



**INTRODUCE YOURSELF TO WHAT'S POSSIBLE.**

**VIMIZIM™ (elosulfase alfa): the only therapy designed for people with Morquio A (MPS IVA).**

Life-threatening allergic reactions, known as anaphylaxis, can occur during VIMIZIM™ (elosulfase alfa) infusions. Typical signs of anaphylaxis include cough, rash, throat tightness, hives, flushing, changes in skin color, low blood pressure, shortness of breath, chest pain, and gastrointestinal symptoms such as nausea, abdominal pain, retching, and vomiting. Contact your doctor or get medical help right away if these symptoms occur during or after VIMIZIM infusions. If you have a respiratory illness, you may be at risk for a sudden worsening of your condition, and you may require additional monitoring.

Please see Important Safety Information, including important warning, on page 14.

**VIMIZIM™**  
(elosulfase alfa)

## NOW THERE'S A MEDICINE DESIGNED FOR MORQUIO A.

It's just for you. And it could change the way you live with your condition. Now you have VIMIZIM™ (elosulfase alfa). It's the only enzyme replacement therapy (ERT) approved by the US Food and Drug Administration for people with Morquio A. This brochure can help you learn about Morquio A and how VIMIZIM can treat it.



**TALK TO US ABOUT STARTING THERAPY WITH VIMIZIM™ (elosulfase alfa).  
JUST CALL 1-855-MORQUIO (1-855-667-7846) TODAY.**

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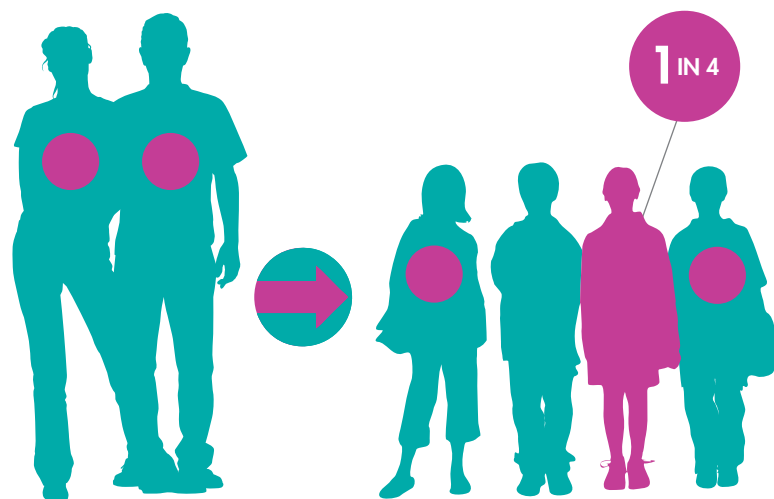
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## WHAT CAUSES MORQUIO A?

Morquio A is a recessively inherited condition. This means that both parents must have a malfunctioning gene to pass Morquio A on to their children.

When **2 parents who are carriers** have children, each pregnancy presents a **25% chance (1 in 4)** of passing Morquio A on to that child.



● = Morquio A carriers

People born with Morquio A are missing, or don't have enough of, an enzyme known as *N*-acetylgalactosamine-6 sulfatase, or GALNS. The purpose of this GALNS enzyme is to break down materials called glycosaminoglycans, or GAGs, the body can't use. Since certain GAGs are not broken down and remain stored in the lysosomes (the parts of a cell that help digest and degrade materials), Morquio A is known as a lysosomal storage disorder.

When the GALNS enzyme is missing, GAGs build up in the lysosomes of cells throughout the tissues and organs of the body. This buildup can cause serious health problems, including heart disease, skeletal abnormalities, vision and hearing loss, and difficulty breathing.



ENZYMES ARE PROTEINS THAT HELP PERFORM SPECIFIC JOBS IN YOUR BODY.

# HOW CAN MORQUIO A AFFECT YOU?

When GAGs build up in the bodies of people with Morquio A, the results can potentially be severe. People with Morquio A have physical problems and organ complications that can lead to difficulties performing daily activities, and may cause them to need walking aids or wheelchairs.

