



# UNDERSTANDING FABRAZYME<sup>®</sup> (AGALSIDASE BETA)

**A Guide for Patients**

**SANOFI GENZYME** 

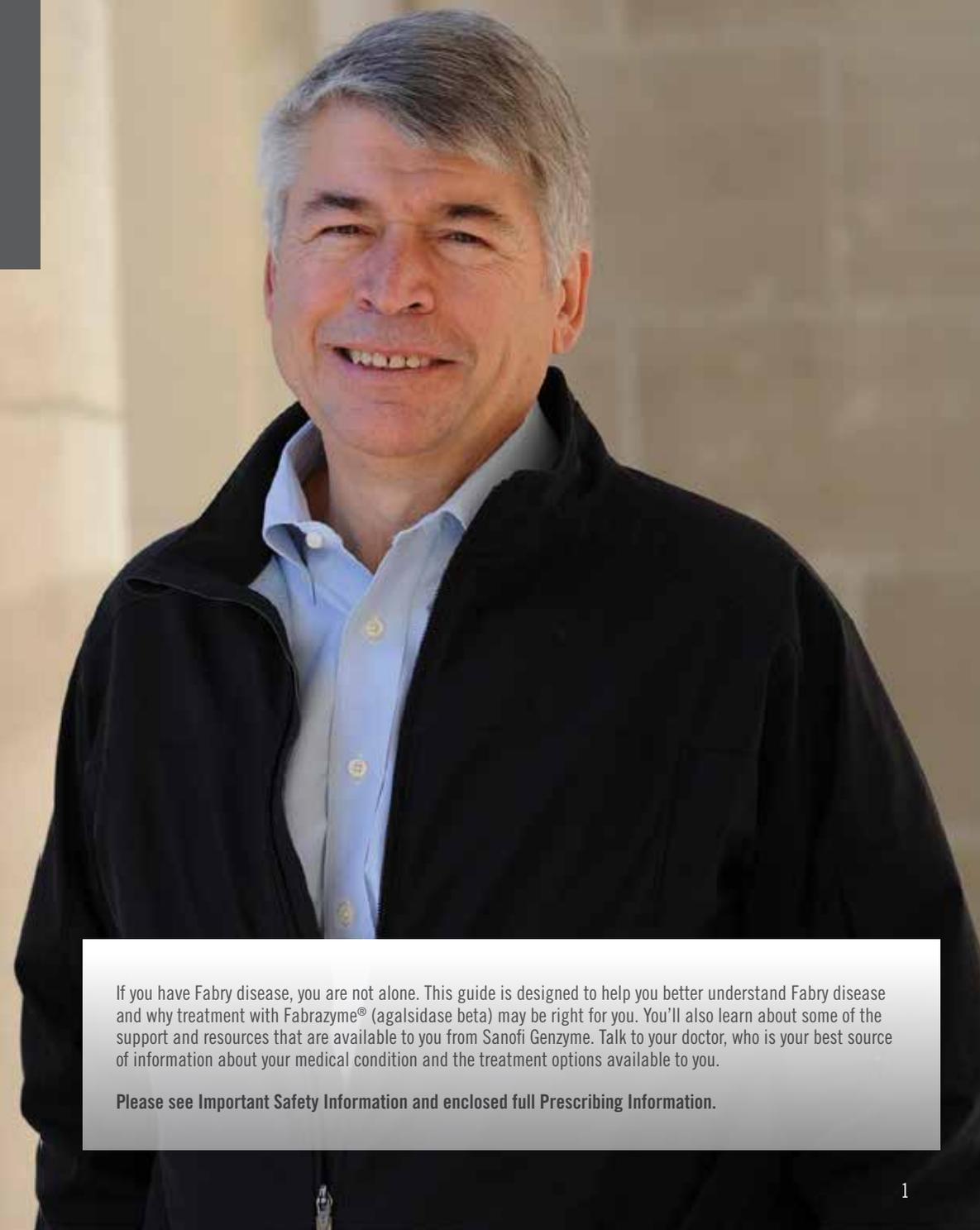
Please see Important Safety Information and enclosed full Prescribing Information.

## UNDERSTANDING FABRY DISEASE

Fabry disease is caused by a faulty gene. A person who inherits this gene is unable to produce enough of an enzyme known as 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, but instead builds up in cells throughout the body.

GL-3 build-up starts before birth and continues over decades. Early signs and symptoms of Fabry disease may include pain in the hands and feet, fatigue, digestive problems, and an inability to sweat. 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, people with Fabry disease are at risk for potentially life-threatening problems such as kidney disease, an enlarged heart, heart valve problems, and early stroke.



If you have Fabry disease, you are not alone. This guide is designed to help you better understand Fabry disease and why treatment with Fabrazyme® (agalsidase beta) may be right for you. You'll also learn about some of the support and resources that are available to you from Sanofi Genzyme. Talk to your doctor, who is your best source of information about your medical condition and the treatment options available to you.

