

### Jeffrey Modell im Foundation

# IMMUNEWS

### Harvard Medical School Establishes Jeffrey Modell Immunology Center

### Newly Constructed Building Will Be Focal Point For Immunologists Worldwide

The Harvard Medical School, in collaboration with the Jeffrey Modell Foundation, completed construction of a new immunology building centrally located on the Harvard Medical School campus. The Jeffrey Modell Immunology Center at Harvard is a unique endeavor serving as a focal point for the most dynamic and diverse community of immunologists in the world. The Center will include:

- Dr. Fred S. Rosen Lecture Hall
- Dr. Robert A. Good Library
- Reception Facilities
- Conference Room
- Visiting Professor Office
- Program Director & Faculty Offices
- Graduate Student "Idea Exchange"
- Graduate Student Reading Room

The Center will maximize collaboration between faculty, postdoctoral fellows and students in the graduate program in Immunology, as well as throughout the Longwood medical area.

#### **SAVE THE DATE**

April 24, 2008 CIPRIANI 42nd St. NYC

# "Spring Ahead"

HONORING JMF
"MAN OF THE YEAR"
JEFF BLAU

President, Related Companies



Architectural rendering of the new Jeffrey Modell Immunology Center at Harvard.

Harvard has been rated the #1 medical school in the United States for the past 19 years. There are more than 7,000 full time faculty working at 17 Harvard teaching hospitals and *To be continued on page 2...* 

### Networking...

# 35 Centers Worldwide Form Jeffrey Modell Network

At the Jackson Hole, Wyoming Expert Meeting on Primary Immunodeficiencies, 35 Jeffrey Modell Center Directors and industry supporters convened to address the ever-expanding Jeffrey Modell Diagnostic and Research Centers. There was a consensus to set up a new network that would frame the scientific agenda; including best practices, clinical guidelines, outcome measures, data capture, novel therapies, and a research platform. A Steering Committee was formed, and several sub-committees are now being organized. The Jeffrey Modell Centers Network is comprised of expert Immunologists from 28 cities, 14 countries and 4 continents.

#### A Message from Vicki & Fred Modell



# Reaching Out... And Reaching Up

Recently we visited Brazil and met with families and patients affected by Primary Immunodeficiencies. When we first met them, it was not a very encouraging picture. Their heads were down, and they expressed to us that they were victims of a dreaded disease, feeling helpless, and unable to change the outcome. Most of these families had never met each other in the past.

We offered some remarks in English that were translated to their native Portuguese. Slowly, they began to ask questions and offer poignant and personal stories. They expressed to us that they were never able to speak about their diseases in the past. We provided answers as best as we could. Then, almost miraculously, one family began talking to another and soon, they came to life, smiling and laughing with heads held high. Before long, they were planning a "KID's Day." They formed a patient organization, found volunteer leadership, established a name, a logo, t-shirt design, and one of the fathers, a musician, even promised to write a song about hope and dreams for the future.

It was so inspirational to us, and brought to mind memories of our very first meetings with patients in Boston and Seattle, New Orleans and Cleveland, Miami and then Budapest, Beijing and Shanghai. We are constantly amazed at the similarities. Parents, brothers, sisters, and friends trying to help a child live with hope for a better tomorrow. We are all so similar and have so much to share. We can't wait to go back and participate in the new Brazilian patient organization event...and, listen to the music they create about hope and dreams for the future.

Sincorol

Vicki and fred

# GGOSA Get Together

#### 2nd Robert A. Good Society Symposium

The Robert A. Good Society conducted its Second Annual Symposium entitled "Immune Reconstitution of Primary Immunodeficiencies," at Harvard Medical School in Boston.

The two day conference focused on unresolved problems of Stem Cell Transplantation (SCT), long-term analysis of immune reconstitution, and novel strategies to expedite immune reconstitution post-SCT. The meeting honors Dr. Robert A. Good, considered to be one of the greatest immunologists of the 20th century. Forty years ago, Dr. Good conducted the first successful Bone Marrow Transplant to cure Severe Combined Immune Deficiency. It has emerged as the treatment of choice for many congenital and acquired blood disorders.

# Harvard Medical School Establishes Jeffrey Modell Immunology Center

(Continued from page one)

research institutes. The Harvard Medical School has produced 12 Nobel Prize winners.

