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Horizons

A Newsletter for the Gaucher Community *From the Genzyme Corporation*

How Will Health Care Reform Affect You?

Everything you need to know about the historic law and how it may affect you and your family.

Understanding the Cerezyme® (imiglucerase for injection) Supply Shortage

Family Assistance Program

A Free Service for Patients with Type 1 Gaucher Disease

Patient Stories

An Artist with Type 1 Gaucher Disease Thrives

A Patient's Support Network Helps Manage His Disease

www.gauchercare.com
www.cerezyme.com

NOW RECRUITING

Genzyme is currently sponsoring two worldwide PHASE 3 STUDIES

Eliglustat tartrate (Genz-112638) is an investigational oral drug which aims to partially inhibit the production of glucosylceramide (GL-1) in Gaucher cells

The ENGAGE and ENCORE studies are designed to determine the safety and efficacy of eliglustat tartrate (Genz-112638) in patients with Gaucher disease type 1.

The ENGAGE study is recruiting patients 16 to 65 years of age with splenomegaly and anemia and/or thrombocytopenia and who have never been or currently not being treated for their disease. The ENCORE study is recruiting clinically stable patients 18 to 65 years of age who have been treated with imiglucerase for injection for at least 3 years.

These studies require:

- Patients to have a confirmed diagnosis of Gaucher disease type 1
- Female patients to use a medically accepted method of contraception
- Patients to be excluded if they have a clinically significant disease, other than Gaucher disease type 1

To participate or learn more about these studies, contact:

- **www.clinicaltrials.gov** search
  ENGAGE: NCT00891202 ENCORE: NCT00943111
- **Genzyme Medical Information** at **1-800-745-4447** (option 2) or **medinfo@genzyme.com**

One of the world's leading biotechnology companies, Genzyme is dedicated to making a major positive impact on the lives of people with serious diseases.

GZGD-08-0210

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Contents

Foreword

Kathleen Coolidge

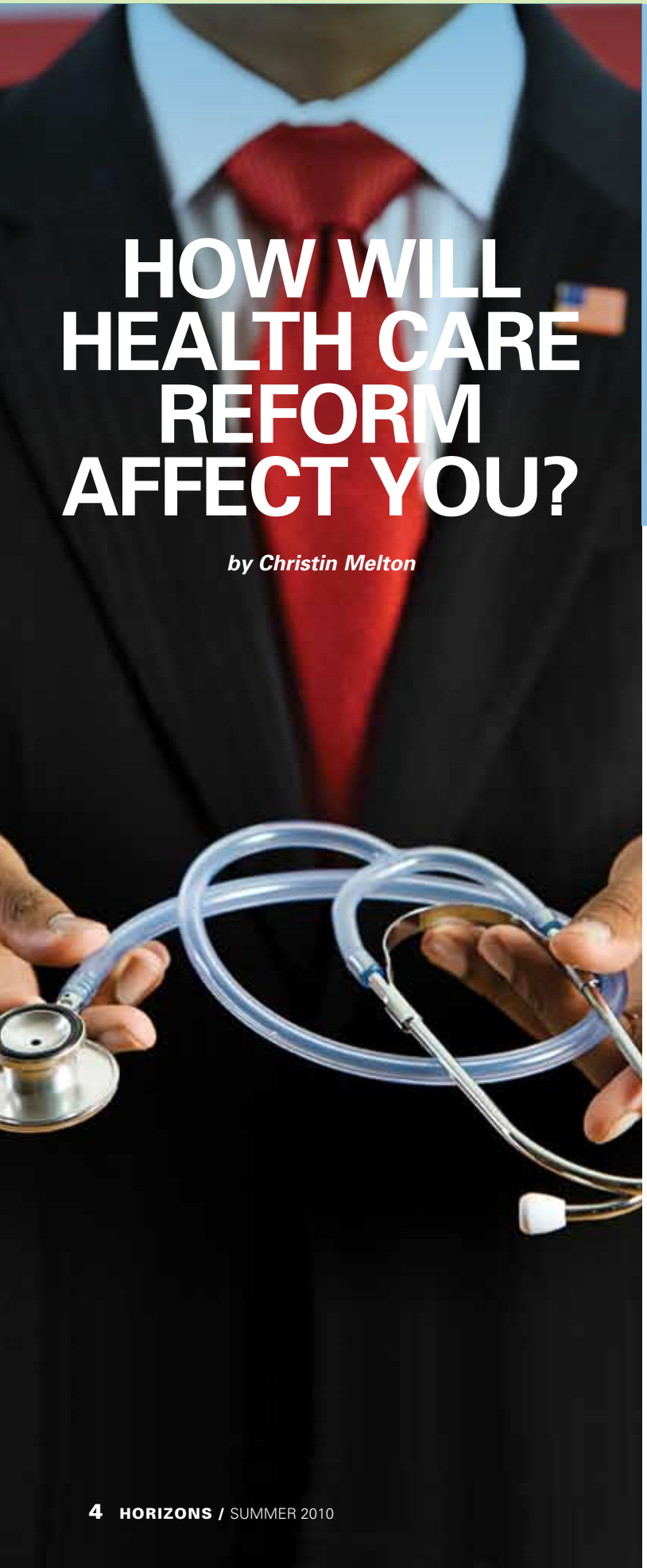
In this issue of *Horizons*, we feature an article on the historic health care reform legislation that President Obama signed into law in March. The new law left many people wondering how it could affect them and their families. The article on page 4 is designed to inform you on the reforms, as well as how—and when—they may affect you.

In the following pages, you'll also find an update on the Cerezyme supply shortage to help you understand the situation. All of us at Genzyme know the critical importance of keeping you updated on the status of Cerezyme® (imiglucerase for injection) supply, not just in the near term, but also in the long term, for your treatment and care.

This issue also includes information on the Family Assistance Program, a free service available to patients with type 1 Gaucher disease to help them deal with stress and personal issues.

We want to hear from you. If you have questions or feedback, please contact Kathleen Coolidge, Associate Director of Patient Advocacy at Genzyme, at Kathleen.Coolidge@genzyme.com.

Thank you and we look forward to hearing from you.



HOW WILL HEALTH CARE REFORM AFFECT YOU?

by Christin Melton

In March, President Barack Obama signed into law HR 3590, "The Patient Protection and Affordable Care Act," and HR 4872, "The Health Care and Education Affordability Reconciliation Act of 2010," two laws passed by Congress with the goal of reducing costs and improving access to health insurance and health care services in America. The Congressional Budget Office (CBO) predicts that the reform package will extend health care coverage to nearly 32 million uninsured Americans at a cost of $938 billion over the next decade. The CBO also projects that the law will reduce the deficit by $130 billion over the next 10 years and by $1.2 trillion in the following decade.

Now that the health care reform legislation has become law, you should know how it could affect you and your family. This article describes some of the changes that might affect you or your family members. Some of theses changes start in 2010 and others phase in between now and 2014 or, in a few cases, even later. The health care reform bills encompass more than 2,400 pages, so it will take time to figure out all their provisions (**Table 1**). How the laws affect individuals depends on personal factors such as their health and employment status, insurance status and options, age, the age of their children, income, and whether they own a small or large business. Your Genzyme Case Manager can help by reviewing your current health insurance plan to determine how these changes will impact your coverage (**Table 2**).

