



Patty and Alexsia, Fabry patients

# UNDERSTANDING TREATMENT GOALS

**A Guide for People Diagnosed with Fabry Disease**

**SANOFI GENZYME** 

# UNDERSTAND FABRY DISEASE

Fabry disease is an inherited disorder that can affect many family members. An altered gene results in decreased function of an enzyme called alpha-galactosidase A, or alpha-GAL. Alpha-GAL breaks down a fatty substance called globotriaosylceramide or GL-3. Without enough alpha-GAL, GL-3 is not broken down, and instead builds up in cells throughout the body. GL-3 build-up may start before birth and continue over decades.

## Signs and Symptoms of Fabry Disease Can Vary Within A Family

Early signs and symptoms that start appearing in childhood or adolescence include:

- Pain in the hands and feet
- Small, reddish-purple spots on the skin
- Digestive problems
- Inability to sweat
- Fatigue



Pain



Fatigue



Gastrointestinal

Over time, GL-3 build-up may cause blood vessels to narrow, which means the kidney, heart and brain do not get the blood flow they need to function properly. As a result, adults with Fabry disease are at risk for potentially life-threatening problems such as:

- Kidney disease
- An enlarged heart
- Irregular heartbeat
- Heart valve problems
- Early stroke



Kidney



Heart

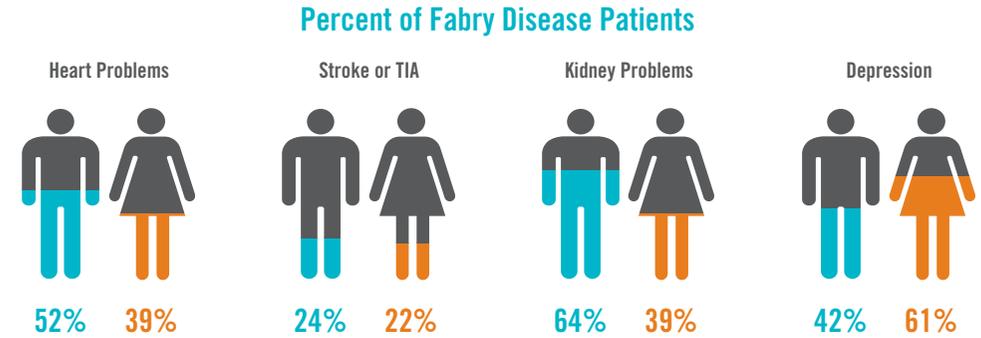


Brain

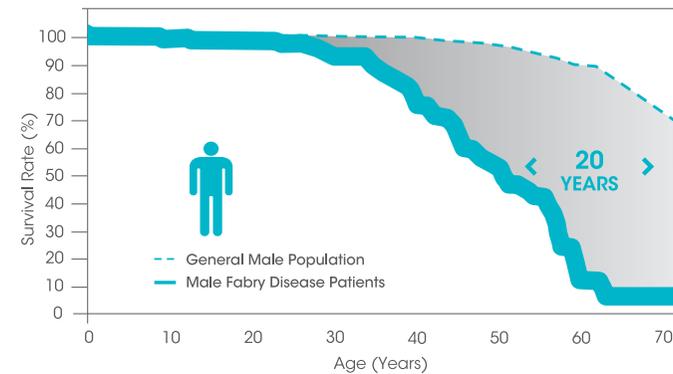
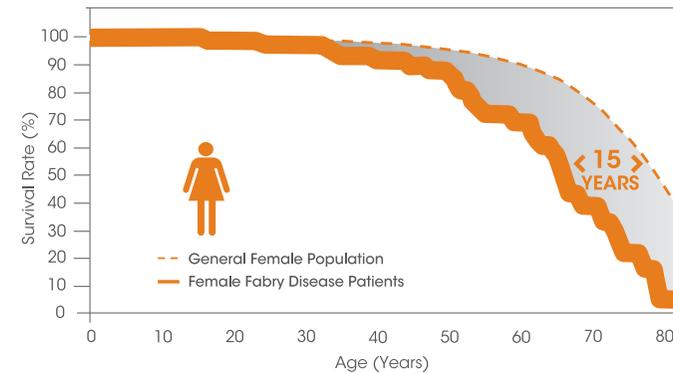
Symptoms may vary within a family, ranging from mild to very severe. Some people feel healthy, but could be experiencing “silent” progression that affects internal organs.

