OUR CHAT WITH EMILY V. GORDON

HOW TO PREPARE FOR AN EMERGENCY

TZ’S CORNER

IDF ADVOCACY DAY
THE SNOW WON’T STOP US!

GRIFOLS
This newsletter is sponsored by an educational grant from Grifols.
Looking Ahead

One of our responsibilities here at IDF is to anticipate the needs of people with primary immunodeficiency diseases (PI) and to monitor any possible threats that would affect their health.

In this issue of the IDF ADVOCATE, you’ll hear about our continued public policy work and see how not even a snowstorm can stop us. You’ll read about dynamic members of the community—whether newly diagnosed or a part of the community for years. You’ll see how IDF is keeping a watchful eye on the future—from emergency preparedness and gene therapy to research and immunoglobulin (Ig) potency.

As we look ahead, there’s one concern that I will address right here: the global plasma supply. Many members of the PI community rely on a lifesaving treatment derived from human plasma: Ig replacement therapy. Once upon a time, the PI community was the majority user of Ig. Our majority, however, has shrunk over the years thanks to other therapeutic uses of Ig, both on label and off label. As a result, people with PI now account for less than half of the usage of Ig in the U.S.

The need for plasma and plasma-derived products is increasing, both here in the U.S. and around the globe. The upside: Ig and plasma-derived products are helping more people. The downside: manufacturing plasma into therapies such as Ig takes a long time, and, if there’s increased demand, increasing supply can take time, which could lead to disruptions.

The good news is that more and more plasma collection centers are opening up across the U.S. to help meet the growing demand. You can find those centers at www.donatingplasma.org.

The global plasma supply is a multi-faceted and complex issue, and IDF is actively looking at the role that it should play in terms of addressing it. While our community’s share of Ig may have grown smaller, the supply is more-or-less keeping with new demands, so the hope is that no one will be left without treatment.

The world needs more plasma, so IDF will remain vigilant and engaged to ensure that those who rely on plasma-derived products have what they need to remain healthy, as we try to do with every need within our community.
What is the difference between co-pay and coinsurance?

Many dealing with insurance have posed this question, and we have the answer. See the answer, and learn more about how IDF is advocating for co-pay choice legislation. [www.primaryimmune.org/copay-coinsurance](http://www.primaryimmune.org/copay-coinsurance).

Where can I learn more about the different types of immunoglobulin (Ig) products that are licensed in the U.S.?

IDF maintains an Ig product chart that features all the Ig products licensed in the U.S. Download your chart at no cost to you. [www.primaryimmune.org/ig-product-chart](http://www.primaryimmune.org/ig-product-chart).

How can I meet another person who has a primary immunodeficiency?

Join us at an IDF event! IDF holds events across the country all year long, including IDF Education Meetings, IDF Get Connected Groups, IDF Walk for PI and more. You can find events near you on our Calendar of Events. [www.primaryimmune.org/events](http://www.primaryimmune.org/events).

If you can't attend an event, the IDF Peer Support Program can connect you to someone with similar experiences and situations. We can connect you with a Peer Support Coach to help you. [www.primaryimmune.org/peer-support-program](http://www.primaryimmune.org/peer-support-program).

How can I make sure I'm updated when there is news about PI?

IDF My Account allows you to update your preferences for communications, ensuring you're getting the information you want from IDF. You can also add your diagnosis and/or birthdate to receive customized updates. Sign up and utilize My Account today. [www.primaryimmune.org/my-account](http://www.primaryimmune.org/my-account).

What materials can I share with my healthcare provider to help them better understand my diagnosis?

IDF offers a range of materials for healthcare professionals, all of which can be found on our website. Many people share the IDF Patient & Family Handbook with their healthcare team to help them better understand PI. [www.primaryimmune.org/healthcare-professionals](http://www.primaryimmune.org/healthcare-professionals).
SCID Newborn Screening Update

Screening infants born in the U.S. for Severe Combined Immune Deficiency (SCID) is close to being official in almost every state across the country. Early identification of SCID can make possible lifesaving intervention before severe infections occur. All types of PI, not just SCID, stand to benefit from early diagnosis as research advances. Currently, 94% of all newborns in the U.S. are receiving SCID screening, and three states (Alabama, Indiana and Louisiana) have plans to begin screening in 2018. For more information on the IDF SCID Newborn Screening Campaign, visit: www.primaryimmune.org/IDF-SCID-Center.

Ensuring Affordable Access to Care

As a rare disease community, those living with PI may find that it can be difficult to get the lifesaving treatment that is needed through affordable rates and flat fee copays. The issues of copay and affordability face many within the rare disease community, and it’s something that IDF is working hard to advocate for across state and federal levels. In 2017, IDF was actively involved in state level advocacy in Iowa to address these issues. Read more about the measures that were taken to lead the charge for our community in states like Iowa: www.primaryimmune.org/access-to-care.

