The Jeffrey Modell Foundation will be celebrating its 25th year in 2011 and will host a series of events, including a World Immunology Conference focused on Primary Immunodeficiencies. The four day symposium - “A Global Get-Together”, will take place June 1st – June 5th at Rockefeller University in New York City. The Keynote Address will be delivered by Nobel Laureate, Dr. David Baltimore. Dr. Baltimore won the Nobel Prize in “Physiology and Medicine” in 1975 for his discoveries concerning the interaction between tumor viruses and the genetic material of the cell. He is President Emeritus of the California Institute of Technology. This conference will bring together more than 300 researchers, scientists, and physicians from 55 countries to further education, expand dialogue, and inspire young scientists to elevate their commitment and dedication to the field of Primary Immunodeficiencies. This scientific event will be Webcast in real-time to physicians throughout the world, who will have an opportunity for interaction with the speakers.

On Thursday evening, June 2nd, JMF will celebrate “25 Years and Still Reaching for the Stars” with a festive and fabulous “Spring Ahead” Birthday Gala, at the American Museum of Natural History, a spectacular New York City landmark.

The JMF “Man of the Year” Award will be presented to David Levinson, Chairman and Chief Executive Officer, L&L Holding Company, a privately owned real estate investment and development company in New York City. He is a longtime, enthusiastic, and generous supporter of the Jeffrey Modell Foundation.
As we begin the Foundation’s 25th year, we are still somewhat perplexed as to how in the world did all of this happen. We are never able to clearly answer that question, so a few weeks ago we dusted off some old files and found the Minutes of our very first Board Meeting. Allow us to share with you the opening remarks expressed 25 years ago:

“Jeffrey Modell died a little more than one year ago of a Primary Immunodeficiency Disease. In the brief span of his 15 years he never knew what was wrong. There was no name for his condition. He just became ill more often than others. There were long periods when he was at school, but too many times when he was not. Each and every day was filled with uncertainty and anxiety. He never met one other child with his condition. We never met one other family struggling with this disease.

Over the years, we were told by his doctors that there was little research on this condition. There was virtually no funding. There were no known cures. There were no effective treatments. There was little hope.

Jeffrey pleaded with his doctors and his family to do something. There had to be an answer somewhere in the world, in some laboratory, in the literature, or at the NIH that might give us some insight as to what we should do. But there were no answers, and regrettably one of those episodes of illness was too much for him to fight off, and it took Jeffrey’s life last January, at the age of 15.

Jeffrey asked for answers and there were none. Jeffrey asked us to do something; and that is why we have created this Foundation, to bring nearer the day when none of our children or grandchildren need suffer as he did.

The question is: can we do something? Will there be advances one day? Will we ever see a treatment that can make a difference in a child’s quality of life for those that follow Jeffrey? Those answers won’t come easy. It will take immense resources, your time, your support and your commitment, but hopefully one day, far off in the future, the rewards will come. If this Board is ready to go forward, let us begin.”

That was 25 years ago and none of us could have imagined the advances in research, diagnosis, and treatment that would follow. No one could have foreseen that we would celebrate the fulfillment of seemingly impossible dreams.

We began with one Center. Now the Jeffrey Modell Centers Network consists of 453 Expert Physicians at 190 Academic Teaching Hospitals, serving 165 Cities, in 55 countries, spanning 6 continents. Our funded Jeffrey Modell Diagnostic and Research Centers will reach more than 100 before the end of the year. Indeed, this years’ World Immunology Conference - “A Global Get-Together” in June 2011 will reach more than 300 researchers, scientists, and physicians from 55 countries. The Public Service Advertising Campaign has increased awareness and education reaching over $130 million in donated media generating a 33% annual growth rate in number of patients referred to our Centers. Our KID’s Days for families and patients reached more than 20 cities this past year. The World Immunodeficiency Network (WIN) has provided grants to over 100 patient organizations around the world. There are four Jeffrey Modell Endowed Chairs in Pediatric Immunology Research at major teaching hospitals and medical schools in the US. JMF has funded more than 25 post doctoral fellowships and recently initiated the first population based Newborn Screening Program for SCID. Our research support has generated remarkable results! There have been 44 new genes discovered at Jeffrey Modell Centers in the past four years. We continue to testify before the United States Congress and have recently advocated at the EU Parliament in support of increasing research, public awareness, and physician education for Primary Immunodeficiency.

