Results from the final Phase II clinical trial of Symphogen’s rozrolimupab shows it is safe and effective in inducing a rapid increase in platelet counts in patients with primary immune thrombocytopenia purpura (ITP). Rozrolimupab is a novel human recombinant mixture of 25 antibodies that are all manufactured simultaneously.

The Phase II open-label, multicenter clinical trial evaluated the efficacy, safety and tolerability of rozrolimupab (SYM001) in adult, RhD positive, non-splenectomized ITP patients. A total of 61 patients were treated with single doses from 75 µg/kg to 300 µg/kg as single intravenous infusions of 15 to 20 minutes’ duration. The trial demonstrated that at 300 µg/kg, eight of 13 (62 percent) patients responded at day seven. Within five to eight hours after rozrolimupab administration, 23 percent of patients achieved platelet responses (≥ 30x10^9/L and increase in platelet count by > 20x10^9/L from baseline). Median time to response was 59 hours (approximately 2.5 days), and the median duration of response was 14 days. The most common adverse events observed were headache, mostly mild or moderate (20 percent), pyrexia (13 percent), chills (10 percent) and fatigue (8 percent). Four serious adverse events considered related to the drug were reported: decreased hemoglobin, extravascular hemolysis/dizziness and two cases of transient rise in D-dimer values without clinical symptoms.

According to Tadeusz Robak, MD, professor at the University of Lodz, Poland: “These results suggest an efficacy and safety profile similar to that seen with plasma-derived immunoglobulin products. It seems promising that rozrolimupab rapidly yields platelet responses. This unique recombinant human monoclonal antibody mixture, rozrolimupab, can be produced indefinitely and may represent a novel and convenient replacement for blood-derived immunoglobulins with more limited supply.”

The study was published in the August 22 edition of Blood.

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A recent study exploring physicians’ knowledge and experience about primary immunodeficiency (PIDD) found a noteworthy deficiency in PIDD workup. The self-administered questionnaire was conducted among 263 pediatricians of wide education backgrounds practicing in the United Arab Emirates (UAE) working in the 27 government hospitals in all regions of the UAE. It included questions on PIDD signs and symptoms, syndromes associated with immunodeficiency, screening tests, interpreting laboratory tests and case management.

Of the 50 questions, 20 percent of pediatricians answered fewer than 60 percent of the questions correctly, 76 percent answered 60 percent to 79 percent of the questions correctly, and 4 percent answered fewer than 80 percent of the questions correctly. Seventeen of the 19 PIDD signs and symptoms were identified by 55 percent to 97 percent of pediatricians. Four of five syndromes associated with immunodeficiency were identified by 50 percent to 90 percent of pediatricians. Appropriate screening tests were chosen by 64 percent to 96 percent of pediatricians. And, attention to the laboratory reference range values as a function of patient age was notably limited. The overall performance of the pediatricians did not differ based on their age, gender, origin of certification, rank or years of experience.

PIDD is a cluster of serious disorders that require special alertness on the part of the medical staff for prompt diagnosis and management of the patient. The conclusion of the study was to implement effective educational strategies to improve the competency of pediatricians to diagnose and manage PIDD disorders.
**Disease Awareness**

**Scleroderma Foundation Launches Video Campaign**

The Scleroderma Foundation has launched the “Walk for Cure” video awareness campaign to support its signature fundraising and awareness event, “Stepping Out to Cure Scleroderma.” Supporters are asked to upload an original video on WalkForCure.org, a video-sharing website, that describes why they walk. The goal is to increase scleroderma awareness and provide hope and inspiration to the entire scleroderma community. WalkForCure.org was open for video submissions until 11:59 p.m. (Pacific), Monday, Oct. 15, 2012. In November, all uploaded videos were edited together, burned onto DVDs and sent to members of Congress and the president of the United States.

