

Clinical Policy: Vestronidase Alfa-vj bk (Mepsevii)

Reference Number: CP.PHAR.374

Effective Date: 01.09.18

Last Review Date: 05.23

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Vestronidase alfa-vj bk (Mepsevii[®]) is a recombinant human lysosomal beta glucuronidase enzyme replacement therapy.

FDA Approved Indication(s)

Mepsevii is indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome).

Limitation(s) of use: The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Centene Corporation[®] that Mepsevii is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mucopolysaccharidosis VII: Sly Syndrome (must meet all):

1. Diagnosis of MPS VII (Sly syndrome) confirmed by one of the following (a or b):
 - a. Two repeated enzyme assay tests demonstrating a deficiency of beta-glucuronidase;
 - b. One DNA testing showing *GUSB* gene mutation;
2. Prescribed by or in consultation with a specialist with expertise in lysosomal storage diseases (e.g., pediatric endocrinologist, pediatric geneticist);
3. Apparent clinical signs of lysosomal storage disease including at least one of the following (a, b, c, or d):
 - a. Enlarged liver and spleen;
 - b. Joint limitations;
 - c. Airway obstruction or pulmonary problems;
 - d. Limitations of mobility;
4. Dose does not exceed 4 mg/kg IV every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Mucopolysaccharidosis VII: Sly Syndrome (must meet all):

1. Member meets one of the following (a or b):
 - a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
 - b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B*);
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS VII disease manifestation profile (*see Appendix D for examples*);
3. If request is for a dose increase, new dose does not exceed 4 mg/kg IV every 2 weeks.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND

criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration

MPS VII: Mucopolysaccharidosis VII

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported
- Boxed warning(s): anaphylaxis

Appendix D: General Information

- The presenting symptoms and clinical course of MPS VII can vary from one individual to another. Some examples, however, of improvement in MPS VII disease as a result of Mepsevii therapy may include improvement in:
 - 6-minute walking distance
 - Breathing difficulties
 - Muscle weakness
 - Vision or hearing problems
 - Hepatomegaly or splenomegaly
 - Reduction of total urinary glycosaminoglycan (uGAG) excretion
 - Stair climbing capacity as measured by the 3 Minute Stair Climb Test
 - Height and weight growth velocity compared to estimated pretreatment growth rate velocity from medical records for pediatric patients
- In individuals with MPS, the circulation of fluid through the blood-brain barrier may become blocked, which can lead to hydrocephalus and cortical atrophy. Seizures are a complication most common among individuals with severe forms of MPS. The clinical benefit on this central nervous system manifestation with treatment of Mepsevii has not yet been determined.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS VII (Sly syndrome)	4 mg/kg IV every 2 weeks	4 mg/kg/2 weeks

VI. Product Availability

Single-dose vial: 10 mg/5 mL

VII. References

1. Mepsevii Prescribing Information. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2020. Available at: www.mepsevii.com. Accessed February 9, 2023.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3397	Injection, vestronidase alfa-vjbjk, 1 mg

Reviews, Revisions, and Approvals	Date	P&T Approval Date
2Q 2019 annual review: added HIM-Medical Benefit; references reviewed and updated.	02.04.19	05.19
2Q 2020 annual review: no significant changes; revised HIM-Medical Benefit to HIM line of business; references reviewed and updated.	02.21.20	05.20
2Q 2021 annual review: no significant changes; revised HIM.PHAR.21 to HIM.PA.154; references reviewed and updated.	02.28.21	05.21
2Q 2022 annual review: no significant changes; references reviewed and updated.	02.24.22	05.22
Template changes applied to other diagnoses/indications and continued therapy section.	09.22.22	
2Q 2023 annual review: no significant changes; references reviewed and updated.	02.09.23	05.23

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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