Importantly, the new law prevents insurers in all states from denying coverage to people with preexisting conditions, capping lifetime and annual benefits, and dropping policyholders who become ill; implements interim high-risk pools in 2010 for uninsured individuals who have pre-existing conditions; and requires states to establish insurance exchanges by 2014, where individuals and small businesses can shop for a plan offered in their state by private insurance companies that meet benefits package and other minimum eligibility requirements.

Beginning in 2014, the new law requires that every American citizen and legal resident have health insurance or pay a penalty. Low-income uninsured people who cannot afford a plan offering a minimum benefits package will receive tax credits to offset their premiums and copayments. The goal is to give incentive to healthy individuals who forgo health insurance because of its expense to purchase insurance, which will help insurers offset and absorb the expense of paying for the medical needs of people with chronic or expensive illnesses and will reduce the burden of free care and bad debt borne by health care providers.

Near-Term Benefits for the Uninsured

As of July 1, 2010, people denied coverage because of a preexisting condition have the option of purchasing insurance through an interim high-risk pool in their state, provided they were uninsured for the previous six months. The U.S. Department of Health and Human Services (HHS) will set up high-risk pools in states that fail to establish one by July 1, 2010.

Plans in this pool are required to cover at least 65% of the cost of care. Premium rates must be equal to what an average person of the same age would pay in the private market, and premiums for the oldest subscribers can be no more than four times the premiums for the youngest subscribers. The yearly maximum out-of-pocket limit for these plans will be $5,950 for an individual and $11,900 for families. The federal government will provide subsidies on a sliding scale for those who cannot afford the premiums. Kaiser Permanente, headquartered in Menlo Park, California, offers an on-line calculator that estimates what subsidy amount you might be eligible to receive (see http://healthreform.kff.org/SubsidyCalculator.aspx).

The interim high-risk pools will be available until 2014, when the state insurance exchanges are scheduled to be operational. A drawback of these interim pools is that individuals paying higher premiums in an existing state high-risk pool or a private policy would have to drop coverage for six months before buying a policy from the interim pool—a risky proposition for someone with a preexisting illness.

Benefits for All

People facing a chronic illness or a life-threatening condition such as cancer worry about reaching their plan's annual or lifetime coverage limit or being dropped by their insurer. As of September 2010, those worries will ease, because insurers in all states will no longer be permitted to impose lifetime benefit maximums or drop enrollees who get sick and, further, they must reduce the use of annual benefit maximums.

Beginning in 2014, older subscribers cannot be charged more than three times the premium rate that younger subscribers pay. Nor can women be charged higher rates than men. The only factors that insurers will be permitted to use to adjust premiums are age, place of residence, family size, and tobacco use. In 2014, policies can no longer include annual benefit limits.

Insuring Children

This September, provisions take effect to prohibit insurers from denying coverage to children because of a preexisting condition. In addition, dependent children will be permitted to remain on the insurance plan of a parent or guardian until they are 26 years old. In 2014, adults who fail to provide coverage for their minor dependents will be fined, unless they meet specified criteria, such as a hardship or religious exemption.

Preventing Disease

The new health care reform legislation implements tools designed to relieve the burden of disease on the economy by guaranteeing access to services intended to prevent disease or treat it early on, before complications arise. For example, as of September 2010, new private policies must cover 100% of preventive services and immunizations, and these will no longer be subject to deductibles. All plans must comply with this requirement by 2018. As another example, the law increases Medicaid payments for primary care services to encourage the provision of disease prevention and wellness services by primary care physicians who treat Medicaid patients.

The law also funds several new prevention programs, such as an evidence-based campaign overseen by the Atlanta-based Centers for Disease Control and Prevention (CDC) to increase awareness of breast health and breast cancer in younger women and a campaign to prevent oral diseases, including oral cancer. In addition, HHS will fund a five-year pilot Wellness Program from 2010 through 2014 that covers public programs to screen people aged 55 to 64 years for cancer, stroke, and diabetes; assists the uninsured in finding coverage; and provides referrals for tobacco cessation and other programs.

Medicare Recipients

The most immediate benefit for seniors who have drug coverage through a Medicare Part D prescription plan is a $250 rebate in 2010 for those who fall into the "donut hole" (i.e., the gap between $2,700 and $6,000 of drug expenses, when seniors must pay 100% of drug costs before Medicare Part D coverage resumes). Beginning in 2011, Medicare Part D subscribers will receive a 50% discount on brand-name drugs during this coverage gap and the coverage gap for generic drugs also will begin to close. By 2020, the donut hole will be phased out entirely.

Other Medicare benefits that take effect in 2011 include a free annual wellness visit and the elimination of copayments for preventive services, such as mammograms, colonoscopies, and other recommended screening tests. Furthermore, preventive services will no longer be subject to deductibles. The law provides new funding to hospitals in counties with the lowest Medicare spending and authorizes a 10% bonus payment to general practitioners and surgeons working in underserved areas. The "frontier provision" raises reimbursement rates for outpatient services (2010) and inpatient services (2011) at hospitals in Wyoming, Montana, North Dakota, South Dakota, and Utah and for physician services (2011) in those same states.

While health reform does authorize new Medicare benefits and funding, Medicare faces $400 billion in cuts over the next 10 years, including $132 billion in cuts to Medicare Advantage plans. The Medicare Advantage program pays 14% more to private insurers to cover enrollees than what it

(Continued on page 10)

Table 1

Health Care Reform

How It Affects Everyone

In 90 Days:
- Adults denied coverage because of a preexisting condition can get insurance through an interim high-risk pool until 2014 provided they have not had insurance for the previous 6 months.
- A 10% tax will be charged to consumers of indoor tanning services.

In 6 Months:
- Lifetime benefit maximums are prohibited and annual maximums are curtailed.
- Plans can no longer exclude children for preexisting conditions.
- Dependent children can remain on their parent/guardian's plan until 26 years of age.
- New private policies must cover 100% of preventive services and immunizations, which will not be subject to deductibles. (Preventive services are those recommended by USPSTF with a grade of A or B.) All plans must comply by 2018.
- New subscribers must have access to effective internal and external appeals processes.
- Subscribers cannot be dropped when they become sick.
- No prior approval will be required to see a gynecologist or go to the emergency room.
- Adoption credit is increased by $1000 per eligible child.

January 1, 2011:
- Insurers must spend 80% to 85% of premiums on medical services or give subscribers premium rebates.
- Distributions from a health savings account that are not used for qualified medical expenses will be subject to a 20% tax.
- Through payroll deductions, employees can voluntarily contribute to a long-term care insurance program for adults who become functionally disabled.
- States can require insurance companies to justify requested premium increases; companies that have excessive rate hikes may be excluded from the health insurance exchanges.

