Personalized Immune Mouse New Tool to Study Autoimmune Disease

Columbia University Medical Center scientists have developed a new “personalized immune mouse,” a new tool that allows them to recreate an individual’s immune system to study autoimmune diseases. The mouse model also could have clinical applications, such as predicting how a particular patient might respond to existing drugs or immunotherapies. And, it could prove useful for developing individualized immunotherapies for fighting infection or cancer or for lessening a patient’s rejection of transplanted tissue.

The mouse model is made by transplanting human bone marrow stem cells (also known as CD34+ cells), along with a small amount of HLA-matched immature thymus tissue, into an immunodeficient mouse. The thymus tissue is implanted into the mouse’s kidney capsule, a thin membrane that envelopes the kidney and serves as an incubator. Within six to eight weeks, the transplanted thymus tissue is seeded by circulating human CD34+ cells (which are infused into the mouse’s bloodstream), and begins generating human immune cells from the CD34+ cells.

While the researchers intend to use the personalized immune mouse to study type 1 diabetes, Dr. Megan Sykes, director for the Columbia Center for Translational Immunology, says that they “hope to find out what is fundamentally different about patients’ immune systems, compared with those of healthy individuals, before any disease develops.”

Correction
Gamunex CoPay Card for CIDP Patients Only

In the June-July issue of IG Living, we incorrectly reported that the Gamunex CoPay Card Program offered by Grifols is a coupon program that helps patients with common variable immune deficiency (CVID) cover the copay costs for Gamunex-C. However, at this time, the CoPay program is intended only for patients with chronic inflammatory demyelinating polyneuropathy (CIDP), not CVID. The program provides up to $2,500 per patient per 12-month period, and it is open to CIDP patients who are not using any state or federally funded healthcare program such as Medicare, Medicaid, Medicare Advantage and Tricare; who are not residing or receiving treatment in the state of Massachusetts; and who are not getting treatments in hospitals or hospital-associated outpatient clinics. For additional information, go to gamunexcard.com.

Medicines
IDF Urges FDA to Exempt IG from Biosimilars Pathways

At the U.S. Food and Drug Administration (FDA) public hearing on draft guidance of biosimilar products in May, the Immune Deficiency Foundation (IDF) urged the FDA to exempt immunoglobulin (IG) therapies from the biosimilars pathways in order to protect the safety of patients with primary immunodeficiency disease (PIDD). The FDA recognizes each IG product as unique with no generic equivalent because current science cannot demonstrate that two products will provide the exact same clinical results for a large number of patients or that switching patients from one product to another will pose no additional risks.

IDF President and Founder Marcia Boyle's testimony urged the FDA to follow the example set by the European Medicines Agency and exempt IG therapy from the biosimilars pathways, or to, at the least, require that biosimilar drug products undergo clinical trials to determine whether a proposed interchangeable therapy will offer patients the same clinical outcome. Additionally, Boyle requested that the FDA prohibit automatic substitution of a biosimilar drug with an original biologic. “We believe the FDA’s foremost responsibility is to ensure that biosimilars are manufactured and prescribed safely,” said Boyle. “All medicines must be thoroughly tested and meet the highest safety standards set by the FDA.”
Legislation

May 16 Was First HAE Awareness Day

The United States Senate unanimously passed a resolution in February recognizing May 16, 2012, as Hereditary Angioedema (HAE) Awareness Day. The resolution is the result of a year-long advocacy effort to generate recognition of the need for increased professional education regarding HAE, which is a rare and potentially fatal genetic disorder. The advocacy effort also highlights the need for further research aimed at improving diagnosis and treatment options for patients.

The goals of an annual HAE Awareness Day are to increase awareness of HAE among the general public and medical community; support better care and an earlier and more accurate diagnosis for HAE patients; raise funds for further national and international initiatives; and enhance the understanding that HAE patients can lead a healthy life. HAE Awareness Day helped launch the first HAE Global Conference held in Copenhagen, Denmark in May. Findings from the conference, to be held biannually, will be the impetus for additional HAE research.