**Resource**

**IDF Publishes New IG Therapy Guide for Nurses**

The Immune Deficiency Foundation (IDF) has released the *IGF Guide for Nurses Immunoglobulin Therapy for Primary Immunodeficiency Diseases — 3rd Edition.* Developed by the IDF Nurse Advisory Committee, the pocket-size guide includes topics on general information about primary immunodeficiency diseases, delivery of immunoglobulin replacement therapy, product selection and characteristics, troubleshooting subcutaneous immunoglobulin therapy and more. It can be ordered by contacting the IDF at (800) 296-4433 or idf@primaryimmune.org, or a PDF of the guide can be downloaded at primaryimmune.org/about-primary-immunodeficiency-diseases/idf-publications.

**People and Places**

**NexDx Inc.,** a science-driven molecular diagnostics company providing next-generation products and services for personalized medicine in rheumatoid arthritis and other autoimmune diseases, announced today the appointment of internationally renowned physician and scientist **Mary K. Crow, MD,** to its scientific advisory board.

The **Scripps Research Institute** has been awarded a new $22.5 million, five-year grant from the National Institutes of Health to study the immune system. The ongoing research examines innovative technologies that may provide data for treating a wide range of human diseases that include viral and bacterial infections and inherited immune disorders.

**Legislation**

**Florida Will Test Newborns for SCID**

The Florida Department of Health has added severe combined immune deficiency disease (SCID), known as bubble boy disease, to the list of conditions that all newborns in the state are screened for at birth beginning October 1, 2012.

SCID is a primary immunodeficiency disease where affected infants lack T lymphocytes or white blood cells that help fight infections from a wide array of viruses, bacteria and fungi, leaving the infants susceptible to serious, life-threatening infections. Babies with SCID appear healthy at birth, but without early treatment, most often by bone marrow transplant, these infants cannot survive.

In the time since the federal recommendation that all states screen for SCID, dozens of babies have been born with this condition and have gone undetected until serious infection made it known. Three babies born in Florida suffered from this fate; one passed away at the age of five months last year, while the other two babies spent months in the hospital for treatment. Both remain in delicate circumstances awaiting recovery. In June 2011, Florida Governor Rick Scott vetoed a program that would add a test for SCID to the list of genetic diseases newborns are tested for in the state.
Did You Know?

IG Living magazine is now inviting teens to submit their stories to be published in a new Teen Talk column. The column will debut in the February-March 2013 issue. If you are a teen living with a primary immune or autoimmune disease that is treated with immune globulin, we encourage you to submit your story of what it is like to live with a chronic illness. Submit your 600- or fewer-word story to Editor@IGLiving.com.

Research

Refined Gene Therapy May Restore Immune Systems in Kids with SCID

Researchers have demonstrated that a refined gene therapy approach safely restores the immune systems of some children with severe combined immunodeficiency (SCID). SCID is a rare condition that blocks the normal development of a newborn’s immune system, causing chronic infections and a lifespan of two years if their immunity cannot be restored.

The 11-year study tested a combination of techniques for gene therapy in 10 patients with ADA-deficient SCID. The researchers used two slightly different DNA insertion vehicles, called retroviral vectors, to deliver the healthy ADA gene into the bone marrow cells of the patients. Retroviruses have the specialized ability to become a permanent part of host cells. Four of the patients remained on enzyme-replacement therapy throughout the procedure. The other six patients stopped enzyme-replacement therapy beforehand and were treated with a low dose of a chemotherapy that depletes stem cells in the bone marrow, making space for the gene-corrected stem cells that had been given the new gene in the laboratory and then returned to the patient’s body. That step proved to be important. The procedure has produced normal levels of immune function for three of those six patients for up to five years and has eliminated the need for enzyme-replacement injections. The researchers suggest that enzyme-replacement therapy during the procedure may dilute the numbers of corrected lymphocytes in the patients’ immune systems, diminishing the treatment’s effect.

