WOMEN WITH FABRY

It may be time to think more seriously about your Fabry.

Understanding the medical history, variable effects, and management of Fabry disease in women

Select Important Safety Information

What is Galafold?

Galafold® (migalastat) is a prescription medicine used to treat adults with Fabry disease who have a certain genetic change (variant) in the galactosidase alpha gene *(GLA)* that is responsive (amenable) to Galafold.

It is not known if Galafold is safe and effective in children.

Before taking Galafold, tell your healthcare provider about all of your medical conditions, including if you:

- have kidney problems.
- are pregnant or plan to become pregnant. It is not known if Galafold will harm your unborn baby.
- are breastfeeding or plan to breastfeed. Galafold may pass into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take Galafold.

Please see full Important Safety Information on the last page and click here for Full Prescribing Information, including Patient Information and Instructions for Use, also available at Galafold.com.

We know that Fabry can significantly affect women.

Before 2001, women with Fabry disease were considered by medical professionals to be asymptomatic "carriers", meaning that they could pass on Fabry to their children without actually having any signs or symptoms.

Despite past misconceptions, we now know that most women who have a variant (or mutation) in the *GLA* gene do experience the effects of Fabry, often as severely as men with Fabry.

"You are going to be told 'no', because as a woman, they're more likely to say that you are just a carrier."

—Rebecca, a woman with Fabry, on seeking the answers she needed

Take charge of your care

Because the misconception that women are only carriers of Fabry has been so prevalent in the medical community, some healthcare professionals may still not take Fabry as seriously in women. But thousands of women around the world have Fabry and suffer from its impact on their health.

The genetics of Fabry—it's X-linked.

Chromosomes vary by sex and carry genetic information that determines your traits.

Males have one X and one Y chromosome, while females have two X chromosomes.

Fabry disease is an X-linked genetic disorder caused by a change—called a variant or mutation—in the *GLA* gene on the X chromosome. This variant leads to a deficient or absent alpha-Gal A enzyme, causing fatty substances (substrates) to build up in the part of the cell called the lysosome.

Without the normal breakdown of substrates by alpha-Gal A, cellular damage and Fabry symptoms may occur in multiple organs throughout the body. And because Fabry is an X-linked disorder and women have two X chromosomes, the *GLA* gene that causes Fabry may work normally in some parts of a woman's body but not others.

Understand Fabry genetics, by looking at calico cats.

Calico cats have multiple colors in their fur and are almost always female. Why? Calico cats' fur color is determined by genes on the X chromosome. Because female cats inherit both their father's and mother's X chromosome, they can have both their mother's and father's fur colors shown in different parts of their coat.

Fabry disease works the same way. Because the *GLA* gene is located on the X chromosome, a woman with Fabry can have one X chromosome with a mutation that causes Fabry and one X chromosome without a Fabry mutation. Throughout the body, only one of the two X chromosomes may be expressed, meaning its effects will be felt. This random expression varies from body part to body part, which is why a woman with Fabry can have one part of her body that is affected by Fabry and another part that isn't.

Most women have symptoms, but a later diagnosis.

Women with Fabry may experience all or just some of the effects of Fabry to different extents, or they might never feel any of them. But if you are a woman who is experiencing symptoms of Fabry, you are in the majority.

Though women may first notice effects of Fabry when they are teenagers, they often have to wait years before finding out the cause.

of women diagnosed with Fabry report having signs and symptoms of the disease.

is the average amount of time between when women first notice effects of Fabry and when they receive a definitive Fabry diagnosis.

For women, enzyme activity doesn't tell the whole story.

Symptoms can still develop, and Fabry can still be severe and progressive, in women with normal to detectable levels of alpha-Gal A enzyme activity. So while an enzyme blood test may show that you have alpha-Gal A enzyme activity, that doesn't mean that you don't have Fabry. And it doesn't mean that your body is working the way it should.

This is why genetic testing is required to diagnose Fabry in women. Genetic testing will also provide important information on your *GLA* variant, which can help determine your potential treatment options.

Symptoms can vary, so it is important that you and your healthcare provider recognize the type of symptoms that may be caused by Fabry and address them appropriately.

Fabry disease requires consistent monitoring.

Some signs and symptoms of Fabry disease are very noticeable. Other changes that are happening in your body may not be as obvious. One survey looking at pain intensity, duration, and frequency as a result of Fabry disease in both genders showed that pain ratings from women were virtually indistinguishable from those of men.

