

# DISCOVER MYALEPT®

Leptin replacement therapy  
for people with generalized  
lipodystrophy (GL)



—Raeya, living with GL

Myalept® (metreleptin) is a leptin replacement therapy used with a doctor-recommended diet to treat problems caused by not having enough leptin in people with generalized lipodystrophy (GL).

**For additional Important Safety Information, please  
see pages 26-27 and enclosed Medication Guide.**

**myalept®**  
**(metreleptin) for injection** 11.3 mg  
per vial



“

I would say Dena’s main love is theater. She really has the self-confidence to get up there and perform, even though she looks different and she is different. But she enjoys it so much, and she loves going to Broadway shows. That’s probably her biggest passion.

”

—Shelly, Dena’s mother

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Photographs in this brochure feature people living with GL and their caregivers.

- It is not known if Myalept is safe and effective when used to treat problems (complications) caused by partial lipodystrophy or to treat liver disease, including non-alcoholic steatohepatitis (NASH)
- Myalept should not be used to treat people with HIV-related lipodystrophy or people with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without signs or symptoms of congenital or acquired generalized lipodystrophy

### Myalept® may cause serious side effects, including:

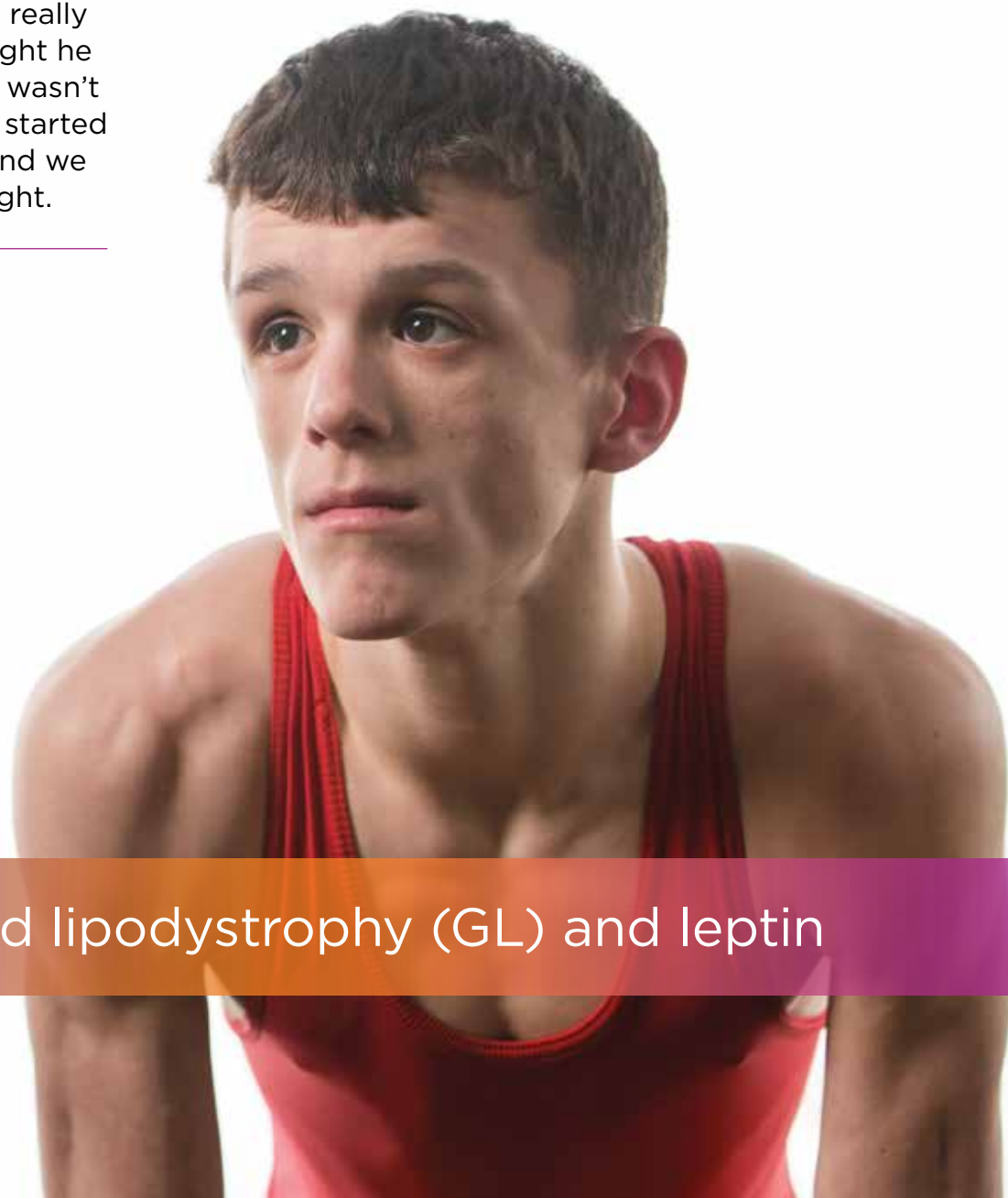
- **Risk for developing certain proteins called neutralizing antibodies** that may reduce how well your own leptin or Myalept works. Side effects of these antibodies may include infection, problems with blood sugar (including diabetes), or an increase in triglycerides
- **Increased risk of a type of blood cancer called lymphoma**

For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.

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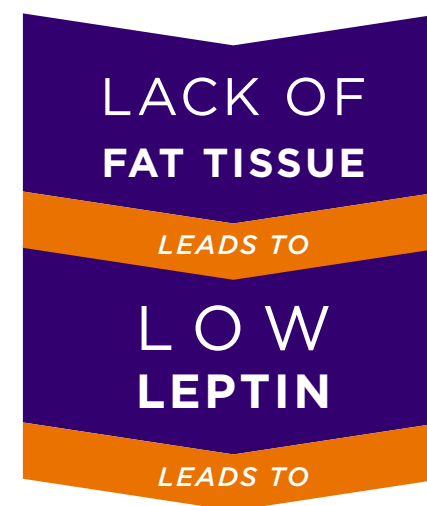
“  
Around age 5, Troy started losing fat in his cheeks and formed really muscular arms. We just thought he was hitting a growth spurt. It wasn't until a few years after that he started losing fat all over his body and we knew something wasn't right.

