

# Take charge of your Fabry



There may be many challenges that come with Fabry, and speaking up for the care you need is an important part of managing your disease.

## 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 Important Safety Information on the back cover and Full Prescribing Information and Instructions for Use, in pocket and also available at [Galafold.com](http://Galafold.com).**



**Galafold**®  
(migalastat)

# Table of Contents

Introduction	4
About Fabry Disease	6
Your Care Team	8
Treatment Options	10
Amicus Assist™	11

# Your care starts with you.



Because Fabry is a rare disease, staying informed and advocating for your needs is an essential part of managing your care.

You may encounter healthcare professionals who are either unaware of Fabry disease, or do not see people with Fabry on a regular basis. Because symptoms can vary greatly, it may be up to you to ensure that your symptoms are recognized and addressed appropriately.

This guide includes key information and tips that may help you work with your care team to manage your Fabry.

## 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.

**Please see Important Safety Information on the back cover and Full Prescribing Information and Instructions for Use, in pocket and also available at [Galafold.com](https://www.galafold.com).**

We'll begin by reviewing how Fabry can affect your body—both in the long term and on a day-to-day basis. Being able to clearly communicate the impact Fabry has on your life may help healthcare professionals develop a plan to manage your disease.

Because each person with Fabry is unique, so too is each care team. This guide identifies the specialists that might be involved in your care, depending on your specific needs. Having one healthcare provider that acts as the primary coordinator can help ensure that all members of your care team are on the same page. But remember—the most important person on your care team is you.

Finally, this guide will cover the treatment options that may be available to you. If you and your healthcare provider decide that Galafold® (migalastat) is right for you, Amicus Assist™, our patient support program, may help you navigate insurance and get started on your treatment journey.

Being able to clearly communicate the impact Fabry has on your life may help healthcare professionals develop a plan to manage your Fabry.

# Your Fabry is unique to you.

Fabry is a rare, progressive disease that can affect many parts of the body.

There are many genetic variants of Fabry disease. The variant you have can affect which of your organs are impacted, what symptoms you have, when they appear, and how severe they become. Some symptoms may be noticeable. Others might not be as obvious. That's why it's important to work with your doctor to monitor all the different parts of your body that could be affected by Fabry. Monitoring your Fabry may also help your doctor determine which treatment(s) may be right for you.

## KEY TERM

**Variant:** A mutation or change in a gene

## WOMEN WITH FABRY

It was once believed that women were “just carriers” of Fabry, meaning they could pass it on to their children but didn't have any of the effects that men do. We now know that both genders can experience symptoms and serious organ damage. Still, women may need to be particularly clear and persistent in speaking up about their disease.

# This is how Fabry may affect your body.

Fabry disease can vary widely from person to person, affecting various organs and resulting in a range of symptoms. Not all people with Fabry will experience the same symptoms.

## HEART

- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

## KIDNEYS

- Protein in the urine
- Decreased kidney function
- Kidney failure

## NERVOUS SYSTEM

- Nerve pain
- Intolerance to heat, cold, or exercise
- Transient ischemic attack (TIA) and stroke
- Pain or burning in the hands and feet
- Vertigo/feeling dizzy

## LUNGS

- Wheezing
- Difficulty breathing, shortness of breath
- Bronchitis
- Chronic cough

## PSYCHOLOGICAL

- Feelings of guilt, worry, depression, isolation, and/or fear

## GASTROINTESTINAL

- Nausea, vomiting, cramping, and diarrhea
- Pain/bloating after eating, feeling full after a small amount of food
- Constipation

## EARS

- Hearing loss, ringing in the ears (tinnitus)

## EYES

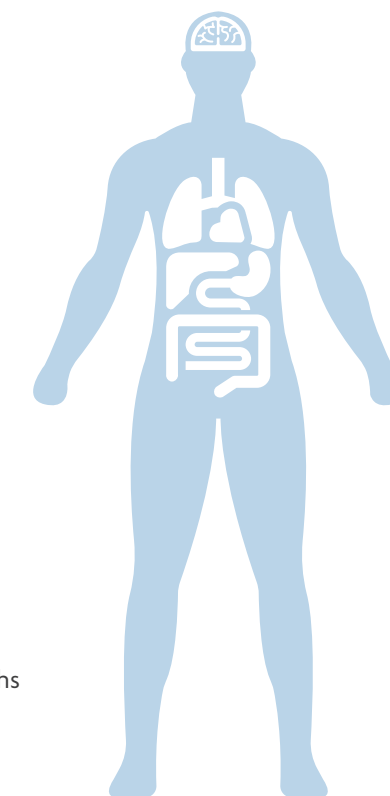
- A whorled pattern in the cornea
- Fabry cataracts

## SKIN

- Sweating less than normal or not at all
- Small, dark, red spots, mostly appearing between the belly button and upper thighs

## OTHER

- Fatigue—a deep feeling of tiredness



The relationship between fatty substance reduction with Galafold and improvement in symptoms, complications, or quality of life has not been established.