No one will doubt that these accomplishments became a reality-only because of the innovation, commitment, and support of all of you. If ever a small group of people could bring their compassion, their determination, and their selfless commitment to change the world, it is you! You are the rewards that we only dreamed of 25 years ago. You deserve our appreciation, our admiration, and our respect. We can assure you that you have the profound thanks of patients from all over the world, whose lives you have forever changed and even saved...

With hope for our Cause,

Vicki and Fred
Kathleen Sebelius, Secretary of the U.S. Health and Human Services, recently acted to add screening of Severe Combined Immunodeficiency Disease (SCID) to the core panel for universal screening of all newborns in the United States. SCID is the first new disease to be added to the Uniform Core Screening Panel. The states of Wisconsin, Massachusetts, and California have already begun to screen every newborn baby for SCID, and 17 states are now preparing to implement screening programs. JMF has championed this cause for more than 10 years.

Following up on Secretary Sebelius’ decision, JMF has been in contact with the Governors and Public Health Departments in all 50 States.

First baby in the World Screened and Cured of Combined Immune Deficiency

JMF Initiative in Wisconsin cures Baby of a fatal disease

In 2008 JMF collaborated and funded the first Newborn Screening Program for SCID in Wisconsin. As a result of that initiative Dawson Bornheimer was cured of a fatal disease.

A Note From Missy Bornheimer
(Dawson’s Mother)

“Everyday is a celebration of life for our family. Vicki and Fred have done so much to ensure the health of so many other little boys and girls around the world. What this newborn screening has done for our family is so incredible and to think that other states are starting to test now is such a great accomplishment for the Jeffrey Modell Foundation. It is so very exciting that many more families will get to experience the joy that we celebrate everyday by having a healthy child!”

DID YOU KNOW?

• When detected in the first few months of life, SCID can be cured with a bone marrow transplant.
• To date there is a better than 95% success rate and these babies can grow up to live normal and healthy lives.
• Late diagnosis of SCID costs an average of $2.2 million, according to the Wisconsin Department of Public Health and Children’s Hospital of Wisconsin.
• Early diagnosis & treatment, in the first 3 months of life, ranges in cost from $10,000 to $100,000.
• The cost to screen using the TREC’s assay is $4-$5 per baby.
• The results – priceless.
MEETING INDUSTRY LEADERS

Meet Peter Turner,
President of CSL Behring
A World Leader in Plasma Therapy

Peter Turner wasn’t always president of a global company that makes lifesaving plasma-derived and recombinant therapies. First and foremost, he was and still is a plasma therapeutics expert at heart whose knowledge encompasses plasma fractionation, R&D, production and engineering.

Since earning Bachelor of Science degrees in chemistry, biochemistry and microbiology and an MBA in his native Australia, the president of CSL Behring, headquartered in King of Prussia, PA, has compiled more than 40 years of experience in the biotherapeutics industry. Peter served on the Board of Directors of the Plasma Protein Therapeutics Association for 10 years including four as Chairman.

Recently IMMUNENEWS asked Peter what drives his passion for plasma protein biotherapeutics. His perspectives on people and health care are revealing and refreshing. CSL Behring makes innovative plasma-derived and recombinant therapies that treat rare and serious diseases including Primary Immunodeficiency (PI). Over the years Peter has had the opportunity to meet people from all over the world and see, firsthand, the difference these life-saving therapies or the lack of them can have. One such story involves a young girl who was in a sports competition for student-athletes with rare diseases.

“I was introduced to her and her mother,” Peter recalls, “She’d been treated for one disease but still wasn’t doing well.” As told to Peter, it turned out the youngster also had undiagnosed PI. “Once the correct diagnosis was made and she was placed on immunoglobulin (Ig) therapy, she did really well.”