According to Joseph B. Martin, MD, PhD, retiring Dean of the Faculty of Medicine, "This Center will make a prominent contribution to the development of a new generation of highly trained immunologists at a time when research in immunology has emerged as the key to the treatment, prevention and cure of a host of deadly and debilitating diseases"

Future programs at the Jeffrey Modell Immunology Center will be announced in early 2008.

# **Did You Know?**

The National Institute of Allergy and Infectious Diseases (NIAID), at the U.S. National Institutes of Health, stated in a recent grant announcement:

"While individual Primary Immunodeficiency Diseases are rare, as a group they may affect 1-2% of the population."

This translates to between 3 million and 6 million Americans, a prevalence far greater than most health agencies have estimated in the past.

# Nation's 1st

### Pilot Program for Newborn Screening of SCID

#### JMF Initiates Collaboration with the State of Wisconsin

Although there are more than 150 Primary Immunodeficiency defects identified, Severe Combined Immune Deficiency (SCID), or "bubble boy disease," will end a child's life before his or her first birthday if not precisely diagnosed and treated within the first weeks of life. This is the reality for the most severe Primary Immunodeficiency disease, and therefore, there is a great need to initiate a screening program. Experts indicate that SCID can be treated and cured with a better than 95% success rate.

JMF has formed a partnership with the Children's Hospital of Milwaukee, the Medical College of Wisconsin, and the Wisconsin State Laboratory of Hygiene to initiate a pilot program to screen for these diseases. The collaboration will utilize the T-cell receptor excision circles (TRECs) to determine whether it is appropriate as a screening test for SCID in newborns. The initial budget is set at \$500,000. It is anticipated that there will be early results by the end of 2008.

# Are We All Immunodeficient?

Dr. Jean-Laurent Casanova, a world-renowned and respected physician at Hôpital Necker Enfants Malades, Paris, France, published a most provocative study in a recent issue of Science, a prestigious medical publication. The article, entitled "Primary Immunodeficiencies: A field in its infancy," expressed the hypothesis that "a paradigm shift is occurring in the field of Primary Immunodeficiencies, with revision of the definition of these conditions and a considerable expansion of their limits. It now appears that most individuals suffer from at least one of a multitude of Primary Immunodeficiencies." The article appeared in Volume 317 of Science, dated August 3rd, 2007. Dr. Casanova has collaborated with the Jeffrey Modell Foundation frequently over the past decade.



# Jeffrey Modell Foundation Endows Chair at Seattle Children's Hospital

# **Dr. Hans Ochs Selected to Hold Chair** in Pediatric Immunology Research

The Jeffrey Modell Foundation has established its first Endowed Chair to support pediatric immunology research. It will assure continuing scientific investigation in Primary Immunodeficiency and Immunology, and bring attention to this field in the medical community. Dr. Hans Ochs, a long-time collaborator with the Foundation, has been selected as the recipient of the Chair. Dr. Ochs is a distinguished professor of Pediatrics at the University of Washington and an acclaimed researcher and clinician.

# JMF's Public Service Advertising Campaign Starts Fifth Year Donated Media Reaches \$80 Million

The JMF Public Awareness Campaign, supported in part by the Centers for Disease Control and Prevention (CDC), is now beginning its fifth year. Local and national television networks, radio stations, newspapers, national magazines, and airport dioramas, have featured the message on PI Diseases, stressing the importance of earliest possible diagnosis. The Campaign includes a targeted effort to reach the African American and Hispanic communities. The ads have had thousands of appearances, including 60 Minutes, Good Morning America, the Today Show,

the Tonight Show, and during most televised sporting events. Full-page 4-color ads have appeared in Time, Newsweek, Sports Illustrated, InStyle, Bazaar, and Parents magazines. Look for the new airport dioramas in Atlanta, Boston, Dallas, Miami, Orlando, Philadelphia, Seattle, JFK and La Guardia in New York. A number of additional airports will display the ad in 2008. CNN airport has continued to run the PSA regularly. A new campaign has been produced and will launch in 2008.