”  
—Jason, Troy's father



## Generalized lipodystrophy (GL) and leptin

## People with GL do not have enough leptin



— HIGH —  
TRIGLYCERIDES  
— HIGH —  
BLOOD SUGAR

Generalized lipodystrophy (GL) is a rare condition that results in having little to no fat tissue all over the body. Not only does the condition affect your appearance, the lack of body fat results in a loss of leptin, an important hormone that helps the body function properly.

Leptin is vital because it helps control some key metabolic processes in the body. Not having enough leptin can lead to health problems such as high triglycerides (fat in the blood) and high blood sugar. **This is where Myalept<sup>®</sup>, the only FDA-approved leptin replacement therapy, may be able to help.**

**You may get low blood sugar (hypoglycemia) if you take** Myalept with other medicines used to lower blood sugar, such as insulin or sulfonylurea. Your doses of these medications may need to be lowered while you use Myalept. Tell your doctor right away if you experience shakiness, sweating, headache, drowsiness, weakness, dizziness, confusion, irritability, hunger, fast heartbeat, or a jittery feeling because these may be signs of low blood sugar (hypoglycemia).

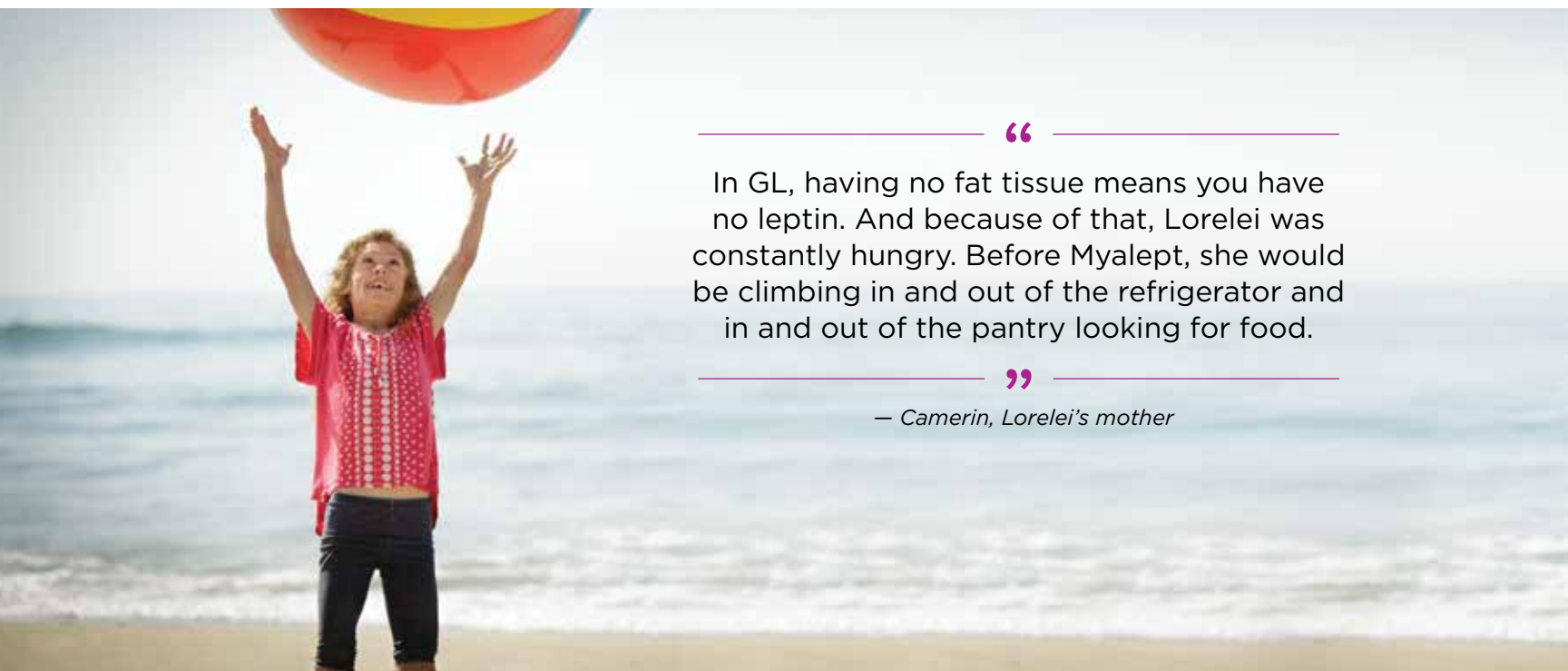
For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.

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## Three things you need to know about leptin

Leptin helps control certain important metabolic processes in the body such as:

- 1 Controlling how fat is broken down or stored.** When fat is not used and stored properly, it can collect in places it should not, for example, in organs, such as the liver and muscles, and also as triglycerides in the blood.
- 2 Helping the body respond to insulin.** Insulin is a hormone produced by the pancreas that helps manage levels of sugar in the blood. Without leptin, the body can develop insulin resistance, which may lead to diabetes in people with GL.
- 3 Telling the body when you have eaten enough food.** Without this signal, people with GL may be constantly hungry, even after eating a full meal.



“  
In GL, having no fat tissue means you have no leptin. And because of that, Lorelei was constantly hungry. Before Myalept, she would be climbing in and out of the refrigerator and in and out of the pantry looking for food.

”  
— Camerin, Lorelei's mother

## When leptin levels are too low, health can be affected

When important metabolic functions in the body are not controlled, it can lead to serious health problems, such as high triglycerides and high blood sugar.

To track these problems, people with GL should be aware of 3 important measures:

- **Triglyceride level.** This is the amount of fat in the blood.
- **Blood sugar level,** also referred to as blood glucose level. This is the amount of sugar in the blood.
- **A1C level.** This is a measure of the average blood sugar level over the course of 2 to 3 months. When blood sugar levels are too high, the risk of developing diabetes can increase in people with GL.

