vestronidase alfa-vjbk (Mepsevii™)

Policy # 00618
Original Effective Date: 05/16/2018
Current Effective Date: 05/16/2018

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.

Continuation:

- Patient has experienced a positive clinical response to Mepsevii therapy (e.g., improved endurance, improved functional capacity, improved pulmonary function, etc.); AND
- Mepsevii dose does not exceed (and is not dosed more often than) 4 mg/kg once every two weeks.

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 the patient selection criteria are not met 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.

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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, Blue Cross and Blue Shield Association technology assessment program (TEC) and other non-affiliated 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. Updated November 2017.
2. Mepsevii [dossier]. Ultragenyx Pharmaceuticals, Inc. Novato, California. Updated December 2017.

Policy History

Original Effective Date: 05/16/2018
Current Effective Date: 05/16/2018
05/03/2018 Medical Policy Committee review
05/16/2018 Medical Policy Implementation Committee approval. New policy.

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Next Scheduled Review Date: 05/2019

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 2017 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.

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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	C9399, J3490, J3590
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. 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 the Blue Cross and Blue Shield Association technology assessment program (TEC) or other nonaffiliated 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.

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

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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.