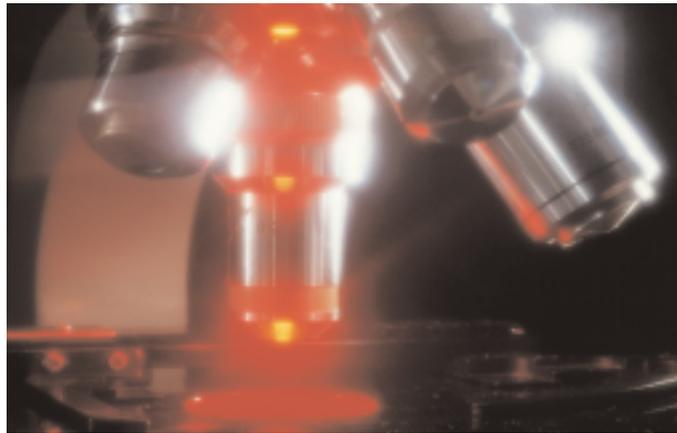


## Gene Therapy Successful in SCID Patients

**G**ene therapy has restored immune system function to two French babies born with the X-linked form of Severe Combined Immunodeficiency (SCID). As reported in the April 28, 2000 issue of the journal *Science*, this gene therapy experiment, conducted by Dr. Alain Fischer of the Hospital Necker-Enfants Malades in Paris, is the first of its kind to provide unequivocal evidence that gene therapy can succeed in this form of SCID.

The babies were born with defective immune systems that could not produce T-lymphocytes, immune cells critical for fighting infection. Without working T-cells, the babies could not fight off infection and could die from even minor infections. Previously, bone marrow transplants have been effective in curing the disease or extending the life of individuals. But because transplants are not always successful, scientists hope gene therapy will offer a viable alternative.

Dr. Fischer started the gene therapy by taking bone marrow from the patients and culling out the stem cells, which are special cells that reside inside the marrow and produce lymphocytes. Then the normal gene was added to the defective stem cells.



Lastly, the genetically corrected cells were transplanted back into the patients' bodies. Once the functioning genes enter the bone marrow cells, the corrected cells will grow rapidly and displace stem cells with the defective gene. Soon, the patients will produce their own germ-killing immune cells and survive without antibiotics or additional treatment.

Three months after treatment, the French babies returned home from the hospital, living like normal children. Today, they have levels of immune system cells comparable to healthy children their own age. It can't be called a complete cure until the children are much older, but the initial findings are extremely positive. This success comes at a crucial time because gene therapy has been harshly criticized over the past years.

The first gene therapy attempted on human patients took place nearly

ten years ago. Two SCID children, Ashi and Cindy, were treated by a team that included Drs. French Anderson, Michael Blaese and Ken Culver. Both girls are doing very well. Since then, however, over 390 gene therapy studies involving more than 4,000 patients have been conducted unsuccessfully. Skeptics have questioned whether gene therapy would ever cure anyone and if the risks were too high to continue studies. "With this resounding success, we can expect to see more gene therapy studies conducted on immune deficiency diseases and others," comments Jerry Winkelstein, MD, professor of pediatric immunology at Johns Hopkins School of Medicine and chairman of the IDF Medical Advisory Committee, "This is a very exciting advance and has positive implications for gene therapy of a number of different genetic diseases." ■

## FEATURED THIS ISSUE

### ■ OPERATION OUTREACH

Sponsored by Aventis Behring, kick-off events held in Minnesota, Maryland, North Carolina and Utah benefit nearly 200 patients and their families

### ■ LEBIEN VISITING PROFESSOR PROGRAM 2000

Combining Grand Rounds and Clinical Presentations, 6 Visiting Professorships are awarded and 2 more are scheduled, thanks to a generous educational grant from FFF Enterprises

### ■ IGIV LICENSURE FDA

revises its policies to expedite the licensure of new IGIV products

### ■ HHS ADVISORY COMMITTEE ON BLOOD SAFETY AND AVAILABILITY

IDF obtains voting membership on the highest governmental committee addressing blood issues



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# Nearly 200 Patients and Their Families Benefit from Operation Outreach

