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Effect: none

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Effect: none

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Font-Style: normal
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Opacity: 1
Effect: none

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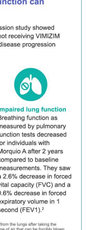
1 **From:** BioMarin <MPSInfusionInsights@bmrn.com>
To: <Recipient's email>
Subject: Long-term benefits for patients with Morquio A
[ALT] MPS Infusion Insights: new edition!
[ALT] Learn about the natural progression of Morquio A

2 Having difficulty viewing this email? You can view it in your browser.

MPS INFUSION INSIGHTS 3 Supporting nurses through the infusion process

4 An eNewsletter for nurses by nurses

5 A team of clinical nurse educators developed this quarterly eNewsletter as a way for nurses from around the country to share insights and best practices.



6 Have an insight to share? Email us >

7 For patients with Morquio A, endurance and lung function can decrease over time

An international disease progression study showed that individuals with Morquio A not receiving VIMIZIM (elosulfase alfa) treatment had disease progression over a 2-year period.[1]

8 **Reduced endurance**
Individuals with Morquio A walked 21.9 meters less after 2 years than they did at the start of the study, as measured by the 6-minute walk test (6MWT), which measures how far someone can walk in 6 minutes.[1]

Impaired lung function
Breathing function as measured by pulmonary function tests decreased for individuals with Morquio A after 2 years compared to baseline measurements. They saw a 2.6% decrease in forced vital capacity (FVC) and a 0.6% decrease in forced expiratory volume in 1 second (FEV1).[1]

9 FVC=volume of air that can forcibly be exhaled from the lungs after taking the deepest breath possible; FEV1=maximum volume of air that can be forcibly blown out in 1 second.

10 **REMEMBER:** MORQUIO A IS HETEROGENEOUS, SO SYMPTOMS AND PROGRESSION MAY BE DIFFERENT FOR EVERYONE.[3,4]

11 VIMIZIM® (elosulfase alfa) can provide long-lasting benefits

In a 6-month clinical trial, people who received VIMIZIM infusions every week improved their endurance as measured by the 6MWT. In a follow-up 2-year extension study, results showed that the benefits of VIMIZIM can be maintained over the long term and are consistent with the results of the primary study.[1,2,5]

58 px

12 **Improved endurance**
Results from the long-term extension study showed a 32.9 meter increase in the 6MWT.[1]

Helped lungs work better
Breathing function as measured by pulmonary function tests improved for patients in the long-term extension compared to baseline. There was a 9.2% increase in FVC and an 8.8% increase in FEV1 compared to baseline.[2]

13 Serious and severe reactions were associated with VIMIZIM, including hypersensitivity reactions as well as life-threatening allergic reactions (anaphylaxis). The most common adverse reactions were infusion-associated and were managed with symptomatic treatment and/or modification of infusion rate.[5]

Please see Important Safety Information, including important warning, near the bottom of this email.

14 VIMIZIM IS THE ONLY ENZYME REPLACEMENT THERAPY THAT TREATS THE ROOT CAUSE OF MORQUIO A.[3]

15 The National MPS Society is a great resource for you and your patients

The National MPS Society exists to find cures for MPS and related diseases. They provide hope and support for patients and their families through research, advocacy, and awareness of these devastating disorders.

Family support is one of the centerpieces of the mission of The National MPS Society. Over the years, they have expanded their efforts to better support families affected by MPS by developing programs specific to MPS patient needs. Support programs that may be available to your patients include:

- Continuing Education Scholarship
- Extraordinary Experiences
- Family Assistance
- Journey Assistance
- Medical Travel Assistance
- Regional Social Events

Membership to The National MPS Society is free! Encourage your patients to sign up and check the website often for new information and programs.

National MPS Society Powerful Stories, Powered by a Cure

16 Visit www.mpssociety.org >

17 NURSE SPOTLIGHT

Nancy Carew, RN, and Diane Doukellis, RN, are home infusion nurses that exhibit exceptional care for two patients, who are siblings with Morquio A. During the school year, they go to the patients' school to administer their weekly dose of VIMIZIM. Nancy and Diane have built professional and trusting relationships with the patients, family, and school staff.

Nancy and Diane visiting VIMIZIM in the school nurse's office.

School infusions can help with both compliance and academics, but they are not for all patients. The prescribing doctor should determine what is appropriate for each patient.

Do you know a nurse that goes above and beyond?

Nominate a nurse here to be featured in the next edition of MPS Infusion Insights.

18 From those of us at BioMarin, thank you for all that you do to help patients with MPS. Be on the lookout for future eNewsletters from MPS Infusion Insights.

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

IMPORTANT SAFETY INFORMATION

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.

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 RareConnections™ at 1-866-906-6100.

Please see full Prescribing Information, including important warning.

20 **References:** 1. Hendriksz CJ, Parini R, AlSayed MD, et al. Long-term endurance and safety of elosulfase alfa enzyme replacement therapy in patients with Morquio A syndrome. *Mol Genet Metab.* 2016;119(1-2):131-143. doi:10.1016/j.ymgme.2016.05.016. 2. Hendriksz CJ, Berger KI, Parini R, et al. Impact of long-term elosulfase alfa treatment on respiratory function in patients with Morquio A syndrome. *J Inherit Metab Dis.* 2018;39(6):839-847. doi:10.1007/s10545-016-9973-6. 3. Morrone A, Caciotti A, Abaoud R, Morquio A syndrome-associated mutations: a review of alterations in the GALNS gene and a new locus-specific database. *Hum Mutat.* 2014;35:1271-1279. 4. Wood TC, Harvey K, Beck M, et al. Proposing mucopolysaccharidosis IVA. *J Inherit Metab Dis.* 2013;36(2):293-307. doi:10.1007/s10545-013-9687-1. 5. VIMIZIM [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; 2014.

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