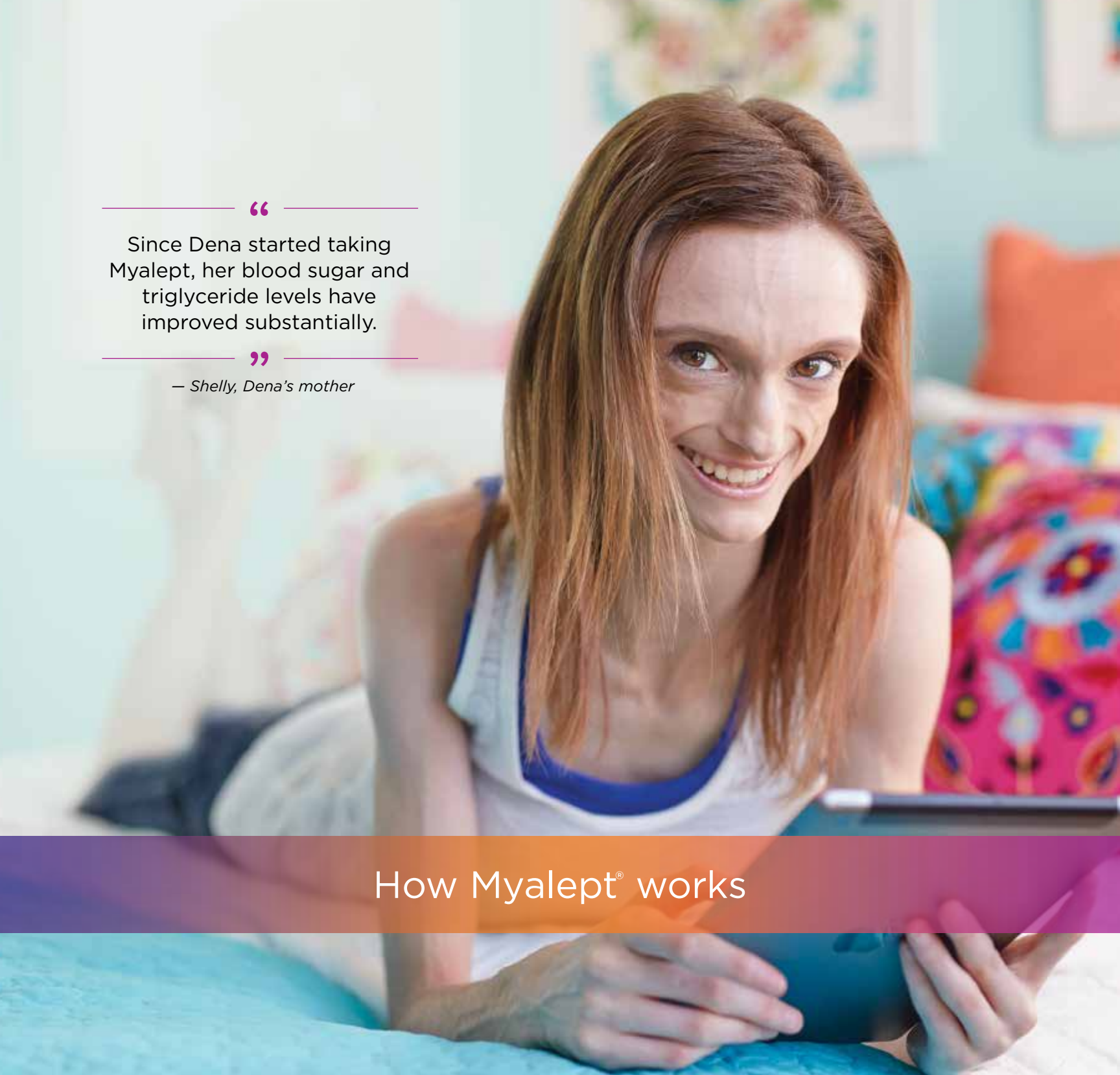
Not having enough leptin can cause these levels to be higher than normal in people with GL. Simple blood tests ordered by your doctor can show the level of triglycerides and sugars in the blood.

Because people with GL are often hungry, the extra fats and sugars taken in from food can make these high levels much worse, resulting in a continuous cycle.

**Myalept® may worsen symptoms caused by certain problems in your immune system (autoimmune disorder).** Ask your doctor about what symptoms you should watch for that may require further testing.

**For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.**

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“  
Since Dena started taking Myalept, her blood sugar and triglyceride levels have improved substantially.

”  
— Shelly, Dena's mother

## How Myalept® works

## Myalept replaces missing leptin

Myalept is a leptin replacement prescription medicine used along with a doctor-recommended diet for people with GL. Myalept treats problems caused by not having enough leptin in the body (leptin deficiency).

Because Myalept acts similarly to the body's naturally occurring leptin, it can help treat problems in people with GL that result from not having enough leptin.

### BY REPLACING — THE LEPTIN — YOUR BODY IS MISSING

#### Myalept

- ✓ Tells the brain when the body has had enough food
- ✓ Helps the body respond better to insulin

