CareSource

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Mepsevii (vestronidase alfa-vjbk)
BILLING CODE	J3590
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Outpatient Hospital
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Product) QUANTITY LIMIT— 4 mg/kg every two weeks
LIST OF DIAGNOSES CONSIDERED **NOT** MEDICALLY NECESSARY	Click Here

Mepsevii (vestronidase alfa-vjbk) is a **non-preferred** product and will only be considered for coverage under the **medical** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

SLY SYNDROME (Mucopolysaccharidosis VII or MPS VII)

For **initial** authorization:

1. Member has documented leukocyte or fibroblast glucuronidase enzyme assay or genetic testing confirming diagnosis of MPS VII; AND
2. Member did **not** undergo a successful bone marrow or stem cell transplant or has any degree of detectable chimaerism with donor cells; AND
3. Member has elevated urinary glycosaminoglycan (uGAG) excretion at a minimum of 2-fold over the mean normal for age; AND
4. Member's chart notes have baseline of at least **two** of the following: six-minute walk test (6MWT), Forced Vital Capacity (FVC), shoulder flexion, visual acuity, and Bruininks-Oseretsky Test of Motor Proficiency (BOT-2) (fine motor and gross motor skills).
5. **Dosage allowed:** 4 mg/kg administered by intravenous infusion every two weeks.

If member meets all the requirements listed above, the medication will be approved for 12 months.

For **reauthorization**:

1. Chart notes have been provided that show the member has shown improvement from baseline of any of the following: six-minute walk test (6MWT), forced vital capacity, motor function, visual acuity, or liver and spleen volume.

If member meets all the reauthorization requirements above, the medication will be approved for an additional 12 months.

CareSource considers Mepsevii (vestronidase alfa-vjbk) not medically necessary for the treatment of the diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
09/13/2018	New policy for Mepsevii created.

References:

1. Mepsevii [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; November, 2017.
2. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT01856218. An Open-Label Phase 1/2 Study to Assess the Safety, Efficacy and Dose of Study Drug UX003 Recombinant Human Beta-

glucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7); January 31, 2018. Available at: https://clinicaltrials.gov/ct2/show/NCT01856218?term=NCT01856218&rank=1.
3. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT02230566. A Phase 3 Study of UX003 Recombinant Human Betaglucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7); February 16, 2018. Available at: https://clinicaltrials.gov/ct2/show/NCT02230566?term=NCT02230566&rank=1.
4. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT02432144. A Long-Term Open-Label Treatment and Extension Study of UX003 rhGUS Enzyme Replacement Therapy in Subjects With MPS 7; November 6, 2017. Available at: https://clinicaltrials.gov/ct2/show/NCT02432144?term=NCT02432144&rank=1.
5. Harmatz P, et al. A novel Blind Start study design to investigate vestronidase alfa for mucopolysaccharidosis VII, an ultra-rare genetic disease. Mol Genet Metab. 2018 Apr;123(4):488-494.

Effective date: 10/01/2018
Revised date: 09/13/2018