BioMarin RareConnections™ Patient Enrollment for Maroteaux-Lamy (MPS VI)

Fax completed form with prescriber's signature to **1-888-863-3361**

Phone: 1-866-906-6100 Hours: M–F 6 AM–5 PM (PST)

PATIENT

First name _____ Last name _____ Birth date _____ Gender ☐ Male ☐ Female

Parent/Caregiver name (if applicable) _____

Home address _____ Suite/Apt _____

City _____ State _____ ZIP _____

Shipping address (for product—no PO box) _____ Suite/Apt _____

City _____ State _____ ZIP _____

Home phone _____ Work phone _____ Cell phone _____

Preferred method of contact ☐ Home ☐ Work ☐ Cell ☐ Email _____

Preferred language ☐ English ☐ Other _____

I authorize BioMarin RareConnections™ to leave a message if I am not available ☐ Yes ☐ No

PRESCRIBER

First name _____ Last name _____ Specialty _____

DEA # _____ NPI # _____ License # _____

Medicaid # _____ Tax ID _____ Site Tax ID _____

Name of institution/Practice name _____

Street address _____ Suite _____

City _____ State _____ ZIP _____

Office contact _____ Phone _____ Fax _____

INSURANCE

Provide copies of all medical and prescription cards—front and back, primary and supplemental coverage.

Primary insurance name _____

Insurance phone # _____

Policy holder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) # _____

Secondary insurance name _____

Insurance phone # _____

Policy holder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) # _____

☐ **Patient does not have insurance**

DIAGNOSIS

If diagnosis is confirmed, please fill out the information below:

Diagnosis code ICD-10 (Other Mucopolysaccharidoses, E76.29), Other _____ Date of diagnosis _____

Lab performing diagnosis _____

Method of diagnostic (select one or more) ☐ Biochemical/Enzymatic testing ☐ Molecular testing

If diagnostic testing is needed, please note below which tests are required:

Biochemical testing (select one or more below)

☐ Mucopolysaccharidosis (MPS) enzyme panel (MPS I, II, III A-D, IV A & B, VI, and VII)

☐ Maroteaux-Lamy (*N*-acetyl-galactosamine-4-sulfatase enzyme analysis) ☐ Urine monitoring MPS VI (total GAGs, DS)

☐ MPS urinalysis (total GAGs, DS)

Molecular testing

☐ Maroteaux-Lamy, MPS VI (ASB) sequencing

Patient name _____ Birth date _____

Optional tests or clinical measurements used only as additional information; not required.

CLINICAL INFORMATION

Category	Test	Pretreatment (completed) √	Pretreatment (scheduled) √	Date	Results
Endurance	6MWT				
Respiratory	FVC				
	FEV_1				
Other (please specify)					

Frequency and type of ongoing assessments _____

PRESCRIPTION

For Use by In-Network Specialty Pharmacy Only—Not for Home Infusion

Product name: NAGLAZYME® (galsulfase), concentrate for infusion, NDC #: 68135-020-01

Patient allergies? ☐ NKDA ☐ Other _____

Patient weight (kg) _____ Date weight was measured _____ Dose _____ mg per week

Dispense

Number of days' supply/Rx: ☐ 30 days ☐ 90 days Refills: One (1) year

Direction for use Infuse _____ mg every week in _____ mL normal saline over _____ hours

_____ _____ _____ _____

Signature/Substitution permitted Date **Signature/Dispense as written** Date

No stamps or initials: If you are a New York prescriber, please use an original New York State prescription form.

SITE OF SERVICE

Preferred site of infusion

☐ Prescribing physician's site of care office (if this is selected, please proceed to next section)

☐ Non-prescribing physician's site of care ☐ Hospital outpatient ☐ Other _____

Name of institution/Practice name _____ Physician or infusion provider name _____

Provider's specialty _____

Street address _____ City _____ State _____ ZIP _____

Office contact _____ Email _____

Office phone (and extension) _____ Office fax _____ Site Tax ID _____

PRESCRIBER DECLARATION

Prescriber declaration

I verify that the patient and prescriber information contained in this enrollment form is complete and accurate to the best of my knowledge and that I have prescribed NAGLAZYME® (galsulfase) based on my professional judgment of medical necessity. I authorize BioMarin or its affiliated companies or subcontractors to forward this prescription electronically, by facsimile, or by mail to a dispensing pharmacy chosen by the above-mentioned patient. I also authorize BioMarin to perform any steps necessary to obtain reimbursement for NAGLAZYME, including but not limited to insurance verification and case management. I understand that BioMarin may need additional information, and I agree to provide it as needed for purposes of reimbursement.