#### You should not take Myalept if you:

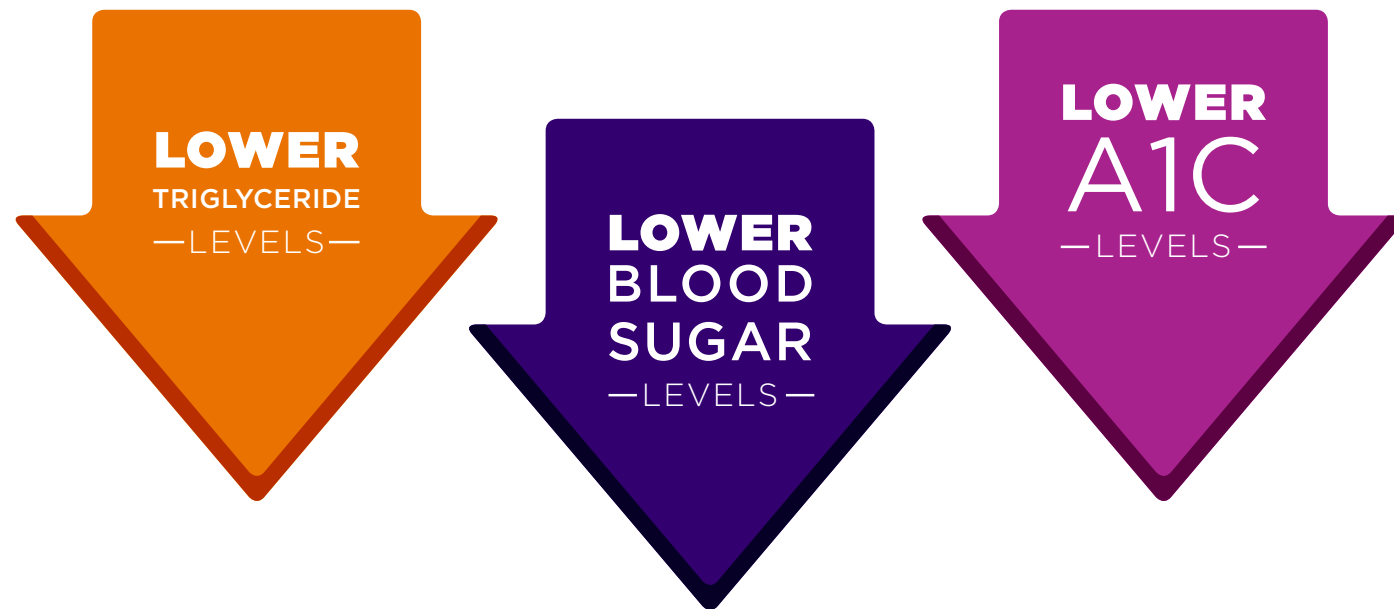
- Have general obesity not caused by a congenital leptin deficiency
- Are allergic to metreleptin or any of the ingredients in Myalept. Symptoms of an allergic reaction include rash, itching (hives), swelling of face, lips, tongue, or throat, problems breathing or swallowing, fainting or dizziness, rapid heartbeat

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## Myalept® treats problems from not having enough leptin

People with GL often have triglyceride and blood sugar levels that are above the normal range. Myalept has been shown to improve these levels, resulting in



### Myalept may cause serious side effects, including:

- **Risk for developing certain proteins called neutralizing antibodies** that may reduce how well your own leptin or Myalept works. Side effects of these antibodies may include infection, problems with blood sugar (including diabetes), or an increase in triglycerides
- **Increased risk of a type of blood cancer called lymphoma**

“  
It must have been 2 weeks after I started taking Myalept that my triglyceride levels started to go down.

”  
— Dwanna, living with GL



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“  
Once I learned about Myalept, I knew Raeya needed to be on it. Leptin is a hormone that Raeya’s body doesn’t produce, and Myalept is a leptin replacement therapy, so it made sense to me for Raeya to add Myalept right away.

”  
— Jilandre, Raeya’s mother

Talking with your doctor

## What you should tell your doctor



Tell your doctor if you have or have had problems with your blood cells (including low blood cell counts, especially your white blood cells), bone marrow, immune system, or pancreas; if you have or have had swollen glands on your neck or other locations, lymphoma, or high triglyceride levels; or if you use insulin or a sulfonylurea (SU).

Tell your doctor if you are pregnant or plan to become pregnant. If you become pregnant while using Myalept®, talk to your doctor about registering with a program to collect information about the outcomes of mothers and babies exposed to Myalept during pregnancy. Additionally, tell your doctor if you are breastfeeding or plan to breastfeed. You should not breastfeed while taking Myalept.

Tell your doctor about all the medications you take, including prescription and nonprescription medicines, vitamins, and herbal supplements. Make sure to keep your doctor and your pharmacist informed.

The most common side effects of Myalept include headache, low blood sugar, decreased weight, and/or abdominal pain.

**For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.**



## Questions for your doctor

- Q How will I know if Myalept® is working?
- Q How often will I need to have blood tests? Which tests will I get?
- Q What can I do if I experience side effects?
- Q Will my dose need to be increased over time?
- Q What should I be aware of if I am also taking insulin?
- Q Will I need to change dosing of my other medications?
- Q What can I do if the injections are too painful?

