The story of Michelle Fox is a play in three acts: Family, Frustration and Advocacy, and one would be hard-pressed to pinpoint which scene elicits the most passionate response.

The curtain rises on her father, Erlend Keenan, a 75-year-old man who was recently diagnosed with Common Variable Immunodeficiency Disease (CVID).

“Almost every story my father tells about milestones in his life is punctuated by the various illnesses he had at the time,” Michelle chuckles, and then runs down a list of medical procedures rivaling only the catalog of available surgeries in a childhood game of “Operation.”

“We could start with the numerous pneumonias and other respiratory issues my father has had,” Fox tells me, “but he has also had his gall bladder and appendix removed, lost half of his intestines and basically has no stomach — and nearly every surgery has resulted in some sort of post-operative infection.”

But it was the most recent surgery — a knee replacement last year — that not only nearly ended Keenan’s life, but also exposed broad gaps in both Medicare coverage for patients with primary immunodeficiency diseases and

the medical community’s continuing ineffectiveness in diagnosing those diseases.

For years, Michelle Fox has accompanied her father during his numerous doctors’ visits. There, primarily to make “sure the right questions were asked,” Fox would leave each appointment frustrated — with both treatment strategies and a perceived tone-deafness among the doctors. Fox was convinced her father’s recurring illnesses and inclination for post-op infections were sure signs of a primary immunodeficiency disease.

“We even had one doctor tell us it was a waste of time to refer us to an immunologist,” Fox explains, “even though we have a family history of the disease we couldn’t even get a test.”

Although she carries not a single medical degree, Michelle Fox — and her husband, Neal — are all-too-familiar with both the symptoms and diagnoses of PIDD: all three of their sons have Common Variable Immunodeficiency Disease.

“After seeing what my dad has been through I feel extremely fortunate that our kids were continued on page 4
While I’m sure numerous members of the primary immunodeficiency community have attended every Immune Deficiency Foundation National Conference, it’s a safe bet that none have done so wearing a pair of walking shoes that have touched the soil of five of the globe’s seven continents.

Arlan Sprague, however, could make such a claim – having purchased the footwear in preparation for such worldly travels, and then comfortably strolling atop every earthly landmass, save for Africa and Antarctica.

And while exotic locales often topped Arlan’s itinerary, one of his most treasured stops was IDF’s celebrated biennial event.

Whether it was Baltimore, St. Louis or Disney World, Arlan – who was diagnosed with common variable immune deficiency (CVID) at 30 years of age – would pack up his family and head toward that year’s host destination.

“We always learned so much and met so many great people, Arlan’s wife, Nancy Wilson, tells me. “After the first day of sessions during the very first conference, Arlan couldn’t wait to get back to the hotel room. He wanted to call his parents and tell them he wasn’t a freak – that there were so many people out there who were just like him.”

As any member of the immunodeficiency community can attest, learning that you are not alone in your fight against the disease is arguably the most important step one can take after diagnosis.

Equally essential, however, is the opportunity to interact with the world-renowned immunologists and medical professionals who anchor the sessions offered at IDF’s preeminent gathering.

“Arlan found it remarkable that Conference attendees could have such beneficial interaction with the doctors,” Nancy shares. “He was amazed at the amount of time those professionals were willing to spend with PIDD patients.”

Last August, shortly after returning home from the 2009 Conference, Arlan was diagnosed with lymphoma of the liver, and according to Nancy, approached that diagnosis in the same manner in which he handled news of his CVID.

“Arlan had such a great attitude,” Nancy states emphatically, “He wasn’t going to let anything stop him.”

In December, Arlan developed an infection, and as in many cases in those persons with compromised immune systems, it became “septic.” On December 29, Arlan passed away, leaving Nancy and their two children – Emma (8) and Ryan (6) - without their husband, father and fellow traveler.

However, thanks to Nancy Wilson’s generosity and Arlan’s love of IDF, Sprague’s legacy – and perfect Conference attendance record – can live on through future patients with PIDD.

“I just hope that another family can experience what we did,” Wilson says, “The IDF Conference is such a valuable resource.”

So valuable that Nancy has made a donation to IDF – in honor of Arlan - to allow a first-time family to attend the National Conference in 2011.

“I want a family to attend – to have the same wonderful and helpful experiences that we did – that couldn’t afford it otherwise. And I hope to continue to do it for many years to come.”