Advocating for the PI Community

The PI Community Won’t Be Stopped: IDF Advocacy Day 2018

In March, 49 advocates from the PI community took to Washington, DC in order to advocate for issues important to individuals with PI. The participants’ motivation was not diminished by the snowstorm that swept the Capitol.

Participants made the most of their time in the nation’s capital, conducting meetings with 81 Congressional offices to request legislative support, thanking congress for their support of various legislation and bringing a face to the PI community. One such piece of legislation extended the Medicare IVIG Access Demonstration, which will allow individuals with PI who are in the demonstration project to continue to have their in-home infusions covered through 2020 while IDF continues advocating for this to become a permanent benefit. The other legislation accelerated the start of the Medicare SCIG home infusion benefit, ensuring that Medicare beneficiaries with PI have continued access to SCIG until the permanent benefit takes effect in January 2021.

Thank you to all those who attended. To learn more about Advocacy Day, please visit: www.primaryimmune.org/advocacy-day-2018.

Want to Make Your Voice Heard?
SIGN UP FOR IDF ACTION ALERTS!

Reaching out to your public officials is simple and quick with IDF Action Alerts. Customize each alert with your name and contact information and hit send. It is that easy to make a positive impact for the PI community.

To sign up to be notified when your voice needs to be heard, go to www.primaryimmune.org/action-alerts.
Remembering Sara LeBien

It is with great sadness that the Immune Deficiency Foundation (IDF) shares the news that Sara LeBien died January 14, 2018.

Sara and her husband Bob helped IDF get started. They, like the other families and physicians in those early days, saw the need for a national organization dedicated to improving the diagnosis and treatment of people with primary immunodeficiency diseases (PI) after their son Mike was diagnosed.

Sara wrote IDF’s popular illustrated book, Our Immune System. She was inspired to write the publication in the mid 1980’s when she accompanied her husband Bob from California to the IDF Board of Trustees meeting in Baltimore. The first edition of the Patient and Family Handbook on Primary Immunodeficiency Diseases was presented, and the board members were thrilled with finally having a comprehensive resource book for the PI community. Although Sara was delighted with the handbook, as a mother of a son with PI and a primary school teacher, she couldn’t help but think, “What about the children?” It was a long flight from Baltimore to southern California, and Sara was not the kind of person to waste time. She started writing a story about how the immune system works and what happens when there is a PI.

“I wanted a story for very small children and didn’t want it to be too medical or scary. I wanted to create a story that children could easily understand and relate to.” Sara laughed, “While on the plane, I even started sketching little medical pictures and the woman sitting next to me wanted to know if I was a doctor!”

Sara’s beginning efforts resulted in the first edition of Our Immune System in 1990, and in 2008 she helped update the second edition. Our Immune System is a simple story of how the immune system works and the treatments necessary for children with PI. Through delightful illustrations, the story is told by friendly characters in the immune system — B cells, T cells, and even phagocytes. Reviewed by physicians, teachers, nurses, psychologists, and parents, hundreds of thousands of copies have been distributed; in fact, it continues to be the most requested publication from IDF. In demand worldwide, IDF has given permission to have it translated into more than a dozen languages.

Sara leaves a legacy that has forever changed how people learn about PI, and she will be fondly remembered for her tremendous dedication and kind spirit. IDF extends our deepest sympathy to Bob and all of Sara’s family and friends.

To see Sara’s official obituary and learn more, please visit www.primaryimmune.org/Sara-LeBien.

Catch Up on What’s New: IDF Publications

Immune Deficiency Foundation Guide to Hematopoietic Stem Cell Transplantation

This guide includes Hematopoietic Stem Cell Transplantation (HSCT) approaches that could potentially benefit patients with several types of PI. Subsequent chapters provide more details as to how a patient is prepared for a transplant, what the transplant experience is like and what life can be like after a transplant.

This publication has been made possible through the IDF SCID Initiative and the SCID, Angels for Life Foundation.

Immune Deficiency Foundation Guide to Immunoglobulin Replacement Therapy for People Living with Primary Immunodeficiency Diseases

This guide was developed for patients and caregivers to help increase understanding of immunoglobulin (Ig) replacement therapy. An increased understanding puts patients and caregivers in a better position to make informed decisions regarding care. Education about the therapy can also build confidence and create a level of understanding that can reduce a patient’s anxiety about a treatment that can be frightening. If a patient and/or caregiver is well educated about the treatment, they will understand the goals they need to work towards in order to improve health and be more motivated to reach those goals, which will in turn improve their overall health.

This publication has been made possible by Shire.

Order or download these publications at no cost to you: www.primaryimmune.org/idf-publications.
THE REAL LIFE INSPIRATION BEHIND THE BIG SICK MOVIE

EMILY V. GORDON

To get the scoop on the story that so many in our community can relate to, IDF President & CEO John Boyle talked with Emily V. Gordon, the real Emily from The Big Sick movie.

Not many people find themselves checking into the hospital with what appears to be a super flu, as the character Emily does in The Big Sick movie, which premiered in theaters June 2017. The sudden onset of symptoms was a shock to not only her but also to her friends and family. While the movie, based on actual events in the life of Emily V. Gordon, portrays the symptoms as a sudden onset, the reality was a bit different.