The Operation Outreach Program, sponsored by Aventis Behring, develops IDF local programs and a network of patient contacts in regions of the country currently without an IDF program. Since Fall 1999, the program has successfully launched local programs in four states and has additional programs planned for this summer. Nearly two hundred primary immune deficient patients and family members attended recent kick-off meetings in Minnesota, Maryland, North Carolina and Utah. The educational meetings featured leading clinical immunologists addressing

primary immune deficiency diseases, pharmacists speaking on intravenous immune globulin therapy, and insurance reimbursement specialists offering advice and resources for patients. Jennifer Bass, IDF Director of Patient Services, introduced participants to the programs and services of the Foundation.

Following the educational sessions, primary immune deficient patients, family members, and



health professionals had the opportunity to meet with each other over lunch and special activities. Meeting participants in Minnesota toured the Underwater World Aquarium; the Maryland group toured Oriole Park at Camden Yards. The North Carolina meeting was held at the Museum of

Life and Science and Magic Wings Butterfly House in Durham; and in Utah, patients had the opportunity to tour the spring gardens at Thanksgiving Point Institute located just outside of Salt Lake City. The following Operation Outreach meetings are planned for this summer:

**Saturday, June 24, 2000 - Point Pleasant, New Jersey**

**Saturday, July 22, 2000 - St. Louis, Missouri**

If you would like to attend the New Jersey or Missouri meeting or contact a local program in your region, please call Jennifer Bass at (800) 296-4433 or write [jb@primaryimmune.org](mailto:jb@primaryimmune.org). ■

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## LeBien Visiting Professorship Program

**T**he Immune Deficiency Foundation has received a generous educational grant from FFF Enterprises to sponsor the LeBien Visiting Professorship Program. The Professorship Program honors IDF Vice Chairman Bob LeBien for his continuing involvement and dedication to the IDF.

The purpose of the

Visiting Professorship is to foster improved knowledge about the diagnosis and treatment of patients with primary immunodeficiency diseases. Six Visiting Professorships have been awarded for the academic year 1999-2000. The Visiting Professorship takes the form of Grand Rounds and Clinical Presentations. Other relevant activities such

as physician and medical student conferences or patient and family conferences

may be included. The schedule of upcoming LeBien Visiting Professors is:

**October 16-17, 2000**

**Dr. Rebecca Buckley - Baystate Medical Center/Children's Hospital, Springfield, MA**

**November 14-16, 2000**

**Dr. Charlotte Cunningham-Rundles - MCP Hahnemann Hospital, Philadelphia, PA**

If you wish to attend a Visiting Professorship program, please call Tamara Brown, IDF Medical Programs Coordinator, at (800) 296-4433. ■

# Revised FDA Policies for New IGIV Licensure

**D**r. Basil Golding presented the U.S. Food and Drug Administration's revised clinical trial proposal for licensure of new IGIV products at the March 17, 2000 FDA Blood Product Advisory Committee (BPAC) meeting. By expediting the licensure of new IGIV products, the proposed policy will help alleviate the IGIV shortage, ongoing since Fall 1997.

Dr. Jay Epstein, Director of the FDA Office of Blood Research and Review, stated during the BPAC meeting that the FDA will accept infection rates established by the historical experience of untreated patients as the basis for control groups in new IGIV clinical trials. This will decrease the number of patients required to participate in trials by eliminating the need for a comparison IGIV product. Additionally, the proposed FDA policy will measure efficacy end-points based on the standard of care in clinical immunology, thereby reducing the burden of unnecessary diagnostic tests for patients participating in new IGIV trials.

E. Richard Stiehm, MD of the University of California, Los Angeles School of Medicine and chairman of the IDF Blood Safety and Availability Advisory Committee, said, "We are pleased that the FDA has agreed to simplify testing of new IGIV products. We believe that these proposed guidelines will increase the supply of IGIV into the U.S. market without compromising product safety. The chief advantage of the guidelines is that large numbers of patients need not be recruited every time a new brand of IGIV is introduced into the market."