A CULTURE OF COMPASSION

“It’s very inspiring,” Peter says, “to meet the patients who use our products and their families. If we ever need a reminder of the importance of what we do, it’s in the faces of the people we help with our medicines. The case of this girl was especially rewarding because she and her mother were quite thankful her condition was not only diagnosed, but that we could provide products to make her healthy.”

“Working with serious, and in many cases unmet medical needs, our mantra has always been to save lives and enhance the well-being of patients,” Peter says of CSL Behring, which traces its beginnings to the 1904 founding of Behringwerke, a company established by Emil von Behring. (Dr. von Behring was the first recipient of the Nobel Prize in Medicine and Physiology.) “That’s where we’ve come from,” Peter continues. “That’s what we know.”

LEADING A NORMAL LIFE

In Peter Turner’s view, it’s all about the choices in treatment that are available to patients. “We try to provide options for people who have PI,” he says. “One of those choices is subcutaneous infusion, which we believe is much more suited to PI therapy and self-infusion, particularly for those people who have time constraints or are located a long way from clinics.”

CSL Behring was the first company to bring subcutaneous Ig products to market. These self-infused medicines give people with PI a much greater degree of independence. “We believe that it’s essential to continue to provide similar advances so that patients can get as close as possible to leading a normal life.”
INNOVATION DRIVES THE BUSINESS

Innovation has long been one of CSL Behring’s core values and a driving force behind the development of its medicines. The company continually invests in emerging technologies to develop new products and improve existing therapies.

As an example, Peter points to the innovative technologies in Ig manufacturing the company introduced in its Center for Excellence for Ig in Bern, Switzerland, that increase the yield of Ig per liter of plasma. Increasing the company’s capacity to make more Ig, ensures reliability of supply and gives patients greater access to important therapies. CSL Behring received the Swiss government’s Tell Award for “significant innovative technology” in the Bern facility for this leading-edge technology and therapy.

PARTNERING WITH PATIENT GROUPS

CSL Behring partners with a number of patient organizations, such as the Jeffrey Modell Foundation (JMF), advocating for access to health care and working together to achieve better results. “The Jeffrey Modell Foundation is remarkable,” Peter observes. “What Vicki and Fred Modell have been able to achieve is just staggering to me. It shows what can be done if you have passion and believe in what you’re doing. They’ve been tireless advocates for testing and diagnosis of children and adults with persistent serious infections, and have done a tremendous job creating awareness of PI and attracting the interest of scientists.”

CSL Behring has shown its support by providing funding for Jeffrey Modell Diagnostic and Research Centers around the world, among other corporate responsibility initiatives.

PATIENTS ADVOCATE FOR CHANGE

Beyond the actual therapies manufactured by the company, Peter says CSL Behring helps people share in the healthcare needs debate through programs such as Raise Your Voice! and Local Empowerment for Advocacy Development (LEAD) grants, which help patients advocate for healthcare policy change. “We support patients and their families in making positive changes in their communities.” CSL Behring’s Voice-to-Voice program features Ig patients who mentor new patients in how to correctly use the therapy, which helps them achieve greater freedom more quickly.

“CSL Behring provides reimbursement services to ensure that people do not get lost in the myriad of demands that are placed on them by the healthcare system,” Peter says. “For patients with serious medical conditions, getting diagnosed, accessing therapies and returning to normal life is a formidable challenge. It’s bad enough you don’t feel well. Dealing with bureaucracy and all the people involved in providing your therapy is an added challenge for many people.”

PRODUCT OUTPUT DOUBLES

Peter is proud of how CSL Behring has developed in the last five or so years. During that time the company has more than doubled its product output. “We have some exciting products in development that I believe our customers will be thrilled about should they be successful.” Peter emphasizes that CSL Behring’s products are driven by its customers. “We listen to patients,” he says.

It’s very inspiring to meet the patients who use our products and their families. If we ever need a reminder of the importance of what we do, it’s in the faces of the people we help with our medicines. Our mantra has always been to save lives and enhance the well-being of patients. We listen to patients.”

Fred Modell with Peter Turner

CSL Behring Plasma Fractionation Facility
SPIRIT...Software for Primary Immunodeficiency Recognition Intervention and Tracking, is now being utilized in the marketplace. The Jeffrey Modell Foundation developed the SPIRIT Analyzer to be used by managed care plans across the country. The new diagnostic tool identifies undiagnosed patients with Primary Immunodeficiencies (PI).