Prescriber signature _____ Date _____

BIOMARIN®

Naglazyme®
(GALSULFASE)

INDICATION

NAGLAZYME® (galsulfase) is indicated for patients with Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). NAGLAZYME has been shown to improve walking and stair-climbing capacity.

IMPORTANT SAFETY INFORMATION

Life-threatening anaphylactic reactions and severe allergic reactions have been observed in some patients during NAGLAZYME (galsulfase) infusions and up to 24 hours after infusion. If these reactions occur, immediate discontinuation of NAGLAZYME is recommended and appropriate medical treatment should be initiated, which may include resuscitation, epinephrine, administering additional antihistamines, antipyretics or corticosteroids. In patients who have experienced anaphylaxis or other severe allergic reactions during infusion with NAGLAZYME, caution should be exercised upon rechallenge; appropriately trained personnel and equipment for emergency resuscitation (including epinephrine) should be available during infusions.

As with other enzyme replacement therapies, immune-mediated reactions, including membranous glomerulonephritis have been observed. In clinical trials, nearly all patients developed antibodies as a result of treatment with NAGLAZYME; however, the analysis revealed no consistent predictive relationship between total antibody titer, neutralizing or IgE antibodies, and infusion-associated reactions, urinary glycosaminoglycan (GAG) levels, or endurance measures.

Caution should be exercised when administering NAGLAZYME to patients susceptible to fluid volume overload because congestive heart failure may result. Consider a decreased total infusion volume and infusion rate when administering NAGLAZYME to these patients.

Consideration to delay NAGLAZYME infusion should be given when treating patients who present with an acute febrile or respiratory illness. Sleep apnea is common in MPS VI patients and antihistamine pretreatment may increase the risk of apneic episodes. Evaluation of airway patency should be considered prior to the initiation of treatment. Patients using supplemental oxygen or continuous positive airway pressure (CPAP) during sleep should have these treatments readily available during infusion in the event of an infusion reaction, or extreme drowsiness/sleep induced by antihistamine use.

Pretreatment with antihistamines with or without antipyretics is recommended prior to the start of infusion to reduce the risk of infusion reactions. If infusion reactions occur, decreasing the infusion rate, temporarily stopping the infusion, or administering additional antihistamines and/or antipyretics is recommended.

During infusion, serious adverse reactions included laryngeal edema, apnea, pyrexia, urticaria, respiratory distress, angioedema, and anaphylactoid reaction; severe adverse reactions included urticaria, chest pain, rash, abdominal pain, dyspnea, apnea, laryngeal edema, and conjunctivitis. The most common adverse events (≥10%) observed in clinical trials in patients treated with NAGLAZYME were rash, pain, urticaria, pyrexia, pruritus, chills, headache, nausea, vomiting, abdominal pain and dyspnea. The most common adverse reactions requiring interventions are infusion-related reactions.

Spinal/cervical cord compression is a known and serious complication that is expected to occur during the natural course of MPS VI. Signs and symptoms of spinal/cervical cord compression include back pain, paralysis of limbs below the level of compression, and urinary or fecal incontinence. Patients should be evaluated for spinal/cervical cord compression prior to initiation of NAGLAZYME to establish a baseline and risk profile. Patients treated with NAGLAZYME should be regularly monitored for the development or progression of spinal/cervical cord compression and be given appropriate clinical care.

To report SUSPECTED ADVERSE REACTIONS contact BioMarin Pharmaceutical Inc. at 1-866-906-6100, or FDA at 1-800-FDA-1088 or go to www.fda.gov/medwatch.

Please see accompanying full Prescribing Information, or visit www.NAGLAZYME.com.

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