### Notes

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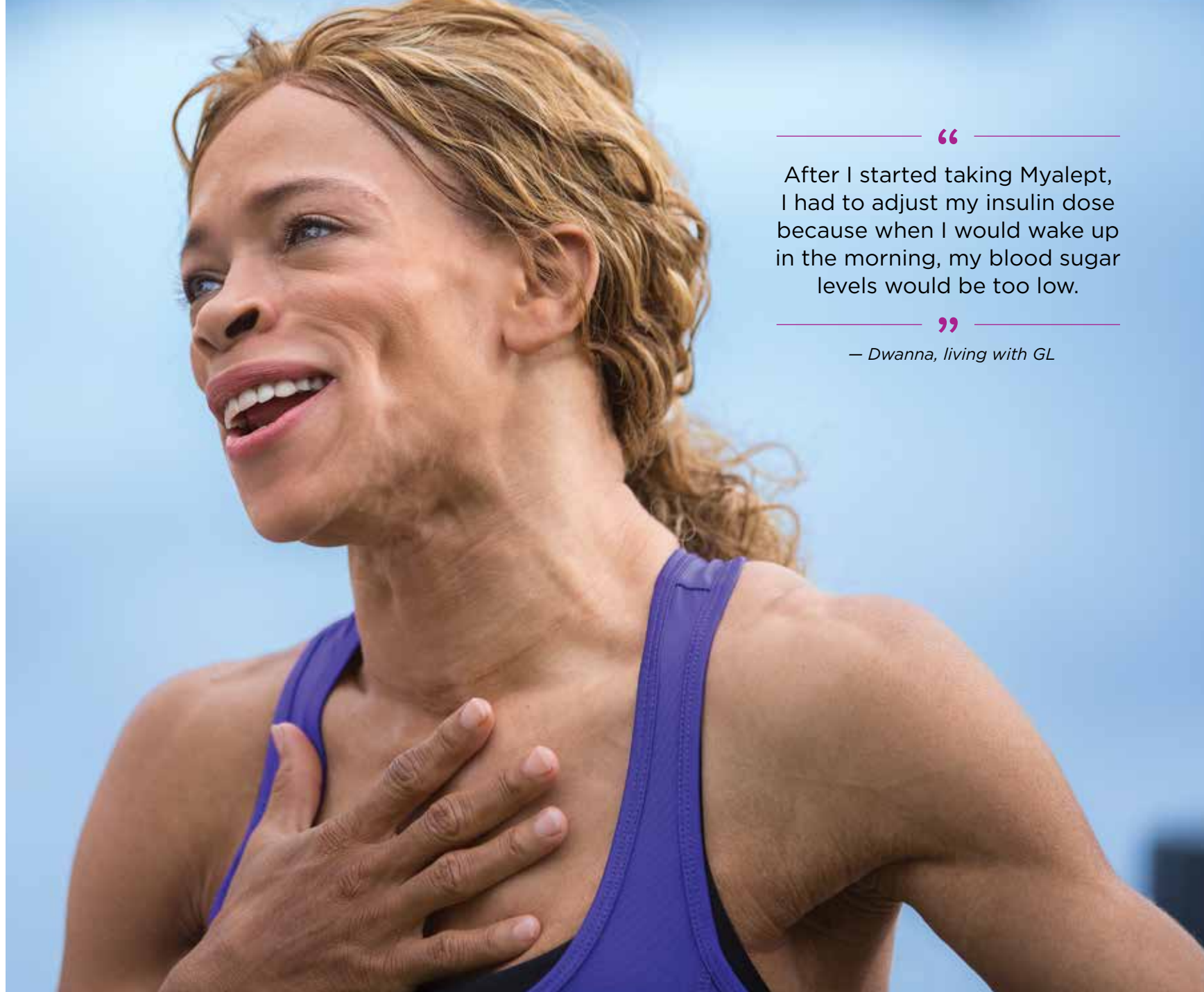
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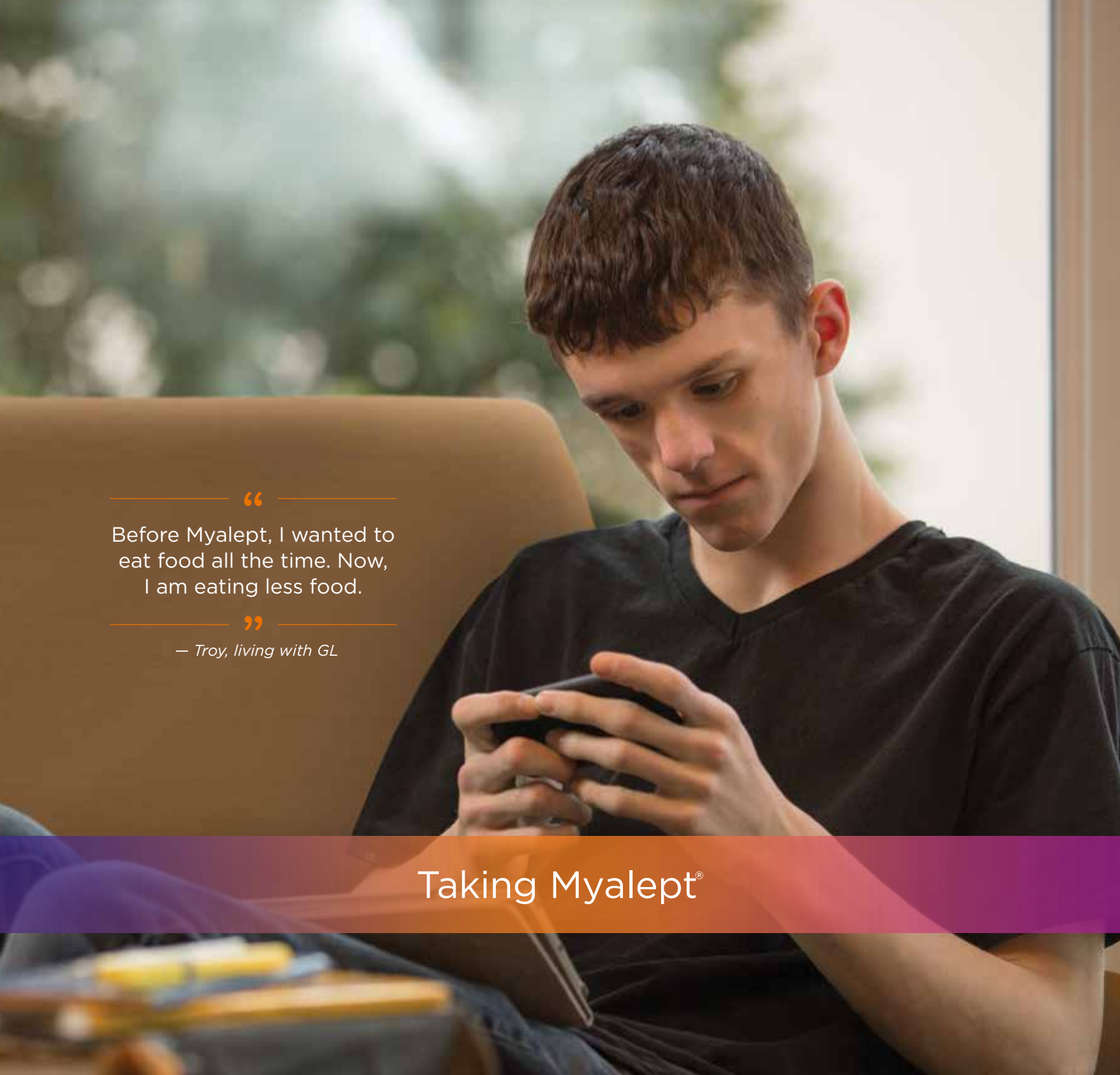
“  
After I started taking Myalept,  
I had to adjust my insulin dose  
because when I would wake up  
in the morning, my blood sugar  
levels would be too low.

”  
— Dwanna, living with GL

For additional Important Safety Information, please see  
pages 26-27 and enclosed Medication Guide.

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“  
Before Myalept, I wanted to eat food all the time. Now, I am eating less food.

