Steps Ahead of Gaucher

Lace up your shoes. Show your support.

Celebrating Gaucher Awareness Month
The National Gaucher Foundation’s “Go For Gaucher” Atlanta Walk/Run
Preventing Bone Problems in Type 1 Gaucher Disease
Gaucher Disease Support Groups: Finding Help

Patient Profile: Kim Keegan

www.cerezyme.com
Lace up your shoes. Show your support.

In honor of Gaucher Awareness Month this October, this issue of Horizons contains a pair of green shoelaces. We invite you to join us as we celebrate how far the Gaucher community has come in the fight against this life-altering disease by wearing green shoelaces during the month of October. Gaucher is a commonly overlooked disease, and wearing green laces will help promote awareness and unity in the fight against Gaucher—and, most importantly, will get people to notice this rare disease!

Anyone can participate: Simply wear the green Gaucher laces throughout the month of October to publicly show your support for the Gaucher community. If your shoes don’t use laces, you can still show your support by turning the laces into a bracelet.

Help spread awareness of Gaucher disease by taking a photo of your shoes (or bracelet!) and uploading it to stepsaheadofgaucher.com. In turn, Genzyme will make a donation on your behalf to the National Gaucher Foundation.

—Your team at Genzyme

Cerezyme® (imiglucerase for injection) is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia (low red blood cell count), thrombocytopenia (low blood platelet count), bone disease, hepatomegaly or splenomegaly (enlarged liver or spleen).

Important Safety Information
Approximately 15% of patients have developed immune responses (antibodies). These patients have a higher risk of an allergic reaction (hypersensitivity). Use Cerezyme® (imiglucerase for injection) carefully if you have had an allergic reaction to the product in the past. Symptoms suggestive of allergic reaction happen in 6.6% of patients, and include anaphylactoid reaction (a serious allergic reaction), itching, flushing, hives, an accumulation of fluid under the skin, chest discomfort, shortness of breath, coughing, cyanosis (a bluish discoloration of the skin due to diminished oxygen), and low blood pressure. Side effects related to Cerezyme administration have been reported in less than 15% of patients. Each of the following events occurred in less than 2% of the total patient population. Reported side effects include nausea, abdominal pain, vomiting, diarrhea, rash, fatigue, headache, fever, dizziness, chills, backache, and rapid heart rate. Because Cerezyme therapy is administered by intravenous infusion, reactions at the site of injection may occur: discomfort, itching, burning, swelling or uninfected abscess. Cerezyme is available by prescription only. For more information, consult your physician.

Please see accompanying full Prescribing Information on pages 9-10.

Patients are encouraged to report negative side effects of prescription drugs to the FDA. Visit FDA.gov/medwatch, or call 1-800-FDA-1088.
Celebrating Gaucher Awareness Month

By Cheryl Alkon

October marks the time of year when we enjoy autumn leaves showing their colors, celebrate Christopher Columbus’s discovery of America, and watch children delight in dressing up for trick or treating. This year it’s also the month of Gaucher Awareness, according to Rosina Papantonio, Marketing Director of the National Gaucher Foundation (NGF).

Gaucher Awareness actually began in April 1988, when President Ronald Reagan designated a week during that month to educate people about the disease. Then, in September 2005, Massachusetts Congressman Barney Frank proclaimed September as Gaucher Awareness Month. The NGF later selected October as the official month to spotlight Gaucher disease.

The NGF plans to reach as many people as possible to educate and inform them about getting diagnosed with the inherited condition, and learning to live and thrive with it.

Type 1 Gaucher Disease

Type 1 Gaucher disease develops when an individual inherits two defective copies of the gene (one from each parent) that controls the production of the enzyme glucocerebrosidase. These individuals lack the ability to produce this enzyme, which processes fatty substances in the body. As a result, the fatty substances build up in the body and can cause a variety of problems that affect the liver, spleen, bones, blood cells, and other body parts.

If an individual inherits only one defective gene, he or she does not develop type 1 Gaucher disease but is a “carrier.” Carriers can pass the defective gene on to their children.

Treatment for Gaucher disease involves enzyme replacement therapy (See Important Safety Information, page 2, and Product Information, centerfold) to help the body process these substances so they don’t build up to dangerous levels.

Gaucher Awareness Month Activities

NGF activities during October include:

• **The 7th Annual Online Gaucher Awareness Auction**—October 1-October 30 at 10:00 PM
  Proceeds raised through the auction go toward funding Gaucher disease medical research, increasing informational efforts about the disease, and supporting assorted NGF programs. The auction features a variety of fun items such as vacation packages, gifts and gift certificates, sports equipment, hotel stays, airline miles, wine, jewelry, electronics, sports tickets and outings, clothing and accessories, books, adventure and experience packages, and health and beauty packages. To donate items for the auction or for more information about becoming an auction sponsor, contact Cyndi Frank at 877-649-2742 or email her at Cyndi@gaucherdisease.org.

• **“Go For Gaucher” Atlanta-area 5K Walk/Run**—October 14 (see related story)

The NGF also is featuring a “Share Your Awareness” campaign that continues to promote information about and support for Gaucher disease, and identifies the many efforts that people and organizations are making to get the word out.

“On our website, Facebook and Twitter accounts, and in press releases and in our newsletter, we will feature what has or will be done to promote education of Gaucher,” said Papantonio.

Activities for this year’s Gaucher Awareness Month are designed to appeal to a larger segment of people than in past years, she added. “This year, it is a fun-for-the-whole-family event, and a very mixed crowd of people, so the general population at large will be involved in education of Gaucher, rather than segments of the population.”

