needed to kill certain microorganisms. Because of this defect in phagocytic cell killing, patients with CGD have an increased susceptibility to infections caused by certain bacteria and fungi. The condition is associated with excessive accumulation of immune cells into aggregates called granulomas at sites of infection or other inflammation.

Children with CGD are usually healthy at birth. Then, sometime in their first few months or years of life, they may develop recurrent bacterial or fungal infections. However, CGD can vary in its severity and there is a certain amount of chance as to when a patient with CGD develops a severe infection. For this reason, some patients with CGD may not have an infection that draws attention to the disease until late adolescence or even adulthood.

The publication, released at the IDF 2013 National Conference, was authored by Jennifer W. Leiding, MD, University of South Florida, Department of Pediatrics, Division of Allergy, Immunology, and Rheumatology; Harry Malech, MD, Laboratories of Host Defenses, NIAID, NIH; and Steven M. Holland, MD, Laboratory of Clinical Infectious Diseases, NIAID, NIH. It was approved by the IDF Medical Advisory Committee.

You can download a PDF version or order copies at http://primaryimmune.org/idf-publications, or if you prefer, contact IDF at 800-296-4433 or idf@primaryimmune.org to order hard copies.

This publication was made possible by an educational grant from Vidara Therapeutics.
You are never too young to raise money for IDF! Recently some of the most creative and ambitious fundraisers were organized by patients under the age of 15.

**Nine-year-old Abigail Addison of Raymond, MS** was diagnosed with Common Variable Immune Deficiency (CVID) when she was 4 years old. Abigail read about raising money through lemonade stands, so she decided to create her own stand to raise money for IDF. Abigail’s grandfather built her a lemonade stand, which she painted like a zebra to represent herself. She sold her lemonade at the Raymond Country Fair as part of Lemonade Day and raised $240. “I was so proud of her for wanting to raise the money and the great job she did,” says Abigail’s mom Kathleen, “I want her to give back and raise awareness of primary immunodeficiency. We have both learned so much from IDF and want to thank everyone who is involved with this organization.”

At just 14, **Maddie Shaw of Southberry, CT** organized her own fundraising campaign with multiple activities, all to help IDF. After being diagnosed with CVID in 2012, IDF connected Maddie with other teens with primary immunodeficiency, and the Foundation gave her mom, Tracy, advice about infusions and locating a specialist. This spring, Maddie utilized IDF’s THINK ZEBRA! campaign and asked her community to join “Maddie’s Herd.” She distributed fliers around her school and community about all of her activities, including encouraging people to donate directly to IDF through www.primaryimmune.org and selling THINK ZEBRA! t-shirts and bracelets. Maddie held her own Team Zebra 5K and Walk and a Zumba for Zebras, and she rallied family and friends to help register athletes at a local triathlon, which would donate money to IDF for every hour volunteered. In addition Maddie partnered with a local pizzeria and bakery to have proceeds from zebra pizzas and zebra cookies go to IDF. Overall, Maddie raised nearly $10,000—an incredible accomplishment and testament to her hard work. Tracy is proud of Maddie, “As a mom, I am so proud of Madison’s ability and willingness to marshal the troops for such essential efforts. Equally, I am thankful to the IDF for providing a forum for her to positive channel her energies while adjusting to her diagnosis and shifting from ‘why me?’ to ‘why not me?’” Maddie feels her work is simply the right thing to do, “Like Robin Roberts, I’m making a message from my mess, especially when there is so much more we could and should do to raise awareness and find a cure.”

IDF truly appreciates the efforts of Abigail, Jack and Maddie—all donations help make a difference. These young patients prove that you are never too young to educate others about primary immunodeficiency and raise money for IDF.
Choosing Health Insurance

Whether it is an individual or family policy offered through your employer (a group health plan) or one you acquired as an individual, there are many things to consider when reviewing your options for health insurance. These considerations usually fall under one of two categories: cost or benefits. Typically most people look at the cost of the plan when making a decision—our goal is to help you evaluate the benefits you receive in relation to the cost of the plan you select. For example, the plan with the least expensive premium may result in more out-of-pocket costs for you in the future.

Questions to ask may include:

• What is the monthly/annual premium for the plan?
• In a given year, what might be the sum total of my out-of-pocket costs, including medical and prescription co-pays, deductibles and/or co-insurance?
• Will the policy cover all the services I need?
• Are my physicians covered or are they out-of-network?
• Are there annual limits? If so, is it a maximum annual benefit limit based on dollars or on number of visits or both? For example, home healthcare coverage is usually limited to a certain number of nursing visits per year.
• Are out-of-network benefits available? If so, what percentage of cost will be my responsibility if I receive out-of-network benefits?
• Am I covered if I get sick while out of state? Out of the country?

For those affected by primary immunodeficiency disease, there are often additional, more specific, questions you must ask that relate to what benefits are covered and how, such as:

• Is immunoglobulin (Ig) therapy covered? If so, is it a part of major medical or a pharmacy benefit and what does that mean for my cost responsibility?
• Do I have a choice of site of care (hospital, home infusion, physician’s office)?
• Do I have a choice of administration of therapy, i.e. subcutaneous (SCIG) or intravenous (IVIG)?
• What is my out-of-pocket cost for my Ig therapy? Is my cost responsibility a flat copayment fee or a percentage of the cost of treatment known as coinsurance?
• Are supplies and nursing services covered?
• Do I need a referral to see a specialist?
• What services require prior authorization?
• Is Ig therapy subject to a restrictive formulary, i.e. are there preferred Ig therapy products that I will have to use?
• Will I be required to switch from my current Ig product to another product?
• Does the plan provide a case manager to assist me with navigating my benefits?

Answers for many of the questions above, both relative to cost and benefits, can be found by reviewing your plan’s summary of benefits, drug formulary list and provider network directory. While this is often viewed as a tedious process, it is one of the most important steps you can take to insure that a plan meets your needs. It is best not to judge the cost solely based on the premium. In some cases, the lower cost premiums may also end up being more expensive for you, given other out-of-pocket costs. If your insurance is through your employer, you can also speak with your Human Resources department about your insurance questions.

Beginning October 1, 2013, you will be able to purchase individual insurance in your state health insurance marketplace.

The Marketplace was created by the Affordable Care Act and is designed to help you find health insurance that fits your budget, with less hassle. Visit Healthcare.gov or call the 24-hour consumer assistance number 800-318-2596 for information on how to enroll.

Download the IDF Health Insurance Toolkit at http://primaryimmune.org/services/patient-insurance-center. The toolkit provides you with the information and resources to make the best possible choice in selecting a private health insurance plan, including a helpful Health Plan Cost Comparison Worksheet. For more information, contact IDF at 800-296-4433 or info@primaryimmune.org.
For an Updated IDF Calendar of Events, Visit
www.primaryimmune.org/event-calendar.