”  
— Troy, living with GL

## Taking Myalept®

## Myalept is an injection you take once a day

Myalept is only available through certified pharmacies that are enrolled in the Myalept Risk Evaluation and Mitigation Strategy (REMS) Program. Your doctor must be enrolled and certified in the program in order to prescribe Myalept.

Your doctor will start you on a specific dose of Myalept based on your weight. Depending on how well you are doing, your doctor may increase or decrease your dose over time.

### Three things to know before you start:

- 1 Myalept comes in a powdered form and needs to be mixed with a liquid before injecting. The liquid used to mix Myalept for newborns is different from the liquid used for children and adults. It is very important to follow your doctor's instructions for mixing Myalept.
- 2 Before starting Myalept, your doctor should show you how to inject Myalept for the first time. It is important that your doctor watch your first injection of Myalept.
- 3 Myalept needs to be taken once a day, at the same time each day. You should find a time that works for you before starting treatment.

Step-by-step instructions on how to mix, store, and inject Myalept are enclosed in the pocket of this brochure and are included with each Myalept shipment.

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## Preparing your Myalept® dose

When you are ready to take Myalept, it is important to mix Myalept with the appropriate liquid as prescribed by your doctor, following these guidelines



### Newborn or infant

Myalept should be mixed with sterile **water for injection (preservative-free) (WFI)** and used right away. You should throw away any medication not used during each injection.

**Serious side effects** including death have happened in newborns or infants who have received the preservative benzyl alcohol. Bacteriostatic water for injection contains benzyl alcohol and it should not be used to mix Myalept for newborns and infants.



### Older child or adult

Myalept should be mixed with **bacteriostatic water for injection (BWFI)** and can be used for more than 1 dose for up to 3 days. Mixed Myalept should be stored in the refrigerator and out of the light. You should throw away any medication not used after 3 days.

Make sure to review the complete **Instructions for Use** before each injection.

## How do I take Myalept?

Always take medications exactly as instructed by your doctor. Below are some important things to keep in mind while taking Myalept.

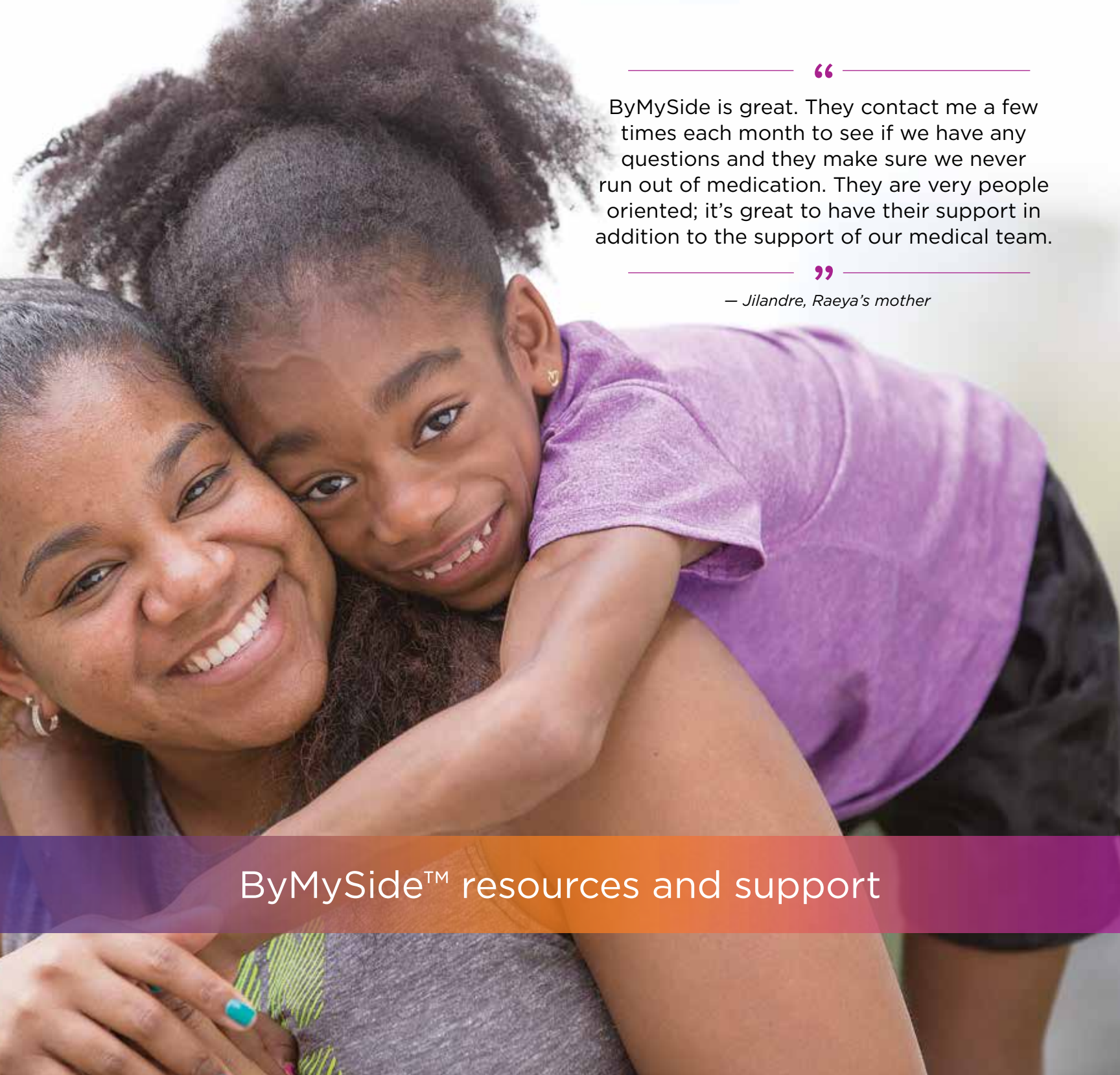
✓ DO	⊘ DO NOT
<b>DO</b> review the complete <b>Instructions for Use</b> before each injection	<b>DO NOT</b> inject Myalept into your muscle or veins
<b>DO</b> inject Myalept once a day, at the same time each day	<b>DO NOT</b> take more than your regular daily dosage in a single day
<b>DO</b> inject Myalept at room temperature	<b>DO NOT</b> take an extra dose or increase the amount of your dose to make up for a missed dose
<b>DO</b> mix Myalept with the right liquid depending on age	<b>DO NOT</b> mix Myalept and insulin in the same syringe or vial
<b>DO</b> inject Myalept in the upper arm, thigh, or abdomen	<b>DO NOT</b> shake Myalept when mixing
<b>DO</b> store Myalept in the refrigerator between 36°F and 46°F (2°C and 8°C) and away from light	<b>DO NOT</b> freeze Myalept
<b>DO</b> follow the diet recommended by your doctor	<b>DO NOT</b> stop taking Myalept without consulting your doctor
	<b>DO NOT</b> use if the liquid is cloudy or colored or has particles after mixing