The Sprague family won’t be in Arizona when the 2011 National Conference rolls into Phoenix, but Arlan’s indomitable spirit will most certainly be there.

Nancy, meantime, will be taking a Disney cruise on the Mediterranean Sea next summer, anxiously awaiting the first African port of call.

“Arlan’s shoes are going with me,” she explains. “They are going to touch that sixth continent after all.”
You won’t want to miss the IDF 2011 National Conference, June 23-25, 2011 at the luxurious JW Marriott Desert Ridge Resort and Spa, in Phoenix, Arizona!

It promises to be an exciting and rewarding event, as members of the primary immunodeficiency community come together to engage in educational sessions, and fun! From basic information about primary immunodeficiencies to discussions on major opportunities and challenges to our community, the conference sessions offer a large selection of topics for you to attend.

The IDF 2011 National Conference will be an incredible opportunity for individuals and family members who live with primary immunodeficiency diseases to gain knowledge about their diseases. World-renowned immunologists will share their time and expertise with families. Attendees will learn about scientific advancements in the diagnosis and treatment of the diseases and gain skills needed to manage their healthcare. Families will have the opportunity to meet other families and age appropriate learning and fun activities are available for youth.

There will be time to talk with the speakers and our generous sponsors in the interactive exhibit hall. We want you to be a part of it!

You’ll enjoy luxurious spa treatments, stunning sunsets, and magnificent mountain views. This award-winning destination encourages leisure, calms the senses and conversely, offers a wide choice of activities ensuring a fun time for the whole family. Desert Ridge also features four acres of cool, sparkling swimming pools, multiple waterways that wind through the landscape and inviting palm-lined pathways. An immense waterfall is the centerpiece of the main pool, and families and adventure seekers alike enjoy floating on the resort’s Lazy River and Serpentine Slide.

For the athletically inclined, Desert Ridge offers two 18-hole championship golf courses and is centrally located for many of Arizona’s awe-inspiring recreation areas. Hiking, mountain biking, horseback riding, fishing, cycling and water sports are just a short drive from the resort.

The Grand Canyon is only four hours away and Sedona is just three, so you might want to consider extending your stay to visit these spectacular natural wonders. Arizona offers lush forests, wildflower meadows, mountain lakes and swift, fish-filled rivers.

Make plans now!

About the JW Marriot Desert Ridge Resort & Spa

When you’re not attending seminars facilitated by top immunologists, participating in life management sessions or networking with peers, you’ll enjoy luxurious spa treatments, refreshing recreational activities, stunning sunsets, and magnificent mountain views. This award-winning destination encourages leisure, calms the senses and conversely, offers a wide choice of activities ensuring a fun time for the whole family. Desert Ridge also features four acres of cool, sparkling swimming pools, multiple waterways that wind through the landscape and inviting palm-lined pathways. An immense waterfall is the centerpiece of the main pool, and families and adventure seekers alike enjoy floating on the resort’s Lazy River and Serpentine Slide.

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Make plans now!

Help IDF Celebrate 30 Years of Advocacy, Education and Research. Join us at the Immune Deficiency Foundation 2011 National Conference!

Registration begins February 2011

Questions? Call 800-296-4433 or email: info@primaryimmune.org
diagnosed as early as they were,” Fox says, “but unfortunately a timely diagnosis is just a small part of the overall battle.”

In 2005, that battle was joined by the passage of the Medicare Modernization Act, a Congressional edict that changed payment policy for Medicare reimbursements. One of the consequences of the Act was it allowed coverage for IVIG for PIDD under Medicare Part B, but it excludes coverage for items and services related to home administration of IVIG. As a result, the current benefit also does not cover the skilled nurses needed to administer the product.

The Medicare Cab Company?

This empty Medicare benefit reached new levels of absurdity for Michelle Fox and Erlend Keenan while the family patriarch was rehabbing after the knee surgery. Keenan, confined to a wheelchair and just recovered from an infection that prompted his daughter to fear for his life, had just begun his IVIG treatments.

“My dad’s story really punctuates the struggle,” Fox clarifies. “Here you have a senior citizen unable to travel, recently diagnosed with a primary immune deficiency disease and dependent on IVIG treatments, and we are unable to get him home infusion.”