“When you make a movie like this you want to both honor the condition but realize that viewers don’t want to get bogged down in the details,” said Gordon during our chat with her.

Like many in our IDF community, the real Emily found herself in and out of the doctor’s office a handful of times, trying to solve her mystery illness. It wasn’t until the ninth visit that she was admitted to the hospital due to impaired breathing. When her parents received a call telling them their daughter was in the hospital, they were shocked. “I was hiding my illness much of the time, pretending it wasn’t as bad as it was,” said Gordon. Her parents were left waiting, hoping for good news as each specialist diagnosed Gordon with a different illness. In fact, an early version of the movie had multiple specialists diagnosing Emily with multiple illnesses, but this was later taken out because of how complicated and confusing it became.

“What I have is not very easily diagnosed or categorized,” continued Gordon. “They didn’t know how to treat me or what to do with me.” It wasn’t until February of 2017 that she received a new diagnosis, Common Variable Immune Deficiency (CVID), a type of primary immunodeficiency disease (PI), on top of her original diagnosis of Adult-Onset Stills Disease.

Regardless, dealing with a chronic disease is no easy feat. “I felt isolated. No one understands this. You feel like a bore. No one wants to talk about how you’re sick again,” says Gordon. “I’d lie about why I wasn’t meeting up with my friends.”

Since her diagnosis though, she has learned to be more transparent and that has been nothing but beneficial. Her doctor appointments are on the calendar kept by her team to ensure nothing gets in the way of her treatments. Now, when nervous about a treatment change, she feels comfortable enough to reach out and share with those around her, which she recently did through an Instagram post where she received overwhelmingly supportive responses.

“This past year has been amazing,” Gordon tells us. “I went from limping through life, technically being ok because I wasn’t hospitalized. That was my bar – that I didn’t need to be hospitalized. I’ve truly embraced it this past year. I’ve found support when I didn’t really know I was looking for it. I’ve learned that I’m not alone,” she says, and that has made all the difference.

Emily is busy working on her next projects. She and her husband, Kumail Nanjiani, were nominated for an OSCAR® for Writing (Best Original Screenplay) for The Big Sick. Follow Emily on Twitter and Instagram: @emilyvgordon.

This article originally appeared on the IDF blog: www.primaryimmune.org/blog.
Let’s Keep Up the Momentum and Walk for PI!

Many within our IDF community have participated in IDF Walk for Primary Immunodeficiency in some way over the last five years. Whether it was by donation, participating in our Virtual Walk, creating a team, wearing a t-shirt or anything else, thank you! We could not have done this without your support and dedication.

You are the reason that in 2017 there were 3,600 walkers on 397 teams in 16 cities that raised more than $940,000. This is the power of the PI community. With the dollars you have raised from IDF Walk for PI, IDF is funding research grants and bringing together thousands of individuals for the opportunity to meet others who are living with these rare, chronic illnesses.

“For me, supporting the walks shows that you’re supporting us: the people that have these illnesses, and that in itself is so important,” says Michelle Searle, an active member of the PI community, during her speech at the IDF Walk for PI in Fort Lauderdale, FL.

Like Michelle, we want everyone in the community to support the walks and support the community. By sharing your story about why you participate in the walks, you can inspire others, raise funds for IDF and increase awareness and understanding of PI.

“I still become overwhelmed each year with the amount of support I receive surrounding IDF Walk for PI, and that carries me throughout the year. When I’m about to do an infusion and I’m feeling sick of it and I don’t want to stick another needle in me, I look over at a walk poster hung next to my desk that I got at the first Fort Lauderdale walk signed by my family and friends and I look at the flags that I’ve kept, and it reminds me that it gets hard at times, but I have so many people on my side.”

Michelle’s story is one of many, but we need even more members of the community to walk for PI!

We Need You in 2018!

Now is the time to step up and join us for IDF Walk for PI 2018! Join a team, create a team, register individually or make a donation. We need your support now more than ever to ensure IDF will be here for future generations! We can’t wait to walk with you.

#WalkforPI
@ifdfcommunity  @TZtheIDFZebra

IDF Walk for PI 2018
National Walks
- Atlanta: 10/27
- Boston: 9/30
- Chicago: 9/30
- Cleveland: 9/23
- Dallas: 11/10
- Fort Lauderdale: 11/17
- Kansas City: 10/27
- Los Angeles: 11/4
- New York City: 10/21
- Philadelphia: 10/14
- St. Louis: 10/13
- Tampa: 11/17

Community Walks
- Detroit: 09/22
- Indianapolis: 10/6

Virtual Walk from Anywhere All Year!

