

**POLICY:** Enzyme Replacement Therapy – Mepsevii™ (vestronidase alfa-vjvk injection, for intravenous use – Ultragenyx Pharmaceutical)

**DATE REVIEWED:** 04/15/2020

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### OVERVIEW

Mepsevii is lysosomal beta glucuronidase (GUS) produced in a Chinese hamster ovary cell line via recombinant DNA technology.<sup>1</sup> It has the same amino acid sequence as human GUS and catabolizes accumulated glycosaminoglycans in lysosomes in affected tissues.

Mepsevii is indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis type VII ([MPS VII], Sly syndrome).<sup>1</sup>

### Disease Overview

MPS VII or Sly syndrome is an extremely rare lysosomal storage disorder characterized by deficient GUS activity.<sup>2</sup> In MPS VII, the partially catabolized glycosaminoglycans, chondroitin sulfate, dermatan sulfate, and heparin sulfate accumulate in the lysosomes, ultimately leading to the signs and symptoms of the disease.<sup>2,3</sup> The onset, severity and rate of progression of MPS VII is heterogeneous. Patients may present at birth with hydrops fetalis and only survive a few months while others may have milder disease and survive into their 40s.<sup>2</sup> However, most patients have mental retardation, hepatosplenomegaly, and musculoskeletal issues including short stature, coarse facial features, loss of range of motion, restricted mobility, scoliosis, and kyphosis. The diagnosis of MPS VII is established by demonstrating deficient GUS activity in leukocytes, fibroblasts or serum, or by genetic testing.<sup>3</sup> Treatment for MPS VII includes enzyme replacement therapy with Mepsevii and hematopoietic stem cell transplantation.<sup>2</sup>

### POLICY STATEMENT

Prior authorization is recommended for medical benefit coverage of Mepsevii. Approval is recommended for those who meet the Criteria and Dosing for the listed indication(s). Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Mepsevii as well as the monitoring required for adverse events and long-term efficacy, approval requires Mepsevii to be prescribed by or in consultation with a physician who specializes in the condition being treated.

### RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Mepsevii is recommended in those who meet the following criteria:

#### FDA-Approved Indications

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1. **Mucopolysaccharidosis Type VII (Sly Syndrome).** Approve for 1 year if the patient meets the following criteria (A and B):
    - 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 glucuronidase gene mutation; AND
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- B) Mepsevii is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

**Dosing.** Each dose must not exceed 4 mg/kg administered intravenously no more frequently than once every 2 weeks.<sup>1</sup>

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### CONDITIONS NOT RECOMMENDED FOR APPROVAL

Mepsevii has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

### REFERENCES

1. Mepsevii injection [prescribing information]. Novato, CA: Ultragenyx Pharmaceutical; December 2019.
2. Montano AM, Lock-Hock N, Steiner RD, et al. Clinical course of sly syndrome (mucopolysaccharidosis type VII). *J Med Genet.* 2016;53:403-418.
3. Tomatsu S, Montano AM, Dung VC, et al. Mutations and polymorphisms in GUSB gene in mucopolysaccharidosis VII (Sly syndrome). *Hum Mutat.* 2009;30:511-519.

### HISTORY

Type of Revision	Summary of Changes	Date Reviewed
New Policy	--	04/17/2019
Annual Revisions	No criteria changes.	04/15/2020