In past years, Gaucher Awareness Month programming has also included a series of live meetings around the country about Gaucher disease; webcasts from the NGF website; and conferences and meetings with the rabbinical, lay, medical, and patient communities—sometimes all together, and other times tailored to specific groups such as medical students, physicians, nurses, and/or geneticists.

With the online auction and the “Go for Gaucher” 5K Walk/Run, the focus is more on reaching people who are not necessarily part of the medical profession to help them learn more about the disease.

Genzyme has demonstrated its support of the NGF’s Gaucher Awareness Month by sponsoring the Atlanta 5K event. “Genzyme is proud to support the National Gaucher Foundation and participate in Gaucher Awareness Month,” said Kathleen Coolidge, Genzyme’s Associate Director of Patient Advocacy-Rare Diseases. “We have a long history with the Gaucher community and are proud and honored to be able to be a leader in supporting them.”
As part of the activities scheduled for October’s Gaucher Awareness Month, the National Gaucher Foundation (NGF) is holding a “Go For Gaucher” 5K Walk/Run in the Atlanta area. The walk/run—the first such event of its kind in that area—is designed to help raise awareness for Gaucher disease and will be held on October 14 in Mason Mill Park in Decatur, Georgia, located in the Atlanta metropolitan area.

Sponsored and organized by the NGF, the Atlanta 5K event is an effort to help people learn more about Gaucher disease, a genetic disorder that affects one in 40,000 to 60,000 people worldwide. The most common form of Gaucher disease is called type 1, which affects about 1 in up to 450 people who are Ashkenazi Jews.

What Is Gaucher, and Why a 5K?
Type 1 Gaucher disease occurs when a person inherits genetic mutations from both his or her mother and father. With this pair of mutations, the affected person lacks a key enzyme and cannot process fatty products, known as glucocerebrosides, in the body. Without the enzyme, the fatty products grow in the body and can cause many different problems, including abnormal liver and spleen enlargement, anemia (low red blood cells), a tendency to bruise, lung problems, and bone issues such as severe bone pain, bones that break easily, and arthritis.

The Atlanta 5K event will help raise awareness by providing information about how people can find out if they have Gaucher disease or are carriers and may be at risk for passing on the disease to future generations. They can also learn what medications are available to help people with Gaucher disease, and just how many people in the world are diagnosed with the condition.
Why a 5K walk/run? “It provides a fun and easy way to get people engaged in our cause to promote awareness,” said Rosina Papantonio, the NGF’s Marketing Director. “It’s not an ad, website, or a press release. It’s fun for the whole family, and it’s something to make parents, friends, and family think about the health of their children, and the fact that Gaucher disease can and does affect anyone.”

The NGF has sponsored several 5K events held in different cities over the past three years, said Papantonio. “Most of the time, it comes about when a mother of a child with Gaucher wants to hold a walk in their area,” she said. “In some cases, it may be because their child is not doing well. In others, it is because their child is doing well—often from treatment.”

Since the early 1990s, Gaucher has been able to be treated by replacing the missing enzyme that helps the body process glucocerebrosides. Genzyme’s enzyme replacement therapy, Cerezyme® (imiglucerase for injection), is administered by infusion. (See Important Safety Information, page 6, and Product Information, centerfold.)

Activities such as the Atlanta race, which make more people aware of Gaucher disease, are important because they can potentially help save lives. “Early diagnosis and early treatment, if needed, may be key to how well a person will do for the rest of their lives,” Papantonio said. “The longer a person goes undiagnosed and untreated, the more damage is caused to the skeletal structure, liver and spleen, kidneys and heart. Many organs are involved with this disease, and the symptoms vary from mild to severe, so one person may not be affected in the same way as the next.”

As an organization devoted to helping people understand Gaucher disease and live well with the condition, the NGF relies both on personal experience and on statistics and research. “Education, outreach, and awareness in all forms is key to helping prevent misdiagnosis, and the pain and suffering that comes from this debilitating and often fatal disease,” said Papantonio. “We’ve been educating for more than 20 years, but many people each year are still undiagnosed or misdiagnosed.”

That is something the NGF hopes to change. The organization has tried to cover every angle while publicizing the Atlanta event. They distributed “hundreds of press releases, posted to fifty or more online calendars, sent thousands of emails and letters to doctors, hotels, Jewish organizations, schools, colleges, attorneys, shops and stores,” said Papantonio. NGF has also spread the word using social media sites such as the foundation’s Facebook and Twitter accounts, the Gaucher discussion group on Yahoo Groups, and by posting details online at the NGF website www.gaucherdisease.org.

Atlanta-area media outlets have been targeted as well, with banner advertising or information about the race appearing on the Dr. Oz website for viewers living in the Atlanta metropolitan area. Ads are running in The Atlanta Jewish Times newspaper and in program books for six games of the Atlanta Braves baseball team. Television banner ads will run on 160

(Continued on the next page)
television sets at Turner Field, the Atlanta ballpark, for six home games when the Braves host the Miami Marlins and the New York Mets, she said.

Papantonio also said that information about the event was disseminated to members of the NGF, and that people reached out to any contacts known through organizations, friends, associates, and publications in the area. The 5K Walk/Run has also been advertised through search engine marketing ads, on runners’ websites in the Atlanta area, and via public service announcements running on television and radio.

Event organizers hope that a successful turnout for this event will set the tone for events in the coming years. “We expect at least 250 participants, but have no benchmark, as this is a first 5K for the Atlanta area,” said Papantonio. “The number could go higher as our Public Service Announcements begin running, along with our ads and the Atlanta Braves programs. We’d like to see 500 or more, but no matter what we do to prepare, there is not an exact science to something like this event, and the number of people who will participate.”

