



# Go farther with Naglazyme

Your step-by-step guide to the first and only treatment for MPS VI

  
**Naglazyme**<sup>®</sup>  
(GALSULFASE)



***“What makes it worth it is knowing in the back of my mind that I am slowing the progression of MPS VI. And that going on Naglazyme now, and slowing the progression, will help me.”***

— Tyler, age 20

# Patient information about Naglazyme and MPS VI

This guide gives you an overview of Maroteaux-Lamy syndrome (MPS VI) and how it can be treated with Naglazyme® (galsulfase), the first and only treatment for MPS VI.

Be sure to read this brochure completely before you begin therapy with Naglazyme. This information should not replace discussions with your doctor about your therapy or your medical condition. Talk to your doctor about Naglazyme before you begin treatment and at regular checkups. If you have any questions about Naglazyme, ask your doctor.

Please see page 9 of this booklet for important safety information about Naglazyme.

  
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# About Maroteaux-Lamy syndrome (MPS VI)

MPS VI is a rare but serious genetic disease. Only about 1100 people in the world are thought to have it.

Maroteaux-Lamy syndrome is named after the 2 doctors who first described the disease in 1963. MPS VI is short for mucopolysaccharidosis VI.

MPS VI is an inherited condition. A child who gets one MPS VI gene from each parent will have the disease. MPS VI affects different people differently. Not everyone shows all the symptoms. Some people have signs of MPS VI almost from birth. Others may only notice problems over many years. Either way, MPS VI can cause serious disabilities over time.

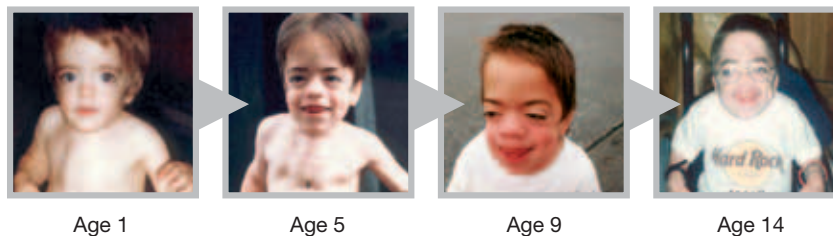
There are varying degrees of MPS VI. Some people have clear symptoms very early that grow worse quickly. Some people may not show signs until later in life and have symptoms that slowly worsen. There is no hard line between rapidly and slowly progressing patients. However, over time people with MPS VI will notice more and more problems, and their condition will progress. That is why it's important to begin therapy as soon as possible.

## People born with MPS VI tend to have a cluster of symptoms that may include:

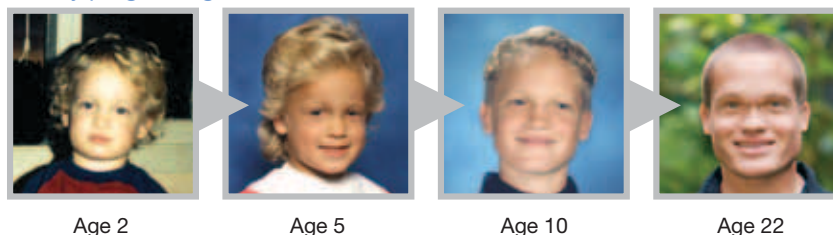
- Enlarged head
- Short stature
- Poor endurance
- Distorted facial features
- Hearing problems
- Ear and sinus infections
- Vision problems, including cloudy corneas and glaucoma
- Enlarged tongue and abnormal teeth
- Problems breathing and sleep apnea
- Lung infections
- Irregular heartbeat
- High blood pressure in the heart
- Enlarged liver or spleen
- Hernias
- Joint stiffness
- Hip problems
- Spinal cord compression
- Carpal tunnel syndrome

