

Medical Policy:

Mepsevii (vestronidase alfa-vjbc) intravenous infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.304	March 17, 2025	2017

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The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as “EmblemHealth”), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Mepsevii, a lysosomal beta glucuronidase (GUS), is indicated for the treatment of Mucopolysaccharidosis type VII ([MPS VII], Sly syndrome). It is produced in a Chinese hamster ovary cell line via recombinant DNA technology. It has the same amino acid sequence as human GUS and catabolizes accumulated glycosaminoglycans in lysosomes in affected tissues

Length of Authorization

Coverage will be provided for 12 months and may be renewed.

Dosing Limits [Medical Benefit]

Each dose must not exceed 4 mg/kg administered intravenously no more frequently than once every 2 weeks. (460 billable units (460 mg) every 14 days)

Guideline

I. INITIAL CRITERIA

- Mucopolysaccharidosis Type VII (Sly Syndrome):** Approve if the patient meets the following criteria:

- A. The diagnosis is established by one of the following (i or ii):
 - i. Patient has a laboratory test demonstrating deficient beta-glucuronidase activity in leukocytes, fibroblasts, or serum; **OR**
 - ii. Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic glucuronidase (*GUS*) gene variants; **AND**
- B. Patient is at least 5 months of age; **AND**
- C. Documented baseline age-appropriate values for one or more of the following have been obtained: 6-minute walk test (6-MWT), motor function [i.e., Bruininks-Oseretsky Test of Motor Proficiency (BOT-2)], liver and/or spleen volume, urinary excretion of glycosaminoglycans (GAGs) such as chondroitin sulfate and dermatan sulfate, skeletal involvement (i.e. Z-score), pulmonary function tests, shoulder flexion, visual acuity; **AND**
- D. Therapy is being used to treat non-central nervous system manifestations of the disease and patient does not have severe, irreversible cognitive impairment; **AND**
- E. Mepsevii is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder subspecialist, or a physician who specializes in the treatment of lysosomal storage disorders.

II. RENEWAL CRITERIA

Coverage may be renewed based on the following criteria:

1. Patient continues to meet Initial Criteria; **AND**
2. Absence of unacceptable toxicity from the drug. *Examples of unacceptable toxicity include: anaphylaxis and severe allergic reactions, etc.*; **AND**
3. Patient has demonstrated a beneficial response to therapy compared to pretreatment age-appropriate baseline values in one or more of the following:
 - i. Stability or improvement in 6-MWT, shoulder flexion, pulmonary function tests, visual acuity, and/or motor functions (i.e., BOT-2)
 - ii. Reduction in liver and/or spleen volume
 - iii. Reduction in urinary excretion of GAGs
 - iv. Stability of skeletal disease (i.e. improvement in Z-score)

Applicable Procedure Codes

Code	Description
J3397	Injection, vestronidase alfa-vjbk, 1 mg

Applicable NDCs

Code	Description
69794-0001-01	Mepsevii 2mg/mL Solution

ICD-10 Diagnoses

Code	Description
E76.29	Other Mucopolysaccharidoses

Revision History

Company(ies)	DATE	REVISION
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EmblemHealth & ConnectiCare	3/17/2025	Annual Review: Initial Criteria: Removed “glucuronidase gene mutation; AND “ from the following statement: “Patient has a molecular genetic test demonstrating glucuronidase gene mutation; AND” replaced with: “biallelic pathogenic or likely pathogenic glucuronidase (GUS) gene variants; AND” Added: Patient is at least 5 months of age; AND Documented baseline age-appropriate values for one or more of the following have been obtained: 6-minute walk test (6-MWT), motor function [i.e., Bruininks-Oseretsky Test of Motor Proficiency (BOT-2)], liver and/or spleen volume, urinary excretion of glycosaminoglycans (GAGs) such as chondroitin sulfate and dermatan sulfate, skeletal involvement (i.e. Z-score), pulmonary function tests, shoulder flexion, visual acuity; AND Therapy is being used to treat non-central nervous system manifestations of the disease and patient does not have severe, irreversible cognitive impairment; AND” RENEWAL CRITERIA: Added: “Coverage may be renewed based on the following criteria: Patient continues to meet Initial Criteria; AND Absence of unacceptable toxicity from the drug. <i>Examples of unacceptable toxicity include: anaphylaxis and severe allergic reactions, etc.</i> ; AND Patient has demonstrated a beneficial response to therapy compared to pretreatment age-appropriate baseline values in one or more of the following: Stability or improvement in 6-MWT, shoulder flexion, pulmonary function tests, visual acuity, and/or motor functions (i.e., BOT-2) Reduction in liver and/or spleen volume Reduction in urinary excretion of GAGs Stability of skeletal disease (i.e. improvement in Z-score)” Updated ICD-10 codes
EmblemHealth & ConnectiCare	2/2/2024	Annual Review: no criteria changes
EmblemHealth & ConnectiCare	4/7/2023	Transfer from CCUM template to Co-Branded medical template Retired MG.MM.PH.122
EmblemHealth & ConnectiCare	4/6/2022	Annual Revision: no criteria changes
EmblemHealth & ConnectiCare	4/7/2021	Annual Revision: no criteria changes

References

1. Mepsevii intravenous infusion [prescribing information]. Novato, CA: Ultragenyx; December 2020.