Corporate Support
The event currently has several corporate sponsors, including pharmaceutical companies Actelion, Pfizer, Shire, and Genzyme. Various Genzyme employees are taking part, said Kathleen Coolidge, Genzyme’s Associate Director of Patient Advocacy-Rare Diseases. “We will have several Genzyme volunteers at the event helping to hand out refreshments, running or walking the 5K event with Gaucher patients, physicians, National Gaucher Foundation staff, and other members of the community.”

Genzyme has long supported the NGF and its efforts to educate people about the condition. “This is one of the most important times of the year when the whole Gaucher community, patients, family members, health care providers, industry, and everyone who cares about Gaucher disease actively talks about it with anyone who’ll listen about how to identify Gaucher disease.”

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Please see accompanying full Prescribing Information on pages 9-10.
If you have enjoyed this issue of Horizons, please let us know by completing and returning the postage-paid Business Reply Card below.

Do you feel you have been kept informed on the Cerezyme supply issue?
[ ] Yes  [ ] No

How can Genzyme communicate better?
________________________________________________________________________

What channels are you using to keep informed?
[ ] Your physician  [ ] Cerezyme supply website
[ ] Your Genzyme Case Manager  [ ] Other ________________________________
[ ] National Gaucher Foundation

What different form of communication would you like to see used if any?
________________________________________________________________________

Would you be interested in sharing your story of living with Gaucher disease?
If so, please fill in the following:

Name________________________________________
Address_____________________________________
City________________________________________ State __________ Zip __________
Email_______________________________________
Phone_____________________________________
DESCRIPTION

Cerezyme® (imiglucerase for injection) is an analogue of the human enzyme β-glucocerebrosidase, produced by recombinant DNA technology. β-Glucocerebrosidase (β-D-glucosyl-N-acylsphingosine glucohydrolase, E.C. 3.2.1.45) is a lysosomal glycoprotein enzyme which catalyzes the hydrolysis of the glycolipid glucocerebroside to glucose and ceramide.

Cerezyme® is produced by recombinant DNA technology using mammalian cell culture (Chinese hamster ovary). Purified imiglucerase is a monomeric glycoprotein of 497 amino acids, containing 4 N-linked glycosylation sites (Mr = 60,430). Imiglucerase differs from placental glucocerebrosidase by one amino acid at position 495, where histidine is substituted for arginine. The oligosaccharide chains at the glycosylation sites have been modified to terminate in mannos sugars. The modified carbohydrate structures on imiglucerase are somewhat different from those on placental glucocerebrosidase. These mannose-terminated oligosaccharide chains of imiglucerase are specifically recognized by endocytic carbohydrate receptors on macrophages, the cells that accumulate lipid in Gaucher disease.

Cerezyme® is supplied as a sterile, non-pyrogenic, white to off-white lyophilized product. The quantitative composition of the lyophilized drug is provided in the following table:

<table>
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<tr>
<th>Ingredient</th>
<th>200 Unit Vial</th>
<th>400 Unit Vial</th>
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<tr>
<td>Imiglucerase (total amount)*</td>
<td>212 units</td>
<td>424 units</td>
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<tr>
<td>Mannitol</td>
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*This provides a respective withdrawal dose of 200 and 400 units of imiglucerase.

An enzyme unit (U) is defined as the amount of enzyme that catalyzes the hydrolysis of 1 micromole of the synthetic substrate para-nitrophenyl-β-D-glucopyranoside (pNP-Glc) per minute at 37°C. The product is stored at 2-8°C. After reconstitution with Sterile Water for Injection, USP, the imiglucerase concentration is 40 U/mL (see DOSAGE AND ADMINISTRATION for final concentrations and volumes). Reconstituted solutions have a pH of approximately 6.1.

CLINICAL PHARMACOLOGY

Mechanism of Action/Pharmacodynamics

Gaucher disease is characterized by a deficiency of β-glucocerebrosidase activity, resulting in accumulation of glucocerebroside in tissue macrophages which become engorged and are typically found in the liver, spleen, and bone marrow and occasionally in lung, kidney, and intestine. Secondary hematologic sequelae include severe anemia and thrombocytopenia in addition to the characteristic progressive hepatosplenomegaly, skeletal complications, including osteonecrosis and osteopenia with secondary pathological fractures. Cerezyme® (imiglucerase for injection) catalyzes the hydrolysis of glucocerebroside to glucose and ceramide. In clinical trials, Cerezyme improved anemia and thrombocytopenia, reduced spleen and liver size, and decreased cachexia to a degree similar to that observed with Ceredase® (alglucerase injection).

Pharmacokinetics

During one-hour intravenous infusions of four doses (7.5, 15, 30, 60 U/kg) of Cerezyme® (imiglucerase for injection), steady-state enzymatic activity was achieved by 30 minutes. Following infusion, plasma enzymatic activity declined rapidly with a half-life ranging from 3.6 to 10.4 minutes. Plasma clearance ranged from 9.8 to 20.3 mL/min/kg (mean ± S.D., 14.5 ± 4.0 mL/min/kg). The volume of distribution corrected for weight ranged from 0.09 to 0.15 L/kg (0.12 ± 0.02 L/kg). These variables do not appear to be influenced by dose or duration of infusion. However, only one or two patients were studied at each dose level and infusion rate. The pharmacokinetics of Cerezyme do not appear to be different from placental-derived α-glucerase (Ceredase®).