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

# WHY FABRAZYME® (AGALSIDASE BETA)?

Fabrazyme is the first approved prescription treatment for Fabry disease. Here's why you should consider Fabrazyme:

- Without treatment, GL-3 continues to build up in people who have Fabry disease.
- With Fabrazyme, GL-3 levels were significantly reduced at 5 months of treatment in clinical trials.
  - Fabrazyme has been shown to lower GL-3 levels in certain cells across 3 key organs: the kidney, heart and skin.
- Fabrazyme has been widely studied for the treatment of Fabry disease.
  - Four clinical trials of Fabrazyme included 162 patients who had a combined 473 years of experience.
- Ask your doctor if Fabrazyme is right for you.



Sophia and Blanca, Fabry patients

## Fabrazyme Indication

Fabrazyme 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.

## How Fabrazyme Works

Fabrazyme is a replica of the alpha-GAL enzyme normally produced by the human body. Just like naturally occurring alpha-GAL, Fabrazyme breaks down GL-3.

The way it works is not based on patient genotype (DNA mutation), phenotype (disease presentation or severity), or level of enzyme in your body.



## 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.

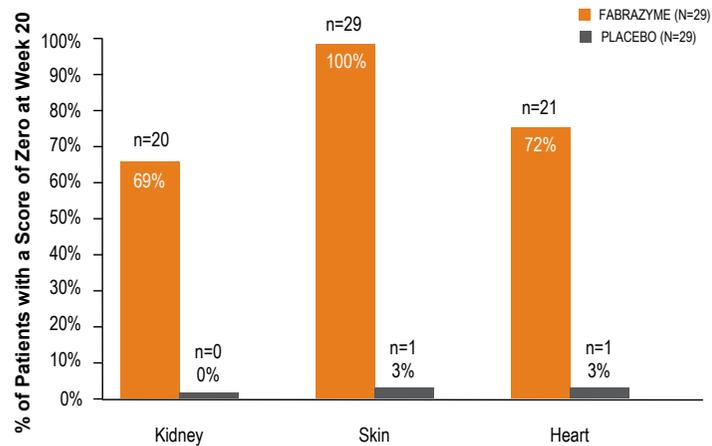
- If you experience a severe allergic or anaphylactic reaction, your healthcare professional will immediately stop the infusion of Fabrazyme and provide you the necessary emergency medical treatment. Because of the possibility that severe allergic reactions may occur, appropriate medical support should be available during your Fabrazyme infusion.

**Please see enclosed full Prescribing Information.**

## Fabrazyme (agalsidase beta) Cleared GL-3 at 5 Months and Sustained Clearance for Up to 5 Years in a Clinical Study

- Fabrazyme reduced GL-3 in cells lining the blood vessels of 3 key organs: the kidney, heart and skin.
  - Patients received a score of 0 to 3 based on the amount of GL-3 in their cells. Most patients (69%) received a score of 0, meaning their GL-3 level was normal or near normal.
  - Fabrazyme reduced GL-3 to normal or near normal levels in almost all patients.
  - 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.
- Fabrazyme demonstrated an effect in 5 months. That means if you started treatment with Fabrazyme in spring, by the end of summer, your GL-3 levels could be lower.

### Fabrazyme normalized GL-3 in certain cells of the kidney, skin and heart at 5 months



- Study included 58 Fabry patients ages 16-61. Patients in this study received either Fabrazyme or placebo every two weeks for five months. Forty-four of the 58 patients completed an additional 54 months of treatment with Fabrazyme in an open-label extension study.
- Similar long-term responses were seen in most patients, with sustained GL-3 clearance in cells lining the blood vessels of the kidney (6 of 8 patients), heart (8 of 8) and skin (31 of 36) at 4.5 years.

## Important Safety Information, continued

**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 enclosed full Prescribing Information.



Ricky, Fabry patient

### Fabrazyme Keeps Working When You Keep Taking It

- Regular infusions of Fabrazyme every two weeks help ensure that the body has an ongoing supply of enzyme to reduce the GL-3 in certain cells in the kidney, heart and skin.
- It's important to continue to take Fabrazyme as directed, even if you don't notice any symptoms, to help ensure that GL-3 doesn't start to build up again.

## FABRAZYME (AGALSIDASE BETA) CAN BE ADMINISTERED AT HOME, AT A DOCTOR'S OFFICE OR AT AN INFUSION CENTER

Fabrazyme is administered by intravenous (IV) infusion. The amount of Fabrazyme you will be given is based on how much you weigh. The recommended dosage of Fabrazyme is 1 milligram (mg) for each kilogram (kg) of body weight, infused once every two weeks. The infusion can take place at your home, your doctor's office, or at an infusion center. Because of the possibility that severe allergic reactions may occur, appropriate medical support should be available during your Fabrazyme infusion. Talk to your doctor about the right location for you as well as infusion expectations.

### 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.

Please see 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

**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.**

# 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 [CareConnectPSS@sanofi.com](mailto:CareConnectPSS@sanofi.com).

## Contact a Case Manager:

1-800-745-4447 (option 3)  
Monday–Friday 8am–6pm EST

## Connect with us online: [careconnectpss.com](http://careconnectpss.com)



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