# How to work with your care team.

While you may have many specialists providing care, you might need to speak up to get the kind of care that's best for you. You may encounter some healthcare professionals who are unfamiliar with Fabry or the severity of your symptoms. Or, because they specialize in one particular area, they may simply not see the full picture of your disease.

## Be sure to discuss the following with your providers:

- The nature and severity of your symptoms
- How your symptoms affect your daily life
- Test results to monitor your Fabry
- Your family history of Fabry disease
- Any goals you may have that are affected by your condition

## NEED HELP?

We've created an interactive guide that will help drive your discussion at your next appointment. You can find it at [Galafold.com](https://Galafold.com) under Support & Resources.

Keeping track of medical information can be a challenge, especially when you see many doctors and specialists. Try recording your symptoms, appointments, and test results in a diary or tracker so you have all of your information in one place.

# Here's who might be on your care team.

Fabry is a progressive disease, so guidelines recommend that your care team monitors each affected system. Depending on your particular symptoms, your care team could include:



## Geneticist

Primary specialist who may oversee your Fabry treatment and management



## Genetic counselor

Gives personalized guidance based on your genes to help make decisions about your health



## Nephrologist

Uses a series of tests to monitor your kidneys and determine how well they are working



## Cardiologist

Uses a series of tests to monitor your heart and determine how well it is functioning



## Neurologist/pain specialist

Monitors your brain and nervous system including how you feel pain, heat, and cold



## Gastroenterologist

Monitors your gastrointestinal (GI) system and helps manage GI symptoms



## Psychologist/Psychiatrist

Supports you in dealing with feelings of depression and/or anxiety



## Others

Based on your particular symptoms

## TIP:

It may be helpful to choose the healthcare provider you're most comfortable with to act as the primary coordinator of your care team. This way, the burden to keep each care team member aligned isn't all on you.

These are just a few examples of the various healthcare providers you may need on your care team. Some people with Fabry may have just a couple of these providers on their care team, while others could have all of them plus more.

# Know your treatment options.

There are many genetic variants of Fabry disease. Depending on your variant, and your doctor's assessment, different treatment options may be appropriate for you.

While some people receive enzyme replacement therapy intravenously (through an IV), others may also be considered for treatment with Galafold® (migalastat). Galafold is the first and only oral therapy for adults with confirmed Fabry disease and an amenable *GLA* variant.

Gene variants are considered "amenable" if they have been determined to be responsive to Galafold. Talk to your doctor to learn which treatment options may be suitable for you.

Galafold uses the enzyme you already have. It isn't enzyme replacement.



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



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

## Select Important Safety Information (continued)

### 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.

**Please see Important Safety Information on the back cover and Full Prescribing Information, including Patient Information and Instructions for Use, in pocket and also available at Galafold.com.**



For adults prescribed Galafold, Amicus Assist™ is a call away.

Amicus Assist™ is a service designed to provide product assistance and support for Galafold.\* As part of the program, you'll be assigned a dedicated case manager who will provide you with ongoing support.

Amicus Assist™ is dedicated to helping identify possible sources of financial assistance, including:



**Co-Pay Assistance<sup>†</sup>:** Eligible, commercially insured patients may pay as little as \$0 per month for Galafold.



**Patient Assistance Program** for eligible, uninsured or underinsured patients.<sup>‡</sup>



**Provide ongoing support** throughout your treatment journey.



**Support from independent charitable foundations:**  
Amicus Assist™ can help identify independent charitable foundations who may be able to provide support. These foundations exist independently of Amicus and have their own eligibility criteria and application processes. Availability of support from the foundations is determined solely by the foundations.

**Reach out at 1-833-AMICUS-A (1-833-264-2872) Monday–Friday | 8AM–8PM ET**

\*Individuals must be prescribed Galafold for an approved indication to be eligible for support from Amicus Assist.

<sup>†</sup>Co-pay assistance is only available for patients with commercial (private) prescription drug insurance. Patients are not eligible for co-pay assistance if they are enrolled in a state or federally funded insurance program, including but not limited to Medicare, Medicaid, TRICARE, VA, DOD, or a state prescription drug assistance program or where prohibited by law. Offer is subject to program maximum limits. Offer is only available for U.S. residents who have a valid prescription for an Amicus product. Amicus reserves the right to rescind, revoke, or amend this offer without notice.

<sup>‡</sup>The Patient Assistance Program provides free product to those who qualify. Contact Amicus Assist for details.

Amicus makes no representation or guarantee concerning reimbursement or coverage for any service or item. Information provided through the Amicus Assist program does not constitute medical advice and is not intended to be a substitute for a consultation with a licensed healthcare provider or applicable third-party payer(s). Amicus reserves the right to modify the program at any time without notice.



# You've got this.

Remember, the most important member of your care team is you—so let your voice be heard.

For additional information and resources, visit [Galafold.com](https://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.

### 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 see Important Safety Information on the back cover and Full Prescribing Information and Instructions for Use, in pocket and also available at [Galafold.com](https://Galafold.com).**