In 2007, an Office of the Assistant for Planning and Evaluation (ASPE) report concluded that home infusion providers generally don’t accept new Medicare beneficiaries with PIDD.

However, while Medicare won’t pay for a medical professional to treat Erlend in his home, they will disburse untold amounts of money to chauffeur Keenan to and from the hospital for his treatments.

For months, Keenan - while in constant pain from the surgery and its post-op consequences - would rise at 5 a.m. on each infusion day, hoist himself into the wheelchair and await the ambulance that would take him to his treatments – a 160-mile, round trip journey that would take 4-to 6 hours, depending on Boston-area traffic jams.

“A very unpleasant trip in a wheelchair,” Keenan says, “lots of banging and bouncing. There were plenty of opportunities to get knocked around.”

“You know there's something very wrong with the system when a senior citizen has to jump through these kinds of hoops to get life-saving treatment,” Fox adds.

“So not only was dad in no physical condition to make these trips,” Fox says, “Medicare also decided it was a great idea to – when he was most vulnerable – send him to a hospital which happens to be the best place to pick up an infection. Healthy people don’t go to hospitals.”

To view Erlend Keenan’s video, go to the IDF Advocacy Channel at http://www.primaryimmune.org/advocacy_center/advocacy_center.asp, and click on the video titled Erlend – PIDD, Medicare, IVIG Access.

You can hear Michelle Fox’s frustration with the medical system with each measured response. Not surprising, when you consider she has spent the last 11 years fighting for the health and quality of life for her three children.

Now, that passion includes standing up for seniors such as her father – our older citizens, as Fox sees it, suffer when it comes to medical care because of not only their age, but also the societal mentality of treating the aged.

“I’ve been concerned about Medicare coverage since long before my father’s situation,” Fox adds. “This is something I’ve always feared – because PIDD isn’t something my kids will outgrow. Medicare has to be fixed.”

And Fox, with nearly a dozen years of struggle and sacrifice for her family, has armed herself – and others – with the tools to begin the renovation.

“There is no better advocate than someone who cares enough to push hard enough to effect change, and this issue couldn’t be any closer to my heart,” Fox declares.

Advocating for Life

It’s been more than seven years since Michelle connected with the Immune Deficiency Foundation’s Volunteer Programs, and in that time, she has participated in IDF’s annual Advocacy Day in Washington, D.C. and held numerous fundraisers – including a highly-successful wine-tasting that raised funds for IDF.

Currently, her focus – along with IDF and the PIDD community – is on legislation now in both the U.S. House and Senate. The Medicare Patient IVIG Demonstration Project (House Resolution 5597) was introduced in June of this year by Congresswoman Doris Matsui (CA) to correct the problems caused by the Medicare Modernization Act.

This bill would mandate a three-year Medicare demonstration project that would provide reimbursement for the items and services necessary to administer IVIG in the home, and include a study into the way that Medicare currently reimburses for IVIG and the appropriateness of implementing a new payment method.

Last year, Senator John Kerry (MA) and Congressman Steve Israel (NY)
introduced the “Medicare Patient IVIG Access Act” (Senate Bill 701 and House Resolution 2002) to address this inequity. These bills continue to be supported by IDF, but the focus has shifted to the Demonstration Project as it is less expensive, making it more likely to pass and become law.

Senator Kerry became a supporter of this issue after one of his staffers met a roomful of children with PIDD. Among them were the sons of Neal and Michelle Fox.

“We spoke to someone who has the power to do something about this Medicare mess,” Fox says proudly. “And once they looked into the faces of those kids they knew something had to be done – it’s easy to see that their medical care is not supposed to be this way.”

Fox, though hopeful for a “fast solution,” knows that the struggle to right this wrong is not easy. Nevertheless, she also knows that a groundswell of grassroots activism can help expedite the process, and encourages everyone to get involved.

“Everyone has time to send their legislators a letter,” Fox explains, “and IDF has made that contact easy through their ‘Action Alerts’. I realize that the more dramatic changes will be made on a national level, but it really begins locally. I’m more hopeful with each passing year.”

You can contact your legislators via IDF’s Action Alert by visiting www.primaryimmune.org/advocacy_center/advocacycenter.asp and clicking the IDF Action Alert link at the top right of the page.

This year has been difficult for new Medicare legislation because of the development and passage of the new health care reform law, but Fox truly believes that the battles and hurdles faced by her family will not plague future patients with PIDD.