## Fabry Disease Affects Both Males and Females

Both males and females are at risk of developing signs and symptoms of Fabry disease. Here are some examples of how common severe symptoms are in the Fabry population:



When unmanaged, Fabry disease reduces life expectancy by approximately 15-20 years. Fabry disease may affect males and females differently, but it reduces life expectancy in both genders.



# GET TO KNOW THE TREATMENT GUIDELINES

Guidelines have been developed by a panel of Fabry experts for the treatment of Fabry disease. Guidelines are a tool for physicians to use when making treatment recommendations for their patients. People who have been diagnosed should receive regular check-ups, even if they are not experiencing symptoms or not currently on treatment. If you have questions about the guidelines, talk to your doctor.

There are guidelines for treatment in men, women, and adolescents with Fabry Disease:



Heather and Amanda,  
Fabry patients

## Enzyme Replacement Therapy (ERT) Should be Considered in Males Aged 8 and Older at the Time of Diagnosis

- Males with an altered *GLA* gene usually have low alpha-GAL enzyme activity
- In published guidelines, Fabry disease experts recommend that ERT should be started in males aged 8 and older at the time of diagnosis

## Enzyme Replacement Therapy Should be Considered in Females with Certain Symptoms

- Disease severity varies widely in females, from virtually symptom-free to the more classical profile of symptoms found in males
- Despite having near normal alpha-GAL activity, studies show that most symptomatic women had heart, kidney, or brain abnormalities
- The guidelines suggest that ERT be started in women with problems in the kidneys, heart or brain, as well as women who experience pain, gastrointestinal distress, difficulty sweating or exercising

## Enzyme Replacement Therapy Should be Considered in Symptomatic Adolescent Patients

- ERT should be considered in pediatric patients with certain kidney, heart, brain, pain, gastrointestinal symptoms, difficulty sweating or exercising
- The safety and effectiveness of Fabrazyme in patients younger than 8 years of age have not been studied

If you or your children experience any of the above symptoms, talk to your doctor about whether you might be a candidate for treatment.

Fabrazyme lowers the amount of a substance called globotriaosylceramide (GL-3), which builds up in cells lining the blood vessels of the kidney and certain other cells. Fabrazyme has not been shown to affect other symptoms of Fabry disease.

## Indication and Usage

Fabrazyme® (agalsidase beta) is used to treat patients with Fabry disease. Fabrazyme lowers the amount of a substance called globotriaosylceramide (GL-3), which builds up in cells lining the blood vessels of the kidney and certain other cells.

The lowering of GL-3 suggests that Fabrazyme may improve how Fabry disease affects your body; however a relationship of lower GL-3 to specific signs and symptoms of Fabry disease has not been proven.

Please see Important Safety Information and enclosed full Prescribing Information.

# FABRAZYME® (AGALSIDASE BETA): TREATMENT FOR FABRY DISEASE

Fabrazyme is the only approved enzyme replacement therapy for Fabry disease in the United States. Here is why you should consider Fabrazyme:

- Fabrazyme introduces an external source of fully functional enzyme to help break down GL-3 in the body
- Fabrazyme is prescribed for patients, regardless of mutation, disease severity or enzyme activity level
- Fabrazyme has been used by families with Fabry disease for over 15 years, backed by the safety and effectiveness shown in clinical studies



## Selected Important Safety Information

Fabrazyme can cause serious side effects, including:

**Severe Allergic Reactions (anaphylaxis):** Life-threatening severe allergic (anaphylactic) reactions have been seen in patients during Fabrazyme infusions. Approximately 1% of patients who have received Fabrazyme either during a clinical study or after Fabrazyme was approved have experienced anaphylactic or severe allergic reactions during their infusion.