To register and for updated information, go to:

www.walkforpi.org

National Presenting Walk Sponsor
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Grifols
Horizon Pharma
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Interested in sponsoring
IDF Walk for PI or have questions?
Contact us: walk@primaryimmune.org.
Reports of impending weather send everyone running to the store to stock up on items they may need during an emergency, such as natural disasters, like snowstorms, hurricanes, earthquakes or wildfires. Ensuring that you have readily available access to food, water and even electricity is often your first thought, but what about your other needs? For those living with a chronic illness, like a primary immunodeficiency disease (PI), you may rely on lifesaving treatments that, despite weather, you still need. While these natural disasters are out of our control, fortunately, our response to them doesn’t have to be. It all starts with a plan that goes over everything you need to get through an extreme weather event, and covers both sheltering at home and evacuating to other accommodations.

“Think extreme camping on steroids,” is how Joanna, a member of the IDF community described the aftermath of Hurricane Sandy in 2012.

Creating a plan that ensures all your specific health needs are met, regardless of the conditions outside takes time, which is why it’s smart to start planning now. Here are some tips to help you weather (pun intended) anything Mother Nature throws your way.

The Three-Day Rule

Most emergency preparedness experts suggest that you need to have food, supplies and medication to last for three days, at least. An emergency supply kit should be your go-to during an extreme event and the sooner you get your supplies pulled together, the sooner you’ll be ready. Stacking up on basics, like nonperishable food, water, flashlight, first aid kit and personal care items is essential; but as someone living with PI, so are any medications and specialty drugs, pumps, thermometers and other healthcare essentials. For how to best prepare, check the “PI Emergency Supply Kit Checklist” on the next page.

Staying Healthy

Even though our world may turn upside down, the best way to get through an emergency is by staying well. To do that, you need to make your health your number one priority, regardless of the events taking place outside your door. Who better to help ensure that then the members of your healthcare team? Your doctors, nurses, social workers or medical case worker, pharmacist, etc. can be contacted now to arrange visits, phone calls and plans with them long before the storm clouds start brewing. They, too, have their own emergency preparedness plan, and will be glad to help you figure out your own. All you have to do is start the conversation.

Here are some questions and topics you can bring up at your next visit:

• I’m worried about running out of medication and not getting access to a pharmacy. Can I get a reauthorization for extra medication refills?
• I’ve been reading that a three-day supply of all medications, at minimum, are necessary. Does that apply to me? How much can I receive and how soon can I get it? I’d like enough medication to keep in my emergency supply kit to have on hand all year round.
• How long can my refrigerated medications last if I lose power?
• I receive my infusions at a center or hospital, what is the contingency plan if the center is closed?
• How can I get in touch with your office if I get sick during a natural disaster?
• Are there immunizations/vaccinations that my family should be up-to-date on and which ones are especially needed to keep me safe in an emergency situation?

Take Advantage of Readily Available Resources

While you may just be starting your emergency ready plan, take advantage of the information and resources that are already out there. Whether it’s using the Center for Disease Control and Prevention’s Disaster and Severe Weather Guides (www.cdc.gov/disasters), finding your local Emergency Management Agencies (www.fema.gov/emergency-management-agencies), using IDF ePHR to easily track your health, at any time and any place (www.idfephr.org), or following your local emergency services for updated information, you can find helpful information and tools that can keep you safe and keep your health in order during emergencies.

Don’t forget to program key contact information into your phones, along with emergency contact information, labeling contacts as “ICE.” You can also have a digital lifeline by staying in contact with fellow members of the PI community through social media, sharing information and speaking to those who understand what you are going through.
## When You Have PI

### Follow the Instructions of Local Authorities
If an evacuation order is issued, follow it immediately rather than “wait and see what happens.” It is much harder to attempt to leave once a storm or wildfire hits. Have an evacuation plan ready for you and your family. If staying in place is an option, stay put and listen for updates on the TV or radio.

You can also see if your local authorities, news stations and emergency services have social media accounts. Often, these are updated with any new information on evacuation and updates.

### Keep Things Business as Usual
Trying to keep your usual schedule as best as possible will both help your physical and mental health during an emergency/natural disaster. Remember to take your medications and infusions on time, try to go to sleep at a decent hour, keep active and aim for a level of normalcy as you wait for the sun to shine again.

### Don’t Be Shy and Know Your Rights
During a natural disaster, be sure to identify yourself as someone with PI. Sharing your story can get you the help you need as soon as possible. If you need to visit a hospital, inform the emergency personnel that you have a chronic, rare disorder and you know how to manage it, sharing with them your medical information, like from your IDF ePHR on your phone, for instance.

Under the Americans with Disability Act and Section 504 of the Rehabilitation Act, individuals with chronic diseases are guaranteed the right to receive reasonable accommodations. If you find yourself needing assistance through disaster relief programs, this status can help you cut through red tape and receive the benefit of community or government programs and resources.

Ensuring you’re ready in the face of an emergency may not be an easy task, which is why taking steps now to prepare for anything will pay off in the long run.

To learn more about how to be prepared in an emergency, visit the IDF website at: [www.primaryimmune.org/emergency-checklist](http://www.primaryimmune.org/emergency-checklist).