“Sure it’s frustrating that we’ve yet to see any marked changes but we can’t continue to let a faceless bureaucracy make decisions for our loved ones,” Fox concludes. “Members of our community need to fight for the opportunity to live, not simply exist.”

Infants With SCID Should Not Receive Live Rotavirus Vaccines

New Brochure for Providers

The IDF SCID Initiative and SCID Angels for Life Foundation has undertaken the development and distribution of a new brochure to educate health care providers against the administration of the Live Rotavirus vaccine to infants with SCID. The brochure, authored by Javier Chinen, MD and reviewed by the IDF Medical Advisory Committee, explains the contraindication of the vaccine with SCID infants and lists the signs and symptoms, as well as actions to take prior to administration of the vaccine. The brochure is intended for the information of healthcare providers. To order copies for your physician, please contact IDF at 800-296-4433, or idf@primaryimmune.org. To download and print the PDF version, visit www.primaryimmune.org, and click on the “Publications” link on the left-hand side.
This summer’s 2 IDF Retreats, held in Rye Brook, NY and San Francisco, CA, were fabulously successful events that attracted a combined 400+ people from 27 states! Attendees had the opportunity to meet and become acquainted with other patients and family members who live with primary immunodeficiency diseases, while also hearing presentations from expert immunologists and experienced professionals who volunteered their time and knowledge. People received the most up-to-date and authoritative information on medical and life management issues, including: Overview of the Immune System and Primary Immunodeficiency; Understanding Immunological Testing; Understanding Antibiotic & Antifungal Therapy; IG Replacement Therapy; Managing GI Issues; Managing Lung Issues; Antibody Production Defects; Cellular, Combined & Phagocytic Defects; Healthcare Reform; Plasma Collection & Safeguards; Living with Chronic Illness; and, even had personal questions answered during the Ask the Medical Expert Seminar.

Youth of all ages participated in fun and educational activities throughout the weekend. From the youngest learning how to really wash their hands and playing IDF Jeopardy to the teens being challenged with their survivor skills, everyone got involved in the fun. The IDF carnival was also a huge hit, however, the best part for many participants was meeting new friends. IDF Teen Council members helped organize and lead the youth program and were integral in its success.

“This conference is an unexpected joy and reward! I don’t feel so alone anymore. Thank You”
Attendees at both retreats enjoyed great entertainment on Saturday Evening. At the Rye Brook Retreat, IDF Volunteer Stephanie Bush performed several of her own songs and joined in with IDF Volunteer, and pianist, Bill Hindin to lead an old-fashioned sing-a-long with all attendees. In San Francisco chart-topping singer and songwriter, Maree McRae performed several songs and shared her story.

“This event was important to learning how to live better, and be more proactive in our own treatment and options.”

Of course, none of this could be possible without our generous sponsors. Much appreciation goes to Baxter Healthcare, CSL Behring, IgG America/ ASD Healthcare, Octapharma, Talecris Biotherapeutics, and Walgreens-Ig Therapy Program for their support.

THANK YOU TO OUR PRESENTERS!

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Becky Wang, MA, LLPC
Meredith Zerbe

IDF Teen Escape Weekend

“Awesome Weekend!”

Fifty teens, young adults and parents got-away for the IDF Teen Escape during the weekend of July 16 in Bloomington, Minnesota. The weekend was an opportunity for teens diagnosed with primary immunodeficiency diseases to meet with others like themselves, learn about and develop skills to live better with their diseases.

The weekend kicked off with informative presentations about getting “In Tune” with your immune system, taking control of your healthcare and need-to-know facts about insurance. Details about treatment options and basic care for dealing with primary immunodeficiencies were shared.

In addition to participating in presentations, there was plenty of time for the teens to meet others, make friends and have fun! An escape to the Mall of America for dinner, to play miniature golf, and enjoy rides at the Nickelodeon Universe were all a huge hit.

While the teens were busy with their sessions, their parents attended their own gathering on Saturday morning. This was an opportunity for a roundtable discussion about parenting issues, insuring teens, and wise words from medical professionals.