- These reactions have included: localized swelling of the face, mouth and throat, narrowing of breathing airways, low blood pressure, hives, difficulty swallowing, rash, trouble breathing, flushing, chest discomfort, itching and nasal congestion.
- People who have experienced these reactions have required treatment including heart/lung resuscitation, oxygen, fluids given through the vein, hospitalization, and have needed treatment with inhaled drugs called beta-adrenergic agonists to help open the breathing airways, antihistamines, epinephrine (also known as adrenalin), and a medication given through the vein called a corticosteroid (or steroid) which helps to decrease the body's allergic reaction by decreasing inflammation.
- If you experience a severe allergic or anaphylactic reaction, your healthcare professional will immediately stop the infusion of Fabrazyme and provide you the necessary emergency medical treatment. Because of the possibility that severe allergic reactions may occur, appropriate medical support should be available during your Fabrazyme infusion.

Please see Important Safety Information and full Prescribing Information.



Braulio and Clara, Fabry patients

# MAKING TREATMENT DECISIONS

Treatment decisions should always be made in collaboration with your doctor. If you have questions about starting treatment, share them with your doctor. Here are some common considerations that people may think about when diagnosed with Fabry disease:

## Severity of Symptoms

- Even if you do not feel sick, symptoms could be progressing silently, affecting internal organs such as the kidney or heart
- Without treatment GL-3 continues to build up in cells

## Expectations about Fabrazyme® (agalsidase beta)

- Talk to your doctor about any questions you may have
- Fabrazyme helps clear and regulate GL-3
- Ask your doctor about options for managing specific symptoms as you continue to manage GL-3 levels with Fabrazyme

## Integrating Treatment into Your Life

- Your CareConnectPSS™ Case Manager can work with you and your healthcare team to help resolve scheduling problems and to ensure that your infusion process goes smoothly
- Fabrazyme can be administered at your doctor's office, an infusion center, or at home; talk to your doctor about which option is right for you
- During the infusion, you can read a book, talk on the phone, write in your journal, visit with friends or family members, use the restroom – even take a nap

## The Right Time to Begin Treatment

- The Fabry panel of experts say that a decision to delay treatment should be based on a comprehensive, long-term medical assessment
- Remember, treatment decisions should be made in collaboration with your doctor



Cameron, whose family is affected by Fabry disease

## Selected Important Safety Information

**Infusion-Associated Reactions:** In clinical studies with Fabrazyme, 59% of patients experienced infusion-associated reactions during Fabrazyme administration, some of which were severe.

- For patients who have had reactions to their infusions, it is recommended that they be given anti-fever and antihistamine medications right before their next infusions. Infusion-associated reactions have happened in some patients even after taking these medications before their infusions.
- If an infusion-associated reaction occurs, slowing the infusion rate, stopping the infusion for a short time and/or giving more anti-fever and antihistamine medications and/or steroids may improve the symptoms.
- If severe infusion-associated reactions happen, your healthcare professional should consider stopping the Fabrazyme infusion right away and should provide medical care for your condition. Severe reactions are generally managed by giving antihistamine medications, corticosteroids, fluids through the vein, and/or oxygen when needed. Because severe infusion-associated reactions may happen, medical treatment should be readily available during your Fabrazyme infusion.

