# vestronidase alfa-vjbk (Mepsevii™)

**Policy #** 00618
Original Effective Date: 05/16/2018
Current Effective Date: 06/12/2023

*Applies to all products administered or underwritten by Blue Cross and Blue Shield of Louisiana and its subsidiary, HMO Louisiana, Inc.(collectively referred to as the "Company"), unless otherwise provided in the applicable contract. Medical technology is constantly evolving, and we reserve the right to review and update Medical Policy periodically.*

# When Services May Be Eligible for Coverage

*Coverage for eligible medical treatments or procedures, drugs, devices or biological products may be provided only if:*

- *Benefits are available in the member's contract/certificate, and*
- *Medical necessity criteria and guidelines are met.*

Based on review of available data, the Company may consider vestronidase alfa-vjbk (Mepsevii™)‡ for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome) to be **eligible for coverage.****

Patient Selection Criteria

Coverage eligibility for vestronidase alfa-vjbk (Mepsevii) will be considered when the following criteria are met:

- Initial:
  - Patient has a diagnosis of MPS VII confirmed by BOTH of the following:
    - Beta-glucuronidase enzyme deficiency in peripheral blood leukocytes or cultured fibroblasts; AND
    - Molecular genetic confirmation of mutations in the *GUSB* gene; AND
  - Patient has clinical signs and symptoms of MPS VII (e.g., skeletal deformities, enlarged spleen, hernias, airway limitations, joint limitations, etc.); AND
  - Mepsevii dose does not exceed (and is not dosed more often than) 4 mg/kg once every two weeks.
- Re-authorization:
  - Patient received an initial authorization for Mepsevii; AND
  - Patient has experienced a positive clinical response to Mepsevii therapy (e.g., improved endurance, improved functional capacity, improved pulmonary function, etc.); AND

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*(Note: This specific patient criterion is an additional Company requirement for coverage eligibility and will be denied as not medically necessary** if not met).*

- Mepsevii dose does not exceed (and is not dosed more often than) 4 mg/kg once every two weeks.

# When Services Are Considered Not Medically Necessary

Based on review of available data, the Company considers the use of vestronidase alfa-vjbk (Mepsevii) when the patient has NOT experienced a positive clinical response to vestronidase alfa-vjbk (Mepsevii) therapy (e.g., improved endurance, improved functional capacity, improved pulmonary function, etc.) to be **not medically necessary.****

# When Services Are Considered Investigational

*Coverage is not available for investigational medical treatments or procedures, drugs, devices or biological products.*

Based on review of available data, the Company considers the use of vestronidase alfa-vjbk (Mepsevii) when patient selection criteria are not met (with the exception of those denoted above as **not medically necessary**)** to be **investigational.***

# Background/Overview

Mepsevii is a recombinant human lysosomal beta glucuronidase indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII. The recommended dose of Mepsevii is 4 mg/kg given every two weeks via an intravenous infusion.

MPS VII (also known as Sly Syndrome) is an ultra-rare, progressively debilitating and life-threatening lysosomal storage disease. The incidence of MPS VII in the United States is 0.05 per 100,000 births. The worldwide overall prevalence for MPS VII is 1:300,000 – 1:2,000,000. There are fewer than 200 patients identified worldwide with MPS VII. Individuals with MPS VII lack the lysosomal enzyme beta-glucuronidase, which leads to an accumulation of glycosaminoglycans (GAGs). These GAGs can lead to coarsened facies, pulmonary disease, cardiovascular complications, hepatosplenomegaly, joint stiffness, short stature, cognitive impairment, dysostosis

multiplex, cognitive impairment, and reduced life expectancy. Prior to the approval of Mepsevii, an enzyme replacement for this condition, treatment for this condition was palliative.

# FDA or Other Governmental Regulatory Approval

**U.S. Food and Drug Administration (FDA)**

Mepsevii was approved in November of 2017 for the treatment of pediatric and adult patients with Mucopolysaccharidosis VII, also known as Sly syndrome.

# Rationale/Source

This medical policy was developed through consideration of peer-reviewed medical literature generally recognized by the relevant medical community, U.S. FDA approval status, nationally accepted standards of medical practice and accepted standards of medical practice in this community, technology evaluation centers, reference to federal regulations, other plan medical policies, and accredited national guidelines.

Mepsevii's efficacy was established in one randomized, placebo-controlled crossover study in patients with MPS VII. Due to the rarity of the disease and the small population, information from a dose exploration study and an expanded access program were also included. Researchers investigated the mean difference in the 6 minute walk test (6MWT) between Mepsevii and placebo. At week 24, the mean difference in the 6MWT between Mepsevii and placebo treatment was 18 meters. Ten patients could perform the 6MWT through week 120. Of these ten, 3 patients demonstrated improvement of at least 60 meters compared to the start of Mepsevii treatment. Liver volumes were normal or below normal at baseline and remained as such during the study. The other 7 patients experienced a relatively stable disease course during the 6MWT. An open label exploration trial with 3 patients demonstrated an improvement over baseline in the Forced Vital Capacity percent predicted in one patient coupled with an increase in 6MWT. The other 2 patients had a reduction in liver and spleen volume.