In patients who developed IgG antibody to Cerezyme, an apparent effect on serum enzyme levels resulted in diminished volume of distribution and clearance and increased elimination half-life compared to patients without antibody (see WARNINGS).

INDICATIONS AND USAGE

Cerezyme® (imiglucerase for injection) is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease that results in one or more of the following conditions:

a. anemia
b. thrombocytopenia
c. bone disease
d. hepatomegaly or splenomegaly

CONTRAINDICATIONS

There are no known contraindications to the use of Cerezyme® (imiglucerase for injection). Treatment with Cerezyme should be carefully re-evaluated if there is significant clinical evidence of hypersensitivity to the product.

WARNINGS

Approximately 15% of patients treated and tested to date have developed IgG antibody to Cerezyme® (imiglucerase for injection) during the first year of therapy. Patients who developed IgG antibody did so largely within 6 months of treatment and rarely developed antibodies to Cerezyme after 12 months of therapy. Approximately 46% of patients with detectable IgG antibodies experienced symptoms of hypersensitivity.

Patients with antibody to Cerezyme have a higher risk of hypersensitivity reaction. Conversely, not all patients with symptoms of hypersensitivity have detectable IgG antibody. It is suggested that patients be monitored periodically for IgG antibody formation during the first year of treatment.

 Treatment with Cerezyme should be approached with caution in patients who have exhibited symptoms of hypersensitivity to the product.

Anaphylactoid reaction has been reported in less than 1% of the patient population. Further treatment with imiglucerase should be conducted with caution. Most patients have successfully continued therapy after a reduction in rate of infusion and pretreatment with antihistamines and/or corticosteroids.

PRECAUTIONS

General

In less than 1% of the patient population, pulmonary hypertension and pneumonia have also been observed during treatment with Cerezyme® (imiglucerase for injection). Pulmonary hypertension and pneumonia are known complications of Gaucher disease and have been observed both in patients receiving and not receiving Cerezyme. No causal relationship with Cerezyme has been established. Patients with respiratory symptoms in the absence of fever should be evaluated for the presence of pulmonary hypertension.

Therapy with Cerezyme should be directed by physicians knowledgeable in the management of patients with Gaucher disease.

Caution may be advisable in administration of Cerezyme to patients previously treated with Ceredase® (alglucerase injection) and who have developed antibody to Ceredase or who have exhibited symptoms of hypersensitivity to Ceredase.
**Carcinogenesis, Mutagenesis, Impairment of Fertility**

Studies have not been conducted in either animals or humans to assess the potential effects of Cerezyme® (imiglucerase for injection) on carcinogenesis, mutagenesis, or impairment of fertility.

**Teratogenic Effects: Pregnancy Category C**

Animal reproduction studies have not been conducted with Cerezyme® (imiglucerase for injection). It is also not known whether Cerezyme can cause fetal harm when administered to a pregnant woman or whether it can affect reproductive capacity. Cerezyme should not be administered during pregnancy except when the indication and need are clear and the potential benefit is judged by the physician to substantially justify the risk.

**Nursing Mothers**

It is not known whether this drug is excreted in human milk. Because many drugs are excreted in human milk, caution should be exercised when Cerezyme® (imiglucerase for injection) is administered to a nursing woman.

**Pediatric Use**

The safety and effectiveness of Cerezyme® (imiglucerase for injection) have been established in patients between 2 and 16 years of age. Use of Cerezyme in this age group is supported by evidence from adequate and well-controlled studies of Cerezyme and Ceredase® (algucerase injection) in adults and pediatric patients, with additional data obtained from the medical literature and from long-term post-marketing experience. Cerezyme has been administered to patients younger than 2 years of age, however the safety and effectiveness in patients younger than 2 have not been established.

**ADVERSE REACTIONS**

Since the approval of Cerezyme® (imiglucerase for injection) in May 1994, Genzyme has maintained a worldwide post-marketing database of spontaneously reported adverse events and adverse events discussed in the medical literature. The percentage of events for each reported adverse reaction term has been calculated using the number of patients from these sources as the denominator for total patient exposure to Cerezyme since 1994. Actual patient exposure is difficult to obtain due to the voluntary nature of the database and the continuous accrual and loss of patients over that span of time. The actual number of patients exposed to Cerezyme since 1994 is likely to be greater than estimated from these voluntary sources and, therefore, the percentages calculated for the frequencies of adverse reactions are most likely greater than the actual incidences.

Experience in patients treated with Cerezyme has revealed that approximately 13.8% of patients experienced adverse events which were judged to be related to Cerezyme administration and which occurred with an increase in frequency. Some of the adverse events were related to the route of administration. These include discomfort, pruritus, burning, swelling or sterile abscess at the site of venipuncture. Each of these events was found to occur in < 1% of the total patient population.

Symptoms suggestive of hypersensitivity have been noted in approximately 6.6% of patients. Onset of such symptoms has occurred during or shortly after infusions; these symptoms include pruritus, flushing, urticaria, angioedema, chest discomfort, dyspnea, coughing, cyanosis, and hypotension. Anaphylactoid reaction has also been reported (see WARNINGS). Each of these events was found to occur in < 1.5% of the total patient population.