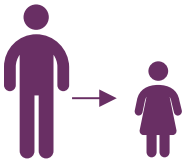
Regardless of if or how you are experiencing its symptoms, Fabry is a progressive disease, meaning that damage may be happening even if you can't see it. Keep an eye on your disease, as it can inform what steps you and your healthcare team take in managing it. Monitoring your Fabry may also help your healthcare provider determine which treatment(s) may be right for you.

"I finally found a doctor with an interest in learning about Fabry. Ultimately, you have to have medical support."

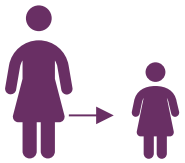
—Joyce, a woman with Fabry, on finding a healthcare team

It may be time to talk to other family members, too.

Fabry can be passed down by either sex, so there may be more people in your family affected by Fabry than you think.



100% of the time, fathers pass Fabry to their daughters.



50% of the time, mothers pass Fabry to their children, male or female.

We know that people with Fabry may have feelings of guilt for possibly passing Fabry on to their children. While those feelings are real, it's important to understand and accept that we can't control our genetics. What you can control is how you care for yourself and your children once you know what you're up against.

From diagnosis through treatment, you are your own best advocate.

From your specific variant (mutation) to how Fabry disease affects your body, the way you experience Fabry is unique. So is when and how it's treated.

The decisions around treating your Fabry are between you and your healthcare providers. You may face resistance to treating your Fabry or hesitation to treat until your symptoms are more severe (a "wait and see" approach).

Remember that you are a part of these decisions. Begin by cultivating a healthcare team and community that will listen attentively to your goals and concerns. It is crucial that you understand the type of symptoms that you may experience, and that you communicate to your healthcare team exactly what you are feeling. Because Fabry symptoms can be variable, it is sometimes difficult to connect the dots and understand what may be caused by your Fabry disease.

> "For women especially, if you feel like you need that treatment, then you need to push for it. That's what I did."
>
> —Rebecca, on speaking up for herself

When it comes to your care, your voice is one of your strongest weapons. Speak up. Ask questions. Make requests. Though it may take some time to build these relationships, cultivating open channels of communication with the members of your healthcare team will be to your benefit.

There are treatments you and your care team can consider.

Galafold® (migalastat) 123 mg capsule

Oral therapy may be an option for adults with a *GLA* gene variant that is amenable (responsive) to Galafold. Your healthcare provider can help you identify if your *GLA* variant is amenable.

Galafold
An oral option for adults with confirmed Fabry disease and an amenable *GLA* gene variant.

Enzyme replacement therapy (ERT)
Administered intravenously (IV) for patients with Fabry disease.

"I had the hardest time with what treatment looked like for life with newborn twins. With Galafold, I am satisfied with my routine."

—Veronica, an actual Galafold patient, on finding what worked for her

Select Important Safety Information (continued)

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Especially tell your healthcare provider if you take medicines or supplements containing caffeine as these medicines or supplements may affect how Galafold works.

Please see full Important Safety Information on the last page and click here for Full Prescribing Information, including Patient Information and Instructions for Use, also available at Galafold.com.

"I am a female with Fabry. Know that what you have is real, what you feel is real."

—Veronica, an actual Galafold® (migalastat) patient

To hear other patients discuss their experiences with Fabry disease and Galafold, visit the Videos & Resources page at Galafold.com.

Important Safety Information

What is Galafold?

Galafold® (migalastat) is a prescription medicine used to treat adults with Fabry disease who have a certain genetic change (variant) in the galactosidase alpha gene *(GLA)* that is responsive (amenable) to Galafold.

It is not known if Galafold is safe and effective in children.

Before taking Galafold, tell your healthcare provider about all of your medical conditions, including if you:

- have kidney problems.
- are pregnant or plan to become pregnant. It is not known if Galafold will harm your unborn baby.
- are breastfeeding or plan to breastfeed. Galafold may pass into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take Galafold.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Especially tell your healthcare provider if you take medicines or supplements containing caffeine as these medicines or supplements may affect how Galafold works.

How should I take Galafold?

Do not eat food, or take or drink any product that contains caffeine at least 2 hours before **and** 2 hours after taking Galafold to give a minimum 4 hour fast.

What are the possible side effects of Galafold?

The most common side effects of Galafold include headache, stuffy or runny nose and sore throat, urinary tract infection, nausea, and fever.

These are not all the possible side effects of Galafold. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to Amicus Therapeutics at 1-877-4AMICUS.

Please click here for Full Prescribing Information, including Patient Information and Instructions for Use, also available at Galafold.com.

Amicus Therapeutics®