“This first annual HAE Awareness Day will put a spotlight on HAE, its symptoms, and the impact this challenging disorder has on patients and their families,” said Janet Long, executive vice president of the Hereditary Angioedema Association. “We hope this national recognition will broaden awareness of HAE and prompt anyone who suffers from repeated bouts of swelling to seek appropriate diagnosis and treatment.”

Support for the public policy program of the Hereditary Angioedema Association, which encouraged the Senate to acknowledge the need for increased awareness and research, was provided by CSL Behring through the company’s Local Empowerment for Advocacy Development (LEAD) program. For more information about HAE Awareness Day, please visit www.haeday.org.

Research

National Psoriasis Foundation Awards $2 Million in Grants

Twenty-six scientists have received a total of $2.06 million in grants from the National Psoriasis Foundation to study psoriasis — the most common autoimmune disease in the country, affecting as many as 7.5 million Americans — and psoriatic arthritis, an inflammatory joint and tendon disease.

Eight scientists each received a one-year $50,000 Discovery Grant, for a total of $400,000, for pilot projects that have potential to lead to breakthroughs in the understanding of psoriatic diseases and to discover better treatments. The grants are also intended to lay the groundwork for additional, long-term funding from the National Institutes of Health and other funding agencies. Six scientists each received a two-year $200,000 Translational Grant, for a total of $1.2 million, for studies that aim to move laboratory and clinical discoveries into projects and treatments that benefit patients. And, 12 early-career dermatologists were awarded Psoriasis Foundation medical dermatology fellowships totaling $465,000. These fellowships provide support of up to $40,000 per year to new doctors training to do research in psoriasis and psoriatic arthritis. The fellowships are intended to increase the number of doctors studying psoriatic diseases.

“National Psoriasis Foundation is committed to funding promising research,” said Chip Newton of the National Psoriasis Foundation’s Scientific Advisory Committee. “This year, due to a record number of applicants, we awarded the highest number of grants and dollars in our organization’s history. Each of these projects has tremendous potential to advance our knowledge of psoriatic diseases, lead to new treatments and, we hope, even a cure for these diseases.”
Medicines

AAN Releases IVIG Guideline for Neurology

The American Academy of Neurology (AAN) has released a new evidence-based guideline on the efficacy of intravenous immunoglobulin (IVIG) — used to treat a range of immune-mediated neurological diseases — for neuromuscular disorders, based on a comprehensive review of the literature by the AAN Therapeutics and Technology Assessment Subcommittee in the 43-year period between 1966 and 2009. The guideline answers the following questions: What are the significant findings for treatment of neuromuscular disorders with IVIG? How would neurologists use these guidelines in practice? What are some of the side effects of the treatment? What are some of the alternate treatments, and how do they measure up? Why is the benefit from IVIG often short-lived? Where should further research be done? The review was published in the March 27 print issue of Neurology.

Disease Guidelines

New Guidelines Issued for Severe Lupus

The American College of Rheumatology has issued new guidelines for the screening and management of lupus nephritis (kidney inflammation). According to the guidelines, patients who have not received treatment for lupus nephritis who show signs of kidney involvement should get a kidney biopsy. If there is kidney involvement, patients should be given the drug hydroxychloroquine. And, if there is any sign of protein in the urine, patients should be prescribed blood pressure-lowering medications called ACE inhibitors or angiotensin-receptor blockers.

When diagnosed with lupus, one in three patients already has kidney inflammation, and during the first 10 years with the disease, as many as 60 percent of patients will have some kidney problems. “Without treatment, lupus nephritis can lead to end-stage renal disease, which requires dialysis or a kidney transplant. But, not all types are this serious. It depends on the pattern of damage to the kidneys,” says Dr. Bevra Hahn, lead author of the new guidelines and a professor of medicine and chief of rheumatology at the David Geffen School of Medicine at the University of California, Los Angeles.

The guidelines were released online May 3 and were published in the June 2012 issue of Arthritis Care & Research.

People and Places

A researcher at the University of Texas Health Science Center at Houston has been awarded a $1.9 million grant from the National Institutes of Health’s National Heart, Lung and Blood Institute to study a novel cell therapy that could help avoid autoimmune problems after stem cell transplantation, as well as potentially treat other autoimmune diseases.