## VARIABLE RATE OF MPS VI PROGRESSION

### Rapidly progressing



### Slowly progressing

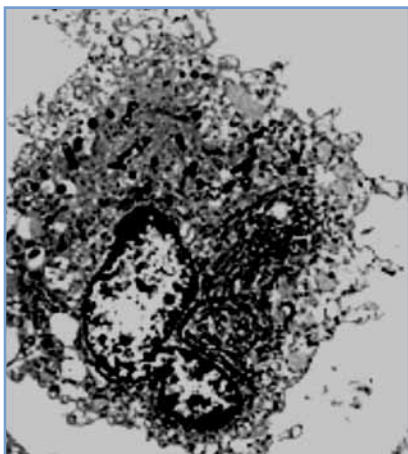


## What happens in MPS VI?

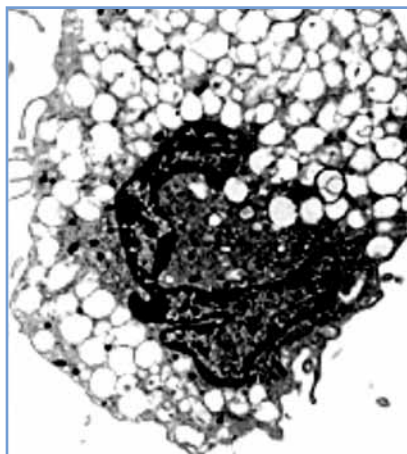
MPS VI is a kind of disease called a lysosomal storage disorder (LSD). There are more than 40 known LSDs. People with these disorders lack specific enzymes that keep waste from building up inside the cells of body tissues and organs. This waste is a gel-like substance called GAG, which is short for glycosaminoglycan (gly-KOH-sah-meen-o-GLY-can).

Packed with too much GAG, the cells don't work right. Over time, major organs and systems in the body can be harmed and fail to function properly.

**NORMAL CELL**



**MPS CELL**



On the left you can see lysosomes (LIE-so-sohms) within a normal cell. These are the clear areas that look like bubbles in the picture. In MPS VI, the lysosomes are missing a specific enzyme needed to break down GAG—a kind of waste material—so that it can leave the cell. As GAG builds up, lysosomes store more and more GAG inside the cell.

In the image on the right you can see the result. As the cell becomes swollen, it can't work as well. This causes problems throughout the body.



# About enzyme replacement therapy with Naglazyme

## Who should receive Naglazyme therapy?

All MPS VI patients are candidates for treatment with Naglazyme® (galsulfase). Naglazyme provides the enzyme the body needs to break down and get rid of the GAG that causes symptoms of MPS VI. In all patients with MPS VI, GAG continues to build up over time. If untreated, the symptoms of MPS VI continue to get worse. So it is important to start treatment with Naglazyme as soon as it is prescribed and to stay with treatment over time.



## How do you take Naglazyme?

Naglazyme is a protein, so it must be given directly into the bloodstream. That means that once a week, you will go to a hospital or infusion center. At the center, a doctor or nurse will give you Naglazyme through an intravenous (IV) line. Your blood then carries the drug to your body's cells and organs so that it can break down GAG.



The infusion takes about 4 hours. You may want to use this time to read, study, play video games, watch DVDs, or talk with friends. Some patients may feel sleepy or tired during and after treatment with Naglazyme, due to other medications taken before or during the infusions. If you start to feel unwell during an infusion, it is important to let the medical staff know.

Every infusion helps keep more GAG from building up. So it's important to keep every infusion appointment.

Occasionally, patients miss an infusion because of illness. If you have a fever or cold, be sure to let your doctor know. You may need to reschedule your next infusion appointment.

If you need help getting to your appointments, talk with your BioMarin BPPS patient advocate. See page 12 for more information about BPPS.



## How does Naglazyme work?

Naglazyme is a kind of treatment called enzyme replacement therapy (ERT). In ERT, the enzyme that is missing from a patient's body is replaced with a synthetic enzyme. Naglazyme, a highly purified protein, is identical to the natural enzyme that is missing in MPS VI. Naglazyme reduces the GAG buildup that leads to MPS VI symptoms, and can help minimize the effects of the disease.

In clinical studies, Naglazyme has been shown to improve endurance. People who took Naglazyme were able to do better on stair-climbing and walking tests.