January 1, 2013:
- The health flexible spending account annual maximum cannot exceed $2500.
- The threshold for itemizing medical expenses increases from 7.5% of income to 10% for people aged <65 years.
- Medical devices manufacturers will pay a 2.3% excise tax on sales of devices (not including eyeglasses, contact lenses, hearing aids, and everyday items bought at the drugstore), which could be passed on to consumers.

January 1, 2014:
- Self-employed and uninsured individuals can select a private plan from a state-based insurance exchange or a national private plan.
- Adults with preexisting conditions cannot be refused coverage or charged more.
- Policies cannot include annual limits.
- Subscribers cannot be dropped when they get sick nor can they be charged more.
- Premiums for older subscribers cannot exceed three times the premium rate for younger subscribers.
- Premiums can vary only by age, place of residence, family size, and tobacco use.
- Subscribers who want an abortion coverage rider must pay for it out-of-pocket (except in cases of rape, incest, or to protect the life of the mother).
- Penalties go into effect for individuals who do not have coverage or fail to provide coverage for their dependents, unless they meet specified criteria.

How It Affects High-Income Adults

January 1, 2013:
- The Medicare payroll tax for couples earning >$250,000 and individuals earning >$200,000 will increase on wages above those thresholds from the current 1.45% to 2.35%.
- These same earners are subject to a 3.8% tax on income from investments.

How It Affects Businesses

Immediately:
- Small businesses (<50 employees* and average annual wages ≤$40,000) that provide group insurance will receive tax credits of up to 35% of premiums as of January 1, 2010.

January 1, 2011:
- A temporary reinsurance program is established to offset costs for companies that provide health benefits to early retirees aged 55 years to 64 years.
- Employers must include the value of health benefits on employees' W2s.

January 1, 2013:
- Employers will no longer receive a tax deduction for subsidizing prescription drug plans for Medicare Part D–eligible retirees.

January 1, 2014:
- Small business tax credits will cover up to 50% of premiums.
- Small businesses can pick a private plan from a state-based insurance exchange.
- Large employers (≥50 employees*) who do not offer coverage or who offer unaffordable coverage will pay a penalty if any full-time employee purchases insurance through a state exchange or receives a subsidy.
- Large employers (≥200 employees*) must automatically enroll new employees in the health insurance plan but give them notice and an opportunity to opt out.
- Large employers (≥50 employees*) will be assessed a penalty for imposing waiting periods >30 days for insurance to become effective.

*Contracted employees, 2% shareholders, and 5% owners are not included in this total.

How It Affects Seniors on Medicare

Immediately:
- Subscribers subject to the Medicare Part D donut hole will receive a $250 rebate.

January 1, 2011:
- Medicare Part D subscribers will receive a 50% discount on brand-name drugs in the donut hole.
- Medicare will no longer charge copayments for preventive services and they will no longer be subject to deductibles. (Preventive services are those recommended by USPSTF with grade A or B.)
- Subscribers will receive one free wellness visit per year.

January 1, 2016:
- The threshold for itemizing medical expenses increases from 7.5% of income to 10%.

January 1, 2020:
- Donut hole will be closed but seniors will be responsible for 25% of prescription costs until catastrophic coverage kicks in.

How It Affects Low-Income Adults Not Eligible for Medicare

In 6 Months:
- States will have the option of offering disabled Medicaid recipients home- and community-based care instead of institutional care.

January 1, 2011:
- Covers 100% of comprehensive tobacco cessation services for pregnant women enrolled in Medicaid.

January 1, 2014:
- Childless adults earning 133% of Federal Poverty Line can apply for Medicaid.
- Premiums will be capped at 2%-3% of income for those earning ≤133% of Federal Poverty Line and
- Out-of-pocket spending on health care will be capped at ~12% of income.

6743 (4/05)

Cerezyme®

(imiglucerase for injection)

200 UNITS **400 UNITS**

DESCRIPTION

Cerezyme® (imiglucerase for injection) is an analogue of the human enzyme ß-glucocerebrosidase, produced by recombinant DNA technology. ß-Glucocerebrosidase (ß-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 mannose 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:

Ingredient	200 Unit Vial	400 Unit Vial
Imiglucerase (total amount)*	212 units	424 units
Mannitol	170 mg	340 mg
Sodium Citrates	70 mg	140 mg
(Trisodium Citrate) (Disodium Hydrogen Citrate)	(52 mg) (18 mg)	(104 mg) (36 mg)
Polysorbate 80, NF	0.53 mg	1.06 mg
Citric Acid and/or Sodium Hydroxide may have been added at the time of manufacture to adjust pH.		

*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-ß-D-glucopyranoside (pNP-Glc) per minute at 37°C. The product is stored at 2-8°C (36-46°F). 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 ß-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 alglucerase (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 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® (alglucerase 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. Pre-treatment with antihistamines and/or corticosteroids and reduced rate of infusion have allowed continued use of **Cerezyme®** in most patients.

Additional adverse reactions that have been reported in approximately 6.5% of patients treated with **Cerezyme®** include: nausea, abdominal pain, vomiting, diarrhea, rash, fatigue, headache, fever, dizziness, chills, backache, and tachycardia. Each of these events was found to occur in < 1.5% of the total patient population.

Incidence rates cannot be calculated from the spontaneously reported adverse events in the post-marketing database. From this database, the most commonly reported adverse events in children (defined as ages 2 – 12 years) included dyspnea, fever, nausea, flushing, vomiting, and coughing, whereas in adolescents (>12 – 16 years) and in adults (>16 years) the most commonly reported events included headache, pruritis, and rash.

In addition to the adverse reactions that have been observed in patients treated with **Cerezyme®**, transient peripheral edema has been reported for this therapeutic class of drug.

OVERDOSE

Experience with doses up to 240 U/kg every 2 weeks have been reported. At that dose there have been no reports of obvious toxicity.

DOSAGE AND ADMINISTRATION

Cerezyme® (imiglucerase for injection) is administered by intravenous infusion over 1-2 hours. Dosage should be individualized to each patient. Initial dosages range from 2.5 U/kg of body weight 3 times a week to 60 U/kg once every 2 weeks. 60 U/kg every 2 weeks is the dosage for which the most data are available. Disease severity may dictate that treatment be initiated at a relatively high dose or relatively frequent administration. Dosage adjustments should be made on an individual basis and may increase or decrease, based on achievement of therapeutic goals as assessed by routine comprehensive evaluations of the patient's clinical manifestations.

Cerezyme® should be stored at 2-8°C (36-46°F). After reconstitution, **Cerezyme®** should be inspected visually before use. Because this is a protein solution, slight flocculation (described as thin translucent fibers) occurs occasionally after dilution. The diluted solution may be filtered through an in-line low protein-binding 0.2 µm filter during administration. Any vials exhibiting opaque particles or discoloration should not be used. DO NOT USE **Cerezyme®** after the expiration date on the vial.