**Please see Important Safety Information and enclosed full Prescribing Information.**

## Common Side Effects

Common side effects reported in 20% or more of patients treated with Fabrazyme (agalsidase beta) in clinical studies compared to placebo were:

- Upper respiratory tract infection
- Chills
- Fever
- Headache
- Cough
- Burning or tingling in the hands and feet
- Fatigue
- Swelling
- Dizziness
- Rash

## Serious Side Effects

- Approximately 1% of patients who have received Fabrazyme either during a clinical study or after Fabrazyme was approved have experienced severe allergic reactions (anaphylaxis) during their infusion
- In clinical studies, 59% of patients experienced infusion-associated reactions during Fabrazyme treatment, some of which were severe
- Infusion-associated reactions tended to decline in frequency with continued use of Fabrazyme in clinical studies; however, they may still occur despite extended duration of treatment
- For patients who have had reactions to their infusions, it is recommended that they be given anti-fever and antihistamine medications right before their next infusion. Infusion-associated reactions have happened in some patients even after taking these medications before their infusions
- People with advanced Fabry disease may have heart problems, which may put them at a higher risk for severe complications from infusion-associated reactions.

## Important Safety Information, continued

**Pre-existing Heart Problems:** People with advanced Fabry disease may have heart problems, which may put them at a higher risk for severe complications from infusion-associated reactions. These patients should be watched closely during their infusion if the decision is made to give them Fabrazyme.

**Immune Response and Continued Treatment After Allergic Reaction:** In the clinical studies, a few patients developed IgE antibodies or a reaction to an allergy skin test specific to Fabrazyme. IgE antibodies are usually produced by the body's immune system during an allergic reaction. Your doctor should consider testing for IgE antibodies if you experience suspected allergic reactions. Providing Fabrazyme to patients who have experienced severe or serious allergic reactions to Fabrazyme should only be done after carefully considering the risks and benefits of continuing the treatment, and only under the direct supervision of a qualified healthcare professional and with appropriate medical support readily available.

### Common and Other Possible Side Effects:

- Common side effects reported in 20% or more of Fabrazyme treated patients in clinical studies compared to placebo were upper respiratory tract infection, headache, cough, burning and/or tingling sensation, fatigue, dizziness, swelling in the legs, and rash.
- Serious and/or frequently occurring side effects (occurring in 5% or more of the patients) thought to be related to Fabrazyme in placebo-controlled and open-label clinical studies have included: chills, fever, feeling hot or cold, trouble breathing, nausea, flushing of the skin, headache, vomiting, burning and/or tingling sensation, fatigue, itching, pain in the hands and feet, high blood pressure, chest pain, throat tightness, abdominal pain, dizziness, rapid heart rate, nasal congestion, diarrhea, swelling in the legs, muscle pain, back pain, paleness of the skin, slow heart rate, hives, low blood pressure, face swelling, rash and sleepiness.
- Other serious side effects that were seen in the clinical studies included stroke, pain, lack of muscle coordination, slow or irregular heartbeat, stopping of the heartbeat, decreased blood pumped by the heart, dizziness, and kidney problems resulting in too much protein leaving the body in the urine (nephrotic syndrome). These side effects also occur as part of Fabry disease.
- Since Fabrazyme has been approved, there have been side effects that resulted in death that may or may not be related to the use of Fabrazyme. These included: the heart and/or lungs stop working (known as cardiorespiratory arrest, respiratory failure, and/or cardiac failure), life-threatening infection in the blood stream (known as sepsis), stroke, heart attack, kidney failure, and pneumonia. Some of these side effects were reported in Fabry disease patients with significant underlying disease.

The safety and effectiveness of Fabrazyme in patients younger than 8 years of age have not been studied.

**Please see full prescribing information for Fabrazyme.**



Charles and Cindy, Fabry patients



# JUST A PHONE CALL OR EMAIL AWAY

CareConnectPSS represents Sanofi Genzyme's more than 25-year commitment to supporting the rare disease community and is designed to support each patient's unique journey.

Whether your needs are large or small, your CareConnectPSS team will work closely with you and your health providers to give you the confidential and personalized support you need. To learn more about our range of support offerings, or to reach your CareConnectPSS Case Manager, please call 1-800-745-4447, and select Option 3, or email us at [info@careconnectpss.com](mailto:info@careconnectpss.com).

**For more information, visit us at  
[careconnectpss.com](http://careconnectpss.com)**

Sophia and Blanca, Fabry patients



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