A nominal 5.0 mL for the 200 unit vial (10.0 mL for the 400 unit vial) is withdrawn from each vial. The appropriate amount of Cerezyme® for each patient is diluted with 0.9% Sodium Chloride Injection, USP, to a final volume of 100 – 200 mL. Cerezyme® is administered by intravenous infusion over 1-2 hours. Aseptic techniques should be used when diluting the dose. Since Cerezyme® does not contain any preservative, after reconstitution, vials should be promptly diluted and not stored for subsequent use. Cerezyme® after reconstitution, has been shown to be stable for up to 12 hours when stored at room temperature (25°C) and at 2-8°C. Cerezyme®, when diluted, has been shown to be stable for up to 24 hours when stored at 2-8°C.

Relatively low toxicity, combined with the extended time course of response, allows small dosage adjustments to be made occasionally to avoid discarding partially used bottles. Thus, the dosage administered in individual infusions may be slightly increased or decreased to utilize fully each vial as long as the monthly administered dosage remains substantially unaltered.

**HOW SUPPLIED**

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<td>400 Units per Vial NDC 58468-4663-1</td>
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Cerezyme® is manufactured by: Genzyme Corporation 500 Kendall Street Cambridge, MA 02142 USA

Certain manufacturing operations may have been performed by other firms.

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6LE0005D
Untreated type 1 Gaucher disease can have a severe effect on a patient’s bones. In patients whose Gaucher disease is untreated, bone abnormalities may be painful and debilitating, according to Elena Lukina, MD, a physician with the National Research Centre for Hematology in Moscow, Russia.

However, enzyme replacement therapy can mitigate some or all of these symptoms, so an early diagnosis is imperative, she said. Treatment with Cerezyme® (imiglucerase for injection) has been shown to improve the bone strength (bone mineral density) of many patients with type 1 Gaucher disease. (See Important Safety Information, below, and Product Information, centerfold.)

Still, the diagnosis can be challenging, according to Lukina, who was part of a panel of speakers at the American Society of Hematology annual meeting. The panel discussed various cases to increase awareness of type 1 Gaucher disease among hematologists.

“Sometimes the diagnosis of Gaucher disease is quite easy to perform, and sometimes it’s very difficult,” Lukina explained. “What can make the diagnosis difficult is that not all patients appear sick right away. Another problem is that some signs and symptoms of type 1 Gaucher disease are similar to other, more common diseases, so that patients may be misdiagnosed. For example, joint pain may be diagnosed as arthritis or growing pains.”

Type 1 Gaucher disease is a progressive, genetic disorder that can lead to many different blood and bone abnormalities. The disease occurs because a person cannot produce enough glucocerebrosidase, an essential enzyme that breaks down a fatty substance called glucocerebroside. As a result, the fatty substance builds up in the cells. These cells are called Gaucher cells.

Although this build-up causes many signs and symptoms, Lukina primarily discussed the damage to the skeleton. The precise way in which the build-up causes skeletal problems is not known, but the disease appears to affect the bone marrow and the minerals in the bone itself. Bone pain can be dull and achy or intense. Patients may suffer bone fractures and breaks.

“Early diagnosis and treatment can help,” Lukina said. “Cerezyme has been shown to help reduce or reverse many of the signs and symptoms of type 1 Gaucher disease. Early diagnosis and treatment is particularly important for avoiding some of the more serious symptoms related to bone, which may become irreversible if treatment is delayed.”

Meet the Experts
Preventing Bone Problems in Type 1 Gaucher Disease

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Please see accompanying full Prescribing Information on pages 9-10.
The idea of support groups for people dealing with chronic diseases is nothing new. Many people find that such groups offer a level of emotional support, beyond what can sometimes be provided by family and friends—namely, the opportunity to share with others who have truly “walked a mile in your shoes,” and really “get it,” when no one else seems to understand.

For those dealing with relatively common conditions, finding an appropriate support group is often as easy as clicking on a link or reading a flyer posted in a physician’s office. But for those with rare diseases, such as Gaucher disease, peer support can sometimes be more difficult to find.

Peer support is especially critical to those learning to cope with a disease such as Gaucher. After all, friends, neighbors, coworkers, and even family members may not even have heard of the disease, let alone be familiar with what patients with Gaucher disease need to deal with. This can intensify the sense of isolation felt by many who are diagnosed with a serious illness.

The good news is that such support does, indeed, exist. And with a bit of research, it is easy to find the kind of support that meets your needs.

No two people experience or cope with a disease in quite the same way, and the population of patients with Gaucher disease and their families is especially varied. For this reason, some people feel most comfortable communicating online or by phone, while others find that they benefit from the camaraderie of face-to-face encounters. Still others find that their emotional needs can be met by accessing resources that empower them with the information they need (see “Online Information Resources”).

Face-to-Face Support
Finding a group that meets in person and in your area may take a bit of detective work. Although an online search can be helpful, talking to your physician, nurse, social worker, or Genzyme Case Manager is a good place to start.

Depending upon your location, you may be able to find a group created specifically as a forum for discussion and support among patients and their families. In some cases, however, groups are created for educational purposes—for instance, medical practices that treat patients with Gaucher disease may host talks by experts on such topics as stress management or nutrition.

Betsy Simon was in her 20’s before meeting another person with Gaucher disease. That encounter was thanks to an educational meeting at a physician’s office, which she attended after receiving an invitation from a Genzyme Case Manager.

“It was an eye-opening experience to learn from others with this disease,” she said. “I really learned to appreciate my healthcare team after hearing what others had been through, and I enjoy sharing my experiences with other patients and helping to guide them through insurance hoops or give suggestions about finding good physicians.”

Acknowledging that it’s “sometimes difficult to hear the stories of those who suffered because they were diagnosed before treatment was available,” Simon loves going to patient meetings. And she even serves as a National Gaucher Foundation (NGF) peer-mentor for those recently diagnosed with the disease.

