



# Improving CIDP Outcomes

Research is contributing to a greater understanding about this rare neurological disease to help in its diagnosis and develop better treatments.

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**CHRONIC INFLAMMATORY** demyelinating polyneuropathy (CIDP) is a type of neuropathy caused by an immune system dysfunction. The true incidence of CIDP is not known since, oftentimes, it is misdiagnosed. People may be diagnosed with CIDP when they don't have it, or the correct diagnosis of CIDP may be missed. However, it is estimated between five and seven in 100,000 people are affected by it, with approximately 40,000 patients in the U.S. dealing with it at any one time. CIDP can strike any age and gender, although the peak period of development is in the sixth or seventh decade of life, and it affects males more than females. There does not seem to be a genetic link to CIDP.<sup>1</sup>

## What Is CIDP?

CIDP occurs when the immune system malfunctions and creates an antibody that attacks the nerve roots and peripheral nerves resulting in inflammation and damage to the myelin sheath, which covers the nerves and assists in nerve signal transmission. The result is a slowing of the nerve signals and subsequent weakness in the muscles they control. It usually starts in the feet and moves slowly over time up the legs and arms,

typically affecting both sides of the body. Both proximal and distal muscles can be involved. Symptoms reported include:<sup>2</sup>

- Initial limb weakness, both proximal and distal
  - Sensory symptoms (e.g., tingling and numbness of hands and feet)
  - Motor symptoms (usually predominant)
  - Symptoms of autonomic system dysfunction (e.g., orthostatic dizziness)
  - Preceding infection (infrequent)
  - A relatively acute or subacute onset of symptoms in about 16 percent of patients
  - Usually a more precipitous onset of symptoms in children
- When the condition is associated with other diseases, symptoms may include:<sup>2</sup>
- Signs of cranial nerve involvement (e.g., facial muscle paralysis or diplopia)
  - Gait abnormalities
  - Motor deficits (e.g., symmetric weakness of both proximal and distal muscles in upper and lower extremities)
  - Diminished or absent deep tendon reflexes
  - Sensory deficits (typically in stocking-glove distribution)
  - Impaired coordination

The rate and severity of progression of weakness varies from person to person; however, CIDP usually presents slowly over several months. This is in contrast to the acute form of demyelinating neuropathy known as Guillain-Barré syndrome (GBS). GBS presents with a rapid progression of symptoms occurring over days or weeks that usually warrants hospitalization due to involvement of the breathing muscles. Respiratory involvement does not occur in CIDP.

### Triggering CIDP

It is unknown what causes CIDP, but it is believed to be an autoimmune disorder. Autoimmune disorders occur when the body's natural defenses (antibodies and lymphocytes) against invading organisms suddenly begin to attack perfectly healthy tissue.<sup>3</sup> In the case of CIDP, the autoimmune disorder causes the immune system to attack the myelin cover of the nerves causing inflammation of nerves and nerve roots.<sup>4</sup>

Healthcare providers also consider CIDP as a chronic form of GBS. And, while the specific triggers of CIDP vary, in many cases, the cause cannot be identified.<sup>4</sup>

In addition, CIDP may occur with other conditions such as diabetes (more than half of people with diabetes develop some type of neuropathy), toxins (e.g., heavy metals or chemicals), infections (including certain viral or bacterial infections, Lyme disease, shingles, Epstein-Barr virus, hepatitis C, leprosy, diphtheria and HIV), vitamin deficiencies (B-1, B-6, B-12, E and niacin), physical stress or injury to a nerve (such as from motor vehicle accidents, falls or sports injuries), medications (especially those used to treat cancer), tumors (cancerous and noncancerous growths that develop and press on the nerves), bone marrow disorders (including an abnormal protein in the blood, a form of bone cancer, lymphoma and amyloidosis), other diseases (including kidney disease, liver disease, connective tissue disorders and hypothyroidism) and alcoholism.<sup>5</sup>

### Diagnosing CIDP

Several years ago, the Peripheral Nerve Society (PNS) and European Federation of Neurological Societies (EFNS) established a task force to create consensus guidelines for diagnosing and managing CIDP.<sup>6</sup> All health plans have medical policies outlining how CIDP therapy will be approved. Many plans base their criteria for approving intravenous immune globulin (IVIG) treatment for CIDP on the PNS/EFNS criteria.

