Medical Coverage Policy | Mepsevii™
(vestronidase alfa-vjbk) for Sly Syndrome

EFFECTIVE DATE: 08|01|2018
POLICY LAST UPDATED: 07|17|2018

OVERVIEW

Lysosomal storage disorders (LSDs) are a group of inherited metabolic disorders that result in the accumulation of undigested macromolecules due to the dysfunction of lysosomes. There are several different LSDs but they share a common feature of an error of metabolism of lipids, glycoproteins, or glycosaminoglycans (GAGs), typically due to a deficiency in of a lysosomal enzyme or transport protein

This policy is applicable to BlueCHiP for Medicare products only. For Commercial Products, see related policy section

MEDICAL CRITERIA

Sly syndrome [Mucopolysaccharidosis VII (MPSVII)]
Initial Evaluation
Mepsevii is medically necessary when ONE of the following are met:

1. There is documentation that the patient is currently being treated with the requested agent
   OR
2. ALL of the following:
   A. The patient has a diagnosis of Sly Syndrome [mucopolysaccharidosis type VII (MPS VII)] and ALL of the following:
      i. ONE of the following:
         1. The patient has a beta-glucuronidase deficiency in leukocytes, fibroblasts, or plasma
            OR
         2. The patient has genetic analysis of disease causing mutation of the beta-glucuronidase gene

      AND

      ii. ONE of the following:
         1. The prescriber is a specialist in the area of practice related to the patient's diagnosis (e.g. endocrinologist, geneticist)
            OR
         2. The prescriber has consulted with a specialist in the area of practice related to the patient's diagnosis

      AND

   1. ONE of the following:
      1. In an overnight sleep study the patient has had either an average of >5 apnea events per hour (>1 apnea event per hour for children) over the patient's total sleep or more than 2 severe episodes of desaturation (mean nocturnal O2 saturation of <85% in adults; <92% in children)
      2. The patient has a forced vital capacity (FVC) < 80% predicted value for height
      3. The patient has reduced ejection fraction of <56% [normal range 56-78%]
      4. The patient has a reduction in fraction shortening to <25% [normal range 25-46%]
      5. The patient has restricted range of movement in joints of > 10° from normal

6. The patient has hepatomegaly
7. The patient has splenomegaly

AND

A. The patient does not have any FDA labeled contraindication(s) to therapy with the requested agent

AND

B. The dose is within the FDA labeled dose

Length of Approval: 12 months

Renewal Evaluation

1. The patient has been previously approved for the requested agent through the Prime Therapeutics or BCBSRI PA or Medical Review process
   AND

2. The patient has received clinical benefit from the requested agent (e.g. stabilization or slowing of disease progression, decrease in symptom severity and/or frequency)
   AND

3. ONE of the following
   a. The requested dose is within FDA labeling
      OR
   b. The requested dose for the requested diagnosis is supported by compendia (NCCN Compendium™[level of evidence 1, 2A], AHFS, DrugDex [FDA approved Class I or Class IIa])
      OR
   c. The requested dose is outside the dose supported by FDA labeling or compendia AND the patient is currently taking and is stable on this dose
      OR
   d. The request is for a change in dose that is outside that supported by FDA labeling or compendia AND the prescriber has submitted documentation (the dose is supported by clinical research in 2 or more peer reviewed medical journals) in support of therapy with a higher dose for the requested diagnosis

PRIOR AUTHORIZATION

Prior authorization is required for BlueCHiP for Medicare.

POLICY STATEMENT

Mepsevii™ (vestronidase alfa-vjbk) for Sly Syndrome is covered when the medical Criteria is met.

COVERAGE

Benefits may vary between groups and contracts. Please refer to the appropriate Evidence of Coverage for applicable physician administered infusion drug benefits/coverage.

BACKGROUND

Lysosomal storage disorders (LSDs) are a group of inherited metabolic disorders that result in the accumulation of undigested macromolecules due to the dysfunction of lysosomes. There are several different LSDs but they share a common feature of an error of metabolism of lipids, glycoproteins, or glycosaminoglycans (GAGs), typically due to a deficiency in of a lysosomal enzyme or transport protein. A deficit in these enzymes results in progressive accumulation of material in organs and tissues which results in

an increase in the size and quantity of organelles and ultimately in cellular dysfunction and organ failure. The majority of these disorders have substantial neurological involvement with developmental regression, seizures and learning difficulties. Most patients affected by these disorders have a decreased life expectancy with considerable morbidity.

Most of these disorders have a birth prevalence of < 1:100,000 with a combined prevalence of around 1 in every 7,000 to 8,000 births. There isn't a cure or definitive treatment available for any LSD. Enzyme replacement therapy (ERT) is available for some LSDs and is generally considered safe.

Sly syndrome[47,51-52]
Sly syndrome, also known as Mucopolysaccharidosis VII (MPS VII), is an autosomal recessive disorder caused by mutations in the gene encoding beta-glucronidase (GUSB). The enzyme deficiency results in accumulation of heparan sulfate, dermatan sulfate, chondroitin-4-sulfate, and chrondroitin-6-sulfate. Sly syndrome is extremely rare, affecting about 1 in 250,00 births. Males and females are equally affected.

Clinical features and complications may be similar to Mucopolysaccharidosis I, with significant soft tissue and skeletal abnormalities. Mental retardation may be mild or absent. Hydrops fetalis is a common presentation and may account for a large proportion of patients not being diagnosed due to death before a diagnosis can be made. The most attenuated form is limited to skeletal abnormalities.

In 2017 an enzyme replacement therapy (ERT), Mepsevii (vestronidase alfa-jvbk) was approved to treat pediatric and adult patients with MPS VII. Other treatments of MPS VII are symptomatic and supportive. Bone deformities, hernias, ocular abnormalities, and cardiovascular abnormalities may require surgical correction. The clinical benefits of ERT include decreased hepatomegaly, improved respiratory function, improved walking ability, increased joint range of motion, decreased left ventricular hypertrophy, improved growth, and improved quality of life.

SAFETY:
There are no FDA labeled contraindications for the target agents, however, Aldurazyme, Elaprase, Lumizyme, Mepsevii, and Vimizim all have black box warnings concerning life-threatening anaphylactic reactions. Also, patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions.

CODING
For claims prior to 1/1/19 there is not a specific HCPCS code for this drug. Claims must be filed with an unlisted HCPCS code and the NDC number.

For claims after 12/31/18 please use the following HCPCS code:
J3397 Injection, vestronidase alfa-vjbk, 1 mg

RELATED POLICIES
Prior Authorization of Drugs

PUBLISHED
Provider Update, September 2018

REFERENCES:

1. N Engl J Med 2001; 344: 182-188.
2. UpToDate. Mucopolysaccharoidosis. Accessed January 2018.
3. UpToDate. Approach to adult patient with anemia. Updated 12/19/16. Accessed January 2018.

4. Greiner-Tollersrud OK and Berg T. Lysosmal Storage Disorders. Madame Curie Bioscience Database. Accessed on January 2018.
5. Mepsevii prescribing information. Ultragenyx Pharmaceutical Inc. November 2017.

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