### JMF Study Published in Scientific Journal

#### **Quality of Life Data Compares Undiagnosed and Diagnosed PI Patients**

"Immunologic Research," a scientific journal published by Humana Press,¹ features data compiled by JMF comparing quality of life issues for diagnosed and undiagnosed patients. The information was provided by physicians/experts at 76 JMF Diagnostic and Referral Centers worldwide. Surveys were collated by JMF and reviewed by members of the Foundation's Medical Advisory Board. 532 Patients were included in the study. JMF assigned economic values to the conditions assessed based on publicly accessible information.²

Condition	Cost per episode per dary	# of episodes pre-period	Cost prior to diagnosis	# of episodes post-period	Costs after diagnosis	Annual savings
Acute Infections	<b>\$2,950</b> per episode	6.4	\$18,880	1.8	\$5,310	\$13,570
Severe Infections	<b>\$5,708</b> per episode	4.3	\$24,544	0.6	\$3,424	\$21,119
Bacterial Pneumonia	<b>\$7,529</b> per episode	2.8	\$21,081	0.6	\$4,517	\$16,564
Chronic Infection	<b>\$36.33</b> per day	44.7	\$1,623	12.6	\$457	\$1,166
Physician/ Hospital/ER Visits	<b>\$125</b> per visit	70.9	\$8,862	11.8	\$1,475	\$7,387
Hospitalizations	<b>\$1,158</b> per day	19.2	\$22,233	5.1	\$5,905	\$16,328
Antibiotics	<b>\$4.25</b> per day	166.2	\$706	72.9	\$309	\$397
School/Work Days Missed	<b>\$136.40</b> per day	33.9	\$4,623	8.9	\$1,213	\$3,410
Totals per patient:			\$102,552		\$22,610	\$79,942

### Results

The U.S. National Institutes of Health (NIH) estimates that at least 500,000 cases of PI remain undiagnosed in the United States.

The economic impact of undiagnosed PI to the healthcare system in the United States totals over \$40 billion annually.

<sup>&</sup>lt;sup>1</sup> Immunologic Research. pp. 43-47, Volume 38, Numbers 1-3, July 2007. Totowa, NJ: Humana Press.

<sup>&</sup>lt;sup>2</sup> Hospital charges and length-of-stay data were obtained from Hospital Cost and Utilization Project (HCUP), Nationwide Inpatient Sample (NIS), under the auspices of the Agency for Healthcare Research and Quality (AHRQ). Hospital counting reports from the Centers for Medicare and Medicaid Services (CMS) supplied additional information.

The study assumed minimum frequency of adverse events, such as recurring infections and multiple hospitalizations.

#### **Industry Leaders**



### In this issue, meet Larry Guiheen, Baxter BioPharm President

### In the beginning...

The 10 Warning Signs campaign was one of the first signs that Baxter and the Jeffrey Modell Foundation (JMF) were going to have a long and successful partnership together. That was the first major awareness and early diagnosis initiative we worked on with Fred and Vicki. Fred had approached the Plasma Protein Therapeutics Association (PPTA) for support to launch the JMF's awareness campaign nationally. Baxter led the way in pledging support, and helped influence others in the industry to join the effort. It was a big victory since the Centers for Disease Control and Prevention (CDC) was matching the contributions. Fred had succeeded in bringing patients, industry and the government together to tackle what was clearly a major problem, lack of Primary Immunodeficiency (PI) awareness and a significant delay in diagnosis.

Ever since that time it has been a huge honor for me, and many people at Baxter to be involved with Fred and Vicki, and all the wonderful initiatives that JMF drives. We support their mission and respect their willingness to work with Baxter, and the plasma industry as a whole. They do things that make sense for the community and the country, and we're proud to be a part of that.

For example, Baxter and JMF are long-standing partners in the effort to raise awareness and increase diagnosis of PI globally. Throughout the years we've supported the JMF's newborn screening program and its highly successful efforts to create awareness, resulting in earlier diagnosis and treatment. **Even today, it takes an average of nine years for many patients to receive an accurate diagnosis.** When patients finally get diagnosed, it can make such a big impact on their ability to work, go to school and to enjoy life. To us, these are all

**the right reasons to help.** I'm saying this not only as an executive, but more importantly, as a father who understands how precious it is to have a healthy, thriving child.