On the day of use, after the correct amount of **Cerezyme®** to be administered to the patient has been determined, the appropriate number of vials are each reconstituted with Sterile Water for Injection, USP. The final concentrations and administration volumes are provided in the following table:

	200 Unit Vial	**400 Unit Vial**
Sterile water for reconstitution	5.1 mL	10.2 mL
Final volume of reconstituted product	5.3 mL	10.6 mL
Concentration after reconstitution	40 U/mL	40 U/mL
Withdrawal volume	5.0 mL	10.0 mL
Units of enzyme within final volume	200 units	400 units

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

Cerezyme® (imiglucerase for injection) is supplied as a sterile, non-pyrogenic, lyophilized product. It is available as follows:

200 Units per Vial NDC 58468-1983-1
400 Units per Vial NDC 58468-4663-1

Store at 2-8°C (36-46°F).

Rx only

U.S. Patent Numbers: 5,236,838
5,549,892

Cerezyme® (imiglucerase for injection) 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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Table 2

Genzyme Care Coordination: Your Connection to Health Care Reform Information

With 20 years of experience providing personalized reimbursement assistance to individuals living with rare genetic disorders, Genzyme Case Managers are available to help you learn more about health care reform and how it may impact your insurance coverage.

Using our expertise and knowledge of the reimbursement process, we examined the Patient Protection and Affordable Care Act (health care reform bill) to identify 10 important changes that may impact you and your family:

1. **Short-term high-risk pool**
   Effective June 21, 2010
   US citizens and legal residents who have been uninsured for at least six months because of a pre-existing condition will be eligible to enroll in a temporary high-risk pool.
2. **Dependent child status extended to age 26**
   Effective for new plan years beginning on or after September 23, 2010. Young adults may qualify for an open enrollment period to join their parents' plan on or after September 23, 2010.
   New health plans and certain grandfathered plans will allow dependents up to age 26 the option to remain on their parents' insurance policy. Until 2014, this is not applicable to adult children who have access to their own employer's insurance offerings.
3. **Children with pre-existing conditions**
   Effective on September 23, 2010
   Health insurance plans cannot deny coverage to children under 19 with pre-existing conditions. Starting in 2014, this will apply to people of all ages.
4. **No lifetime maximums, limits or caps on coverage**
   Effective for new plan years beginning on or after September 23, 2010. For calendar year plans, it will take effect on January 2, 2011. For most plans this will take effect on your plan's renewal date.
   Bans health insurance companies from putting lifetime maximums on coverage for essential benefits.
5. **Restricted annual limits on coverage**
   Effective on September 23, 2010
   Prior to January 2014, plans may impose restricted annual limits on essential benefit coverage only as permitted by the Secretary of Health and Human Services. Starting in 2014, the use of any annual limits on essential benefit coverage will be banned.
6. **No Rescissions on coverage**
   Effective on September 23, 2010
   If you get sick, health insurance companies cannot drop you from their coverage.
7. **Medicare Part D donut hole rebate & phase-out**
   Effective beginning calendar year 2010
   Medicare Part D beneficiaries who hit the donut hole in 2010 will receive a $250 rebate check. Starting in 2011, the law mandates a 50% discount on brand-name prescription drugs in the donut hole and the donut hole will gradually shrink until fully eliminated in 2020, although co-pays will remain.
8. **Free preventive care**
   Effective for new private plans written on or after September 23, 2010
   New Private Plans: Preventative services cannot be subject to co-pays or deductibles.
   Effective starting January 1, 2011
   Medicare Program: Preventative services will no longer be subject to co-pays or deductibles.
9. **Premium payment requirements for insurers**
   Effective on January 1, 2011
   Individual and small group plans will have to spend 80 percent of premium dollars on medical services while large group plans will have to spend 85 percent. If these percentages are not met, the plan is required to provide rebates to policyholders.
10. **Requirement to have health insurance coverage**
    Penalties for not having coverage start in tax year 2014 (Some states already require insurance coverage)
    US citizens and legal residents are required to have minimal essential health insurance coverage for themselves and their dependents unless they meet specific criteria. This coverage can be purchased through employers or through new exchanges that will be set up by 2014. Subsidies will be available to eligible individuals and families.

How can my Genzyme Case Manager help?

Your Genzyme Case Manager can help by reviewing your current health insurance plan to determine how these changes will impact your coverage. As these changes take place, your Genzyme Case Manager will work with you throughout the process to help ensure a smooth transition, especially during open enrollment periods. If you have any questions at all, please call your Genzyme Case Manager at **1-800-745-4447, Option 3**.

The information provided in this resource is an interpretation of the Patient Protection and Affordable Care Act (PPACA).
More information can be found at www.HealthReform.gov.
Please note that some effective dates may vary as the terms of your individual insurance plan and the state where you live may influence how the PPACA affects you.

(Continued from page 5)

costs to cover them through traditional Medicare. To reduce this disparity and save money, subsidies to insurers offering Medicare Advantage plans will be frozen in 2011 and start to drop in 2012, likely increasing premiums and other expenses for Medicare Advantage plans and decreasing perks, such as gym memberships. Beginning in 2012, however, high quality Medicare Advantage plans will be eligible for bonus payments, which will offset some of these funding cuts.

Some of the legislated Medicare cuts come in the form of reduced reimbursement fees to hospitals and drug companies and to private offices or facilities that provide radiology services (excluding radiation oncology). The bulk of the Medicare spending reductions is expected to come from savings derived by eliminating waste, fraud, and abuse in the system.

How Insurance Exchanges Work

By 2014, each state must have an American Health Benefit Exchange and a Small Business Health Options Program Exchange in place. At these exchanges, individuals and businesses with fewer than 101 employees can choose from four different tiers of private insurance coverage meeting or exceeding minimum benefit standards, with co-insurance requirements ranging from 60% to 90%. Deductibles in the small-group market are capped at $2,000 per individual and $4,000 per family. Out-of-pocket requirements cannot exceed Health Savings Account limits, which will be reduced to $2,500 as of January 1, 2013. The exchanges must also offer a catastrophic-only plan to people less than 30 years of age or to individuals exempt from the law's mandate (requirement) that they obtain health care coverage.

Individuals who do not want to select a local private plan from the state exchanges will have the option of choosing from among multi-state private plans offering coverage similar to that enjoyed by members of Congress. As with all policies after 2014, none of these plans can exclude or charge more to anyone for a preexisting condition. Nor can they cap lifetime benefits or drop subscribers who become ill.

What If You Can't Afford Insurance?

Beginning in 2014, low-income non-elderly individuals who are not already eligible for Medicaid under their state's eligibility rules have the option of applying for Medicaid if their household income is less than 133% of the Federal Poverty Level (FPL). The FPL for 2010 is $22,050 for a family of four, $18,310 for family of three, $14,570 for family of two, and $10,830 for a single adult.

Individuals and families whose incomes are between 133% and 400% of the FPL for 2014, making them ineligible for Medicaid but still in need of financial assistance, can apply for subsidies to purchase a plan from the new insurance exchange. People with incomes at 133% of the FPL will have to pay at most 4% of their income for insurance; this maximum increases to 9.5% for people earning 300% to 400% of the FPL. Health care plans offered on the exchange for low-income adults cannot require them to contribute any more than 15% of their annual income for health-related expenses, such as copayments and coinsurance fees.