Investigative tests for diagnosing CIDP are presented in Table 1. While there is no single test for a proper diagnosis, neurologists will conduct a thorough history and physical, including a neurological exam, rule out other causes and evaluate the results of

**Table 1. Investigations to Be Considered<sup>4</sup>**

#### For Diagnosing CIDP

- Electrodiagnostic studies, including sensory and motor nerve conduction studies, which may be repeated, performed bilaterally or used in proximal stimulation for motor nerves
- CSF examination, including cells and protein
- MRI spinal roots, brachial plexus and lumbosacral plexus
- Nerve biopsy

#### For Detecting Concomitant Diseases

##### *Recommended studies:*

- Serum and urine paraprotein detection by immunofixation\*
- Fasting blood glucose
- Complete blood count
- Renal function
- Liver function
- Antinuclear factor
- Thyroid function

##### *Studies to be performed if clinically indicated:*

- Skeletal survey\*
- Oral glucose tolerance test
- Borrelia burgdorferi serology
- C reactive protein
- Extractable nuclear antigen antibodies
- Chest radiograph
- Angiotensin-converting enzyme
- HIV antibody

#### For Detecting Hereditary Neuropathy

- Examination of parents and siblings
- Appropriate gene testing (especially PMP22 duplication and connexin 32 mutations)
- Nerve biopsy

\* Repeating these should be considered in patients who are or become unresponsive to treatment

electrodiagnostic studies, blood tests and other tests to make an accurate diagnosis. Based on findings, tests will be ordered to further evaluate how the nerves and muscles are functioning. Tests that assist in diagnosing CIDP include:

- Electromyography (EMG), which measures muscle activity to show which muscles and nerves are affected;
- Nerve conduction study (NCS), which measures the speed and efficiency of electrical signals of nerves;

- Lumbar puncture, which taps into the cerebral spinal fluid (CSF) looking for abnormalities that will show the cause of the neuropathy (in CIDP, protein may be high in the CSF); and
- Nerve biopsy, which looks at a section of a nerve to assess the cause of the damage (not typically performed unless the diagnosis is unclear).

## Treating CIDP

The primary goals for treatment of CIDP are to reduce symptoms, improve functional status and, if possible, maintain long-term remission.<sup>7</sup> Therapies that have proved to successfully treat CIDP include corticosteroids, intravenous immune globulin (IVIG), subcutaneous immune globulin (SCIG), plasma exchange (PE) and physiotherapy. Other therapies that have been tried but are not as effective include immunosuppressive agents. Most patients see improvement using these treatment options alone or in combination. In addition, hematopoietic stem cell transplantation (HSCT) is being looked at to put CIDP in remission. Table 2 lists therapies for CIDP.

Corticosteroids have been commonly used to treat CIDP for many years. Initial treatment with oral prednisone is typically high dose at 60 mg to 100 mg per day and then tapered once the patient is stabilized. Unfortunately, there is no strong evidence from randomized controlled trials that corticosteroids are effective, and they cause many undesirable side effects. As a result, several trials have been conducted to evaluate alternative dosing regimens with no difference in efficacy.<sup>8,9</sup>

Over the last 15 years, IVIG has been considered first-line treatment for CIDP with fewer side effects than corticosteroids. IG therapy protects the nerves in the body from being attacked. IVIG brands that have a U.S. Food and Drug Administration (FDA)-approved indication for CIDP include Gamunex-C (Grifols), Gammaked (Kedrion) and, most recently, Privigen (CSL Behring). The approvals for Gamunex-C and Gammaked were based on the Phase III ICE (IVIG in CIDP Efficacy) trial conducted by Talecris (now Grifols).<sup>10</sup> The approval of Privigen was based on the Phase III PRIMA (Privigen Impact on Mobility and Autonomy) and PATH (Polyneuropathy and Treatment with Hizentra) studies conducted by CSL Behring, which showed a 61 percent and 73 percent response rate, respectively (see CSL Behring's Privigen Approved to Treat CIDP on page 12).<sup>11</sup>

SCIG therapy is also sometimes used as a successful off-label (not FDA-approved) indication to treat CIDP. Recently, CSL Behring, manufacturer of the SCIG product Hizentra, completed the PATH study, the largest CIDP trial designed to demonstrate the efficacy, safety and tolerability of two different doses of

**Table 2. Therapy for CIDP<sup>7</sup>**

**Proven therapies from randomized controlled trials:**

**Level I evidence**

- Intravenous immune globulin
- Prednisone
- Plasma exchange
- Pulse oral dexamethasone