# Supporting the PI community today...

# SPONSORSHIP OF JMF DIAGNOSTIC CENTERS

When Fred and Vicki first told us about their plan to provide the regional resources communities need to educate healthcare professionals and get patients diagnosed earlier through the opening of JMF Diagnostic Centers, we immediately pledged Baxter's support. **Baxter is a sponsor of nine JMF Diagnostic Centers at prestigious medical institutions around the world.** 

# PROVIDING RESOURCES FOR PATIENTS AND FAMILIES

Through the years many people at Baxter have had the opportunity to work with the PI community and meet patients and families at JMF events. In fact, Pete O'Malley, a Vice President in Baxter's BioScience division, was at the very first JMF Kids' Day in Central Park. Our encounters with patients and families always bring great insight into the programs and resources Baxter can provide.

That's how Charlie and Emma's books came about. Our product manager for Gammagard, David Bond, met Dayna, the mom of three children with PI, at a conference. They got to talking and as a result, Baxter became aware of a book that Dayna wrote for her son, Charlie. Dayna's goal was to help eliminate some of the anxiety Charlie was having about going for his IVIG infusions. Baxter supported Dayna and Charlie's wish to publish the book and share it with other families.

Another PI patient, Emma, approached Baxter about publishing her book, The ABCs of Kids Like Me. Emma used each letter of the alphabet to create an inspirational tale about the courage and determination needed to succeed in managing life with PI. Both books are available free of charge on Baxter's ImmuneDisease.com website.

# WORKING WITH POLICYMAKERS TO ASSURE ACCESS AND CHOICE

In my almost 30 years at Baxter, I'd have to say that one of the most rewarding parts of my job is the advocacy work we do with patients in Washington D.C. Patients need access to all brands at all points of care. We work with the JMF and other patient organizations, government agencies and members of Congress to create awareness about the insurance and reimbursement needs of patients and healthcare professionals. Our mutual end goal is to be sure PI patients can access the IVIG they need, regardless of brand or site of care.

When I go to Washington, it's easy for me to speak passionately, because we have met so many PI patients, and I personally know the hurdles they've had to overcome.

For example, JoJo was 13 years old when we first met her. She was a state-ranked soccer player and honor roll student. But from the time JoJo was born she was in and out of the hospital constantly with pneumonia and severe sinus infections. JoJo was finally diagnosed with CVID and prescribed Gammagard. Now, at age 17, she is looking forward to entering a pre-med program and becoming a trauma surgeon.

# Special programs for special patients...

Addressing the needs of special patient populations, such as those requiring a low IgA IGIV therapy, is important to us too. In fact, we are the only manufacturer who offers an IVIG therapy with IgA levels less than 1mcg/mL in a 5% solution. That was important for Evin, a young girl I met many years back. Throughout her childhood Evin was often ill with various infections. After months of severe reactions to her original treatment regime she was offered Gammagard S/D. She tolerated it well and regained her zest for life. Evin has kept in touch with us over the years as she went to college, pursued a career and got married. It means a lot to us that she considers Baxter part of her extended family.

#### **COMMITMENT INTO THE FUTURE**

I think what I am most proud of is Baxter's history of providing Gammagard to patients who depend on it. In fact, Baxter is the only name that has remained constant in the IGIV market over the last 20 years and we're going to continue to be here for patients into the future.

As a market leader, Baxter has always taken its responsibility of consistently providing the highest quality IVIG to our patients very seriously. First we introduced Gammagard, then Gammagard S/D and then Gammagard Liquid. And we're dedicated to continually advancing the science of IVIG. We're investigating other opportunities, both in SubQ administration and other novel ways of delivery of this product. Baxter continues to look at formulations, packaging and enhancements that would make IVIG even more pure and utilization, even more convenient.

The PI community can continue to expect great things from Baxter. Fred and Vicki's dedication and seemingly endless energy to make strides in research and awareness has inspired us to pursue our own excellence in meeting the needs of PI patients and families. We look forward to many more victories we can accomplish together.

Larry Guiheen is the president of Baxter Healthcare Corporation's BioScience BioPharmaceuticals business. In this position, he is responsible for setting the strategic direction for Baxter in the North American region including sales, marketing, manufacturing expansion and new product requirements. Larry joined Baxter in 1978 and through the years he has held various senior management positions in sales, marketing and operations. In 1997, Larry became president of Hyland Immuno U.S., which is now known as Baxter BioScience.