An additional eight children, most of whom are 1 year old or younger, have been added to a second phase of the study. And, according to the researchers, the younger patients are showing even more favorable response rates to the therapy.

Resource

IDF Introduces New Patient Insurance Center

The Immune Deficiency Foundation has introduced a Patient Insurance Center on its website that provides information regarding insurance issues, as well as other possible sources of assistance, for patients and their families. The center includes a patient advocate corner that provides individualized assistance for patients who face insurance problems, including denials for therapy, procedures related to primary immunodeficiency diseases, reimbursement complications, help getting insurance, as well as the ability to locate a specialist, connect with peer support, request educational materials and more. Also offered is a FAQs page where patients can read answers to frequently asked questions, the American Academy of Allergy, Asthma & Immunology (AAAAI) IVIG Toolkit that outlines patients’ rights in terms of treatment and standards of care, a guide for how to appeal a health insurance denial, a list of manufacturers’ patient assistance programs, a list of insurance and treatment resources, an explanation of how healthcare reform affects patients, and a list of questions and answers submitted by patients over the years. The center can be accessed at primaryimmune.org/patients-and-families/patient-insurance-center.
Kineta Inc. has received regulatory approval in the Netherlands to initiate a first-in-human clinical trial of ShK-186, an autoimmune drug candidate that specifically inhibits the Kv1.3 potassium ion channel. ShK-186 is the first Kv1.3-specific inhibitor to advance to the clinic in hopes of developing an immune-sparing therapy for autoimmune diseases such as multiple sclerosis (MS), rheumatoid arthritis (RA) and lupus (SLE).

Kv1.3 instigates activation of effector memory T cells that are major mediators of autoimmune disease. Kineta scientific adviser and University of California, Irvine, professor K. George Chandy, MD, PhD, and his collaborators discovered the Kv1.3 channel and invented ShK-186 by modifying natural sea anemone-derived peptide inhibitors of Kv1.3. They found that by blocking the Kv1.3 channel, ShK-186 can reduce disease symptoms and pathology in animal models of MS, RA and SLE without broadly suppressing the immune system.

According to Dr. Tim Coetzee, chief research officer for the National Multiple Sclerosis Society and an early supporter of Dr. Chandy’s research, “There is a clear, unmet medical need for new therapies to treat MS that have novel mechanisms of action and may offer freedom from the side effects that accompany broad suppression of the immune system.”

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ImmusanT has initiated clinical trials in New Zealand, Australia and the U.S. to evaluate Nexvax2, a therapeutic vaccine for patients with celiac disease. Nexvax2 is designed to re-establish patients’ tolerance to the toxic effects of gluten, a protein in wheat, barley and rye, and allow them to return to a normal diet. There are currently no approved medicines available for people with celiac disease, who must manage their condition by eliminating gluten-containing foods from their diet.

Advancing the earlier Nexvax2 clinical trial, the new trial in Australia and New Zealand is a randomized, double-blind, placebo-controlled Phase I study evaluating multiple ascending doses of Nexvax2 for the induction of gluten tolerance in patients on a gluten-free diet. ImmusanT expects to enroll 84 subjects at approximately four study sites in the two countries in order to evaluate the safety, tolerability and pharmacokinetics, and to select doses for investigation in subsequent studies.

The second study, a randomized, double-blind, placebo-controlled Phase I trial being conducted in the U.S., will determine the safety, tolerability and pharmacokinetic profile of Nexvax2 in patients with celiac disease well controlled by a gluten-free diet. ImmusanT plans to enroll 30 adult subjects at approximately four trial sites.

“We are kicking off a robust clinical program that we hope demonstrates Nexvax2 dramatically reduces the body’s immune response to dietary gluten so patients can resume a normal diet and return to good health,” said Patrick H. Griffin, MD, chief medical officer of ImmusanT. “Our clinical development program will allow us to further examine the role of antigen-specific T cells in celiac disease activation and in the re-establishment of tolerance to gluten.”