Who Pays for the Reforms?

The bulk of the reforms are expected to be covered by cost savings, reduced Medicare payments to health care providers, and new fines, taxes, and fees assessed on insurers, pharmaceutical companies, employers, and individuals.

Here are a few examples of how the government expects the reforms to be covered. Drug manufacturers will pay an annual fee on brand name drugs beginning in 2011 and will make higher payments to the Medicaid program. Fees will be assessed on health insurance companies, including a fee to fund research comparing different treatments and methods of delivering care and an annual fee totaling $8 billion in 2014, which will increase in subsequent years.

Effective January 1, 2013, the Medicare payroll tax for couples earning more than $250,000 and individuals earning more than $200,000 will go from 1.45% to 2.35%, but only on the amount by which their income exceeds these limits. People meeting these income criteria will also be required to pay a 3.8% Medicare tax on investment income (currently set at 0%). In addition, the current threshold for any taxpayer to itemize and deduct itemized medical expenses on federal income tax returns, which is 7.5% of annual income, will increase to 10% of annual income.

Large employers—those with more than 50 full-time employees—will shoulder some of the burden of these reforms. Beginning in 2014, those large employers that do not offer affordable group coverage or that have an employee who obtains insurance through an exchange or with a subsidy will be fined. Beginning in 2014, adults without coverage for more than three months will be fined $95 or 1% of income (not to exceed the cost of a basic health plan), whichever is greater. In 2016, the penalty increases to $695 per uninsured adult, up to $2,085 per household, or 2.5% of income, whichever is greater. Penalties may be waived for individuals who show they are unable to afford a plan.

Dealing With Demand

To help meet an increased demand for services, the law includes $11 billion to fund staff increases and expand facilities at the nation's 1,500 nonprofit community health centers. The goal is to double the number of patients served at these clinics over the next five years. States can apply for federal grants to develop programs for medical homes, and insurers will be allowed to cover services provided at an eligible medical home. Although medical home models vary, they generally involve having the patient receive primary care at one center, which coordinates any other mental or physical health care needs the patient has and maintains the records in one centralized location. To reduce

the burden on pharmacists and clinicians and offer convenience to patients, these experts will be permitted to use telehealth resources (electronic forms of communication), rather than face-to-face visits, for some services.

Beginning with fiscal year 2010, HR 3590 designates $1.5 billion for training programs designed to increase the number of primary care physicians, nurses, and public health professionals. It also establishes a task force to examine ways to shore up the health care workforce.

States, already forced by economic conditions to make cuts to Medicaid programs, are worried that they will be unable to afford an influx of new enrollees. The federal government is slated to fund 100% of costs for new Medicaid enrollees through 2016, when it is hoped that states will be in better financial shape to bear the additional expense. Governors from both parties have expressed concern that shifting Medicaid costs onto states in 2017 will require cuts to other important state programs and they say federal assistance may be needed well beyond 2016.

Who Wins With the New Legislation?

The most obvious winners appear to be the uninsured, people with preexisting conditions, people with expensive or chronic conditions, and small business owners. Some say the real winners are the pharmaceutical companies and the health insurers, who stand to gain 32 million more paying customers. Others say the economy is the winner, because more focus on prevention, early diagnosis, and treatment will reduce the burden of illness in this country and the percentage of the gross domestic product that goes toward health care costs.

Public reaction to these health care reforms has been mixed, with some polls showing majority support and others showing majority opposition. Many people will not feel the effects of the reforms until 2014, and their views may evolve over time as the law's benefits and drawbacks become more apparent. Public reaction to both the Social Security and Medicare programs was also mixed when those laws were enacted—yet few people today want to see either program eliminated. ■

Family Assistance Program

The Family Assistance Program is a confidential service provided to patients with type 1 Gaucher disease to help them resolve personal and family issues and improve their health and well-being. The program offers a variety of services, including short-term counseling, resources, consultation, and referrals for stress and personal issues to help patients cope with their personal concerns.

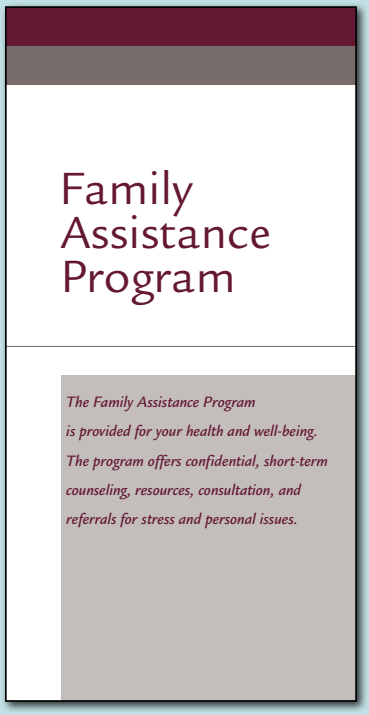
Any personal problem is appropriate for the Family Assistance Program. The most common issues that patients seek help with include:

- Stress management
- Depression, anxiety, and panic attacks
- Grief or loss
- Relationship and family problems
- Chemical dependency
- Eating disorders and nutritional questions
- Domestic violence
- Job stress and career frustration
- Child care and elder care resources
- Legal issues
- Financial concerns
- Work-life balance issues
- Meditation training

The Family Assistance Program offers various types of services, including legal assistance, referral for childcare needs, career exploration services, and convenience services to help with balancing work and personal life.

To access the Family Assistance Program, call 1-800-648-9557. A counselor is available to help you 24 hours a day, 7 days a week. The counselor will gather some information, evaluate your needs, and suggest a possible plan of action. The next step will depend on your unique situation. If you are calling about an emotional or family issue, the counselor will suggest a face-to-face or telephone meeting. This will provide you with a private opportunity to talk about your concerns in depth. If a face-to-face meeting is recommended, it will take place at a convenient, private office within 30 minutes of your home or workplace.

If you need additional counseling, the counselor will help you find the right resources to address your specific problem and will refer you to helpful services beyond the Family Assistance Program.



Understanding the Cerezyme Supply Shortage


The Allston, Massachusetts manufacturing facility

A Supply Challenge

Many Gaucher patients experienced a disruption in therapy when Genzyme Corporation (Cambridge, MA) discovered a virus at its Allston, Massachusetts manufacturing plant where Cerezyme® (imiglucerase for injection) is made. **(Please see Cerezyme indication and important safety information on page 7 and accompanying prescribing information.)**

The virus, called Vesivirus, is not known to infect humans, but is known to affect cells that are used to make Cerezyme. Genzyme had to shut down the Allston facility starting in June 2009 in order to completely sanitize the plant. Drug material that was in production also had to be discarded.

The plant resumed operations in late July/August 2009. Since it takes 4 to 5 months to make a new batch of Cerezyme, this meant that new drug wouldn't become available until the very end of 2009. *(To learn about the complex process of making therapeutic proteins in a bio-manufacturing environment, visit http://www.genzyme.com/research/technology/tech_home.asp).*

From June to December 2009, Genzyme could only supply Cerezyme that it had in inventory. It quickly became clear that there was not enough drug available to treat all current patients in the months when no new drug would be available.