Dr. Jordan Orange, an internationally recognized leader in studying and treating primary immunodeficiency disorders in children, will lead the new Center for Human Immunobiology at Texas Children’s Hospital. His research, which will be funded by the National Institute of Allergy and Infectious Diseases, as well as the United States Immunodeficiency Network, will focus on the biology of natural killer cells and the innate immune system, with a clinical focus on primary immunodeficiency disease.

Baxter International announced plans to spend up to $1 billion over the next five years to open a new production center near Atlanta that will employ more than 1,500 workers. Construction will begin in 2012, and the plant is expected to start production in 2018.

Did You Know?

A new column titled “IG Chronicles” will debut in IG Living magazine’s December-January issue featuring autobiographical stories written by a reader about his or her life with a chronic illness. Submit your stories to editor@IGLiving.com.
Did You Know?

Organizations

New Organization for PIDD in United Kingdom

The United Kingdom Primary Immune Deficiency Patient Support (UKPIPS) is a new national patient-run and patient-led organization in the United Kingdom dedicated to people with primary antibody and immune deficiencies and their caregivers. The organization, which offers them information, advice, support and hope, is currently seeking charitable status and has a highly regarded medical advisory panel.

The launch of UKPIPS will coincide with World Primary Immune Deficiency Week. “There is currently little support or information for people living with a primary antibody deficiency in the U.K., with many people often suffering from extreme ill health for a number of years — or even decades — before they are diagnosed,” says Liz Macartney, UKPIPS coordinator and trustee. “There is also a lot of misunderstanding of the condition, including even the basic principles of caring for a patient with a compromised immune system.”

For more information on UKPIPS, visit www.ukpips.org.uk.

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The U.S. Food and Drug Administration (FDA) approved Baxter International Inc.’s Advate (antihemophilic factor [recombinant] plasma/albumin free method) for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with hemophilia A. Advate is the only antihemophilic factor approved in the U.S. for prophylactic use in both adults and children.

The approval is based on a Phase IV prophylaxis study that demonstrated a statistically significant reduction in the median annual bleeding rate. Patients receiving on-demand treatment experienced 44 bleeds (per patient per year) compared with one bleed (per patient per year) while on either of the prophylactic regimens evaluated (a 98 percent reduction in annual bleed rate). Forty-two percent of study patients experienced zero bleeds during one year on prophylaxis. And, of the two prophylactic regimens approved for use, the dosing schedule of every three days (a pharmacokinetic-driven regimen based on patients’ clinical response) offered some patients the option of fewer infusions over one year of treatment.

FDA Approves Advate to Treat Hemophilia A

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A recent study conducted by Grifols suggests that the plasmapheresis process may reduce levels of low-density lipoprotein (LDL), or “bad” cholesterol, as well as total cholesterol in individuals who have high baseline levels. The study also suggests that plasmapheresis could increase levels of high-density lipoprotein (HDL), or “good” cholesterol, among individuals with low baseline levels. The multicenter longitudinal study was conducted in nine plasma donor centers in the U.S., with blood analyses performed prior to plasma donations to measure initial levels of total cholesterol, HDL and LDL. Plasma was collected from first-time donors or from donors who had not donated plasma for at least six months. The researchers estimated from the study results that plasmapheresis could reduce the levels of LDL by more than 30 mg/dl among individuals with high levels (greater than 160 mg/dl) or higher than desirable levels (greater than 130 mg/dl) when plasmapheresis procedures are performed two to four days apart. This effect was more significant in women, in whom cholesterol could be reduced by up to 35 mg/dl. A similar reduction pattern is estimated to occur in individuals with high total cholesterol levels (greater than 240 mg/dl) or higher than desirable levels (greater than 200 mg/dl), with the reductions in these cases potentially reaching 45 mg/dl and 32 mg/dl, respectively.

However, the cholesterol-lowering effects of plasmapheresis appeared to last only as long as the procedure continued at regular intervals, with cholesterol levels gradually returning to baseline following long periods without plasmapheresis. The same pattern of reductions was seen, although to a lesser degree, when subsequent plasmapheresis procedures were performed more than 10 days apart. Among individuals with normal baseline cholesterol levels, the study results suggested that plasmapheresis would not cause significant changes.