**Therapies ineffective based upon randomized controlled trials:**

**Level I evidence; These studies all had difficulties in trial design**

- Azathioprine
- Interferon B1a (Level II evidence)
- Methotrexate

**Therapies of unproven benefit but probably helpful as steroid-sparing agents, based upon clinical experience and > 1 case series: Level IV evidence**

- Cyclosporine A
- Cyclophosphamide
- Azathioprine

**Other therapies of unproven benefit: Level IV evidence**

- Mycophenolate mofetil
- Pulse weekly oral prednisolone
- Pulse weekly oral dexamethasone
- Pulse weekly intravenous methylprednisolone
- Rituximab
- Interferon alpha 2a
- Etanercept
- Tacrolimus
- Alemtuzumab
- Natalizumab
- Hematopoietic stem cell transplantation

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Hizentra, compared with placebo (Privigen), in the maintenance treatment of CIDP patients previously treated with IVIG.<sup>12</sup> Study results have not yet been published; however, in July, FDA accepted for review the company's supplemental biologics license application for Hizentra for the treatment of CIDP as maintenance therapy to prevent relapse of neuromuscular disability and impairment.<sup>13</sup> In addition, Shire is currently investigating its SCIG product HyQvia in two Phase III studies for treating CIDP. One is measuring the long-term tolerability and

safety of HyQvia.<sup>14</sup> The other study is testing the efficacy, safety and tolerability of HyQvia and Gammagard Liquid, the latter of which can be infused through both the IV and SC route.<sup>15</sup>

Two randomized controlled trials have shown PE to be beneficial in CIDP patients. PE, a process of replacing the plasma in a patient's blood, begins by withdrawing blood from the patient, removing plasma from the blood, and exchanging it with red blood cells reinfused in a plasma substitute (usually human albumin and saline). The result is proteins located in plasma that are responsible for attacking the nerves are removed from the blood. PE is more time-consuming and invasive than IVIG. And, while improvement in disability and nerve conduction after treatment can be rapid, it is also short-term, and patients often relapse after stopping PE. As such, PE is restricted to second-line treatment in most cases.<sup>8,9</sup>

Physiotherapy can also play an important role in the assessment and management of CIDP, especially helping to maximize a patient's physical potential. The goals of physical therapy are to:<sup>16</sup>

- Maximize muscle strength and minimize muscle wastage by exercise using strengthening techniques;
- Minimize the development of contractures (stiffness) around joints;
- Facilitate mobility and function; and
- Provide a physical assessment that may help in planning future management.

Currently, there is one clinical trial being conducted to determine the effect of resistance and aerobic exercise in CIDP and multifocal motor neuropathy (MMN). The study's aim is to evaluate changes in muscle strength during high-intensive resistance training and changes in maximal oxygen consumption ( $VO_2$ -max) during high-intensive aerobic training in patients with CIDP and MMN in maintenance therapy with SCIG. The hypotheses are that muscle strength and  $VO_2$ -max are significantly increased during training sessions.<sup>17</sup>

Because 25 percent of patients fail to respond or do not respond adequately to corticosteroids, PE and IVIG, and the likelihood of progression of the disease is high, immunosuppressants designed to weaken the immune system so it doesn't attack the nerves are considered in CIDP patients. These drugs, which are not approved by FDA to treat CIDP, include rituximab (Rituxan), mycophenolate mofetil (Cellcept), azathioprine (Imuran) and methotrexate (Rheumatrex, Trexall, Otrexup, Rasuvo).

Rituximab is a monoclonal antibody that targets a certain portion of B cells, including those that play a role in the immune response thought to occur in autoimmune conditions.

Rituximab has been used in several autoimmune neurological conditions, including CIDP. In one multicenter study of 13 CIDP patients who were partial or nonresponders to conventional therapy, nine improved clinically or maintained the improvement seen with IVIG/PE after a median time of two months following the course of rituximab, and the effect lasted for up to one year. In another similar study of 18 patients, six responded to rituximab, showing at least a 1-point improvement on the Rankin scale (commonly used for measuring the degree of disability or dependence in the daily activities of people who have suffered a stroke or other causes of neurological disability). In addition, a number of case studies have reported on the efficacy of rituximab treatment in CIDP when there is a coexistent B cell disease such as idiopathic thrombocytopenic purpura, SLE75 and Morvan syndrome, and myasthenia gravis. However, so far, a significant treatment effect has not been proven.<sup>8,9</sup>

## THE PRIMARY GOALS FOR TREATMENT OF CIDP ARE TO REDUCE SYMPTOMS, IMPROVE FUNCTIONAL STATUS AND, IF POSSIBLE, MAINTAIN LONG-TERM REMISSION.