In order to figure out how to best distribute the very limited supply of Cerezyme, Genzyme consulted with expert physicians and representatives from the National Gaucher Foundation. The Cerezyme Stakeholders Working Group (CSWG) was formed in June 2009.

Cerezyme Conservation Efforts

On June 24, 2009, the CSWG issued a *Guidance* to the Gaucher community to conserve the supply of Cerezyme and protect the most vulnerable patients.[1] At that time, the available supply of Cerezyme was projected to meet about 60% of normal demand during the shortage period.[1] In August 2009, the CSWG issued a *Revised Guidance* following Genzyme's decision to discard most Cerezyme "work in process" material (material that was not yet purified and put into vials).[2] At that point, the available supply of Cerezyme was projected to meet only about 20% of normal worldwide demand during the remaining expected period of shortage.[2]

The *Revised Guidance* defined a group of the medically most vulnerable patients—children 18 years old and younger and patients with Gaucher disease type 2 or 3—who should continue to receive Cerezyme therapy, and established criteria for a Cerezyme Emergency Access Program (CEAP) that would allow physicians to request Cerezyme for adult type 1 patients who would be in imminent life-threatening clinical situations without continued Cerezyme treatment.

Ongoing Product Constraints

In January 2010, Genzyme resumed shipping Cerezyme to all patients for whom it was prescribed. However, meeting global demand for Cerezyme when the company had completely depleted its inventory was challenging. Delays in shipping Cerezyme led Genzyme in February 2010 to announce plans to reduce the amount of Cerezyme supplied by 50% for eight weeks in order to build a small inventory of the drug. In April, however, Genzyme announced that the timeframe for this reduced supply had to be extended due to an interruption in operations at the company's Allston facility late in the quarter.[3]

"Due to limited inventory, for the coming months we continue to expect intermittent delays in supplying Cerezyme," according to a statement issued by the company. "Genzyme will be working throughout 2010 and 2011 to rebuild normal levels of global inventory of Cerezyme, and we expect to have additional manufacturing capacity for Cerezyme available with the approval of our new facility in Framingham, Massachusetts in 2011."[2] Genzyme recently announced that it expects to have enough Cerezyme supply for patients to resume normal dosing during the fourth quarter.[4]

Options for Treatment

Physicians who treat patients with type 1 Gaucher disease should be aware of the shortages and may need to consider which patients should receive an individualized dose reduction or other treatment. According to the *Revised Guidance*, patients who undergo an interruption or reduction of Cerezyme treatment "should, at minimum, be monitored by obtaining a hemoglobin level, platelet count, and chitotriosidase activity level (if available) at baseline and every four to eight weeks." For all clinical decisions related to restarting Cerezyme therapy, the guiding principle is that "the treating physician should determine how and where to restart Cerezyme based on each patient's current clinical status and previous infusion history."[5] During this period of Cerezyme supply shortage, alternative therapies may be available for patients who would like to explore other treatment options. A second enzyme replacement therapy, velaglucerase alfa, was recently approved by the FDA for sale in the US. Clinical studies of investigational therapies for Gaucher disease are also ongoing. Information about these studies is available at http://www.clinicaltrials.gov.

In January, Senior Vice President of Global Product Quality, Ron Branning, joined the ranks at Genzyme in an effort to transform the company's operations. A 30-plus-year pharmaceutical veteran, Mr. Branning is responsible for the quality of all Genzyme products manufactured at 17 sites worldwide. Here, he discusses the future of Genzyme.

What are Genzyme's priorities during the Cerezyme supply shortage?

In the short term, we have a number of priorities. First of all, we want to continue our focus on our patients and need to make certain that we are delivering products they need in a very predictable and reliable way. We're also using good risk management principles internally to be certain that we're focusing on the most important things related to the manufacturing operations in our quality systems. Last but not least, we are strengthening our team by building on the good folks that we already have working here and by bringing in industry experts from around the world to make sure that we have the best production operations team.

In the long term, we want to foster a culture of compliance and continuous improvement to be sure that we are strengthening as we move forward. All of this will help us foster an FDA and global regulatory confidence in Genzyme.

Where do you see Genzyme in the future?

I believe Genzyme has a very bright future. Our patient focus has brought us to the point where we are and will take us into the future. We need to be very transparent with the regulators and ensure that we have a fast lane for delivery of our products to our patients. As we build this foundation, I believe we will emerge a stronger company and will be one of the leaders in biotechnology.

Genzyme's Commitment

In the spirit of transparency and collaboration, Genzyme created a supply update Web site (http://supplyupdate.genzyme.com/weblog) as a way to communicate about the Cerezyme and Fabrazyme supply shortages. According to the site, the company remains focused on returning to a full, reliable supply of its products and is working closely with treating physicians, other health care providers, patient organizations, and regulatory officials around the world during this period of constrained supply. The Genzyme Transformation Web site (http://www.genzymetransformation.com) provides information on the changes Genzyme is making to improve their manufacturing and quality control processes. In a video interview on that site, Pamela Williamson, senior vice president and global head of Regulatory Affairs and Corporate Quality Compliance, notes that the company has taken steps to strengthen and improve operations at the Allston manufacturing facility. "We have implemented significant training, we have changed out equipment, and we are confident that we are on the road to recovery."

Patients who depend on Cerezyme may wonder whether Genzyme is still committed to the communities of people affected by Gaucher disease. Henri A. Termeer, chairman and chief executive officer at Genzyme, in a letter to the patient community dated April 21, 2010, expressed the company's regret for the shortage and its commitment to those affected. "We deeply regret and understand the disappointment for those in the Gaucher disease community affected by this change in our ability to supply," said Mr. Termeer. "We take our responsibility to provide the therapy you depend upon very seriously. We are completely focused on confronting and resolving the manufacturing challenges that we currently face and restoring a consistent supply of these products. Each and every one of us here at Genzyme is committed to re-earning your trust." He added, "The only way this will happen is if we return to a reliable supply of products, produced in world-class manufacturing facilities. We are working hard to make this happen and will not let anything distract us from our responsibility and commitment to you."■