### PI Emergency Supply Kit Checklist

#### Medical/First Aid Supplies
- Three-day supply of prescription medications and treatment supplies, at minimum
- Mechanical infusion pump for home-based self-infusion
- Any necessary syringes, needles, tubing, gauze pads and alcohol pads
- Disposal bins for syringes, needles or lancets
- Sterile gloves
- Thermometer
- Antibiotic ointment
- Bandages
- Cleansing agents
- Nonprescription drugs (antacids, ibuprofen, etc.)
- Cooler with room for re-freezable gel packs for medical supplies that need to be kept cold
  - Note: Do not use dry ice and avoid freezing the medicine
- Portable oxygen tank

#### Food and Water*
- Three-day supply of nonperishable food (peanut butter, canned items, granola, crackers, etc.)
- Three-day supply of water (plan one gallon per person, per day)
- Manual can opener
- Paper plates and plastic silverware

#### Electronics
- Flashlight
- Extra batteries
- Charged cellphone
- Radio (battery powered, solar or hand crank powered)

#### Safety Supplies
- First aid kit
- Emergency blanket
- Swiss army knife or other multipurpose tool
- Matches in a waterproof container

#### Personal Care Items
- Soap
- Toothbrush, toothpaste, floss
- Baby wipes
- Contact lenses or glasses
- Personal hygiene items

#### Storage
- Pack all supplies in lightweight, insulated and waterproof containers
- Store the containers in a closet or other spot in your home that is high and dry, but still easily accessible in case you need them in a hurry

*Check the expiration dates on food, water, medicine and batteries two times a year to ensure all items are functional at the time of any emergency.
Gene Therapy: What You Should Know

Many primary immunodeficiency diseases (PI) are caused by errors, or mutations, in specific genes. It’s because of this that there has been the hope that it would be possible to fix the mutation that causes the disease, essentially curing it. The Human Genome Project, which was the international, collaborative research program whose goal was the complete mapping and understanding of all the genes of human beings, and similar efforts have allowed researchers to identify specific genes involved in many types of PI.

There are more genes being identified regularly. While not every genetic disorder will be able to be corrected by gene therapy, it’s still important for members of the PI community to understand their options when it comes to treatment, including gene therapy.

What Is Gene Therapy?

In short, gene therapy is a technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patient’s cells instead of using drugs or surgery. Gene therapy only works for conditions where a single gene is the cause. Researchers are testing several approaches to gene therapy, including:

- Replacing a mutated gene that causes disease with a healthy copy of the gene
- Inactivating, or “knocking out,” a mutated gene that is functioning improperly
- Introducing a new gene into the body to help fight a disease

Challenges of Gene Therapy

While gene therapy is a promising option for some conditions, it is still being researched and developed through ongoing trials. Currently, there are FDA approved gene therapy treatments, but there are not yet any for PI.

Overall, the experience with gene therapy in PI has demonstrated that it is possible to cure the disease by inserting a normal copy of the gene into the patient. There are some risks, however, that need to be overcome and the safety needs to be improved. As of now, gene therapy must be regarded as an experimental therapy that is being researched through on-going clinical trials with individuals with PI, of which there are quite a few.

To learn more about gene therapy and to find out more about the process, please visit: www.primaryimmune.org/gene-therapy.

DID YOU KNOW?

The first clinical trial of gene therapy was at the National Institutes of Health in 1990 and treated Ashanthi DeSilva, a 4-year-old girl with ADA-SCID, a type of Severe Combined Immune Deficiency (SCID) with mutations in a gene that encodes an enzyme called adenosine deaminase (ADA).

Marian Blaese, MD with Ashanthi DeSilva (left) and Cindy Kisik, who was also born with ADA-SCID and treated with gene therapy, at the IDF 2013 National Conference, June 29.

Cindy and Ashanthi in 1992 with the pioneer physicians of gene therapy: (from left) French Anderson, MD; Michael Blaese, MD; and Kenneth Culver, MD.

Don’t Miss Out on All Things IDF

IDF MY ACCOUNT

Whether it’s new publications, e-mails for upcoming events near you, opportunities to get involved or getting our print materials delivered to your new address, IDF My Account is your way to stay connected to us! The IDF My Account feature not only gives you access to our resources and materials, it’s also your chance to let us know how to reach you best so you can always be informed of what’s happening in the PI community. You can add your diagnosis and/or birthdate to receive communications customized for you.

If you haven’t created your account yet, sign-up today! If you already have an account make sure you’re logging in to select your preferences, update your information or check out what’s new. www.primaryimmune.org/my-account.
Be a Part of Research: How the Data You Submit Advances Research in Primary Immunodeficiency Disease

There are many ways to help the primary immunodeficiency (PI) community, and one of those ways is to be a part of research. By participating in IDF surveys or being a part of PI CONNECT, IDF’s Patient-Powered Research Network, you can help researchers learn more about PI and how it affects people like you, bringing us closer to a better quality of life, more accurate diagnoses, treatment options and more. You may wonder, “How is the information I share used? Where does it go?”