“Anna” (not her real name), who has attended educational meetings and similar gatherings on and off for many years, views them mainly as “an excuse to get together and talk about issues.” At the very least, the meetings tend to produce a sense of camaraderie. She does, however, stress the need for what she calls a good patient-group match. “A woman in her 60’s doesn’t want to sit and listen to someone talk about fertility issues,” she said, “and someone who isn’t dealing with treatment issues might not want to attend a group in which treatment is the topic of discussion.”

Online and Phone Support
For those who prefer online or phone communication, or who want to supplement face-to-face meetings with other forms of support, a number of resources are available.

The National Gaucher Foundation (NGF; http://www.gaucher disease.org; 800-504-3189) is a nonprofit organization for Gaucher-related support and information. NGF advocates for those dealing with Gaucher disease by funding research, supporting legislative issues, promoting education and awareness, and offering financial assistance. It also offers outreach programs and services aimed at
helping those in the Gaucher community cope with the day-to-day issues related to their diagnosis, symptoms, and treatments.

One such program links newly diagnosed individuals, and those with newly diagnosed family members, with peer-mentors trained to provide support over the phone or via e-mail. The mentors, all of whom either have Gaucher disease or have a family member with the disease, undergo two days of intensive training, learning about “all things Gaucher,” and also learning about the importance of confidentiality, explained NGF Marketing Director Rosina Papantonio.

Most patients learn about the program on the NGF website (where they can actually select a mentor), from their Genzyme Case Manager, or through word of mouth from healthcare professionals or other patients. No referral is needed; patients can just contact the NGF, which will then put them in touch with a mentor.

Mentors can discuss issues related to nearly every aspect of Gaucher disease (patients with medical questions are referred to one of the physicians on the NGF medical board). Often, a patient will use the time with his or her mentor to discuss family and relationship issues, the best way to talk with others about the disease, feelings about genetic transmission, and practical issues such as insurance coverage or finding the best care. And while some patients find that a single encounter is sufficient, others talk with their mentors (or converse via e-mail) as issues arise.

Simon, who considers her role as a NGF mentor as an opportunity to give back, noted that newly diagnosed patients typically have questions about how their treatments will affect their lives or the lives of their children. “People whose children have Gaucher are, of course, concerned about whether they’ll grow up healthy, and if they’ll be able to have children of their own,” she said.

Forming a Group
A diagnosis of Gaucher disease can be overwhelming, and patients may feel as if they’re being bombarded with information before they’ve even processed the reality of the diagnosis and how it will impact them.

Some people might not be able to find the type of support group that’s best for them. In that case, they sometimes start their own group that meets their needs.

While starting your own support group might sound like a daunting task, asking yourself some important questions can help you to get started:

• What do I want to accomplish in my group?
• Who do I want to include (patients, patients and family members, patients in a specific age group, etc)?
• Do I want to hold my group online or in person? If in person, how many people should attend each session? Where will the sessions be held? What type of support will be needed? How can I get the word out? Who will run the group? If the group will be conducted online, who will manage, host, and sponsor the website?

When beginning any new endeavor, it’s always a good idea to look at what others are doing—to see what works and what doesn’t. And keep in mind that help is available. The NGF provides advice and free printed educational materials.

Online Information Resources
Here’s a list of some online resources and groups offering information (and, in some cases, additional types of support) related exclusively to Gaucher disease. These Web pages and their content are maintained by the organizations listed below.*

Cerezyme.com (www.cerezyme.com)
Created by Genzyme for people living with or caring for someone with Gaucher disease, this site provides supportive information about the condition and treatment of type 1 Gaucher disease, as well as links and other Gaucher-related information.

Children’s Gaucher Research Fund (www.childrensgaucher.org)
This nonprofit organization raises funds to coordinate and support research aimed at finding a cure for types 2 and 3 Gaucher disease, as well as providing support to families.

GaucherCare.com (www.gauchercare.com)
This Genzyme-sponsored site was created to help people living with Gaucher, as well as their friends and family members. It is also intended to educate healthcare professionals about the disease and available resources.

National Gaucher Foundation (www.gaucherdisease.org)
This national not-for-profit patient organization offers a broad range of comprehensive educational and support services and programs for the Gaucher community.

* With the exception of Genzyme websites, Genzyme does not endorse any particular organizations or the content contained on those websites.

Online Support Group Websites
NGF Facebook Discussion Group (http://www.facebook.com/pages/National-Gaucher-Foundation/333921202263)

Yahoo Gaucher Disease Group (health.groups.yahoo.com/group/gaucherdisease) This group deals with Gaucher disease and related disorders.

Other groups can be found with a simple Internet search. The key is to participate only in a group whose reputation you know and trust. When in doubt, contact the NGF or ask a healthcare professional.
Kim Keegan

By Cheryl Alkon

Kim Keegan knows what it’s like to be tough. As a child, she experienced bruising, fatigue, low blood platelet counts, and an enlarged liver and spleen. At age 9, a bone marrow test identified the cause of her symptoms, type 1 Gaucher disease.

But the test didn’t scare Keegan, now 42, of Schenectady, New York. Although the procedure involves inserting a long needle deep into the lower back and taking a sample of bone marrow, Keegan recalled she didn’t cry or scream. “The doctor and nurses were so amazed. I got extra lollipops when I was finished.”

Type 1 Gaucher disease occurs when the body does not produce a particular enzyme, known as glucocerebrosidase, which helps process fatty byproducts. Unchecked, the fats build up in the body, causing the spleen and liver to enlarge. Type 1 Gaucher disease can also affect the bones, causing bone pain, and the blood, causing low platelet counts and low red blood cell counts, which bring on bruising/easy bleeding and fatigue.