**The symptoms that go along with Morquio A can lead to damage in multiple organs and systems.**

<b>SKELETAL SYMPTOMS</b>	<b>NONSKELETAL SYMPTOMS</b>
Skeletal deformities	Breathing problems
Short trunk	Heart valve problems
Weakness in the neck	Muscle weakness
Spinal cord compression	Eye problems
Knock knees	Hearing loss
Overly flexible joints	Dental problems
Abnormal walk	Enlarged liver
Spinal problems	Enlarged spleen
Chest deformities	Limited energy

## **A geneticist can help.**

As Morquio A progresses, different parts of the body could be affected and different doctors may be needed to help manage the condition. One of the most important doctors in the management of Morquio A is the geneticist, who plays a major role in diagnosis and your ongoing treatment. Your geneticist will take the lead in your treatment and coordinate with the other specialists who make up your healthcare team.

### **Other doctors you might need:**

- *Cardiologist (heart doctor)*
- *Ear, nose, and throat (ENT)*
- *Surgeons with experience in Morquio A (eg, an orthopedic surgeon)*
- *Pulmonologist*

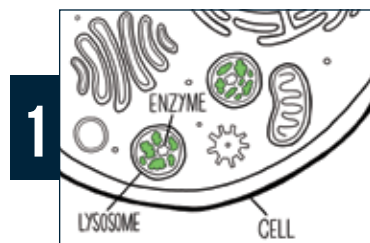
**FOR MORE INFORMATION ABOUT MORQUIO A, VISIT [WWW.MORQUIOSITY.COM](http://WWW.MORQUIOSITY.COM).**

# TAKE ACTION WITH VIMIZIM™ (elosulfase alfa)

## New medicine. New possibilities.

VIMIZIM™ (elosulfase alfa) is the only ERT to address the cause of Morquio A. VIMIZIM may be able to help you go further, but it is not a cure.

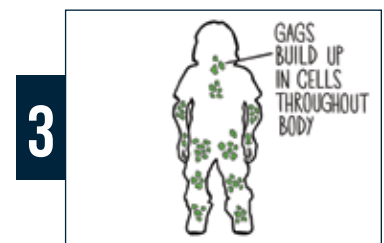
## How does VIMIZIM work?



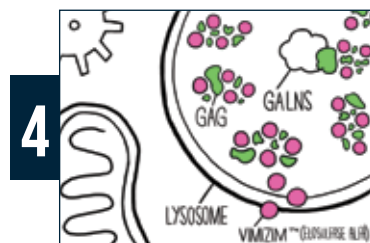
Inside every cell are structures called lysosomes. Lysosomes contain enzymes that help break down and recycle materials known as glycosaminoglycans, or GAGs.



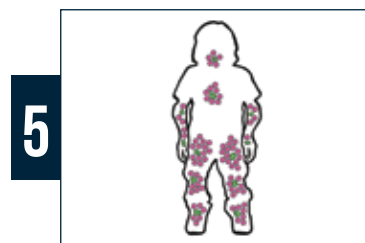
People with Morquio A do not have enough of the GALNS enzyme. This important enzyme is a kind of protein that gets rid of certain GAGs that build up in the lysosomes.



In people with Morquio A, GAGs build up in the lysosomes of cells throughout the tissues and organs of the body, potentially causing serious physical problems.



A weekly infusion of VIMIZIM replaces the deficient GALNS enzyme activity your body needs to break down the buildup of certain GAGs.



Taking VIMIZIM every week can help people with Morquio A reduce buildup of certain GAGs.

## How can VIMIZIM help you?

Simply put, if you have Morquio A, you have deficient enzyme activity. VIMIZIM can replace the missing enzyme to restore cell function.

**Life-threatening allergic reactions, known as anaphylaxis, can occur during VIMIZIM™ (elosulfase alfa) infusions. Typical signs of anaphylaxis include cough, rash, throat tightness, hives, flushing, changes in skin color, low blood pressure, shortness of breath, chest pain, and gastrointestinal symptoms such as nausea, abdominal pain, retching, and vomiting. Contact your doctor or get medical help right away if these symptoms occur during or after VIMIZIM infusions. If you have a respiratory illness, you may be at risk for a sudden worsening of your condition, and you may require additional monitoring.**