Naglazyme also helps lower GAG, which is measured in the urine. Once you are on therapy, your doctor can measure your GAG levels during checkups to track how well Naglazyme is working.

## What changes can you expect from taking Naglazyme?

When you start Naglazyme therapy, you may not feel different right away. However, over time Naglazyme has been shown to improve endurance. This means you may be able to walk farther or climb more stairs. You may feel stronger and not tire as easily.

Even though you may feel better, it is important to continue your therapy with Naglazyme regularly. In clinical studies, Naglazyme reduced the levels of GAG in urine. This is evidence that Naglazyme was working.

## How long will you take Naglazyme?

MPS VI is a lifelong disease, and Naglazyme therapy is a lifelong commitment. Replacing the missing enzyme with regular infusions is necessary for Naglazyme therapy to have optimal effects.



***“My son Nathan was diagnosed with MPS VI when he was 5 years old. Nathan is now 11 years old and has been on Naglazyme for about a year and a half now. Without Naglazyme, I think Nathan would be in a different situation than he is now. It makes me happy to know that he’s happy.”***

—Margo, Nathan’s mother



## What does your doctor need to know before you take Naglazyme?

Before you begin treatment, your doctor will need to examine you to make sure Naglazyme® (galsulfase) is right for you. Your doctor may also want to know if:

- You are pregnant or plan to become pregnant
- You are breastfeeding
- You have current or past allergies or medical problems—such as breathing problems—even if they are not related to MPS VI
- You take any other prescription or over-the-counter medications or dietary supplements

**Note:** Do not stop taking any medications without first discussing it with your doctor.



## Using other medications with Naglazyme

When people take 2 or more medications at the same time, it is possible that they will interact. Always discuss your medications with your doctor. Before you begin therapy with Naglazyme, be sure your doctor is aware of any medicines you are currently taking, as well as any medicines you have recently stopped taking.



***“Today, people affected with Maroteaux-Lamy syndrome have a treatment, one that did not exist 10 years ago. Of course, I recommend starting enzyme replacement therapy as soon as possible.”***

—Chester Whitley, MD, PhD  
Department of Pediatrics and Institute of Human Genetics,  
University of Minnesota

## What are the possible side effects of Naglazyme?

**Important Safety Information:** The most common adverse events in patients treated with NAGLAZYME were headache, fever, joint pain, vomiting, upper respiratory infections, abdominal pain, diarrhea, ear pain, cough, and ear infections. Severe reactions included swollen blood vessels, low blood pressure, difficulty breathing, respiratory distress, stopping breathing, and hives. The most common symptoms of infusion reactions included fever, chills/shakes, headache, rash, and mild to moderate hives. Nausea, vomiting, elevated blood pressure, chest pain, abdominal pain, malaise, and joint pain were also reported.

No patients discontinued NAGLAZYME treatment because of reactions. Nearly all patients developed antibodies as a result of the treatment, but the level of immune response did not correlate with the severity of the adverse reaction. Because antihistamine use may increase the risk of stopping breathing, airways should be checked to ensure they are not blocked or obstructed. Treatment may be delayed if you have a fever or respiratory illness.

Please see patient product information. If you have any questions about this information, please talk with your doctor.

Please see full prescribing information.

**NAGLAZYME is indicated for patients with mucopolysaccharidosis VI (MPS VI).**

**NAGLAZYME has been shown to improve walking and stair-climbing capacity.**





## Getting started with Naglazyme

Once you have a prescription, your physician and the BioMarin Patient and Physician Support program (BPPS) can help you find an infusion center and help make sure that your Naglazyme® (galsulfase) arrives at the center for you. If you don't have a prescription for Naglazyme but think that it may be right for you, talk to your physician. If you need help finding a clinic, call BPPS. See page 12 for more information about BPPS.

You'll go to the infusion center for your appointment. Before treatment, the nurse will make sure you don't have a fever or infection. You may get an antihistamine or pain reliever to help you during the infusion.