The IDF Teen and Young Adult Council served as competent leaders over the course of the weekend. Special thanks go to Brian Rath and Elizabeth Hart, who served as mentors to the teens and shared their experiences with the parents in the roundtable discussion. Our speakers were exceptional as they provided support and information to the teens and parents throughout the weekend: Dr. Ralph Shapiro, Dr. Jason Raasch, Dr. Tamara Pozos, Dr. Laura Hoyt, Kristin Epland, FNP-C, and Frank Quintieri.

Although it is difficult to get a group of teens to agree totally on anything, based on their experience, 100% of the participants agreed that they would recommend the IDF Teen ESCAPE Weekend to others.

And as this teen expressed, “This was my first time attending the event and I really enjoyed it! I hope to attend many more of these.” We expect to see many repeat attendees in the future.

A generous appreciation goes to Baxter Healthcare for sponsoring the IDF Teen Escape with an unrestricted educational grant.
A Parent’s Perspective

-by Rosalind Cardia

In August, my son William and I, alongside 30 other families, including 30 children, from around the world, met an international gathering of amazing specialists, clinicians, and researchers at the first-ever Wiskott-Aldrich Syndrome Conference held in Bloomingdale, Illinois.

Wiskott-Aldrich Syndrome (WAS) is a rare primary immunodeficiency disease affecting four per million live male births. Patients with WAS are prone to significant bleeding due to low platelets and are at an increased risk of serious infections. Most of these patients have eczema of the skin and have an increased incidence of autoimmune diseases as well as malignancies such as lymphoma and leukemia.

Eighteen years ago, my son was born with this very rare, fickle, and seemingly, random disease that often claims its victims early in childhood. Words cannot describe the isolation I felt when William was diagnosed, a feeling shared by other parents as they too confront the challenges of WAS. Each of us desperately yearns for contact with others who understand what we are going through and can offer support and guidance as we cross the emotional, intellectual, and sometimes bleak territory of treating our sons’ conditions.

In only three days, the WAS conference transformed this landscape. This may not sound exciting to some, but to the WAS families it was a miracle. On the first night of the conference, I quickly realized I was among peers; people who truly understood me and who offered acceptance, support, and love. Very little needed to be said; the usual elaborate explanations were all but unnecessary.

This amazing conference of 160 attendees, was brought together by Dr. Sumathi Iyengar, another parent of a son with WAS, Dr. Fabio Candotti of the NIH Human Genome Research Institute and the Immune Deficiency Foundation. The presentations ranged from the discovery of the disease and its affected gene to current challenges. The physicians discussed the latest data on Hematopoietic Cell Transplant and on various clinical trials underway around the world such as gene therapy, IL-2 therapy, and eltrombopag. These experts openly and willingly shared their data, their clinical experiences, and their hearts. It was a godsend to be able to ask questions of them and to be treated with such equanimity and respect. In return, the doctors gained a feel for our lives and our concerns, and appeared to connect with us a little differently, a little more intimately. They shared their passion and commitment to treating our sons’ conditions and shared their enthusiasm for finding new—and improved—treatments that will increase the quality of life for WAS patients and their families.

Finally, William and the other boys just like him were graciously accepted into the fold of this wonderfully supportive community. And, for the first time in their lives, they found themselves amid their peers. Under the care of a team of dedicated camp counselors, the children had a fabulous time, forming friendships and creating wonderful memories, prompting many to ask “When is the next conference?”

The older teens and young adults apparently gained something else: a deeper understanding of and compassion for what we, their parents, had experienced while raising them, how we faced those agonizingly tough decisions on their behalf, and the ways we inevitably carry the weight of those decisions forever forward in our own lives.

Near the end of the conference, my son sat next to me in our hotel room, hugged me, and said “Mom, I realized today that you were one of those scared Moms! Thank you!” It was a very sweet moment that captured, in terms of the conference’s value, so much more than just his words.

This conference was sponsored by National Human Genome Research Institute, NIH Office of Rare Diseases Research.
Syndrome Conference

A Physician’s Perspective

- by R. Michael Blaese, MD

This first WAS Family Conference was not just for families. The scientists had the chance to learn of work from laboratories in the U.S. and Europe and stimulate new collaborations directed toward generating progress in this disease. An important part of the conference included the active participation of WAS patients and their family members providing a unique perspective not often represented in scientific-medical meetings.