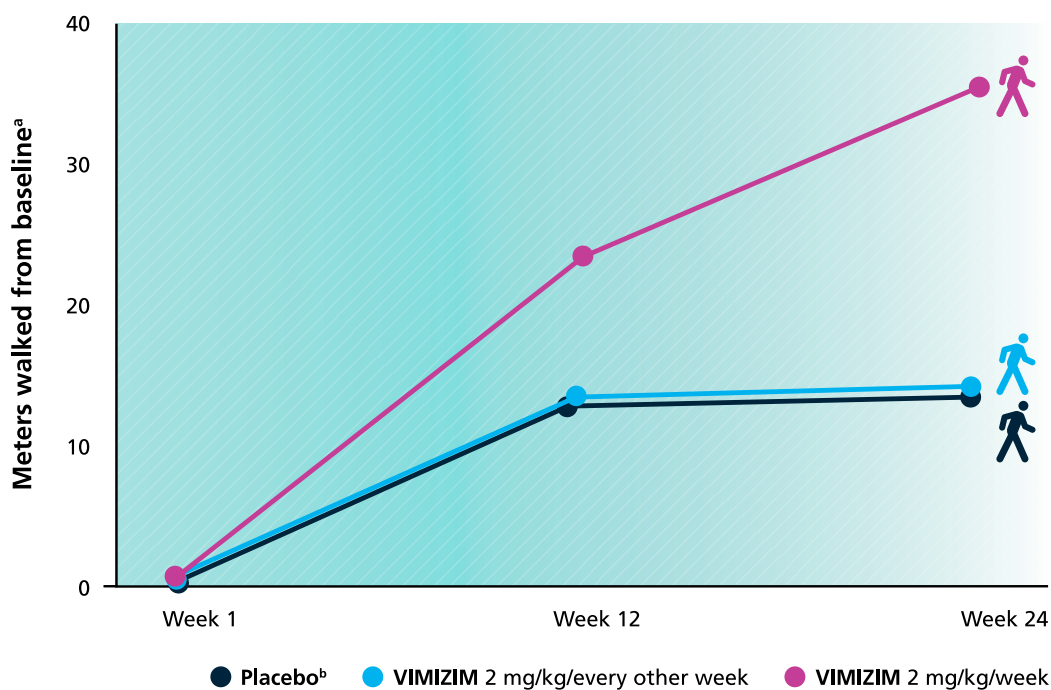
**// NOW THE BOYS HAVE MORE ENERGY TO  
PLAY AFTER SCHOOL AND BE THEMSELVES. //**

- Debbie, mother of Luke (age 16) and Zane (age 14)



# VIMIZIM™ (elosulfase alfa) COULD TAKE YOU FURTHER.

In a 6-month clinical trial, people who took VIMIZIM every week improved their endurance as measured by the 6-minute walk test (6MWT).



This graph shows that by the end of a 24-week trial, people who took VIMIZIM™ (elosulfase alfa) once a week walked an average of 23.9% farther than at baseline.

The trial also included a group of people who took VIMIZIM 2 mg/kg every other week. Their results did not show much improvement and were, in fact, similar to the people who took placebo.

## Why use the 6MWT?

A 6MWT measures how far you can walk in 6 minutes. More importantly, it measures your endurance—or how far you can push yourself physically. Endurance measures how well your whole body performs—from heart and lungs to bones and muscles. When your endurance improves, your body's systems are working better together.

<sup>a</sup> A baseline is a measurement of a person's performance before they begin treatment in a clinical trial. In order to participate in the clinical trial for VIMIZIM, people had to be able to walk at least 30 meters.

<sup>b</sup> A placebo is a harmless substance that does not contain any medicine.





## VIMIZIM™ (elosulfase alfa) CAN HELP TO CHANGE HOW YOU LIVE WITH MORQUIO A.

### Committing to VIMIZIM is a commitment to your health.

In clinical studies, VIMIZIM™ (elosulfase alfa) helped most people with their endurance as measured by the 6MWT and helped them go even further—no matter how serious their symptoms of Morquio A were.

Morquio A affects every person in a different way, so the results you'll see from VIMIZIM will be specific to you. You might not notice a difference right away, but remember that VIMIZIM is working on a cellular level to replace the deficient GALNS enzyme.

### Safety considerations

In clinical trials, the most common side effects occurring during VIMIZIM infusions included fever, vomiting, headache, nausea, abdominal pain, chills, and fatigue.

Please see Important Safety Information, including important warning, on page 14.

