The U.S. Food and Drug Administration (FDA) has approved Shire’s CUVITRU (immune globulin subcutaneous [human] 20% solution) to treat adult and pediatric patients 2 years and older with primary immunodeficiency disease (PI). CUVITRU is the only 20% subcutaneous IG (SCIG) treatment option without proline and with the ability to infuse up to 60 mL (12 grams) per site and 60 mL per hour per site, as tolerated, resulting in fewer infusion sites and shorter infusion durations compared with other conventional SCIG treatments.

FDA approval was based on results of a Phase II/III study of CUVITRU in which 74 patients (aged 3 years to 83 years) received 4,327 infusions over a median of 380.5 days. The rate of validated serious bacterial infections was 0.012 event per patient-year, and the annualized rate of infection was 2.41 events per patient. Median IgG trough levels were greater than 14.5 g/L. The median maximum infusion rate was 60 mL per hour per site, resulting in a median infusion duration of 0.95 hours. A volume of greater than or equal to 30 mL was infused per site in 74.8 percent of infusions. Most (84.9 percent) infusions were administered using less than or equal to two infusion sites. For 99.8 percent of infusions, there was no need to interrupt or stop administration or reduce the infusion rate. No related serious adverse event (AE) occurred during treatment, and related nonserious AEs occurred at a rate of 0.036 event per infusion. The incidence of related local AEs was 0.015 event per infusion, and related systemic AEs was 0.021 event per infusion. Most were mild in severity; none was severe. Increased infusion rates or volumes were not associated with higher AE rates.

“In the clinical study, primary immunodeficiency patients tolerated CUVITRU favorably despite the use of higher infusion site volumes and more rapid rates than have been routine in the past,” said Richard L. Wasserman, MD, PhD, medical director of pediatric allergy and immunology at Medical City Children’s Hospital. “The availability of CUVITRU as a high-concentration, subcutaneous IG provides primary immunodeficiency patients with the dosing flexibility that allows them to customize their therapy to best fit their individual needs.”

CUVITRU was approved in 17 European countries in June 2016. And, the company expects to initiate additional global regulatory submissions for CUVITRU in late 2016 and 2017.


Kedrion 10% Safe and Effective for Treating PI

In a prospective, multicenter, open-label, single-arm controlled Phase III study, researchers evaluated the safety, efficacy and pharmacokinetics of Kedrion 10% (intravenous immune globulin) in 45 patients with antibody deficiency and found it to be safe, efficacious and well-tolerated. Over the 12-month study period, patients were infused every 21 to 28 days, and only two episodes of acute serious bacterial infections (both bacterial pneumonias) were recorded, for a mean annual event rate of 0.04 per subject. The primary safety endpoint was met with the rate of infusion temporarily associated with greater than or equal to one adverse event. Secondary efficacy endpoints were comparable to records in similar studies.

Kedrion 10%, manufactured by Kedrion, S.p.A, is a new preparation of an immunoglobulin G (IgG) solution being studied for treating primary immunodeficiency patients.

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Survey

MMN Quality-of-Life Patient Survey Is Released

The Neuropathy Action Foundation (NAF) has released the results of its first international multifocal motor neuropathy (MMN) quality-of-life patient survey. Of the 214 patients who completed the survey, 71.15 percent are from the U.S., 6.73 percent are from the United Kingdom and the remainder are from 22 other countries.

Key findings from the survey include:

- The majority of respondents (56.52 percent) were diagnosed between the ages of 41 and 65 years.
- More than 67 percent reported it took more than one year to be diagnosed, 44 percent of whom reported it took between two years and three years or longer to be accurately diagnosed.
- The majority of misdiagnoses were reported by neurologists (54.60 percent) and family care providers (20.69 percent).
- 51.96 percent of respondents had conduction block, a characteristic of MMN.
- 91.43 percent receive intravenous immune globulin (IVIG) therapy, and 8.57 percent receive subcutaneous immune globulin (SCIG) therapy. And, if recommended by their neurologist, 81.05 percent would consider switching to SCIG therapy.
- Within one week of the first IG treatment, 36 percent of respondents reported a reduction in symptoms; only 6.67 percent reported IG did not help manage symptoms.
- 50 percent of respondents reported that MMN often or always interferes with employment.
- 56 percent said MMN impacts overall quality of life. Basic tasks such as turning a key in a lock are either difficult or impossible for 53.24 percent; 61.19 percent are able to shop and run errands by themselves); and 58.38 percent have difficulty falling asleep at night, with 77.66 percent reporting daytime sleepiness.
- “Early and accurate diagnosis can mean the difference between MMN patients being successfully managed or becoming permanently disabled,” said NAF President Dominick Spatafora.
- “Timely intervention can make a difference in patients’ lives. The survey results illustrate the complexity of MMN and the impact it can have on peoples’ lives. The good news is that the data also show that treatment clearly helps these individuals.”

A more detailed analysis of the survey findings is available on the NAF website at www.neuropathyaction.org/downloads/MMN_article%209-26-2016.pdf.