# References

1. Mepsevii [package insert]. Ultragenyx Pharmaceutical, Inc. Novato, California.
2. Mepsevii [dossier]. Ultragenyx Pharmaceuticals, Inc. Novato, California.

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# Policy History

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|---|---|
| 05/03/2018 | Medical Policy Committee review |
| 05/16/2018 | Medical Policy Implementation Committee approval. New policy. |
| 05/02/2019 | Medical Policy Committee review |
| 05/15/2019 | Medical Policy Implementation Committee approval. No change to coverage. |
| 05/07/2020 | Medical Policy Committee review |
| 05/13/2020 | Medical Policy Implementation Committee approval. Updated re-authorization criteria to match similar policies. Also added a missing not medically necessary statement regarding re-authorization treatment. |
| 05/06/2021 | Medical Policy Committee review |
| 05/12/2021 | Medical Policy Implementation Committee approval. No change to coverage. |
| 05/05/2022 | Medical Policy Committee review |
| 05/11/2022 | Medical Policy Implementation Committee approval. No change to coverage. |
| 05/04/2023 | Medical Policy Committee review |
| 05/10/2023 | Medical Policy Implementation Committee approval. No change to coverage. |

Next Scheduled Review Date: 05/2024

# Coding

*The five character codes included in the Blue Cross Blue Shield of Louisiana Medical Policy Coverage Guidelines are obtained from Current Procedural Terminology (CPT®)‡, copyright 2022 by the American Medical Association (AMA). CPT is developed by the AMA as a listing of descriptive terms and five character identifying codes and modifiers for reporting medical services and procedures performed by physician.*

*The responsibility for the content of Blue Cross Blue Shield of Louisiana Medical Policy Coverage Guidelines is with Blue Cross and Blue Shield of Louisiana and no endorsement by the AMA is intended or should be implied. The AMA disclaims responsibility for any consequences or liability attributable or related to any use, nonuse or interpretation of information contained in Blue Cross Blue Shield of Louisiana Medical Policy Coverage Guidelines. Fee schedules, relative value units, conversion factors and/or related components are not assigned by the AMA, are not part of CPT, and the AMA is not recommending their use. The AMA does not directly or indirectly practice*

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*medicine or dispense medical services. The AMA assumes no liability for data contained or not contained herein. Any use of CPT outside of Blue Cross Blue Shield of Louisiana Medical Policy Coverage Guidelines should refer to the most current Current Procedural Terminology which contains the complete and most current listing of CPT codes and descriptive terms. Applicable FARS/DFARS apply.*

CPT is a registered trademark of the American Medical Association.

Codes used to identify services associated with this policy may include (but may not be limited to) the following:

| Code Type | Code |
|---|---|
| CPT | No codes |
| HCPCS | J3397<br>Delete codes effective 06/01/2023: J3490, J3590, C9399 |
| ICD-10 Diagnosis | E76.2-E76.9 |

*Investigational – A medical treatment, procedure, drug, device, or biological product is Investigational if the effectiveness has not been clearly tested and it has not been incorporated into standard medical practice. Any determination we make that a medical treatment, procedure, drug, device, or biological product is Investigational will be based on a consideration of the following:

A. Whether the medical treatment, procedure, drug, device, or biological product can be lawfully marketed without approval of the U.S. Food and Drug Administration (FDA) and whether such approval has been granted at the time the medical treatment, procedure, drug, device, or biological product is sought to be furnished; or
B. Whether the medical treatment, procedure, drug, device, or biological product requires further studies or clinical trials to determine its maximum tolerated dose, toxicity, safety, effectiveness, or effectiveness as compared with the standard means of treatment or diagnosis, must improve health outcomes, according to the consensus of opinion among experts as shown by reliable evidence, including:
    1. Consultation with technology evaluation center(s);
    2. Credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community; or
    3. Reference to federal regulations.

**Medically Necessary (or "Medical Necessity") - Health care services, treatment, procedures, equipment, drugs, devices, items or supplies that a Provider, exercising prudent clinical judgment, would provide to a patient for the purpose of preventing, evaluating, diagnosing or treating an illness, injury, disease or its symptoms, and that are:

A. In accordance with nationally accepted standards of medical practice;
B. Clinically appropriate, in terms of type, frequency, extent, level of care, site and duration, and considered effective for the patient's illness, injury or disease; and
C. Not primarily for the personal comfort or convenience of the patient, physician or other health care provider, and not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

For these purposes, "nationally accepted standards of medical practice" means standards that are based on credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, Physician Specialty Society recommendations and the views of Physicians practicing in relevant clinical areas and any other relevant factors.

‡ Indicated trademarks are the registered trademarks of their respective owners.

**NOTICE:** If the Patient's health insurance contract contains language that differs from the BCBSLA Medical Policy definition noted above, the definition in the health insurance contract will be relied upon for specific coverage determinations.

**NOTICE:** Medical Policies are scientific based opinions, provided solely for coverage and informational purposes. Medical Policies should not be construed to suggest that the Company recommends, advocates, requires, encourages, or discourages any particular treatment, procedure, or service, or any particular course of treatment, procedure, or service.