VIMIZIM™  
(elosulfase alfa)

**// IT REALLY SEEMS TO BE WORKING BECAUSE  
HE CAN STAY IN THE GAME LONGER. //**

- Solange, mother of Pablo (age 15)



# WHAT ARE THE POSSIBLE SIDE EFFECTS OF VIMIZIM™ (elosulfase alfa)?

## The most common side effects seen with VIMIZIM that occurred more often than with a placebo

Side effect	VIMIZIM 2 mg/kg/wk N=58 n (%)	Placebo N=59 n (%)
Fever	19 (33%)	8 (14%)
Vomiting	18 (31%)	4 (7%)
Headache	15 (26%)	9 (15%)
Nausea	14 (24%)	4 (7%)
Abdominal pain	12 (21%)	1 (1.7%)
Chills	6 (10.3%)	1 (1.7%)
Fatigue	6 (10.3%)	2 (3.4%)

Serious and severe reactions are also associated with VIMIZIM™ (elosulfase alfa). These include hypersensitivity reactions as well as life-threatening allergic reactions (anaphylaxis) with symptoms such as hives, swelling, cough, shortness of breath, and flushing. In 6 clinical trials, 18.7% of patients treated with VIMIZIM experienced hypersensitivity reactions, and 7.7% experienced signs and symptoms consistent with anaphylaxis.

As a precautionary measure you should receive medication such as antihistamines before VIMIZIM infusions to reduce the risk of reactions. If a reaction occurs, the infusion should be slowed or stopped and you may be given additional medication. If a severe reaction occurs, the infusion should be stopped immediately and you will receive appropriate medical treatment.

Anaphylaxis can happen as a result of sensitivity to protein-based molecules (like enzymes) when delivered through an IV. Your infusion nurse will check on you while you receive treatment to make sure everything is going well. If you have a reaction, the nurse will stop the infusion immediately and give you medicine to treat the reaction.

Anaphylaxis can occur during any VIMIZIM infusion and up to 3 hours after any infusion.

It's important to be aware of how you are feeling after the infusion and once you are home. If you notice any signs that you are experiencing anaphylaxis, call your doctor immediately.

If you have a respiratory illness, it may become worse due to a hypersensitivity reaction, and you may require additional monitoring by your nurse.

These are not all of the possible side effects with VIMIZIM. Talk to your doctor if you have any symptoms that bother you or that do not go away.

Safety and effectiveness in pediatric patients below 5 years of age has not been established and is currently being evaluated.

**Life-threatening allergic reactions, known as anaphylaxis, can occur during VIMIZIM™ (elosulfase alfa) infusions. Typical signs of anaphylaxis include cough, rash, throat tightness, hives, flushing, changes in skin color, low blood pressure, shortness of breath, chest pain, and gastrointestinal symptoms such as nausea, abdominal pain, retching, and vomiting. Contact your doctor or get medical help right away if these symptoms occur during or after VIMIZIM infusions. If you have a respiratory illness, you may be at risk for a sudden worsening of your condition, and you may require additional monitoring.**



## INFUSION TIME CAN BECOME YOU TIME.

VIMIZIM™ (elosulfase alfa) is an IV medicine that is delivered by infusion directly into a vein in your body. Infusions take place once a week. You should know that infusions take at least 3.5 to 4.5 hours. You should also be prepared to schedule travel time, depending on how close you live to the infusion clinic. Talk to your employers or teachers about your treatments and try to come up with a plan to make up for missed work or school. If you are a student, arrange to get schoolwork ahead of time or ask about getting a tutor to help you catch up on any missed classes or assignments.

To feel comfortable during your infusion, bring

- A game
- A book
- A movie
- A familiar pillow, blanket, or stuffed animal
- Your favorite snack

### **You are not alone.**

If you haven't yet started on VIMIZIM, you may be worried about barriers that could stand in the way. For example, how will you get to the clinic? Don't worry—there's help and real people standing by who care. On the next page, you'll learn an easy way to get direct access to VIMIZIM.

# YOU CAN GET STARTED ON VIMIZIM™ (elosulfase alfa) TODAY.

BioMarin has a service for people with Morquio A. It's called BioMarin Patient and Physician Support (BPPS)—and it is focused on helping you get access to VIMIZIM™ (elosulfase alfa) therapy. This service is available—at no cost—to everyone with Morquio A and provides confidential and personalized support to assist with your healthcare needs. The goal of this service is to help you navigate the healthcare system and get access to VIMIZIM therapy as well as other resources to help you manage Morquio A. Just call **1-855-MORQUIO (1-855-667-7846)** or email **bpps@bmrn.com** to find out how BPPS can help you.

