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PLANS IN SCOPE

Aspirus Health Plan

BACKGROUND & PURPOSE

Background

Mucopolysaccharidosis Type IV (MPS IV) is a rare condition wherein the body cannot break down the sugar molecule glycosaminoglycans resulting in a build-up of these sugars in lysosomes (organelles in a cell that break down and repurpose chemicals). There are two forms of MPS IV caused by different genetic abnormalities and can be inherited or occur randomly in cells. It is estimated that fewer than 5,000 people in the U.S. have this disease.

Symptoms of MPS IV can start at any age and vary between individuals. Some common symptoms include abnormal dentation (teeth, including cavities); abnormal bone morphology (arrangement of parts); abnormal heart morphology; coarse facial features; corneal opacity (loss of transparency); delayed skeletal maturation (growth); gait (how you walk) disturbance; hearing impairment; hernia (organ that pushes out of the abdominal cavity); reduced bone mineral density

(thickness); short neck; short stature; short thorax; spinal canal stenosis (narrowing); and wide mouth. Less common symptoms may include macrocephaly (abnormally large head) and cognitive impairment. MPS IV type A (MPS IVA or Morquio Syndrome Type A) is an inherited form of MPS IV wherein the body does not make enough of a specific enzyme (protein that breaks down chemicals in the body) called N-acetylgalactosamine-6-sulfatase (GLANS). GLANS breaks down and recycles glycosaminoglycans (GAGs). Without this enzyme, the GAGs; keratan sulfate (KS), and chrondroitin-6-sulfact (C6S) builds up in tissues, bones and major organs resulting in multiple progressive complications. Diagnosis of MPS IVA includes a combination of unique clinical features with biochemical markers in blood and urine including GALNS enzyme assay, KS level, and C6S level. Like other MPS IV diseases, symptoms can appear at any age. Currently there is no cure for MPS IVA.

Although, in October 2024, the U.S. Food and Drug Administration granted a rare pediatric and orphan drug treatment for Morquio A Syndrome through the Bespoke Gene Therapy Consortium (BGTC), Vimizin (elosulfase alfa) is currently the only FDA approved treatment for Morquio A (MPS IVA). Vimizin (approved in 2014) has been shown to improve endurance of the 6-minute walk test; the 3-minute stair climbing test; and improvement in forced vital lung capacity, forced expiratory volume and left ventricular mass index in patients taking the medication weekly. Vimizin does not cross the blood-brain barrier, therefore it is unable to improve neurologic function in MPS IVA. Fortunately, cognitive, and/or neurologic symptoms are not routinely associated with MPS IVA. There are currently no biosimilars for Vimizim.

There have been some cases of hematopoietic stem cell transplantation (HSCT) resulting in improvement. Although HSCT may be a treatment option for some patients with MPS IVA, and this method is significantly cheaper than enzyme replacement ($100K one time vs $400K annually); however, limited cases for treatment were identified and more evidence is required. Additional surgical management of complications may be needed during the disease course, including ophthalmologic and orthopedic procedures.

Purpose

The purpose of this policy is to provide coverage guidelines for Vimizin.

Please refer to the member's benefit document for specific information. To the extent there is any inconsistency between this policy and the terms of the member's benefit plan or certificate of coverage, the terms of the member's benefit plan document will govern.

COVERAGE INDICATIONS:

Vimizin is prescribed by or in consultation with a physician who specializes in Morquio Syndrome. Vimizin is considered medically necessary when the following criteria are met:

- Must meet **ALL** the following criteria:
    a. Be diagnosed with MPS IVA
    b. Be aged 5 years or older
    c. Be able to take the medication weekly

d. Show improvement in a 6-minute walking test or 3-minute stair test or spirometry values within 6-12 months of continued use

Dosing

Due to the high-risk of anaphylaxis and complex dosing/administration recommendations, Vimizin is to be administered under the supervision of a physician by a trained healthcare professional in a location where appropriate medical support is readily available such as an infusion clinic. Pre-treatment with antihistamines with or without antipyretics is recommended 30-60 minutes before starting the infusion. A 0.2 micrometer filter should be used for administration. Infusions should be provided by infusionists trained in Vimizin administration. Emergency medications should remain on hand during infusions.

The dose for Vimizin:

- 2 mg/kg administered once a week intravenously over a minimum of 3.5 to 4.5 hours.
- Volume/infusion recommendations:
    - Patients weighing < 25 kg:
        - Mix in 100 ML of 0.9% Sodium Chloride
        - Begin infusion at 3ML per hour for the first 15 minutes and increase to 6 mL per hour for the next 15 minutes
        - If 6mL per hour is tolerated, you can increase 6mL per hour every 15 minutes to a maximum of 36 mL
    - Patients weighing >= 25 kg:
        - Mix in 250 ML of 0.9% Sodium Chloride
        - Begin infusion at 6ml per hour for the first 15 minutes and increase to 12 mL per hour for the next 15 minutes
        - If 12mL per hour is tolerated, you can increase by 12mL per hour every 15 minutes to a maximum of 72 mL per hour

Exclusions

Given the complexity and need for on-site emergency supplies during administration, home infusion services are not covered unless the member is homebound.
Other exclusions may include:

- Pregnancy: Effects in pregnancy are unknown at this time
- Lactation: Effects on presence of in human breastmilk is unknown at this time
- Pediatric Patients: Although there was a small study supporting use in children under age 5 years, safety and effectiveness in this age group has not been proven.
- Geriatric Patients: There is not enough evidence to judge effectiveness on individuals over age 65

Medical Records Documentation

Benefit coverage is determined by review of member specific benefit plan information and all applicable laws. Medical records documentation may be required to assess if the member meets criteria; however, provision of records does not guarantee coverage.