Understanding the Wiskott – Aldrich Syndrome

Understanding about the Wiskott-Aldrich syndrome has expanded greatly since the gene responsible for this disease (WASP) was identified. The classic presentation of WAS is one of recurrent infections with all classes of microorganisms associated with defects in each of the components of the immune system, bleeding because of low numbers of blood platelets, eczema, several different kinds of serious autoimmune problems and a higher than normal incidence of lymphoid malignancy. In the years just after the disease was first described, the mean survival of WAS patients was about three years. The introduction of bone marrow transplantation in 1968 offered the first real chance to cure patients with WAS, but the scarcity of suitably matched bone marrow donors limited the number of patients who could benefit from this treatment. As a result, work to solve the problem of donor availability and the search for alternative treatments intensified throughout the 80s, 90s and into the new millennium.

One of the advances that resulted from the identification of the WASP gene was the realization that another disorder, X-linked thrombocytopenia (XLT), shared mutations in the same gene as did the WAS. Careful studies of XLT patients showed that these boys sometimes showed only low blood platelets, but none of the immune system problems found in classic WAS. However, there were also some XLT patients who did have variable degrees of immunodeficiency. This demonstrated that defects in the WASP gene could result in a broad range of clinical problems from relatively mild to very severe. This insight poses a dilemma in deciding what treatments are most appropriate for a particular patient that takes into consideration the balance between benefits offered and potential risks involved.

Recommended Treatment for Classic WAS

A near consensus regarding a recommendation for the treatment of a patient with classic WAS was reached. For a child newly diagnosed with classic WAS, an immediate search for a related or unrelated HLA-matched hematopoietic stem cell (HSC) donor should be initiated. Over the past three decades, the National Marrow Donor Program has developed a database of tens of thousands of potential HSC donors. The results of transplantation using HSC from unrelated matched donors is now as successful as transplants from matched sibling donors, if performed at a center experienced in transplanting patients with primary immunodeficiency. The age of the WAS patient at the time of transplant is also important with higher rates of success in the first few years of life.

In the absence of imminent HSC transplantation, the potential use of splenectomy continues to be debated as a treatment for both WAS and XLT. Removing the spleen is helpful in restoring the platelet count to near normal levels in most WAS patients. Restoration of normal blood clotting can have a significant positive impact on the quality of life for many WAS boys whose activities otherwise would be restricted for fear of serious or even fatal hemorrhage. However, it was recognized early that splenectomized WAS patients were more susceptible to serious or lethal septic infections with encapsulated organisms such as S. pneumonia or H. flu. By contrast, observations also noted that the use of daily prophylactic antibiotics could largely prevent these serious infections and this led to the increased use of splenectomy for many boys with both classic WAS and XLT.

Unfortunately, with the appearance of antibiotic resistant strains of these organisms, fear that antibiotic prophylaxis would become less reliable again raised a question about the long-term safety of splenectomy. Fortunately, the introduction of regular immunoglobulin infusions for the routine treatment of WAS patients provided an answer to the infection problem following splenectomy. Antibody deficiency is a problem for patients with WAS because they are unable to produce normal antibody responses to capsular polysaccharide antigens. IG replacement has been life-saving for

continued on page 10
A Physician’s Perspective

continued from page 9

both non-splenectomized and splenectomized WAS and XLT patients and is an important strategy for treating all WAS patients, particularly now that antibiotic resistant pneumococci are becoming a more common problem.

The conferees heard about early studies evaluating the use of drugs to promote the production of more platelets as a treatment for WAS and XLT. Data should be coming in soon to indicate whether this approach will be a useful addition to our treatment arsenal. Another study employing treatment with an important factor for T cell and NK cell growth and signaling (interleukin 2, IL-2) has also recently gotten underway. It is hoped that additional patients will enroll in these studies soon so that we can learn whether these ideas will provide needed new tools for treatment of this disease.

Optimistic Expectation

The development of another new treatment for both WAS and XLT brought a great deal of optimistic expectation at the conference. It is 20 years since gene therapy was first used (in a patient with severe combined immunodeficiency) and we have been waiting expectantly for news that it has been successful in WAS and other types of primary immunodeficiency. The conference heard reports of gene therapy trials for WAS that are underway in Germany and about to begin in Italy and the UK.

Ten patients have been treated so far with early results showing promise. The advantages of gene therapy over transplantation of HSC from another donor include the absence of potential graft vs. host disease (GVHD) and the need for reduced levels of chemotherapy conditioning prior to treatment. It is still too early to make a final judgment on the efficacy and safety of gene therapy for the WAS, but these early results are very encouraging. This approach may become the treatment of choice for both WAS and XLT in the coming years.

