BioMarin RareConnections™ Patient Enrollment for Morquio A (MPS IVA)

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 number _____

Policy holder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) number _____

Secondary insurance name _____

Insurance phone number _____

Policy holder name _____

Relationship to patient _____

Group ID _____

Employer _____

Member ID (policy) number _____

☐ **Patient does not have insurance**

DIAGNOSIS

If diagnosis is confirmed please fill out the information below:

Diagnosis code ICD-10 (Morquio A, Mucopolysaccharidosis, E76.210), 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)

☐ Morquio syndrome enzyme panel, MPS IV, types A & B

☐ Morquio, type A (*N*-acetyl-galactosamine-6-sulfatase enzyme analysis) ☐ Urine monitoring MPS IV (total GAGs, KS, CS)

☐ MPS urine analysis (quantitative HS/DS/CS/KS & total GAGs)

Molecular testing

☐ Morquio syndrome A, MPS IVA (*GALNS*) sequencing

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Patient name _____ Birth date _____

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

CLINICAL INFORMATION

Category	Test	Pre-Treatment (completed) √	Pre-Treatment (scheduled) √	Date	Results
Endurance	6MWT	☐	☐		
	T25FW	☐	☐		
Respiratory	FVC	☐	☐		
	FEV_1	☐	☐		
	MVV	☐	☐		
Other (please specify)		☐	☐		

Frequency and type of ongoing assessments _____

PRESCRIPTION

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

Product name: VIMIZIM® (elosulfase alfa), concentrate for infusion, NDC Number: 68135-100-01

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

☐ Non-Prescribing MD'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 VIMIZIM® (elosulfase alfa) 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 VIMIZIM, 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®

VIMIZIM®
(elosulfase alfa)

INDICATION

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

IMPORTANT SAFETY INFORMATION

Life-threatening anaphylactic reactions have occurred in some patients during VIMIZIM® (elosulfase alfa) infusions. Anaphylaxis, presenting as cough, erythema, throat tightness, urticaria, flushing, cyanosis, hypotension, rash, dyspnea, chest discomfort, and gastrointestinal symptoms (e.g., nausea, abdominal pain, retching, and vomiting) in conjunction with urticaria, have been reported to occur during VIMIZIM infusions, regardless of duration of the course of treatment. Closely observe patients during and after VIMIZIM administration and be prepared to manage anaphylaxis. Inform patients of the signs and symptoms of anaphylaxis and have them seek immediate medical care should symptoms occur. Patients with acute respiratory illness may be at risk of serious acute exacerbation of their respiratory compromise due to hypersensitivity reactions, and require additional monitoring.

Due to the potential for anaphylaxis, appropriate medical support should be readily available when VIMIZIM is administered and for an appropriate period of time following administration. In clinical trials, cases of anaphylaxis occurred as early as 30 minutes from the start of infusion and up to three hours after infusion, and as late into treatment as the 47th infusion.

In clinical trials, hypersensitivity reactions have been observed as early as 30 minutes from the start of infusion but as late as six days after infusion. Frequent symptoms of hypersensitivity reactions (occurring in more than 2 patients) included anaphylactic reactions, urticaria, peripheral edema, cough, dyspnea, and flushing.

Because of the potential for hypersensitivity reactions, administer antihistamines with or without antipyretics prior to infusion. Management of hypersensitivity reactions should be based on the severity of the reaction and include slowing or temporary interruption of the infusion and/or administration of additional antihistamines, antipyretics, and/or corticosteroids for mild reactions. However, if severe hypersensitivity reactions occur, immediately stop the infusion of VIMIZIM and initiate appropriate treatment.

Consider the risks and benefits of re-administering VIMIZIM following a severe reaction.

Patients with acute febrile or respiratory illness at the time of VIMIZIM infusion may be at higher risk of life-threatening complications from hypersensitivity reactions. Careful consideration should be given to the patient's clinical status prior to administration of VIMIZIM and consider delaying the VIMIZIM infusion.

Sleep apnea is common in MPS IVA patients. Evaluation of airway patency should be considered prior to initiation of treatment with VIMIZIM. 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 acute reaction, or extreme drowsiness/sleep induced by antihistamine use.

Spinal or cervical cord compression (SCC) is a known and serious complication of MPS IVA and may occur as part of the natural history of the disease. In clinical trials, SCC was observed both in patients receiving VIMIZIM and patients receiving placebo. Patients with MPS IVA should be monitored for signs and symptoms of SCC (including back pain, paralysis of limbs below the level of compression, urinary and fecal incontinence) and given appropriate clinical care.

All patients treated with VIMIZIM 2 mg/kg once per week in the placebo-controlled trial developed anti-drug antibodies. The relationship between the presence of neutralizing antibodies and long-term therapeutic response or occurrence of anaphylaxis or other hypersensitivity reactions could not be determined.

VIMIZIM should be used during pregnancy only if the potential benefit justifies the potential risk to the fetus. It is not known if VIMIZIM is present in human milk. Exercise caution when administering VIMIZIM to a nursing mother. There is a Morquio A Registry that collects data on pregnant women and nursing mothers with MPS IVA who are treated with VIMIZIM. Contact MARS@BMRN.com for information and enrollment.

Safety and effectiveness in pediatric patients below 5 years of age have not been established and are currently being evaluated. In clinical trials, the most common adverse reactions (≥10%) occurring during infusion included pyrexia, vomiting, headache, nausea, abdominal pain, chills, and fatigue. The acute reactions requiring intervention were managed by either temporarily interrupting or discontinuing infusion, and administering additional antihistamine, antipyretics, or corticosteroids.

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, including boxed warning, or visit www.VIMIZIM.com.

BIOMARIN®

US/VIM/0516/0055(1)