APPLICABLE CODES

Note: The code list below is provided for guidance. Not all clinical trials will contain these codes. Code coverage will depend on the coverage guidelines above. All clinical trial coverage requests will require medical review.

Code Type	Code	Description
ICD10	E76.21	Morquio mucopolysaccharidoses
HCPCS	J1322	Elosulfase alfa injection, 1 mg
NDC	6813-100	Elosulfase alfa injection, 1 mg/mL, 5mL
HCPCS	E0780	Ambulatory infusion pump, mechanical reusable for infusion less than 8 hours
CPT	96365	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); initial, up to 1 hour
CPT	96366	Intravenous infusion, for therapy, prophylaxis, or diagnosis (specify substance or drug); each additional hour (List separately in addition to code for primary procedure)

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POLICY/REVISION HISTORY

Date	Summary of Changes	Approval By
07/XX/2025	Initial Policy Development	Optum Coverage Policy Committee

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Nondiscrimination & Language Access Policy

Discrimination is Against the Law. Aspirus Health Plan, Inc. complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability, or sex, (including sex characteristics, including intersex traits; pregnancy or related conditions; sexual orientation, gender identity and sex stereotypes), consistent with the scope of sex discrimination described at 45 CFR § 92.101(a)(2). Aspirus Health Plan, Inc. does not exclude people or treat them less favorably because of race, color, national origin, age, disability, or sex.

Aspirus Health Plan, Inc.:
Provides people with disabilities reasonable modifications and free appropriate auxiliary aids and services to communicate effectively with us, such as:

- Qualified sign language interpreters.
- Written information in other formats (large print, audio, accessible electronic formats, other formats).

Provides free language assistance services to people whose primary language is not English, which may include:

- Qualified interpreters.
- Information written in other languages.

If *you* need reasonable modifications, appropriate auxiliary aids and services, or language assistance services, contact the Nondiscrimination Grievance Coordinator at the address, phone number, fax number, or email address below.

If *you* believe that Aspirus Health Plan, Inc. has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, *you* can file a *grievance* with:

Nondiscrimination Grievance Coordinator
Aspirus Health Plan, Inc.
PO Box 1890
Southampton, PA 18966-9998
Phone: 1-866-631-5404 (TTY: 711)
Fax: 763-847-4010
Email: customerservice@aspirushealthplan.com

You can file a *grievance* in person or by mail, fax, or email. If *you* need help filing a *grievance*, the Nondiscrimination Grievance Coordinator is available to help *you*.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1.800.368.1019, 800.537.7697 (TDD)

Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html. This notice is available at Aspirus Health Plan, Inc.'s website: https://aspirushealthplan.com/webdocs/70021-AHP-NonDiscrim_Lang-Assist-Notice.pdf.

Language Assistance Services

Albanian: KUJDES: Nëse flitni shqip, për ju ka në dispozicion shërbime të asistencës gjuhësore, pa pagesë.Telefononi në 1-800-332-6501 (TTY: 711).

Arabic تنبيه: إذا كنت تتحدث اللغة العربية، فإن خدمات المساعدة اللغوية متاحة لك مجاناً. اتصل بن اعلى رقم الهاتف1-800-332-6501(رقم هاتف الصم والبك : 711)

French: ATTENTION: Si vous parlez français, des services d'aide linguistique vous sont proposés gratuitement. Appelez le 1-800-332-6501 (ATS: 711).

German: ACHTUNG: Wenn Sie Deutsch sprechen, stehen Ihnen kostenlos sprachliche Hilfsdienstleistungen zurVerfügung. Rufnummer: 1-800-332-6501 (TTY: 711).

Hindi: _यान द_: य_द आप ि हंदी बोलते ह_ तो आपके ि लए मु_त म_ भाषा सहायता सेवाएं उपल_ध ह_।1-800-332-6501 (TTY: 711) पर कॉल कर_।

Hmong: LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1-800-332-6501 (TTY: 711).

Korean: 주의: 한국어를 사용하시는 경우, 언어 지원 서비스를 무료로 이용하실 수 있습니다.1-800-332-6501 (TTY: 711)번으로 전화해 주십시오.

Polish: UWAGA: Jeżeli mówisz po polsku, możesz skorzystać z bezpłatnej pomocy językowej. Zadzwoń pod numer1-800-332-6501 (TTY: 711).

Russian: ВНИМАНИЕ: Если вы говорите на русском языке, то вам доступны бесплатные услуги перевода.Звоните 1-800-332-6501 (телетайп: 711).

Spanish: ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al1-800-332-6501 (TTY: 711).

Tagalog: PAUNAWA: Kung nagsasalita ka ng Tagalog, maaari kang gumamit ng mga serbisyo ng tulong sa wika nangwalang bayad. Tumawag sa 1-800-332-6501 (TTY: 711).

Traditional Chinese: 注意：如果您使用繁體中文， 您可以免費獲得語言援助服務。請 致電 1-800-332-6501 (TTY: 711)

Vietnamese: CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1-800-332-6501 (TTY: 711).

Pennsylvania Dutch: Wann du Deitsch (Pennsylvania German / Dutch) schwetzscht, kannscht du mitaus Koschte ebbergricke, ass dihr helft mit die englisch Schprooch. Ruf selli Nummer uff: Call 1-800-332-6501 (TTY: 711).

Lao: ໂປດຊາບ: ຖ້າວ່າ ທ່ານເວົ້າພາສາ ລາວ, ການບໍລິການຊ່ວຍເຫຼືອດ້ານພາສາ,ໂດຍບໍ່ເສັງຄ່າ, ແມ່ນມີພ້ອມໃຫ້ທ່ານ. ໂທຣ 1-800-332-6501 (TTY: 711).

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