### One more thing...

Although Myalept and insulin doses may be given at the same time, do not inject Myalept and insulin at the same injection site.

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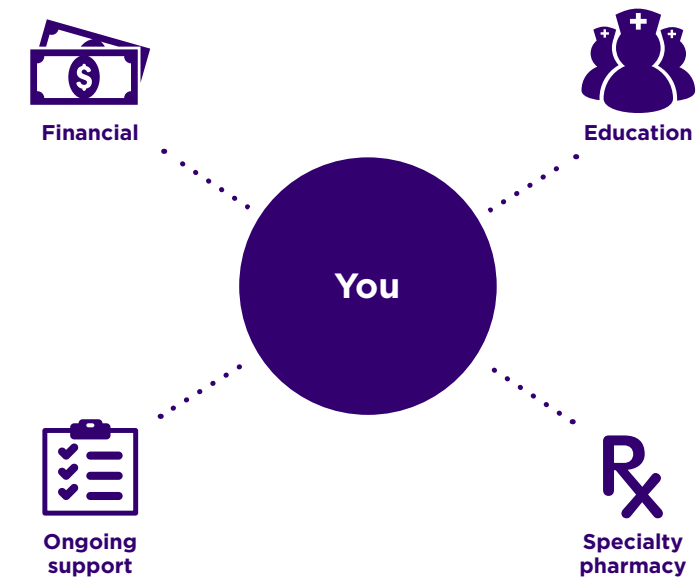
“  
ByMySide is great. They contact me a few times each month to see if we have any questions and they make sure we never run out of medication. They are very people oriented; it's great to have their support in addition to the support of our medical team.

”  
— Jilandre, Raeya's mother

ByMySide™ resources and support

## ByMySide is ready to help


To help you add Myalept® to your current generalized lipodystrophy (GL) treatment plan, the ByMySide team is built around you. ByMySide provides the support services and resources you need when you need them. When you sign up for the ByMySide Program, you can access these support services at no cost to you.



### We're here to help.

Hours: Monday through Friday, 8:00 AM to 8:00 PM (ET)

 1-855-6MYALEPT (1-855-669-2537)

 Myalept.com

For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.

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## Personalized assistance and guidance



### Insurance and financial assistance options

ByMySide aims to make Myalept® available and affordable to people with GL. Your dedicated ByMySide Coordinator works to make the insurance process easier for you. He or she can help you understand the cost of your treatment and refer you to independent financial assistance programs. Your Coordinator will also help determine if you qualify for the ByMySide Copay Program,<sup>a</sup> which covers costs of copayments or coinsurance at 100%, up to the program maximum, for eligible patients.



### Nurse education sessions

ByMySide registered nurses personally guide you through what you need to know about managing GL with Myalept. You can even schedule an in-person injection training session—at no cost to you—at your home or your doctor's office to learn how to mix and inject Myalept.



### Ongoing support

The ByMySide team can help you stay on track with your Myalept treatment by calling to remind you about refills and to ensure you have enough Myalept day to day. They can also provide you with additional supplies necessary for injecting Myalept and sharps disposal containers upon request.

<sup>a</sup>The ByMySide Copay Program is not valid for prescriptions eligible to be reimbursed, in whole or in part, by state or federally funded healthcare programs such as Medicaid, Medicare, TRICARE, VA/DOD, or any state prescription drug assistance programs. Other eligibility requirements may apply. Aegerion Pharmaceuticals, Inc. reserves the right to discontinue or modify the ByMySide Copay Program at any time.



“  
After I was diagnosed, it was a totally different world and it opened up a lot of possibilities. I thought it was cool because now I had a name to associate with what I have.

”  
— Dwanna, living with GL



### Specialty pharmacy services

Myalept is available only through the ByMySide Specialty Pharmacy Partners. Your Specialty Pharmacy will fill your prescription each month and deliver it directly to you whether you are at home or at work or even if you are traveling or moving. The Specialty Pharmacy also provides information on possible interactions with any other medications you may be taking, so be sure to tell the pharmacist and your doctor about all your medications, including vitamins and herbal supplements. In the event of an emergency, call 1-855-6MYALEPT (1-855-669-2537) for 24/7 pharmacist and nurse assistance.

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# Frequently asked questions about Myalept®

## Q Is Myalept approved by the FDA?

A In 2014, Myalept was approved by the FDA to be used along with a doctor-recommended diet to treat complications of not having enough leptin in people with generalized lipodystrophy (GL). Myalept has been studied for over 10 years.

## Q What diet should I follow while taking Myalept?

A Your doctor is the best resource for nutrition recommendations. Work with your doctor to develop a nutrition plan that is best for you.

## Q Can I get Myalept at my local pharmacy where I get all my other prescriptions?

A Myalept is not available at local pharmacies. It is available only through certified pharmacies that are enrolled in the Myalept REMS Program. Myalept is delivered right to your door through one of our Specialty Pharmacy Partners.

## Q How much does Myalept cost?

A Once your doctor has made the decision to prescribe Myalept, your Specialty Pharmacy will do a benefit verification with your insurance company to identify your out-of-pocket costs related to Myalept. Financial assistance is available to eligible patients to help cover these out-of-pocket costs. Call ByMySide at 1-855-6MYALEPT (1-855-669-2537) to learn about your options including the ByMySide Copay Program,<sup>a</sup> which covers costs of copayments or coinsurance at 100%, up to the program maximum, for eligible patients. (Please see page 22).