Mycophenolate mofetil (MMF) inhibits white blood cell proliferation and formation of adhesion molecules (that play a major role in the recruitment of neutrophils to the site of inflammation) and migration.<sup>18</sup> In a study, researchers analyzed a database of 184 patients with CIDP to obtain clinical, laboratory and electrophysiological information for patients with CIDP treated with MMF. Eight patients who met the inclusion criteria received 2 grams of MMF per day for a median duration of 15.2 months. The average Neuropathy Impairment Score of those patients improved from baseline ( $72.3 \pm 35$ ) to after initiation of MMF therapy ( $37.8 \pm 37$ ). Six patients were either able to stop concomitant medications (corticosteroids, IVIG) or reduce their doses and frequency by equal to or greater than 50 percent.<sup>19</sup> Currently, a Phase III clinical trial is ongoing to investigate if MMF could decrease the proportion of patients who relapse during the IVIG tapering period after IVIG withdrawal.<sup>20</sup>

Proven **safe**. Proven **effective**.

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### **Important Safety Information**

Privigen is approved to:

- Treat types of primary immunodeficiency (PI).
- Raise platelet counts in patients over 15 with chronic immune thrombocytopenic purpura (ITP).
- Treat chronic inflammatory demyelinating polyneuropathy (CIDP) in adults. Talk with your doctor about the length of your therapy.

10+  
Years

Experience in treating  
PI and chronic ITP\*



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now available for people with CIDP†

To see what Privigen can do for you, visit **Privigen.com**.

\*Primary immunodeficiency and chronic immune thrombocytopenic purpura.  
†Privigen is the #1 IVIg used in US hospitals since 2010 in PI and chronic ITP.‡

## Important Safety Information

### WARNINGS:

- **Thrombosis (blood clotting) can occur with immune globulin products, including Privigen. Risk factors may include advanced age, prolonged immobilization, a history of blood clotting or hyperviscosity (thick blood), use of estrogens, installed vascular catheters, and cardiovascular risk factors.**
- **In predisposed patients, kidney malfunction and acute kidney failure, potentially fatal, can occur with the administration of human immune globulin intravenous (IGIV) products. Kidney problems occur more commonly in patients receiving IGIV products that contain sucrose. Privigen does not contain sucrose.**
- **If you are at high risk of thrombosis or kidney problems, your doctor will prescribe and administer Privigen at the minimum dose and infusion rate practicable, and will monitor you for signs and symptoms of thrombosis and viscosity, as well as kidney function. Always drink sufficient fluids before administration.**

**See your doctor for a full explanation, and the full prescribing information for complete boxed warning.**

Treatment with Privigen might not be possible if your doctor determines you have hyperprolinemia (too much proline in the blood) or are IgA-deficient with antibodies to IgA and a history of hypersensitivity. Tell your doctor if you have previously had a severe allergic reaction (including anaphylaxis) to the administration of human immune globulin. Inform your physician if you notice early signs of hypersensitivity reactions to administration of Privigen, including hives, tightness of the chest, wheezing, or shock.

Immediately report to your physician the following symptoms, which could be signs of serious adverse reactions to Privigen:

- A decrease in urine output, sudden weight gain, fluid retention, and/or shortness of breath following infusion (possible symptoms of kidney problems).

- Pain and/or swelling or discoloration of an arm or leg, shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, and numbness or weakness on one side of the body (possible symptoms of a blood clot).
- Headache; a stiff neck; excessive drowsiness or fatigue; fever; sensitivity to light or painful eye movements; nausea; increased heart rate; yellowing of the skin or eyes, and/or dark-colored urine (possible symptoms of other conditions that may require treatment).

Privigen is made from human blood. The risk of transmission of infectious agents, including viruses and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent and its variant (vCJD), cannot be completely eliminated.

Before receiving any vaccine, tell the immunizing physician if you have had recent therapy with Privigen, as the effectiveness of the vaccine could be compromised.

In clinical trials of Privigen, headache was the most common side effect seen in all conditions treated (PI, ITP, and CIDP). Other common side effects that can be seen with treatment include fatigue, nausea, fever, and high blood pressure. These are not the only side effects possible; see the full prescribing information for a complete list of adverse reactions possible with treatment for each condition. Alert your physician to any side effect that bothers you or does not go away.