Recently, the United States Immunodeficiency Network (USIDNET), an NIH-funded research program of IDF, had two publications released using the data from people living with PI, like you. The USIDNET patient-consented registry is the only registry for people with PI.

The following articles were written using USIDNET data:

Complications Associated with Underweight Primary Immunodeficiency Patients: Prevalence and Associations Within the USIDNET Registry.

The point prevalence of underweight status and obesity in PI is unknown, despite the described associations between PI and weight loss and failure to thrive. The goal of this study is to estimate the prevalence of underweight status and obesity in patients with PI and to investigate the associations between abnormal body weight and complications of PI.

Visit: www.primaryimmune.org/USIDNET-data-articles

Latent therapeutic demand model for the immunoglobulin replacement therapy of primary immune deficiency disorders in the USA

This research aim is to model latent therapeutic demand (LTD) for the immunoglobulin (Ig) replacement therapy of PI in the U.S. Given the high level of variability of Ig replacement therapy use and major differences among American and European practices in the management of people with PI, the researchers developed a USA specific LTD model for Common Variable Immune Deficiency, Hyper IgM Syndrome, Severe Combined Immune Deficiency, Wiskott–Aldrich Syndrome and X-linked Agammaglobulinemia.

Visit: www.primaryimmune.org/USIDNET-data-articles

Enhanced USIDNET Website

HIGHLIGHTS CAPABILITIES OF THE USIDNET REGISTRY

Recently, USIDNET launched a redesigned website, allowing visitors to more easily access information about the valuable programs and services, and to improve the overall user experience. The site features a new, expanded view of real-time aggregate data that highlights the USIDNET Registry’s capability to contribute to clinical immunology research.

The USIDNET Registry serves as a critical resource for researchers. For example, researchers can request to query the patient-consented registry and can receive de-identified clinical information to help further understanding of these rare, chronic diseases. Patients can help advance research by joining IDF PI CONNECT and consenting to have their experiences shared with the USIDNET registry. Learn more about USIDNET, and check out the new website: www.usidnet.org.

The Immune Deficiency Foundation (IDF) participated in the Workshop on Immune Globulin Potency in the 21st Century, November 8-9, 2017, in cooperation with the Center for Biologics Evaluation and Research (CBER) of the Food and Drug Administration (FDA), the Plasma Protein Therapeutics Association (PPTA), and the National Institute of Allergy and Infectious Diseases (NIAID).

Immunoglobulin (Ig) replacement therapy is used to treat many people with primary immunodeficiency diseases (PI). For those with antibody deficiencies, such as Common Variable Immune Deficiency and X-linked Agammaglobulinemia, among other types of PI, Ig therapy is a lifesaving and lifelong treatment. IDF along with the CBER, FDA, PPTA, NIAID and other stakeholders have worked over the years to ensure that those Ig products currently available as well as those manufactured in the future will contain sufficient levels of antibodies to provide protection against infectious agents of special concern to individuals who have antibody deficiencies.

ABOUT THE IMMUNE GLOBULIN POTENCY IN THE 21ST CENTURY WORKSHOP

The goals of the workshop were (1) to understand and predict the impact on immunoglobulin (Ig) products of declining measles antibodies in the plasma donor population, (2) to evaluate the impact of world-wide polio eradication on Ig lot release testing, and (3) to discuss potential solutions to challenges to the continued use of measles and polio antibody levels, or titers, as some of the tests used by the FDA and manufacturers to assure that individual lots of Ig meet the quality standards expected by the patients with PI who depend on these products to protect them. The workshop also addressed improving accuracy of diagnostic testing for PI, particularly regarding measurement of antibodies to encapsulated bacteria like pneumococci, and the exploration of other surrogate markers, e.g., specific antibodies in Ig products that can be used to ensure Ig quality standards.

To read a full account of this educational workshop, visit www.primaryimmune.org/FDA-Ig-workshop

IDF Board of Trustees: Honoring Robert LeBien

After 34 years, Robert (Bob) LeBien has stepped down from the IDF Board of Trustees. Bob and his late wife Sara were integral in the founding of IDF in 1980. Bob, whose son was diagnosed with a primary immunodeficiency disease (PI) at a young age, has been an active member of our community since the early 80’s and dedicated countless hours to the Board of Trustees over the years. In 1992, Bob helped establish the International Patient Organization for Primary Immunodeficiencies (IPOPI) and is the IPOPI Life President. IPOPI is dedicated to improving the lives of those living with PI worldwide. We would like to thank Bob for his tremendous devotion to IDF and all those living with PI.

IMMUNE DEFICIENCY FOUNDATION BOARD OF TRUSTEES

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Kathleen Sullivan, MD, PhD, Medical Advisory Committee Vice Chair
Amy Walsh

Receiving Immunoglobulin (Ig) Replacement Therapy?

SIGN UP FOR THE PATIENT NOTIFICATION SYSTEM!