“I couldn’t play on the playground with other kids because my parents and teachers were afraid if I got hit in the stomach or fell that my spleen would rupture,” Keegan explained. Instead of sliding down slides or swinging or climbing on the monkey bars, she would sit inside, pretending to read, but instead watching the other kids having fun.

Finding Support Where She Could

“I knew I was different, but at that age I didn’t understand why,” Keegan said, “nor did the other kids. So bullying and being picked on were a constant for me in grade school and in high school. Kids would make fun of my swollen belly and say to the other kids that I was pregnant.”

Thankfully, not all of the children were unkind and insensitive to her challenges. “Other kids felt sorry for me, and some would sit at the table with me during play time,” she said. “I formed a special bond with these kids, and we are still friends to this day.” Later on, in high school, Keegan would connect with those who understood what she was dealing with. She didn’t interact with the athletic kids, and her grades were poor so she didn’t fit in with the smart kids, according to Keegan.

“I learned to be funny, outgoing and tough, and I managed to make friends with some of the popular, the musically talented, and the oddball kids,” she said. “They protected me because they knew about my health condition and took me under their wing, so to speak.” Decades later, these people are also still close friends.

A Family of Fighters

Learning how to find the right situation for herself—be it her friends from school, classes that helped her focus, or later, the right employer—has helped Keegan determine how to survive and thrive while being surrounded with type 1 Gaucher disease. Keegan’s mother, Colleen, lived with the disease her whole life; her subsequent illnesses, which included infections, spinal meningitis, and bones cracking from simply walking up steps, kept her hospitalized for long periods of time. Keegan’s aunt, Sylvia McGahay, and her late great-grandmother, Grandma Smith, would come to help care for Keegan and her younger siblings, Melissa and Richard, Jr., who later were also diagnosed with type 1 Gaucher disease.

Keegan was diagnosed in the late 1970s, when treatment was not yet available. As a result, she lived with the disease’s unrelenting effects. Low red blood cell counts left her continually exhausted. “I had trouble staying awake in class, and I always fell asleep, especially in English and Social Studies,” she recalled. “When you’re that tired, you can’t concentrate. I was sent to the nurse’s office a lot, and was often in trouble for falling asleep and missing a lot of school.” The only things that held her interest were music, choral, and photography classes. “Anything that had to do with moving around,” she said.

Thinking back, Keegan now says she wishes she had had a tutor to help her, but at the time, “I didn’t even know there was one,” she said.

Eventually, she began skipping school because she had so much trouble staying awake, and it put her graduation in question. Her mother had to go to the school and threaten legal action so that Keegan could graduate on time, which she did.

Keegan’s parents were fighters, too. Her father, Richard Keegan, was an Albany police detective who helped Colleen receive experimental treatment with Ceredase® (alglucerase injection), before it was approved by the Food and Drug Administration. “Because of Genzyme and my Dad’s persuasiveness, Mom got the medication that saved her life,” said Keegan. “My mom was sick most of my life, and our family spent a lot of time at the hospital. Although she was ill, she was the best mother anyone could ask for—she took us...
places, played with us, and spoiled us rotten, so my Dad would say."

Keegan credits her mother’s ability to embrace life despite her illness as a life lesson, calling her “the most courageous, loving, caring, fighting, beautiful spitfire of a lady you could ever meet.”

“The person I am today, I owe all to my mother from watching her struggle and seeing how much she enjoyed and loved life, especially people,” said Keegan. “She treated others and everyone around her with such care and compassion.”

Her mother’s fighting spirit was evident at Keegan’s milestone 40th birthday party in early 2010.

“She planned the party and stayed for the entire night, as sick as she was, even though at the time she did not know she had stage 4 lung cancer,” remembered Keegan. Her mother would learn she had cancer, which wasn’t related to her type 1 Gaucher disease, Keegan believes, just a few weeks later. Colleen passed away that April. “That sums my mom up: No matter what, no matter how much she suffered or how sick she was, she had to be there for her family,” said Keegan.

**Working for Her Life**

After graduating from high school and attending college classes while working full time for insurance coverage, Keegan found that she had to advocate for herself at just about every job she had.

“Working for larger corporations was brutal for me,” she said. “I spent a lot of time in the human resources department for being out of work a lot due to being sick. I was always looked at as a nuisance, rather than a good employee, which meant I had to work even harder to make up for the times I did not feel well.”

Keegan began receiving Cerezyme® (imiglucerase for injection; see Important Safety Information, below, and Product Information, centerfold) in 1992, and began working for her current employer, a retirement plan administration service called Creative Pension Consultants in Albany, New York, in 2000.

“It is a locally owned family business, and for the first time, I found a company to work for that is actually sympathetic and understanding in regard to my health,” she said. In turn, Keegan said her health history has made her sympathetic to clients who call the company and need to take out loans against their retirement plans, for whatever reason. “I’ve seen and heard it all,” she said.

When it comes to her health needs today, Keegan works with the company’s owner each year to select a health insurance plan that will cover her Cerezyme® treatment. She also works from home on the days she receives treatment, as well as whenever she is sick or too fatigued to commute.

“There is something to be said for working for a small company,” said Keegan. “To them, you are not just an employee number as you are with a big corporation. You are actually a person.”