## BPPS can help you get started on VIMIZIM

When you connect with BPPS, you will be introduced to your case manager. Your BPPS case manager will help you coordinate all the details of your treatment, including confirming that your insurance will pay for VIMIZIM. Your case manager will also put you in touch with other people at BioMarin who can help you work with your healthcare team, and who can provide VIMIZIM or Morquio A education to you and your family. Spanish-speaking case managers are also available.

## Ready to get started?

Two things need to happen for you to benefit from BPPS:

- 1** Sign and return the Patient and Provider Authorization forms. You can get these forms by reaching out to either your geneticist or to BPPS, or by ordering the VIMIZIM Introductory Kit, available on **www.vimizim.com**.
- 2** If you have not done so already, work with your doctor to confirm your diagnosis of Morquio A with an enzyme test that measures levels of *N*-acetylgalactosamine-6 sulfatase (GALNS) in your blood or skin cells.

## It's easy to contact BPPS.

You are always welcome to contact BPPS whenever you have a question or concern. Use any of the contact information below to see how BPPS can help you start treatment with VIMIZIM.



**1-855-MORQUIO (1-855-667-7846)**



**1-888-863-3361**



**bpps@bmrn.com**



**www.vimizim.com**

Spanish-speaking case managers are also available.

*También se dispone de coordinadores de casos de habla hispana.*

# IMPORTANT SAFETY INFORMATION

## INDICATION

VIMIZIM™ (elosulfase alfa) is indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

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VIMIZIM is a prescription medicine. Before treatment with VIMIZIM, it is important to discuss your medical history with your doctor. Tell your doctor if you are sick or taking any medication and if you are allergic to any medicines. Also tell your doctor if you are pregnant, planning to become pregnant, or are a nursing mother. Your doctor will decide if VIMIZIM is right for you. If you have questions or would like more information about VIMIZIM, contact your doctor.

Anaphylaxis can occur during any VIMIZIM infusion and up to three hours after any infusion, and hypersensitivity reactions have been observed as early as 30 minutes from the start of infusion but as late as six days after infusion.

Serious and severe reactions can happen with VIMIZIM treatment, including life-threatening allergic reactions (anaphylaxis), hives, swelling, cough, shortness of breath, and flushing. You should receive medication such as antihistamines before VIMIZIM infusions to reduce the risk of reactions. If a reaction occurs, the infusion should be slowed or stopped and you may be given additional medication. If a severe reaction occurs, the infusion should be stopped immediately and you will receive appropriate medical treatment.

If you have acute febrile or respiratory illness at the time of VIMIZIM infusion you may be at higher risk of life-threatening complications from hypersensitivity reactions. If you use supplemental oxygen or continuous positive airway pressure (CPAP) you should have it available during your infusion in the event of a sudden reaction, or extreme drowsiness/sleep from antihistamines.

Spinal cord damage may occur due to the natural MPS IVA disease process. Signs of spinal cord injury include back pain, numbness and paralysis, and loss of bladder and bowel control. Contact your doctor immediately if you develop any of these symptoms.

The most common side effects reported during VIMIZIM infusions included fever, vomiting, headache, nausea, abdominal pain, chills, and fatigue. These are not all of the possible side effects with VIMIZIM. Talk to your doctor if you have any symptoms that bother you or that do not go away.

**Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.**

For more information, call BioMarin Patient and Physician Support (BPPS) at 1-855-MORQUIO (1-855-667-7846).

Please see accompanying full Prescribing Information, including important warning, or visit [www.VIMIZIM.com](http://www.VIMIZIM.com).

**// THERE'S A LOT MORE I WANT TO DO WITH MY LIFE. //**

- Melissa (age 19)



## READY TO GET STARTED?

VIMIZIM™ (elosulfase alfa), the only ERT for Morquio A, could help you

- Replace deficient GALNS enzyme
- Improve endurance, as shown by the 6MWT

When you're ready to start taking VIMIZIM, please visit our website at [www.vimizim.com](http://www.vimizim.com) or simply contact BPPS:

 **1-855-MORQUIO (1-855-667-7846)**

 **1-888-863-3361**

 **[bpps@bmrn.com](mailto:bpps@bmrn.com)**

 **[www.vimizim.com](http://www.vimizim.com)**

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