<sup>a</sup>The ByMySide Copay Program is not valid for prescriptions eligible to be reimbursed, in whole or in part, by state or federally funded healthcare programs such as Medicaid, Medicare, Tricare, VA/DOD, or any state prescription drug assistance programs. Other eligibility requirements may apply. Aegerion Pharmaceuticals, Inc. reserves the right to discontinue or modify the ByMySide Copay Program at any time.

## Q If I have questions about mixing, injecting, or storing Myalept, where can I get answers?

A Please review the complete **Instructions for Use** included in each Myalept shipment. It is also enclosed in the pocket of this brochure. If you have trouble finding what you need, you can also contact ByMySide for assistance. You can schedule an in-person injection training session, at no cost to you, with a ByMySide registered nurse. The nurse will come to your home or to your doctor's office to teach you how to mix and inject Myalept.

## Q Are there smaller needles available for injecting my child?

A Ask your doctor about prescribing the smallest possible needle to use for injecting Myalept to your child. Also, a ByMySide registered nurse can come directly to your home or doctor's office to provide training, at no cost to you, on how to properly inject Myalept.

## Q Can I stop taking Myalept at any time?

A Do not stop taking Myalept without consulting with your doctor. Stopping Myalept suddenly may cause a serious problem with your pancreas (pancreatitis) and very high triglycerides. You should take Myalept once a day every day unless otherwise directed by your doctor.

## Q How should I travel with Myalept?

A Myalept is portable, as long as you have a cool place to store it. Myalept should be stored between 36°F and 46°F (2°C and 8°C). Contact a ByMySide Coordinator at 1-855-6MYALEPT (1-855-669-2537) to learn how to travel with your Myalept.

For additional Important Safety Information, please see pages 26-27 and enclosed Medication Guide.



# Important Safety Information for Myalept®

## What is Myalept?

Myalept® (metreleptin) is a leptin replacement therapy used with a doctor-recommended diet to treat problems caused by not having enough leptin (leptin deficiency) in people with generalized lipodystrophy.

- It is not known if Myalept is safe and effective when used to treat problems (complications) caused by partial lipodystrophy or to treat liver disease, including non-alcoholic steatohepatitis (NASH)
- Myalept should not be used to treat people with HIV-related lipodystrophy or people with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without signs or symptoms of congenital or acquired generalized lipodystrophy.

## Important Safety Information

Myalept is only available through a restricted program called the Myalept Risk Evaluation and Mitigation Strategy (REMS) Program. Your doctor must be enrolled and certified in the program in order to prescribe Myalept

### Myalept may cause serious side effects, including:

- **risk for developing certain proteins called neutralizing antibodies** that may reduce how well your own leptin or Myalept works. Side effects of these antibodies may include infection, problems with blood sugar (including diabetes), or an increase in triglycerides
- **increased risk of a type of blood cancer called lymphoma**

### You should not take Myalept if you:

- have general obesity not caused by a congenital leptin deficiency
- are allergic to metreleptin or any of the ingredients in Myalept. Symptoms of an allergic reaction include rash, itching (hives), swelling of face, lips, tongue, or throat, problems breathing or swallowing, fainting or dizziness, rapid heartbeat.

### Before using Myalept, tell your doctor if you have any medical conditions including if you:

- have or have had problems with your blood cells, including low blood cell counts (especially your white blood cells), bone marrow, immune system, pancreas, swollen lymph nodes, lymphoma, high blood triglyceride levels, or use insulin or a sulfonylurea

- are pregnant or plan to become pregnant as it is unknown if Myalept will harm your unborn baby. If you become pregnant while using Myalept, talk to your healthcare provider about registering with a program to collect information about the outcomes of moms and babies exposed to Myalept during pregnancy. You can enroll in the Myalept program by calling 1-855-669-2537
- are nursing or plan to nurse. You should not nurse while you take Myalept

## Other possible side effects

The most common side effects of Myalept include headache, low blood sugar, decreased weight, and/or abdominal pain.

**You may get low blood sugar (hypoglycemia) if you take** Myalept with other medicines used to lower blood sugar, such as insulin or sulfonylurea. Your doses of these medications may need to be lowered while you use Myalept. Tell your doctor right away if you experience shakiness, sweating, headache, drowsiness, weakness, dizziness, confusion, irritability, hunger, fast heartbeat, or a jittery feeling because these may be signs of low blood sugar (hypoglycemia).

**For newborns and infants, mix Myalept with sterile water for injection (preservative-free) (WFI).** Serious side effects including death have happened in newborns or infants who have received the preservative benzyl alcohol. Bacteriostatic water for injection contains benzyl alcohol and it should not be used to mix Myalept for newborns and infants.

**Myalept may worsen symptoms caused by certain problems in your immune system (autoimmune disorder).** Ask your doctor about what symptoms you should watch for that may require further testing.

Talk to your doctor about any side effect that bothers you or that does not go away. These are not all the possible side effects of Myalept. For more information, speak to your doctor or pharmacist. Tell your doctor about all the medications you take, including prescription and nonprescription medicines, vitamins, and herbal supplements. Take Myalept exactly as your doctor instructs you.

Please read the Medication Guide and Instructions for Use for Myalept enclosed with this brochure.

**You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.FDA.gov/medwatch](http://www.FDA.gov/medwatch) or call 1-800-FDA-1088.**



# Get the leptin you need with Myalept®

- Myalept replaces leptin, an important hormone that people with GL are missing
- Myalept is used with a doctor-recommended diet and treats problems caused by not having enough leptin
- Myalept has been shown to:
  - Lower triglyceride levels
  - Lower blood sugar levels
  - Lower A1C levels

Talk to your doctor to see if Myalept is right for you.



Support  
Access  
Resources

## We're here to help.

Hours: Monday through Friday, 8:00 AM to 8:00 PM (ET)



1-855-6MYALEPT (1-855-669-2537)



Myalept.com

**For Important Safety Information, please see pages 26-27  
and enclosed Medication Guide.**

**Aegerion®**  
Pharmaceuticals

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