The Patient Notification System is a program developed by the Plasma Protein Therapeutics Association (PPTA) to notify patients who receive plasma products, such as immunoglobulin (Ig) replacement therapy, about product recalls. This system is a free, confidential communication system that is available 24-hours a day and provides you with information on withdraws on plasma-derived therapy, like Ig.

Getting the information is easy. Anyone interested in participating registers with the Patient Notification System and provides general contact information, including their preferred method of notification. If a therapy has been withdrawn or recalled, you will be notified. Learn more by calling 888-UPDATE-U (888-873-2838) or visiting www.patientnotificationsystem.org.
SEARCHING FOR INFORMATION ABOUT PI? YOU NEED IDF. AND THOUSANDS LIKE YOU DO TOO.

Support IDF Now, Help More People Living with PI in Years to Come

“I just want to say again thank you for being there and for helping us. There is so much information and yet at the same time a lack of knowledge as a parent being thrown into having a child with a chronic disease. So speaking with someone who understands and is knowledgeable was very helpful and encouraging.” — A mother of a son with PI

“Because of you and your advice I finally got the right diagnosis (CVID)!… Not good but knowing I have not lost my mind is good… Thanks for the really important work you do.” — A woman with PI

“Thanks first of all for everything, the response, the information that was sent to me, and the overall concern for the people with a PI … I found out about the IDF on a website when I was researching a 20 year old diagnosis for my mom … Thank you for the information. I’m not in the dark about her condition.” — A daughter whose mother has PI

Each day we receive requests for information about primary immunodeficiency diseases (PI) from individuals and families living with PI, and we never take for granted how important basic information can be to someone facing this diagnosis. Education is at the core of our mission, and we know there are hundreds of thousands more out there searching for a diagnosis, searching for information. And you can help us reach those people by donating to IDF!

Much like IDF won’t take for granted the basic needs of our community, we don’t want those helped by IDF to take the Foundation for granted. We can only broaden our reach with the generosity of our community—whether you make a personal donation or you fundraise among your family and friends.

And now IDF enables you to designate your donations to support the core services of IDF, like providing educational information, or you can choose to direct your funds to a specific area of focus that is meaningful to you and your family.

Donate
• Make a one-time gift, or join the President’s Club with a monthly gift.* Simply choose “One Time Gift” or “Monthly.” www.primaryimmune.org/give
• Choose to donate to our Core Service Programs or other initiatives.

Fundraise
• Create a team for IDF Walk for PI: www.walkforPI.org (See page 7 for details.)
• Use Facebook fundraiser. Ask your friends to donate in honor of your birthday or other milestone: www.facebook.com/fundraisers

No matter how you choose to support IDF, now is the time to do so.

Think of the next person who is overwhelmed and seeking information to better understand how to care for themselves or their loved one. Think of that mother, that daughter. Think of yourself and how IDF helped you. And make your contribution now.

www.primaryimmune.org/give

Questions about donating or fundraising? Contact IDF: 800-296-4433 or development@primaryimmune.org.

*Those who enroll in monthly giving are members of the President’s Club. Members receive special recognition in the IDF Annual Report and other communications, and they receive exclusive updates from John G. Boyle, IDF President & CEO.

IDF is a 501 (c)(3) nonprofit organization, and your contributions are tax deductible to the maximum extent allowed by IRS regulations. Please consult your tax advisor to determine the tax deductibility of your contribution.
IDF Volunteer Spotlight: Frank Meuers

Because the health of many people living with primary immunodeficiency diseases (PI) relies on plasma protein therapies, the Immune Deficiency Foundation (IDF) considers plasma donations a critical component of the PI community.

For many living with PI, immunoglobulin (Ig) replacement therapy is the only treatment available for their chronic condition. This important relationship led IDF to develop the Plasma Partners Program—to help increase the understanding of the interconnectedness between people with PI, plasma donation centers and the plasma donors themselves.

The vital work of this program is made possible by people like Frank Meuers, one of more than 75 IDF Plasma Awareness Coordinators who visit plasma donation centers nationwide to share personal stories to donors and center staff. IDF is so grateful for our volunteers like Frank, who make so many positive connections with the plasma donation centers. With the tremendous support of our Plasma Partner Volunteers, this work ultimately serves to put a face to the people who really depend on plasma products to maintain their health.

Frank, a retired middle school math teacher, started volunteering with IDF more than 10 years ago to let people know how much their donation means to both him and others in the PI community. “I am beyond thankful for the plasma donors, whose donations have allowed me to be alive today,” he explains. “These donors have a special place in my heart.”

Frank maintains that speaking to donors from the heart and from his own experiences is much more effective than crafting a generic message. “Over the years, I have met people from all walks of life through the Plasma Partners Program,” Frank says. “My visits put a personal touch to the process.”

When asked why he volunteers for IDF, Frank explains that his favorite part of volunteering is the ability to make a positive difference. “You don’t realize how important donating plasma is until you need it.” With Ig replacement therapy, his health has stabilized, and he is able to live a fuller life. “It’s remarkable how few center staff and donors understand where the plasma is going, and they really respond positively when they learn the valuable roles they all play to help people like myself.”

