

# What is MPS VI?

MPS VI (Mucopolysaccharidosis VI, also known as Maroteaux-Lamy Syndrome), is a rare inherited lysosomal storage disorder (LSD) estimated to occur once in every 340,000 live births.

As a result of a deficiency of the enzyme N-acetylgalactosamine 4-sulfatase (arylsulfatase B), individuals with MPS VI are unable to break down substances known as glycosaminoglycans (GAGs), which are found in connective tissue throughout the body. The GAGs accumulate in the lysosomes of cells, leading to multisystemic abnormalities and numerous clinical manifestations including respiratory and cardiovascular complications, gastrointestinal symptoms, joint stiffness and restricted movement, and loss of vision and hearing. MPS VI is a progressive disease and leads to severe disability and a shortened life span in virtually all cases.

## Diagnosis Often Challenging

MPS VI varies considerably among individuals in terms of the severity of symptoms, the organ systems affected, and the rate of disease progression. This variability poses a challenge to physicians who, due to the rareness of the disease, may not be familiar with it. To diagnose MPS VI, physicians must first suspect that an unusual cluster of symptoms may be related to an MPS disorder and then refer their patient to a medical geneticist for evaluation. The geneticist will then rely upon a specialized enzyme assay to make a conclusive diagnosis. While individuals with rapidly advancing MPS VI are usually diagnosed by one to five years of age, those with more slowly progressing disease may initially present more subtle symptoms and thus be diagnosed at a later age.

## Naglazyme® the First and Only Treatment for MPS VI



Until recently, treatment of MPS VI was limited primarily to symptom-based care aimed at treating complications as they occurred. Today, individuals with MPS VI have a therapeutic option developed specifically to treat the underlying cause of their disease. Naglazyme (galsulfase), an enzyme replacement therapy provides a recombinant version of arylsulfatase B to individuals diagnosed with MPS VI. It is administered weekly by intravenous infusion, allowing the medication to be delivered directly to the body's cells and organs to break down the GAG build up. As the first drug approved for

the treatment of MPS VI, the U.S. Food and Drug Administration and the European Commission have granted Naglazyme orphan drug status, a designation given to therapies that are the first to be approved for the treatment of diseases that affect fewer than 200,000 individuals in the United States and fewer than five out of 10,000 people in the European Union. Naglazyme is developed, manufactured and commercialized by BioMarin. Naglazyme was approved in the United States and Europe in May 2005 and January 2006, respectively, and granted orphan drug status in both regions.

*Please see reverse for full indication and important safety information.*

## Resources

### BPPS (BioMarin Patient and Physician Support program)

BioMarin is committed to assisting MPS VI patients with obtaining access to Naglazyme through the BioMarin Patient and Physician Support program.

[www.mpsvi.com](http://www.mpsvi.com)

[www.naglazyme.com](http://www.naglazyme.com)

[www.bmrn.com](http://www.bmrn.com)

## Full Indication and Important Safety Information

**Naglazyme® (galsulfase)** is indicated for patients with MPS VI. Naglazyme has been shown to improve walking and stair-climbing capacity.

The most common adverse events observed in clinical trials in Naglazyme-treated patients were headache, fever, arthralgia, vomiting, upper respiratory infections, abdominal pain, diarrhea, ear pain, cough, and otitis media. Severe reactions included angioneurotic edema, hypotension, dyspnea, bronchospasm, respiratory distress, apnea, and urticaria. The most common symptoms of infusion reactions included fever, chills/rigors, headache, rash, and mild to moderate urticaria. Nausea, vomiting, elevated blood pressure, retrosternal pain, abdominal pain, malaise, and joint pain were also reported. No patients discontinued Naglazyme infusions for adverse events and all patients that completed the double-blind portion of the trial continued to receive weekly infusions of Naglazyme. Nearly all patients developed antibodies as a result of treatment, but the level of the immune response did not correlate with the severity of adverse events or impact the improvements experienced in endurance. Because antihistamine use may increase the risk of apneic episodes, evaluation of airway patency should be considered prior to the initiation of treatment. Consideration to delay Naglazyme infusion should be given when treating patients who present with an acute febrile or respiratory illness.