Since she receives infusion therapy regularly, Keegan said she spends the time watching a lot of films and listening to music—paralleling the music and photography classes that she enjoyed the most in school. "I watch a lot of movies—cop movies, funny comedies. "Walk the Line”—I love that movie. And I love country music, Alice Cooper, and 80s “hair bands” like Led Zeppelin.” Legally separated, Keegan spends her free time caring for her two dogs, a beagle named Fredo and an Australian shepherd-black lab mix named Sophie.

**Sharing Her Insights for Others With Gaucher Disease**

When she thinks of others living with type 1 Gaucher disease, Keegan hopes that children aren’t experiencing the bullying that she dealt with, and that adults aren’t faced with the employment issues she had to deal with when having to explain why she needed sick days off, before finding her more understanding current employer.

“But the challenges I have experienced have made me into the caring, understanding, strong, empathetic woman that I am today,” she said. “If someone told me I couldn’t do something, I basically did it anyway, or at least tried to.”

Keegan urges others living with type 1 Gaucher disease to embrace the toughness she’s cultivated. “Act and believe that living with a health condition should make you a stronger person, as we face challenges every day that others do not, nor can relate to,” she said. “Live as though the sky is the limit, and reach for the stars even though it is harder for us to reach, as we most likely will be fatigued, anemic, and bruised up, and will definitely need to rest!”

**Indications and Usage**

Cerezyme® (imiglucerase for injection) is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia (low red blood cell count), thrombocytopenia (low blood platelet count), bone disease, hepatomegaly or splenomegaly (enlarged liver or spleen).

**Important Safety Information**

Approximately 15% of patients have developed immune responses (antibodies). These patients have a higher risk of an allergic reaction (hypersensitivity). Use Cerezyme® (imiglucerase for injection) carefully if you have had an allergic reaction to the product in the past. Symptoms suggestive of allergic reaction happen in 6.6% of patients, and include anaphylactoid reaction (a serious allergic reaction), itching, flushing, hives, an accumulation of fluid under the skin, chest discomfort, shortness of breath, coughing, cyanosis (a bluish discoloration of the skin due to diminished oxygen), and low blood pressure. Side effects related to Cerezyme administration have been reported in less than 15% of patients. Each of the following events occurred in less than 2% of the total patient population. Reported side effects include nausea, abdominal pain, vomiting, diarrhea, rash, fatigue, headache, fever, dizziness, chills, backache, and rapid heart rate. Because Cerezyme therapy is administered by intravenous infusion, reactions at the site of injection may occur: discomfort, itching, burning, swelling or uninfected abscess. Cerezyme is available by prescription only.

For more information, consult your physician. To learn more, please see the enclosed full product information or contact Genzyme at 1-800-745-4447 (option 2).

**Please see accompanying full Prescribing Information on pages 9-10.**
Genzyme Co-Pay Assistance Program
Cerezyme® (imiglucerase for injection)

Get Started Today in 3 Easy Steps!

1. You complete the program application

For more information about the program and to complete the online application, please visit: www.cerezyme.com/copay.aspx
You can also call your Genzyme Case Manager directly to learn more about the program and application process at 1-800-745-4447, Option 3

2. Your Genzyme Case Manager verifies eligibility

Your Genzyme Case Manager will review your application to verify eligibility. If you are eligible, you will be automatically enrolled in the program. Enrollment in the program is subject to confirmation of eligibility.

3. You’re enrolled

Once approved, you will receive confirmation from your Genzyme Case Manager and an enrollment card will be mailed to you within 7-10 days. Your doctor or specialty pharmacy will also receive a confirmation letter with instructions on how to submit claims for reimbursement through the program. Your enrollment in the program is effective from the date of approval through the end of 2012.

Genzyme reserves the right to make eligibility determinations, to set program benefit maximums, to monitor participation, and to modify or discontinue the program at any time.

Genzyme Co-Pay Assistance Program

The Genzyme Co-Pay Assistance Program will help eligible individuals who are prescribed treatment with Cerezyme® (imiglucerase for injection) with their eligible drug related out-of-pocket expenses, including co-pays, co-insurance and deductibles, regardless of financial status.

Once enrolled in the Genzyme Co-Pay Assistance Program, Genzyme will pay 100% of your eligible out-of-pocket Cerezyme drug costs up to the program maximum. The 2012 Co-Pay Program runs from January 1, 2012 through December 31, 2012.

Who is eligible for this program?
Regardless of financial status, the program is open to individuals who are:
- U.S. citizens or legal residents who have primary commercial insurance
- Prescribed treatment with Cerezyme® (imiglucerase for injection)

Who is NOT eligible?
As required by law, the program is not available to individuals who:
- Are residents of Massachusetts
- Have coverage or prescriptions paid for in part or full under any state or federally funded healthcare program including:
  - Medicare
  - Medicare Advantage Plans (Example: FreedomBlue offered through Blue Cross Blue Shield)
  - Medicaid
  - Medigap
  - Veterans Affairs, Department of Defense or Tri Care
  - High Risk Pool or Pre-existing Condition Insurance Plan (PCIP)

Please call your Case Manager if you have any questions about your eligibility. If you are not eligible for our Co-Pay Assistance Program and need help with your out-of-pocket expenses, your Genzyme Case Manager is available to help review your coverage options and refer you to other financial assistance programs that may offer financial support for eligible individuals.

Genzyme reserves the right to make eligibility determinations, to set program benefit maximums, to monitor participation, and to modify or discontinue the program at any time.
This program assists patients with their out-of-pocket Cerezyme drug costs only, not the cost of infusions, medical evaluations/appointments, testing, or other related services.

For full Prescribing Information for Cerezyme® (imiglucerase for injection) go to www.cerezyme.com