The nurse will start an IV, prepare the Naglazyme, and start the infusion. The infusion will take about 4 hours. The nurse will check with you regularly to see how you are feeling. The rate of infusion may be adjusted if needed. If you don't feel well or if you have questions, talk to your nurse. The nurse can answer your questions, and may be able to help you feel more comfortable.



After the infusion, you'll wait a short time to make sure you feel well enough to leave. Then you'll go home until next week.

### **Home infusion**

Sometimes your doctor and health plan will agree that you can get your infusions at home. If so, the Naglazyme will be sent to your home and a nurse will come to you. The nurse will prepare the infusion, give you the infusion, and make sure everything goes well. BPPS will continue to help make sure the home appointments and your reimbursements go smoothly.



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## Help is just a call away!

### **Programs and services to help with your Naglazyme therapy**

BioMarin, the company that makes Naglazyme® (galsulfase), has a program to help all MPS VI patients get the treatment they need. The BioMarin Patient and Physician Support program (BPPS) can help you with questions about your treatment.

### **Your BPPS patient advocate is ON CALL for you!**

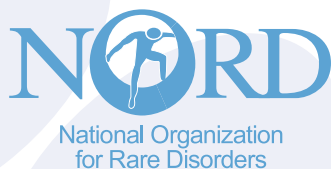
BPPS is a free support service that comes with your Naglazyme treatment. BPPS can help you with problems or questions about your Naglazyme treatment or insurance coverage.

When you get an MPS VI diagnosis and a prescription for Naglazyme, call or e-mail BPPS. When you call, you'll get your own helpful BPPS advocate—a person you will talk with every time you call.

Your advocate works with your insurance plan to submit any required paperwork, investigates your health benefits, researches what your co-pay/deductible amounts will be, and identifies any additional insurance plan requirements for coverage. He or she also keeps you, your doctor, and the infusion center updated as your treatment plans move forward. Please be sure to call your patient advocate back when a message is left for you!

It is important to start Naglazyme as soon as you can and to keep getting your treatments. Call BPPS as soon as you have a prescription for Naglazyme. If you have any concerns or problems, always feel free to call your BPPS advocate.

BPPS is your gateway to other services as well, including the National Organization for Rare Disorders, or NORD. The NORD Naglazyme Assistance Program is dedicated to helping individuals with private insurance coverage who are prescribed Naglazyme for MPS VI, but who cannot afford the out-of-pocket premiums or co-payment.





To learn more about BioMarin's support programs and services for Naglazyme therapy, please contact BPPS at **866-906-6100** or e-mail **bpps@bmrn.com**.

To learn more about Naglazyme enzyme replacement therapy, visit **www.naglazyme.com**.

To learn more about MPS VI, visit **www.MPSVI.com** or **www.MPSSociety.org**.



## The MPS VI Clinical Surveillance Program

BioMarin, the manufacturer of Naglazyme® (galsulfase), is committed to MPS VI treatment. It has established the MPS VI Clinical Surveillance Program (CSP) to learn more about the natural history of MPS VI and the effect of Naglazyme in the treatment of this disease.

The MPS VI CSP, established in 2005, is a multicenter, multinational, observational program for patients with MPS VI. This program is open to individuals with MPS VI who have been treated with Naglazyme as well as those who do not receive Naglazyme treatment. Bone marrow transplant recipients may also enroll in the CSP.

This voluntary program uses information collected by your healthcare provider. To participate, you must agree to share your lab and other MPS VI-related test results. This allows researchers to learn more about your condition or the effects of the medication you are taking to treat MPS VI. No additional tests or changes to your usual treatment are needed for you to be part of the MPS VI CSP.

**To learn more about the BioMarin MPS VI Clinical Surveillance Program, please contact your healthcare provider or call [866-906-6100](tel:866-906-6100).**



To learn more about BioMarin's support programs and services for Naglazyme therapy, please phone BPPS at **866-906-6100** or e-mail **bpps@bmrn.com**.

To learn more about Naglazyme enzyme replacement therapy, visit online at **www.naglazyme.com**.

To learn more about MPS VI, visit **www.MPSVI.com** or **www.MPSSociety.org**.



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