**Please see the following brief summary of prescribing information for Privigen, including boxed warning.**

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088.

**Reference: 1.** Data on file. Available from CSL Behring as DOF PVG-002.

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IgIQ is ready to help

Call for support **1-877-355-IGIQ (4447)**  
Monday–Friday, 8 AM to 8 PM ET

All the **support** you need. All in the **same place**.

For people taking Privigen, CSL Behring offers a comprehensive set of services to help make Ig therapy accessible and affordable. IgIQ provides:

- Financial assistance
- Patient support
- Insurance navigation
- Referral triage
- General information

#### BRIEF SUMMARY OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use Privigen safely and effectively. See full prescribing information for Privigen. Privigen® Immune Globulin Intravenous (Human), 10% Liquid Initial U.S. Approval: 2007

#### WARNING: THROMBOSIS, RENAL DYSFUNCTION AND ACUTE RENAL FAILURE

See full prescribing information for complete boxed warning.

- **Thrombosis may occur with immune globulin products, including Privigen. Risk factors may include: advanced age, prolonged immobilization, hypercoagulable conditions, history of venous or arterial thrombosis, use of estrogens, indwelling vascular catheters, hyperviscosity, and cardiovascular risk factors.**
- **Renal dysfunction, acute renal failure, osmotic nephrosis, and death may occur with immune globulin intravenous (IGIV) products in predisposed patients. Renal dysfunction and acute renal failure occur more commonly in patients receiving IGIV products containing sucrose. Privigen does not contain sucrose.**
- **For patients at risk of thrombosis, renal dysfunction or failure, administer Privigen at the minimum dose and infusion rate practicable. Ensure adequate hydration in patients before administration. Monitor for signs and symptoms of thrombosis and assess blood viscosity in patients at risk for hyperviscosity.**

#### INDICATIONS AND USAGE

Privigen is an Immune Globulin Intravenous (Human), 10% Liquid indicated for the treatment of:

- Primary humoral immunodeficiency (PI)
- Chronic immune thrombocytopenic purpura (ITP) in patients age 15 years and older
- Chronic inflammatory demyelinating polyneuropathy (CIDP) in adults

Limitations of Use:  
Privigen maintenance therapy in CIDP has not been studied beyond 6 months.

#### CONTRAINDICATIONS

- History of anaphylactic or severe systemic reaction to human immune globulin
- Hyperprolinemia (Privigen contains the stabilizer L-proline)
- IgA-deficient patients with antibodies to IgA and a history of hypersensitivity

#### WARNINGS AND PRECAUTIONS

- IgA-deficient patients with antibodies to IgA are at greater risk of developing severe hypersensitivity and anaphylactic reactions.
- Monitor renal function, including blood urea nitrogen and serum creatinine, and urine output in patients at risk of developing acute renal failure.
- Hyperproteinemia, increased serum viscosity, and hyponatremia may occur.
- Aseptic meningitis syndrome (AMS) may occur, especially with high doses or rapid infusion.
- Hemolysis that is either intravascular or due to enhanced red blood cell sequestration may occur. Risk factors include high doses and non-O blood group. Closely monitor patients for hemolysis and hemolytic anemia.
- Elevations of systolic and diastolic blood pressure (including cases of hypertensive urgency) have been observed during/shortly following Privigen infusion. These blood pressure

elevations were resolved or significantly improved within hours with either observation alone or changes in oral anti-hypertensive therapy. Check patients for a history of hypertension and monitor blood pressure during and following Privigen infusion.

- Monitor patients for pulmonary adverse reactions (transfusion-related acute lung injury [TRALI]).
- Carefully consider the relative risks and benefits before prescribing the high dose regimen (for chronic ITP and CIDP) in patients at increased risk of thrombosis, hemolysis, acute kidney injury, or volume overload.
- Privigen is made from human blood and may contain infectious agents, e.g., viruses, the variant Creutzfeldt Jakob disease [vCJD] agent and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent.