Treatment for Patients with XLT

There was no clear consensus reached about the recommended treatment for patients with XLT. Many patients with this form of the WASP gene defect can live nearly normal lives for many years as evidenced by reports of individuals living into their 70s without special specific treatment. However, some patients that have milder forms of disease that could be called XLT, have more immune system abnormalities than expected and later develop symptoms more like classic WAS. We are still struggling to understand how to distinguish those mild cases from those destined to become more severe – and until this can be reliably determined, it is difficult to feel fully confident in what to advise concerning treatment.

Even though it has developed into a treatment with a high level of success and cure, HSC transplantation still has an unsuccessful outcome in approximately 10% of cases. In someone with classic WAS it is not difficult to bet on a 90% chance of success when compared to the lower long-term success of alternative treatments. However, in someone with mild XLT, even taking a 10% chance of an unfavorable outcome may be greater than one is willing to accept.

This is just one example of some of the critical questions that remain to be explored in the coming years. Hopefully the results with approaches such as gene therapy will prove to be an answer that we can all be comfortable recommending to all patients with a mutation in their WASP gene.
Talecris Biotherapeutics Receives FDA Approval for Gamunex®-C

Talecris Biotherapeutics (Nasdaq: TLCR) announced today that the U.S. Food and Drug Administration (FDA) approved Gamunex®-C (Immune Globulin Injection [Human], 10% Caprylate/Chromatography Purified) for subcutaneous administration in the treatment of primary immunodeficiency (PI).

The newly approved Gamunex-C provides both the intravenous route of administration and a new subcutaneous route of administration. The intravenous delivery mode is approved to treat PI, chronic inflammatory demyelinating polyneuropathy (CIDP), and idiopathic thrombocytopenic purpura (ITP). The subcutaneous mode is approved to treat only PI.


Privigen® Demonstrates Efficacy and Tolerability Among Patients With Various Immunodeficiencies, Data Show

Privigen®, the first and only 10% liquid intravenous immunoglobulin (IVIG) therapy stabilized with proline, is effective and well tolerated in patients with several primary and secondary immunodeficiencies, according to data presented today at the XIVth Meeting of the European Society for Immunodeficiencies. Results also demonstrate that Privigen offers significant protection against infection. In a separate observational study, patients reported higher overall satisfaction with Privigen compared to previous treatments, along with fewer adverse events and improvements in quality of life. Even at higher rates of infusion, Privigen tolerability appeared superior to Sandoglobulin.


Data Support Hizentra® Efficacy, Safety After Dose-Equivalent Switch in Adult and Pediatric Patients with Primary Immunodeficiency

Hizentra® (IgPro20) provides primary immunodeficiency (PI) patients with a safe and effective alternative to other immunoglobulin therapies when given in equivalent doses, according to a Phase III pivotal trial data presented today at the XIVth Meeting of the European Society for Immunodeficiencies (ESID). Hizentra, the first and only 20 percent SCIg developed for subcutaneous (i.e., under the skin) administration, can be stored at room temperature over its entire 24-month shelf life given its formulation with L-proline.


Study Demonstrates Safety and Efficacy of Subcutaneous Vivaglobin® as First-Line Therapy for Primary Immunodeficiency

Vivaglobin® (subcutaneous immunoglobulin [IgG]) (SCIg) is an effective and safe initial therapy for treatment-naïve patients with primary immunodeficiency (PI) and may offer an attractive alternative to intravenous IgG (IVIG) therapy in the newly diagnosed, according to data presented today at the XIVth Meeting of the European Society for Immunodeficiencies. The study found that IgG replacement therapy initiated with Vivaglobin in patients with PI provided adequate serum IgG levels, protected the patients from infections, and improved health-related quality of life, without raising safety concerns.


Baxter Presents Data from Interim Analyses of Phase III Clinical Trial of HyQ at European Society for Immunodeficiencies Meeting

Data Presented Include Analyses of Tolerability, Bioavailability and Infusion Volumes, Intervals and Rates of Baxter’s HyQ

Data from interim analyses of a Phase III clinical study in patients with primary immune deficiency (PID) who received Baxter’s HyQ were presented at the 26th meeting of the European Society for Immunodeficiencies (ESID) in Istanbul, Turkey. HyQ is an immune globulin (IG) therapy facilitated subcutaneously by recombinant human hyaluronidase, a dispersion and permeation enhancer.