IDF advocacy directed at resolving the IGIV shortage prompted numerous private and public meetings with the FDA and included public testimony focused on the need for increased IGIV supply. "Thanks in large part to the patient community, we were able to convince the FDA to revise their thinking on this issue," notes IDF President Tom Moran. "Patients actively participated in every step of the process." A major turning point on this issue was IDF's meeting with FDA Commissioner, Dr. Jane Henney. IDF presented Dr. Henney with a bound volume of over 300 letters which were received in a three-week period from patients and their families expressing concern about the ongoing IGIV shortage. ■

## Immune Deficient Patients Now Represented on HHS Advisory Committee

Jerry Winkelstein, MD, chairman of the IDF Medical Advisory Committee, will represent primary immune deficient patients on the U.S. Department of Health and Human Services (HHS) Advisory Committee on Blood Safety and Availability, also known as the Secretary's Advisory Committee. IDF has worked for three years to obtain membership on the Advisory Committee, the highest governmental committee focused on issues pertaining to blood.

The Committee meets three times per year to review public health concerns and make recommendations to the Secretary regarding blood safety and availability. With voting membership on the Advisory Committee, IDF has greatly advanced our opportunity to push forward the organization's initiatives in blood safety and availability. IDF is honored to have Dr. Winkelstein representing primary immunodeficient patients as a member of the Secretary's Advisory Committee.

## A New Address for IDF National Headquarters

Over the past year and a half, IDF has experienced a greatly increased level of demand for patient services, advocacy, and research-related activity, requiring the addition of staff and expansion of office space. In order to serve you better - and accommodate our phenomenal growth - we are pleased to announce the relocation of IDF National Headquarters to a larger suite across the street. Please note: while we have a new address, the phone and fax numbers remain the same.

**40 West Chesapeake Avenue, Suite 308**  
**Towson, MD 21204**  
**(800) 296-4433**  
**(410) 321-6647**  
**FAX (410) 321-9165**

# Unified Effect Results in IGIV Therapy Reimbursements

**O**n March 31, 2000, the Health Care Financing Administration (HCFA) ruled on the long anticipated Prospective Payment System for Hospital Outpatient Services. Under the new ruling, biologics such as IGIV will be reimbursed on a special provision (pass-through) equal to 95% of the average wholesale price. This ruling is cause for celebration, as 1997's Balanced Budget Act discontinued adequate reimbursement for biologics by bundling them into a single (non pass-through) reimbursement code (APC906) for cost containment. IDF was among those providing public comment on APC906, calling for HCFA to exempt IGIV from the bundled reimbursement. HCFA received more comments on this proposed rule than any to date.

These changes are the result of a unified effort by

IDF, other plasma consumers, physicians, and the biologics industry, which collaborated to tackle this issue. Additionally, the Advisory Committee on Blood Safety and Availability focused their agenda on this critical issue, prompting Secretary Shalala to take personal action. Thanks in part to John Walsh, President of the Alpha One Foundation, consumers were well represented during the Advisory Committee's discussions. Finally, the HCFA ruling has provisions for new technologies, giving hope to immune deficient patients that reimbursement will be available for alternative routes of administration when they become available.

According to IDF Chairman Marcia Boyle, "It is a pleasure to announce this hard won victory, fought over a three-year period, that sets a positive precedent for appropriate therapeutic reimbursement." ■

## NOTICE: Class Action Settlement for Gammagard(r) IGIV Therapy Hepatitis C Claims

Baxter Healthcare Corporation has agreed to a class action settlement for patients who may have contracted Hepatitis C through the use of Gammagard(r) IGIV. The legal settlement is designed to provide anyone who used Gammagard(r) between January 1, 1993 and February 24, 1994 the opportunity to receive a medical test to ascertain whether they have Hepatitis C, and then the opportunity for financial support for medical treatment as necessary.

The settlement makes an initial payment for Hepatitis C testing to all patients who received Gammagard(r) between January of 1993 and February of 1994. Those who test positive for Hepatitis C will receive further compensation. In the rare event that patients suffer any severe complications from the disease today or in the future, additional compensation for medical treatment will be provided. Patients who would like to inquire about class action participation should contact the settlement administrator at (888) 921-4476.

Change of Address Requested

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