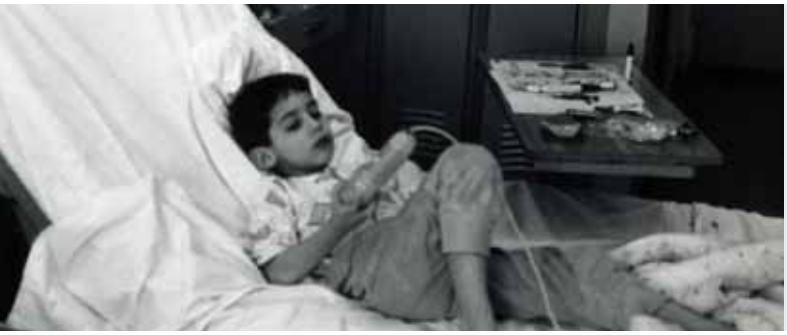
References

1. National Gaucher Foundation. Guidance to the U.S. Gaucher Community: Restarting Cerezyme® (imiglucerase for injection). Available at: http://www.gaucherdisease.org/Guidance%20to%20the%20Gaucher%20Community%20-%20US_Cerezyme_Stakeholders_Working_Group.pdf. Accessed May 17, 2010.
2. Genzyme Reports Progress Related to Allston Plant (http://www.genzyme.com/corp/media/GENZ%20PR-062509.asp)
3. Genzyme Corporation. Genzyme reports financial results for the first quarter of 2010: Provides update on product supply and consent decree terms. [press release]. April 21, 2010. Available at: http://www.businesswire.com/portal/site/genzyme/index.jsp?ndmViewId=news_view&ndmConfigId=1019673&newsId=20100421006162&newsLang=en. Accessed May 17, 2010.
4. Cerezyme® (imiglucerase for injection) and Fabrazyme® (agalsidase beta) Supply Outlook – Understanding Genzyme's Press Release of July 21, 2010 (7/21/2010) Available at: http://supplyupdate.genzyme.com/weblog/2010/07/cerezyme-imiglucerase-for-injection-and-fabrazyme-agalsidase-beta-supply-outlook-understanding-genzy.html. Accessed July 21, 2010.
5. National Gaucher Foundation. Revised Guidance to the U.S. Gaucher Community: Management of Cerezyme® (imiglucerase for injection) Supply. Available at: http://www.gaucherdisease.org/genzyme_guidelines.php. Accessed May 17, 2010.

The article entitled "Understanding the Cerezyme Supply Shortage" and the Sidebar include statements about our future operations and business plans, such as: our plans to rebuild product inventories in 2010 and 2011and to restore a reliable and consistent supply of products; and our expectations to have additional manufacturing capacity in late 2011 and to have enough supply for Cerezyme patients to resume normal dosing during the fourth quarter of 2010. You should be aware that there are risks and uncertainties associated with these plans and expectations that may cause our actual outcomes to be materially different than the ones we described in this publication. Those risks include: the risk that production and shipment of Cerezyme does not continue as planned due to any reason, including contamination, equipment malfunctions, cell growth at lower than expected levels, fill-finish inefficiencies, power outages, human error or regulatory issues; the risk that we cannot obtain or maintain regulatory approvals for manufacturing facilities, including our Allston manufacturing facility and our new Framingham facility or that those approvals are delayed for any reason; that compliance with the FDA Consent Decree, including third-party oversight and/or the implementation of the remediation plan, results in additional or unexpected delays in product releases; and the risks and uncertainties described in our reports filed with the U.S. Securities and Exchange Commission (SEC) under the Securities Exchange Act of 1934, including the factors discussed under the caption "Risk Factors" in Management's Discussion and Analysis of Financial Condition and Results of Operations in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2010. We caution you not to place substantial reliance on the forward-looking statements contained in this publication. These statements speak only as of July 21, 2010.

Drawing Inspiration From Pain, an Artist with Gaucher Disease Thrives

Ted's Story



It is difficult to find one word to describe Ted Meyer. He is an artist, writer, photographer, designer, and teacher. Ted Meyer is also an individual with type 1 Gaucher disease. After hearing Ted's life story and viewing his work, perhaps the word that comes closest to summarizing the man is inspirational.

Ted began producing artwork in the mid 1980s, when type 1 Gaucher disease was affecting him. By the early 1990s, he was in near-constant pain. His paintings from this period sprang from the anxiety and suffering borne of his debilitating illness. Although his artistic horizons have expanded since those early days, Ted continues to include disability as a theme for some of his works.

Art as Medicine

When 5-year-old Ted began to experience bone pain and excessive bleeding, his parents and health care providers readily identified the cause as type 1 Gaucher disease. Ted's older brother had been diagnosed with the disease previously, and the hereditary nature of the condition was well known. An enlarged spleen is common in individuals with type 1 Gaucher disease, and physicians removed Ted's spleen in the first grade. "This alleviated the bleeding I had," Ted said, "but immediately, I started getting bone crises."

Ted credits his illness as his artistic muse. "One of the main themes of my artistic expression stems from this disease," he explained. During his hospitalizations for Gaucher-related symptoms, a hospital volunteer traveled around the children's ward pushing a cart stocked with art supplies. "I got comfortable doing art around medical things," Ted said.

The Ted-Centric Era

Ted's bone degeneration and pain escalated over the next few years. He started painting seriously shortly after college, "when I was very sick and my legs caused me constant pain." In the early 1990s, physicians recommended a hip replacement. One of his friends had recently undergone hip replacement surgery with a terrible outcome, and Ted was not certain he wanted to go through with it. "I struggled with whether or not to have the operation," he said.

Ted's internal debate took shape in a set of paintings completed during that time, the "Structural Abnormalities" series. Each image contains a solitary skeletal figure trapped in a box. In some, the figure struggles to break free; in others, he contorts awkwardly within his constraints. The colors are dark and bold, the brush strokes assertive. There is nothing serene about these paintings. Ted refers to this early period of his work as "Ted-centric," reflecting a low point in his life.

Better Health, Brighter Art

Around age 34, Ted had his right hip replaced. Shortly afterward, in 1996, he started treatment with Cerezyme® (imiglucerase for injection). Since then, his symptoms have improved. A problem with his initial hip surgery left him in excruciating pain, requiring repair. This time he had bilateral replacements.

Just as Ted's darker emotions took over the canvas when he was severely ill, a brighter outlook emerged in his art following improvements to his health. "The healthier I got, the more my art changed, especially in terms of colors and imagery." He transitioned to using lighter colors and whole figures interacting with other people. "I stopped wanting to do stuff just about me," he said. During his convalescence from surgery, and unable to stand for extended periods to paint, he branched out into photography.

Although Ted elected to move beyond self-exploration and Gaucher disease in his works, he revisited the topic for a show at the American Association of Orthopedic Surgeons in 2007. As adjunct pieces to the Structural Abnormalities series, he painted "Silver Hips," in which squares of metal stand in for hip bones (**Figure 1**).

Figure 1

Figure 2

A friend persuaded the artist not to totally abandon ability/mobility as a theme in his work. Challenged, he created a series entitled "Scarred for Life, Monoprints of and Documentation of Human Scars," chronicling people's physical scars of life-changing events with monoprints, photography, and personal stories.

More Than an Artist

Ted has worked with children in various hospitals. He feels suited to it and "very comfortable being around kids in a hospital." Ted also operates a full-service design studio called Art Your World, (**Figure 2**) in Los Angeles, California, that designs print pieces, logos, and websites, and handles programming, illustration, and photography. He has written and illustrated four books.

Ted is also an active and accomplished lecturer, speaking to medical and patient groups about his experiences. For example, Yale Medical School in New Haven, Connecticut, engaged him to speak to first-year medical students. The artist lectures to highlight the twin progressions of his artwork and his health. He points out how his art transformed as his health improved and he began "making plans to live."