# " We're Hearing from You"

#### Dana in Sedalia, Missowri:

"Your website and radio ad, truly saved us. I didn't know what was going on with our little one and finally we have an answer. I had never heard of PI so I took the 10 warning signs to our pediatrician and our first pediatrician told me it was so rare that most children die and therefore our child could not have it. Thank you for airing the radio ads and having the website available. Grace does have PI and I could have never found out if it wasn't for info4pi.org. Thanks so much. We appreciate you so much."

# Mandy in Milwankee, Wisconsin:

"Thanks for all the help and generosity of the Jeffrey Modell Foundation to make our first KIDS day a success at Miller Park! The Foundation gave an evening to all the families to have fun and forget just what it was that brought the group together in the first place."

### Cara in Plymouth, Wisconsin:

"I wanted to let you know that the Jeffrey Modell Foundation helped our small group from Milwaukee, Wisconsin take our kids with PI to a baseball game of the Milwaukee Brewers and we had a wonderful time. Keep up the good work with your organization!"

#### Marisol in Thiensville, Wisconsin:

"I want to thank you for trying to reach out to the community, especially the Spanish speaking community, because that is how I found out about PI. I believe it to be the answer to my child's illness. He never gets rid of the cough and the cold. Thank you so much and I hope I find the answer and that you keep up the great work."

### Tim in Minneapolis, Minnesota:

"I first heard of PI from the radio and then saw a billboard. I mentioned PI as a possibility as to why my son was ill all of the time. Doctors continued to tell me that my son was still "thriving" so he couldn't have PI, or any immune problems. Finally, the pediatrician tested his Ig levels and we were immediately sent to see an Immunologist who said to my son, 'You are lucky to be alive!" and "...I can't believe you don't have lung damage or hearing loss." He is 16 years old.

### Terry in Houston, Texas:

"I wanted to thank you for working with us to make the Houston JMF K.I.D.'s Day such an enjoyable and exciting event for all of our patients and families who attended. You have been so helpful and such a great resource in all of our planning. Thanks once again for making this wonderful K.I.D.'s Day at the Astros game a reality."

### Winnie in New Hope, Pennsylvania.

"I've been researching for years to try to help my kids stay well and I found your site along the way. Unfortunately, we are still in the dark ages of immune deficiency. It's still not on the doctors' radar. I know it takes time for change and your PSA, print and radio ads will certainly help. I think there's little emphasis in today's medicine on recurrent infections, especially if they seem benign. I have hope in doctors again. Thank you Jeffrey Modell Foundation. Keep at it, spread the word, educate the doctors. Keep the faith and hope alive."

### Anne in Fazetteville, Arkansas:

"I just received my first Jeffrey Modell 'Update' and wanted to thank you for all the wonderful work you have done for those of us with immune system diseases. I am so glad that there are people like you who are out there educating not only the public, but the medical community. My heartfelt thanks for all that you have accomplished and for giving us hope for the future."

### Rosemarie in London, England:

"We held our Nurse Study Day yesterday and I would like to thank WIN once again for its support. It was found very valuable by the nurse delegates who attended and all were returning to share the knowledge with their colleagues. At the end of quite an intensive day of sessions, evaluation sheets were completed that both indicated what sessions were most helpful and containing suggestions on how to further work to raise awareness amongst medical professionals. Once again, thank you so much to WIN for the support to make the day possible"

### JMF Recieves 4 Stars... 4th Year in a Row

Once again, the Jeffrey Modell Foundation has received the highest 4-star rating from Charity Navigator, America's largest independent evaluator of charities. This on-line service applies data-driven analysis to the charitable sector, evaluating the financial health of over 4,000 charities.

In a letter to the Foundation from Trent Stamp, Executive Director, "Charity Navigator salutes JMF's commitment to prudent fiscal management and congratulates the JMF on receiving our highest 4-star rating. A 4-star rating from Charity Navigator gives your supporters confidence in JMF's ability to maximize its charitable return on its donations and demonstrates that it is worthy of the public's trust. And this exceptional 4-star rating means that the JMF is able to efficiently manage its funding, exceed industry standards and outperform most charities in its cause."