Update: Product Withdrawal/Product Recall - octagam® (octagam 50mg/ml) and octagam 10% (octagam 100mg/ml)

In response to current measures taken related to the recent increase in the reporting frequency of thromboembolic events (TEEs) in connection with octagam® (human normal immunoglobulin for i.v. administration, 50 mg/ml), Octapharma would like to provide the following update.

On August 20, 2010, in the interest of patient safety, Octapharma USA Inc. initiated a voluntary market withdrawal of selected lots of octagam® (Immune Globulin Intravenous (human)) 5% Liquid Preparation). This was performed as a result of an increased number of reported thromboembolic events, some of which were serious. A total of 31 lots were voluntarily withdrawn at that time.

Effective immediately, Octapharma USA Inc. is initiating a voluntary market withdrawal of ALL lots of octagam® (Immune Globulin Intravenous (human) 5% Liquid Preparation) currently in the US market. While Octapharma has not received any reports of thromboembolic events since the August 20, 2010, voluntary market withdrawal was performed, Octapharma has determined, through consultation with the public health authorities at FDA, that until a root cause of the previously reported thromboembolic events can be determined and the problem corrected, the most prudent course of action is to suspend further administration of octagam®.


Heidelberg to Host New Octapharma R&D Facility

The Octapharma Group, announced that work is proceeding according to plan on the development of its new research and development site, located in the new Technology Park in Heidelberg, Germany. The 10,000 square meter facility will combine both laboratories and office space for the continued development of recombinant products and is expected to cost in excess of €20 million to complete.


Urgent: Voluntary market withdrawal - August 27, 2010 octagam® and octagam®10% (Immune Globulin Intravenous (human) 5% and 10%) Liquid Preparation

In the interest of patient safety, Octapharma has, in collaboration with the authorities, initiated a voluntary market withdrawal of selected lots of octagam® and octagam®10% (Immune Globulin Intravenous (human)) 5% and 10%) Liquid Preparation as a result of an increased number of reported thromboembolic events, some of which were serious.

While Octapharma has not determined that the thromboembolic events reported were caused by the use of its product, globally there was an increase in the number of thromboembolic events reported in the second quarter of 2010 potentially associated with certain lots, and these lots have been voluntarily withdrawn from the market in collaboration with respective regulatory authorities.

All customers and healthcare professionals, should they have any questions in relation to this message, should contact their product supplier or Octapharma representative for further information.


CSL Behring Receives FDA Approval to Extend Shelf Life of Hizentra® from 18 Months to 24 Months

CSL Behring announced today that the U.S. Food and Drug Administration (FDA) has approved a supplemental Biologics License Application (sBLA) to extend the shelf life of Hizentra®, Immune Globulin Subcutaneous (Human), 20% Liquid, from 18 months to 24 months. Hizentra, the first and only 20 percent subcutaneous immunoglobulin (SCIg) approved in the U.S. by the FDA, is also the first and only SCIg in the U.S. that may be stored at room temperature.

Excerpted from CSL Behring news release August 18, 2010.
Supporting IDF at the Highest Levels

The Immune Deficiency Foundation is fortunate to have the continual support of leaders in the healthcare industry. These companies understand the importance of the continuity of services IDF provides for individuals and families living with primary immunodeficiency diseases. Being chronic conditions, these diseases never go away and neither does the need for vital resources and programs. Because of their generous commitment to IDF, our sponsors help to ensure that our services will be available.

IDF Core Services consist of essential programs like direct patient services-patient advocacy, peer support, local patient meetings and volunteer activities. Medical programs like the IDF Consulting Immunologist Program and the LeBien Visiting Professor Program, as well as medical meetings and exhibits are included. Vital advocacy efforts to help ensure access to quality healthcare and support research priorities also fall into this category.

IDF Core Service Sponsors are dedicated partners that support IDF at the highest level.

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These sponsors make an enormous difference in the Foundation’s ability to plan and provide long-term resources to the primary immunodeficiency community. IDF greatly appreciates these devoted sponsors and their commitment to our mission.

For an updated IDF Calendar of events, visit www.primaryimmune.org/idfcalendar.