**How Does SPIRIT Work?**

A list of more than 350 weighted ICD 9 codes are matched to the 10 Warning Signs of PI to establish low, moderate, and high risk categories. Patients that score moderate and high risk are flagged as potentially in need of further testing for PI.

SPIRIT can analyze 1,000,000 pharmacy and medical claims in approximately 30 minutes. Health plans are able to automatically alert the physicians of those patients with recurring infections that are high and moderate risk, encourage appropriate assessment, thereby improving patient outcomes and saving enormous health care costs.

**SPIRIT Analyzer Reaches Managed Care Executives**

Recently, JMF was honored to present the SPIRIT software to the Managed Care Network Executives, at their annual meeting, in Philadelphia, PA. MCN includes more than 100 senior executives and medical/pharmacy directors who are the decision makers and thought leaders at regional and national health plans. Together, they represent nearly 200 million covered lives.

**MCN Senior Executives Were Surveyed**

- **81%** agreed or strongly agreed that “Primary Immunodeficiency is a concern that should be monitored in my health plan”.

- **75%** agreed or strongly agreed that “Early identification of Primary Immunodeficiencies can improve patient’s quality of life and reduce health care costs.”

- **66%** agreed or strongly agreed that “JMF’s SPIRIT Analyzer represents an excellent opportunity to screen for undiagnosed patients in my health plan.”
European Union Parliament Endorses JMF’s “Call to Action”

121 Members of Parliament Sign Document Calling for Early Diagnosis and Access to Treatment

This past Spring, the Jeffrey Modell Foundation launched a European initiative, “Driving Diagnosis for Optimal Care” and a “Call to Action” at the EU Parliament. The objective of the initiative is threefold: to raise public awareness of Primary Immunodeficiencies, to attain earliest possible diagnosis, and to advocate for optimal and equal access to care for patients throughout Europe.

To launch the Campaign, JMF hosted an event in Brussels, on April 28th, at the European Parliament, where Members of Parliament (MEP’s), the European Commission, and European medical and patient community representatives, signed and endorsed the Primary Immunodeficiencies “Call to Action”. Members of the European Parliament met with their respective Jeffrey Modell Center Directors and patients to learn about the important work being carried out by the Centers, to appropriately diagnose young children and adults living with these devastating conditions. These key milestones mark the beginning of an important European initiative, and will enhance further relationships at the global, EU, and national Member State levels. There are more than 121 signatories to date.
Looking Back at 25 Years
years of “Spring Ahead” Galas

Thursday, June 2nd, 2011
at the
Museum of Natural History
New York City

SAVE THE DATE

“Man of the Year”
David W. Levinson
Chairman & Chief Executive Officer
L&L Holding Company
Jeffrey Modell Centers Network (JMCN)  
Growing 26% Annually

Networking...
The Jeffrey Modell Centers Network now consists of 76 funded Jeffrey Modell Diagnostic and Research Centers. The Referral Network consists of 453 Expert Immunologists at 190 academic teaching hospitals and medical schools in 191 cities, 55 countries, spanning 6 continents.

Annual Growth

33% Annual Increase in Number of Patients Referred
33% Annual Increase in Number of Patients Followed
25% Annual Increase in Number of Patients Identified with a Specific Disorder
25% Annual Increase in Number of Patients Receiving Treatment

36% Annual Increase in Number of Patients Receiving Immunoglobulins
26% Annual Increase in Number of Jeffrey Modell Centers
71% Annual Increase in Number of Patient Tests Conducted

JMF Supports
Pediatrics Congress in South Africa

The Jeffrey Modell Foundation proudly provided support for a Symposium at the annual Pediatric Association Congress of Pediatrics in Johannesburg, South Africa in August 2010, entitled “Primary Immunodeficiency Diseases in Resource-Limited Settings”. Expert immunologists from the continents of South America, Asia, and Africa presented topics concerning physician awareness, diagnosis, and treatment options in countries with limited resources. More than 100 people, mostly African physicians, attended this symposium and discussions led to the creation of the “Primary Immunodeficiencies in Resource-Limited Settings Group”, which will link the various continental societies together to promote and publish simple and effective means for awareness and clinical care for PI.