Charity Navigator evaluated two broad areas of financial health: Organizational Efficiency and Organizational Capacity. These two areas combine to contribute to the overall rating. Fundraising efficiency, administration, program expenses, and working capital ratio all received the exceptional 4-star rating.

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# JMF Dedicates 4 New Centers

# of Excellence









New Initiative in Brazil will be 36th

Four new Jeffrey Modell Diagnostic Centers have recently

been dedicated in New Orleans, Seattle, San Francisco, and

Oxford, England. Currently, plans are underway to establish

the first new initiative in South America, in São Paulo, Brazil.

98% annual increase in diagnosed patients

492% annual increase in diagnostic tests

65% annual increase in patients receiving IVIG

The most recent survey of the existing Jeffrey Modell

Diagnostic and Referral Centers reports:

79% annual increase in referrals

**Center Worldwide** 

# **52** Patient Organizations "WIN" Grant Awards



"WIN," the World Immunodeficiency Network established by JMF, supports over 65 patient groups in 50 countries around the world. An esteemed Advisory Board reviews all grant applications, and monetary awards are made within 30 days of approval. WIN is supported by JMF, multiple U.S. government agencies, and global pharmaceutical corporations.

WIN has awarded grants to patient organizations worldwide, providing them with financial and technical support to enhance physician education and public awareness within their communities.



# SYMPOSIUM

### on PRIMARY IMMUNODEFICIENCY DISEASES





JMF was honored to have been selected once again to organize the World Health Organization / IUIS Expert meeting. Previously, JMF organized these high-science events in England, Austria, Switzerland, Portugal and Hungary. This year's meeting in Jackson Hole, Wyoming was a collaboration of the World Health Organization and the International Union of Immunological Societies (IUIS). The three day meeting addressed molecular biology and the genetics of Primary Immunodeficiency Disorders. Subjects included innate immunity, T and B cell deficiencies, disorders of immune regulation, genetic instability and cancer.

The prestigious meeting drew expert immunologists from 21 countries including Australia, China, Iran, Israel and Japan.



































Smiles on kid's faces and relaxed

smiles on kid's faces and relaxed

pavents, the kid's day at the 200

was an ungualified success.

was an ungualified success.

we cannot thank you enough

for your generosity.

Mel Berger

# **Program expands to 22 regions in the United States**

JMF "KIDS DAYS" began in 1988 and were created to provide a joyous occasion for Primary Immunodeficient patients, their families, doctors and nurses. It has become a valuable opportunity for patients to meet with one another, share personal experiences and form supportive, long lasting friendships. JMF has helped organize more than 60 "KIDS DAYS" across America and many others in Europe, Middle East, Asia and Latin America. Plans are already underway for 22 regional "KIDS DAYS" in 2008.









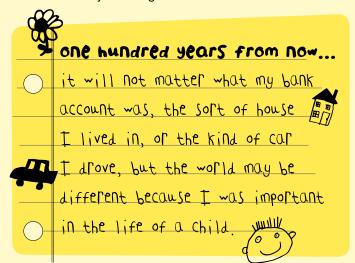
### To Becky Wilson's Fifth Grade Class,

On behalf of the Jeffrey Modell Foundation, we would like to express how thankful, surprised, happy and honored we were to receive your donation of \$1,354 from the "Pay It Forward" program at the Village Green Elementary School. There are no words to completely describe our gratitude and appreciation, so please accept a simple, yet heartfelt, thank you.

We are so proud of all of you for expressing your kindness, working hard to achieve your goals and caring so deeply about other children like your "class buddy", Tanner, who has to cope with and deal with health problems each day. Each and every one of you is special—and we promise you that we will do something very special with your hard earned money.

We have asked Tanner's doctor to make a "wish," and we will grant his wish with your donation and in honor of the Village Green Elementary School Fifth Grade Class. It will be used for a special research project to improve the lives of children with Primary Immune Deficiency and to buy some game boys and DVD's that the children can use while they receive treatment.

What a beautiful gift—what a difference you will make in so many precious lives. We will end with our favorite quote and one that is very meaningful:



With Thanks & Love,

Vicki & Fred Modell
Jeffrey Modell Foundation