Research

ProMetic Completes Adult Enrollment in IVIG Phase III Clinical Trial in Canada

ProMetic Life Sciences has completed its enrollment of adults in its Phase III open-label, single-arm, two-cohort multicenter clinical trial investigating the safety, tolerability, efficacy and pharmacokinetics of its plasma-derived intravenous immune globulin (IVIG). The study will include a total of 50 adults (cohort one) and 25 children (cohort two). Cohort-two enrollment was expected to be completed soon after. Completion of the clinical trial is anticipated to be in the second quarter of 2017, after which the company will submit a biologics license application, making IVIG the second plasma-derived therapeutic targeted for commercialization. This follows ProMetic’s orphan disease plasminogen drug candidate currently in final clinical trial stages for the treatment of plasminogen congenital deficiency.

“Canadian patients are amongst the largest consumers of IVIG on a per capita basis worldwide, and the demand continues to grow at a rapid pace. As a Canadian-based company, we intend to play an important role in facilitating the pursuit of national self-sufficiency by being the first Canadian-based provider to locally manufacture and provide access to mainstream plasma-derived therapeutics such as IVIG and orphan drug candidates such as plasminogen,” said Pierre Laurin president and chief executive officer of ProMetic. According to recent market data, Canada is ranked as the second country in the world after the U.S. for the average consumption of IVIG (kilograms per million people).
Dysautonomia International Awards Research Grants

Dysautonomia International has awarded its first four research grants to support research on postural orthostatic tachycardia syndrome (POTS). The grants will go to Svetlana Blitshteyn, MD, clinical assistant professor in the Department of Neurology at the University at Buffalo School of Medicine and director of the Dysautonomia Clinic in Williamsville, N.Y.; Kamal R. Chemali, MD, associate professor of medicine in the Department of Neurology at Eastern Virginia Medical School and the director of the Neuromuscular and Autonomic Neurology Program at Sentara Hospital in Norfolk, Va.; Satish R. Raj, MD, MSCI, associate professor in medicine and pharmacology at Vanderbilt University’s Autonomic Dysfunction Center in Nashville, Tenn.; Julian Steward, MD, PhD, professor of pediatrics, psychology and medicine and director of the Center for Hypotension at New York Medicine College, and Andrew T. Del Pozzi, PhD, a post-doctoral trainee working in Dr. Stewart’s autonomic lab.

Dr. Blitshteyn will study autoimmune serum markers and the rate of autoimmune co-morbidities seen in POTS patients. Dr. Chemali will study the use of music therapy in POTS patients who continue to be symptomatic despite maximized standard treatment. Dr. Raj will assist with an investigation of a drug with the potential to combat the cognitive impairment experienced by many POTS patients. Drs. Stewart and Del Pozzi will study cerebral blood flow in response to nitric oxide in POTS patients.

NIH Report Provides Insight on ALPS

Researchers at the National Institutes of Health (NIH) have analyzed results from 20 years of research on people with the most common form of autoimmune lymphoproliferative syndrome (ALPS-FAS), a rare inherited disorder caused by mutations in the FAS gene in which the body cannot properly regulate the number of lymphocytes (a type of white blood cell). The study’s report, published online in Blood on Jan. 7, describes an easy-to-measure biomarker for diagnosing ALPS, identifies the major causes of death among ALPS patients, and suggests strategies to improve disease treatment and management.

In the study, clinical and laboratory findings from 150 ALPS-FAS patients and 63 healthy relatives with FAS mutations were analyzed. Results showed that elevated levels of vitamin B12 reliably indicate ALPS-FAS and could be useful in diagnosing the disorder. Some of the current diagnostic tests for ALPS are time-consuming and costly, but doctors can measure B12 levels with a simple, inexpensive blood test.

The study also found that ALPS-FAS patients have a higher-than-average risk of developing lymphoma. The researchers found that this increased risk is even greater than they had originally reported in 2001. Ten of the 150 ALPS-FAS patients developed Hodgkin’s lymphoma during the 20-year period, indicating that the risk is nearly 150 times greater for people with ALPS-FAS than for the general public. ALPS-FAS patients also have a 61-times greater risk of developing non-Hodgkin’s lymphoma, with six patients developing this form of cancer.

An additional study finding was that another major cause of severe illness and death among ALPS-FAS patients is sepsis, or life-threatening whole-body inflammation, following spleen removal. Forty percent of ALPS-FAS patients in the study (66 people) had their spleens removed to help manage anemia and low neutrophil and platelet levels, collectively known as cytopenias. Of these patients, 27 developed sepsis following spleen removal surgery, with six of them dying as a result. Cytopenias can be treated with immune-suppressing medications as well.

The study’s findings promise to facilitate rapid diagnosis and successful treatment of ALPS-FAS. Specifically, they suggest that avoiding spleen removal surgery by managing cytopenias with medication and routinely monitoring ALPS-FAS patients for lymphoma will improve outcomes.