Seated from L-R: A. Alghonaim, Saudi Arabia; Y.L. Lau, Hong Kong; Dr. Brian Eley, South Africa; M.R. Barbouche, Tunisia; A. Condino-Neto, Brazil.
Standing from L-R: A. Davidson; Monika Eser, South Africa; Aziz Bousfiha, Morocco; Shen-Ying Zhang, Rockefeller University; D. Moore; Brian Eley, South Africa.

Chairperson Aziz Bousfiha, Morocco

“I am sure that this will be a landmark for Africa, and that the Educational Programs for African countries will grow exponentially, and patient registries will soon follow.”
- Dr. Antonio Condino (Brazil)
Recent Jeffrey Modell Center Dedications

London

St. Louis

Rotterdam

Freiburg

Leipzig

Tampa / St. Pete

Mexico City

Warsaw

Rome

Medellín
The Jeffrey Modell Foundation KID’s Days Program offers a welcome opportunity for patients and their families to form friendships with one another and to get to know their physicians and nurses in a casual environment. KID’s Days have reached many cities throughout the U.S. and are held at a range of exciting locations, including: amusement parks (Atlanta, Dallas, Los Angeles, Miami); Baseball Games (Buffalo, Milwaukee, Philadelphia, Seattle, Tampa Bay); Zoos (Chicago, Cincinnati, Cleveland, Omaha); Children’s Museums (Houston), and Bowling Alleys (New Orleans, New York); just to name a few. The children enjoy face painting, balloons, magicians, clowns, and other fun activities...not to mention lots of fun food!

Sponsors include Baxter, BioRx, Biotest, CSL Behring, Futures & Options for Kids, Grifols, Octapharma, and Talecris. The Jeffrey Modell Foundation, the doctors, and all the “Kids” greatly appreciate the ongoing kindness and generosity of these donors.

“Thank you for the lovely day at the Mariners game. It was my son’s first ballgame and he loved it. Thank you again for all the work you do around the world. The KID’s Day program truly makes a difference in the lives of children and families with PI.”

- A mother of a child with PI from, Seattle, WA
WIN Awards Grants to More than 100 Patient Groups Worldwide

The World Immunodeficiency Network (WIN) has awarded grants to patient organizations around the globe. This year, WIN grants have been awarded to: Germany, Brazil, Iceland, Poland, Serbia, England, Canada, Morocco, Mexico, Arizona, North Carolina, Sacramento, Argentina...just to name a few! WIN Grants fund events like Kid’s Days, camp weekends, medical conferences, printing and distribution of educational materials, as well as many other activities in various cultural and ethnic communities. There was a 34% increase in the past year.

To date, WIN has awarded $500,000, which has increased public awareness and physician education around the world!

The Netherlands, Iceland, Germany, Poland, Serbia, England, Brazil

Morocco, France, USA, Canada, United Kingdom, Argentina, Shanghai
JMF and NIH Communicate with Every Pediatrician Nationwide!

New Illustrated 10 Warning Signs Poster Alerts 46,000 Members of the American Academy of Pediatrics

The Jeffrey Modell Foundation, working in collaboration with the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) at the NIH, distributed the new illustrated 10 Warning Signs Poster to more than 46,000 pediatricians in the United States. This Pediatrician Public Awareness Campaign is supported by Director of the NICHD, Dr. Alan Guttmacher, whose personal letter accompanied the poster. The purpose of the Campaign is to alert physicians and other healthcare professionals to the 10 Warning Signs of PI, so that affected children and adolescents receive early diagnosis and appropriate treatment.

JMF Launches New PSA Campaign

When I Grow Up...