#### ADVERSE REACTIONS

- **PI** – The most common adverse reactions, observed in >5% of study subjects, were headache, fatigue, nausea, chills, vomiting, back pain, pain, elevated body temperature, abdominal pain, diarrhea, cough, stomach discomfort, chest pain, joint swelling/effusion, influenza-like illness, pharyngolaryngeal pain, urticaria, and dizziness. Serious adverse reactions were hypersensitivity, chills, fatigue, dizziness, and increased body temperature.
- **Chronic ITP** – The most common adverse reactions, observed in >5% of study subjects, were laboratory findings consistent with hemolysis (hemoglobin and hematocrit decrease without blood loss in conjunction with positive direct antiglobulin test [DAT] and elevated blood lactate dehydrogenase [LDH] and/or indirect bilirubin), headache, elevated body temperature, anemia, nausea, and vomiting. A serious adverse reaction was aseptic meningitis.
- **CIDP** – The most common adverse reactions observed in >5% of study subjects were headache, asthenia, hypertension, nausea, pain in extremity, hemolysis, influenza like illness, leukopenia, and rash. Serious adverse reactions were hemolysis, exacerbation of CIDP, acute rash, blood pressure diastolic increased, hypersensitivity, pulmonary embolism, respiratory failure, and migraine.

To report **SUSPECTED ADVERSE REACTIONS**, contact **CSL Behring Pharmacovigilance at 1-866-915-6958 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

#### DRUG INTERACTIONS

The passive transfer of antibodies may:

- Lead to misinterpretation of the results of serological testing.
- Interfere with the response to live virus vaccines.

#### USE IN SPECIFIC POPULATIONS

- Geriatric: In patients over age 65 or in any patient at risk of developing renal insufficiency, do not exceed the recommended dose, and infuse Privigen at the minimum rate practicable.

See 17 for **PATIENT COUNSELING INFORMATION**.

Based on September 2017 revision

Azathioprine is controversial in treating CIDP. In some small cases studies, azathioprine in combination with glucocorticosteroid therapy at higher doses led to improvement.<sup>18</sup>

Methotrexate has been studied in patients with CIDP with no significant results. In one study, 60 patients were enrolled in a multicenter trial to receive either a placebo or methotrexate, all of whom were co-treated with glucocorticoids or IVIG. The primary endpoint was a 20 percent reduction in mean weekly dose of either glucocorticoids or IVIG by the end of the trial, but findings showed no significant difference in the primary outcome between the methotrexate and placebo groups.<sup>18</sup> In another multicenter study, researchers compared 60 CIDP patients requiring corticosteroids or IVIG, 27 of whom were prescribed oral methotrexate and 32 of whom were given a placebo. Primary outcome was a greater than 20 percent reduction in mean weekly dose in the last four weeks of the trial compared with the first four weeks. Secondary outcomes analyzed separately at mid-trial and final visits measured activity limitations and strength. Fourteen (52 percent) taking methotrexate and 14 (44 percent) taking a placebo had a greater than 20 percent reduction in mean weekly dose of corticosteroids or IVIG. However, there were no clinically and statistically significant differences in secondary outcomes.<sup>21</sup>

Lastly, limited HSCTs have been performed in refractory CIDP patients.<sup>22</sup> In one small, uncontrolled trial, autologous HSCT (AHSCT) was performed in 11 consecutive patients with CIDP refractory to first-line immunomodulatory treatments and one or more second-line treatments. The total median Inflammatory Neuropathy Cause and Treatment and Rankin scores improved significantly within two months to six months after AHSCT. Eight of the 11 patients maintained drug-free remission, but three of the 11 relapsed during the follow-up period, requiring retransplantation with AHSCT in one. Complications occurred following six of the transplantations, but resolved spontaneously or with treatment. The authors concluded that “AHSCT can be efficacious in therapy-refractory CIDP, with a manageable complication profile, although randomized controlled trials are needed.”<sup>23</sup>

Currently, a Phase II study is being conducted to examine whether treating patients with high-dose cyclophosphamide (a drug that reduces the function of the immune system) and ATG (an immunosuppressive agent that selectively destroys T lymphocytes), followed by return of the previously collected blood stem cells, will stop the progression of CIDP. The purpose is to evaluate whether this treatment will produce a normal immune system that will no longer attack the body.<sup>24</sup>

## A Rare, Complicated Disease

CIDP is a rare, complicated disease that has many different causes, with a great deal of variability in symptoms from patient to patient. Much research is being conducted to advance scientific understanding of the underlying pathogenesis of CIDP, homing in on diagnostic criteria and establishing optimum treatment doses and durations for established therapies, as well as to further investigate alternative, less-well studied treatments.<sup>8</sup> For now, many therapies have proven successful in managing the symptoms in CIDP patients. But, it is hoped that in the future, more effective therapies and, perhaps, even a cure may be available. ■

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