Deciding to become involved with IDF was an easy decision for Frank, as he finds it rewarding to give back. He enjoys seeing the work at the plasma donation centers and how it directly affects the PI community. “For the years I’ve done this, I know I am helping myself and others,” he concludes. “It’s certainly gratifying to be able to come full circle.”

This article originally appeared on the IDF Blog: www.primaryimmune.org/blog.

Ways to Lend a Hand

Find what IDF Volunteer position would be good for you below, or view all volunteer opportunities at www.primaryimmune.org/volunteer.

- Interested in building connections between plasma centers and those that receive Ig therapies, like Frank?
  Help “put a face” to the PI community by becoming a Plasma Awareness Coordinator.

- Do you believe that you can affect change by reaching out to elected officials?
  Become a Health Access Advocate (HAA)! Make change happen by reaching out to local government officials.

- Are you interested in gathering people together and providing support?
  We’d love to have you as an IDF Get Connected Leader! You can help plan and organize regular group meetings in your local area for people with PI and their family members.

- Do you spend a lot of time on Facebook and love engaging with others in the PI community?
  We’re always looking for IDF Facebook Group Moderators! Help provide a welcoming and safe environment on Facebook.

- Do you love attending the IDF Walk for PI events, but don’t have one in your area?
  You would be a great Community Walk Organizer! IDF Community Walks are vitally important and are created by members of the PI community who want to have a walk in an area that does not yet have an existing walk.
 Hey Fellow Zebras, TZ the IDF Zebra here! On social media, we see great photos of our community and hear amazing stories—all inspire others in the community and help us raise awareness for primary immunodeficiency diseases (PI). We saw many posts during April, National Primary Immunodeficiency Awareness Month and World PI Week (April 22-29). I want to encourage you to keep up the good work, and I’ve got some tips for how you can continue to promote awareness of PI. Check it out!

1. **Post on Social Media (and Hashtag #piawareness):** If I can use my hooves to post on social, you can too! Get inspiration on what to post by following me on Twitter and Instagram (@TZtheIDFZebra) and use the following hashtags:
   - #piawareness
   - #thinkzebra
   - #zebrastrong
   - #primaryimmunodeficiency
   - #livingwithPI

2. **Share IDF Resources:** There are so many IDF resources available on the IDF website—my personal favorite is *A Zebra Tale*.

3. **Join Us at an IDF Event (or Create Your Own Awareness Event):** While many who attend IDF events will already know what PI is, it’s a great place to stay informed, connect with your herd and get materials to share with others. Not to mention, it seems like a great time to take a group picture and share on social media with one of the hashtags above. If you attend an IDF event or create your own, post about it!

4. **THINK ZEBRA! WEAR ZEBRA!** I’m always rocking my stripes, and encourage you to take pride in yours! Whether you’re at work, school, home or on-the-go, you can rock your favorite zebra attire and accessories. When you get a compliment on it (which I’m sure you will), you can tell the person why you wear zebra!

Thanks, everyone! Until next time, THINK ZEBRA!

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**TZ the IDF Zebra – Official Mascot of the Immune Deficiency Foundation**

The primary immunodeficiency (PI) community often identifies with zebras. This is based on an old saying. In medical school, many doctors learn the saying, “when you hear hoof beats, think horses, not zebras” and are taught to focus on the likeliest possibilities when making a diagnosis, not the unusual ones. However, sometimes physicians need to look for a zebra. People with PI are the zebras of the medical world. So IDF says THINK ZEBRA!

A few years ago, the saying truly came to life through the official IDF mascot, TZ the IDF Zebra. The name “TZ” comes from THINK ZEBRA! TZ travels across the country to promote awareness of PI and meet fellow zebras. Children, young and old, can't help but smile when they see TZ, serving as a reminder to members of the PI community that they are not alone and together we are #zebrastrong.

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**TZ and Friends**

Our pal TZ is a popular zebra! At IDF events, like our walks or the National Conference, seeing TZ is an exciting opportunity for our IDF community. We hope that TZ inspires you to show your stripes and have zebra pride, and we want to see it! Whether you’re sporting your favorite piece of zebra clothing or accessories, or you’re posing with TZ, share your pictures with us on social media with #TZandFriends!

By using #TZandFriends, you could be featured in an upcoming IDF ADVOCATE Newsletter, like our friends below!
For an Updated IDF Calendar of Events, Visit www.primaryimmune.org/event-calendar.

Follow IDF!

Check out the upgraded IDF Friends!

IDF Friends is a supportive, private online community where you can discuss topics, ask questions, share stories, get support and connect with others living with PI.

Log in or register www.idffriends.org

Thank You

Thank you to the following companies for their support of the Immune Deficiency Foundation and the primary immunodeficiency community!

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Interested in supporting IDF? Contact us: info@primaryimmune.org.