Although Ted makes light of the health crises he has endured, joking that his "football career" is definitely over, coming to terms with his body was difficult. He has traveled the same long road that is familiar to many patients with Gaucher disease. Yet, even during his worst days, he found a way to turn his pain into something positive.

Ted serves as an inspiration not only to people with type 1 Gaucher disease but to anyone whose life has been affected by chronic illness or trauma. His story and his paintings demonstrate that a debilitating disease need not rob life of its vibrancy. You can view Ted's paintings, scar work, and photographs, or contact him for speaking engagements at http://www.tedmeyer.com. ■

Patient–Family–Physician Partnership Helps Mitigate Gaucher-Related Consequences

Michael's Story

All too often, the Gaucher disease patient profiles that appear in *Horizons* contain heartbreaking narratives of cascading adversity: an inaccurate diagnosis results in unnecessary suffering as the disease progresses unchecked, complications mount, and permanent damage is inflicted. Patients sometimes endure years of ill health and severe acute events (e.g., easy bleeding and bruising, excessive fatigue, weak bones fracturing too easily, bone and joint pain) before their type 1 Gaucher disease is identified and therapy is initiated.

Fortunately, the story of Michael Harris, a 23-year-old student from Connecticut, is neither heartbreaking nor distressing. Although Michael has had his share of difficulties, his experience demonstrates the profoundly positive results yielded when an educated patient with a well-developed support network is able to obtain a proper diagnosis in a timely manner and receive appropriate treatment.

Family Awareness Facilitates Early Intervention

Michael was initially diagnosed with type 1 Gaucher disease at age 5. During a physical exam before entering kindergarten, his physician noticed that Michael's liver and spleen were swollen. His family knew that type 1 Gaucher disease is hereditary and they suspected that he had inherited the genetic condition. Michael explained, "It (type 1 Gaucher disease) has been in my family. Right away, my family realized that and thought it was a strong possibility."

Had it not been for his family's knowledge of the genetic disease, Michael's condition might have been overlooked. He had not been symptomatic and Michael had no idea that anything was wrong. When his swollen liver and spleen were first detected, the initial reaction of his physician was dismissive. "They noted that my liver and spleen were a little enlarged," recalled Michael, "but expected that I would 'grow' into [them]."

The advocacy of Michael's family helped to ensure that he received proper screening. "My mother and father stepped up. They pushed to get me tested. My uncle and my aunt both had Gaucher. When my uncle was diagnosed, it was much later in the progression of his disease. They (mistakenly) thought he had many diseases before that. I remember seeing pictures of him in braces from his bones deteriorating. The connection—that it's in my family—just scared my mother and father (both of whom are carriers but were unaware of this at the time of Michael's diagnosis). That's why they wanted to follow through and make sure everything was okay," recalled Michael.

The testing process, which included a magnetic resonance imaging test, a computed tomography scan, and blood work, confirmed the type 1 Gaucher diagnosis. For a five-year-old, the procedures were intimidating. "It was frightening," said Michael. "I'd never had to go through anything like that. It was not the most pleasant thing."

Following his diagnosis, Michael's parents travelled near and far to provide young Michael with the best medical care possible. "When I was diagnosed, type 1 Gaucher disease was pretty much unheard of in Connecticut, [so] we had to go to Albany Medical Center (Albany, NY)," disclosed Michael, who spoke highly of Marilyn L. Cowger, MD, Albany Medical College, and Robert M. Greenstein, MD (Farmington, CT), his locally based physician.

During his childhood, Michael's care evolved into a basic routine. "I remember travelling to New York a lot," he said, "I went to a local area hospital, John Dempsey Hospital, Farmington, CT, to get tested and treated. We would travel to Albany for yearly assessments." The long journeys to Albany could have been tiresome, even traumatic. However, Michael's parents strove to make the trips fun. "(Going to Albany) used to be a big deal," reminisces Michael. His parents would stop in Lake George, New York (a resort area and winter sports Mecca). "It wasn't nice getting tested," he said, "but we had a little vacation getaway every year." Overall, concluded Michael, "my folks did a really good job with not making the disease a burden."

Life After Treatment

Another providential occurrence was the fact that Ceredase® (alglucerase injection), the forerunner to Cerezyme® (imiglucerase for injection) and Genzyme's original type 1 Gaucher disease treatment, became available just as Michael received his diagnosis. He declared, "It was really good timing and I was very lucky. I was able to get treated right away."

Shortly after his type 1 Gaucher disease was confirmed, Michael initiated treatment and has never looked back. "I never had any complications from Ceradase or Cerezyme. My side effects were manageable," he reported. "Pretty quickly, my liver and spleen decreased to normal size, and my platelet and blood levels normalized." Owing to the speed and competence of his diagnosis and treatment, Michael's symptoms abated.

Consequently, Michael is an example of successful treatment and the positive outcomes that can result. With the aid of regular Cerezyme infusions, Michael has been able to lead an active life. "I play sports all year round. I've played baseball my entire life," he stated. "I was never held back from playing outside. "

For the first three or four years of his treatment, Michael received infusions at the John Dempsey Hospital-University of Connecticut Health Center in Farmington. Thereafter, Michael's physicians carefully assessed and determined that he was a candidate for home infusion. The convenience of home infusion, explained Michael, significantly improved his comfort level. "I still remember the little-sized La-Z-Boy® chair that my mom bought me so I could sit and watch movies while I was getting my infusions," reminisced Michael. "I was treated like a prince."

Michael's parents and physicians teamed up to create the best care environment possible. "The needles were scary at first," Michael admitted, "but the way it was handled by the doctors and my family made it as comfortable as possible. That made it easier." When receiving infusions at the hospital, toys were provided to distract the youngster. "At home," he mused, "I had my special chair, the movies, and television." Aside from providing the various comforts, Michael's family and physicians also looked after his emotional well being. "My parents and the doctor always presented infusion therapy as the right thing to do. They always let me know that what was going on was not a bad thing."

Shaped by Type 1 Gaucher Disease

For Michael Harris, his condition has had a formative, influential effect on his future. As Michael put it, "Type 1 Gaucher disease made me who I am today." His experience with the illness led him to study biology and nutrition, and he is currently in graduate school pursuing a career as a physician assistant at Springfield College (Springfield, MA). "I never had a bad experience in a hospital," explained Michael, adding that the time he spent in the care setting because of his disease "made hospitals feel not so scary and more like a second home."

Michael is also a volunteer, performing patient outreach initiatives in conjunction with Genzyme. One program in which he participates allows children with type 1 Gaucher disease and their parents a chance to talk with Michael and his parents. "I tell them it's not necessarily something that has to take over [their] life," said Michael. "I show them that I have lived an active life, put them at ease, and let them know that probably everything is going to be okay."

"I believe that a lot of people look at the illness as a debilitating disease," he concluded. "I don't believe it's that way at all. In the right hands it's very treatable. It's not something to be scared of. You have to be conscious of maintaining a regular infusion schedule (when you go on vacation, for example). However, as long as you keep up with your infusions, you may be able to lead a full life." ■