JMF created the first ever Public Service Advertising Campaign for Primary Immunodeficiencies. The Campaign reaches the general public through television, radio, print, Web, shopping malls, outdoor billboards, and airports. To date, the Campaign has generated over $130 million in donated media. The new PSA Campaign When I Grow Up is sure to push awareness even further! This National Public Awareness Program is supported by the U.S. Congress, the National Institutes of Health (NIH), and the U.S. Centers for Disease Control and Prevention (CDC).
Research Update from JMF

JMF Supports Highly Ranked NIH Research Grants
Two physician/scientists were awarded Fred Rosen/Jeffrey Modell Research Awards in 2009, totaling $500,000.

Harvard Medical School Research Prize
There were three recipients of the Jeffrey Modell Research Prize, awarded to young investigators at Harvard Medical School in a rigorous peer reviewed process.

Fellowship Program Expanded
In 2009, JMF awarded four Robert A. Good/Jeffrey Modell Fellowships. The Fellowships were granted to investigators in Paris, Zurich, Ulm, and Rockefeller University in New York City. Three additional Fellowships were provided to the Mount Sinai Medical Center in New York City.

Trough Level Study Progresses
JMF has piloted and funded a multicenter research initiative to determine optimum IVIG trough levels in patients receiving antibody replacement therapy.

New Discoveries
There have been 44 new genes discovered and defined at Jeffrey Modell Diagnostic, Research, and Referral Centers worldwide over the past four years. The interaction of investigators within the Jeffrey Modell Centers Network and the support of JMF has contributed significantly to these discoveries.

Dr. Ronald H. Laessig Memorial Newborn Screening Fellowship
The Jeffrey Modell Foundation is pleased to announce a new funding opportunity; the “Dr. Ronald H. Laessig Memorial Newborn Screening Fellowship.” This Fellowship is available to post doctoral candidates to conduct newborn screening research focused on Primary Immunodeficiencies.

The two-year fellowship program will give the awarded candidate an opportunity to attend professional meetings, publish research, and participate in a two-week bench training workshop at the CDC.

Dr. Laessig was Director of the Wisconsin Laboratory of Hygiene from 1980-2006. He initiated the nation’s first Newborn Screening program for SCID in the state of Wisconsin. Sadly, he passed away shortly after this ground-breaking and courageous initiative started.
The 12th annual Dina LaVigna “Breath of Life” Triathlon was held in Ventura, California on June 27th. The 1st Triathlon was held in 1999 and was created by the family of Dina LaVigna, a very special young woman who suffered from PI, and sadly lost her courageous battle. This year’s Triathlon had more than 950 registrants and raised more than $150,000. Since its inception, the “Dina LaVigna” Triathlon has raised more than $1.3 million. All proceeds are matched by the Jeffrey Modell Foundation, and are used to build and enhance infusion facilities in children’s hospitals, fulfilling Dina’s wish for brighter, happier places to receive treatments.

Connie and James Ramos, parents who are dedicated to raising awareness and enhancing research, organized and hosted the second annual Wiffle Ball Tournament on July 4th weekend, on St. Pete Beach in St. Petersburg, Florida to benefit All Children’s Hospital and the University of South Florida Department of Allergy and Immunology.

The event began with a festive Havana Nights party and continued into the next day, with the Wiffle Ball Tournament and a Beach Bash BBQ, accompanied by music, fun food, incredible memories, and exciting athletic competition.

The Wiffle Ball Tournament attracted 450 people, and raised more than $175,000. The Jeffrey Modell Foundation is proud to provide matching funds.

JMF congratulates the Ramos family, and all their friends and volunteers who worked hard to make this event not only possible, but very successful!

The Jeffrey Modell Foundation is proud to partner with Steve Bursley as he competes on his bicycle, to honor his son, Nicholas, and all the children who endure the challenges of PI each and every day. The cycling events cover nearly 1,000 miles and 100,000 feet of climbing.

“One Tough Ride” is a fund raising effort to raise money for research to find a cure for Primary Immunodeficiency. Steve will use the 2010 cycling season to ride in 6 of the toughest Ultra Endurance Cycling Events. All funds will benefit the Lucile Packard Children’s Hospital at Stanford.

The Jeffrey Modell Foundation is proud to congratulate “one tough guy” for his courage and commitment and we are pleased to announce that JMF will match all donations made on behalf of Steve’s “tough rides”.

Fundraising for PI at the “Grass Roots”