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Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

P&T Date: 06/11/2026

Fayuvi™ (rebisufligene etispavovec-hopf)

HPCPS: J3590

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

- A. Coverage of the requested drug is provided when all the following are met:
 - a. FDA approved indication
 - b. FDA approved age
 - c. Confirmation of diagnosis by serum assay showing a decrease of heparan-N-sulfatase activity followed by genetic testing showing the member has homozygous pathogenic mutations or is heterozygous for two known pathogenic mutations of the SGSH gene
 - d. Must not have an anti-AAV9 antibody titer greater than or equal to 1:100
 - e. Must not have received prior treatment with any gene therapy for MPS IIIA or are being considered for treatment with any other gene therapy for MPS IIIA
 - f. The requesting physician attests to providing clinical outcome information within the appropriate provider portal as requested by BCBSM
 - g. Trial and failure, contraindication, OR intolerance to the preferred drugs as listed in BCBSM/BCN's utilization management medical drug list
- B. Quantity Limitations, Authorization Period and Renewal Criteria
 - a. Quantity Limit: Align with FDA recommended dosing
 - b. Authorization Period: 3 months
 - c. Renewal Criteria: Not applicable as no further authorization will be provided

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- MPS type IIIA is an autosomal recessive lysosomal storage disorder caused by a mutation on the SGSH gene
- It is characterized by lysosomal accumulation of heparin sulfate due to a deficiency of the enzyme heparan N-sulfatase
- A mild speech delay is often one of the first presenting features
- Mental and motor development peak by 3 - 6 years of age, after which intellectual decline usually occurs
- Symptoms include severe behavioral disturbances, hirsutism, slightly coarse facial features, severe sleeping problems, mildly enlarged liver and/or spleen, speech delay, respiratory and ear infections, diarrhea, hernias, seizures, hearing loss, vision impairment, and a wobbly and erratic walk
- The 2022 Sanfilippo syndrome: consensus guidelines for clinical care state early diagnosis is critical to ensure the optimal care for patients
- A diagnosis of MSP IIIA should be considered when characteristic signs and symptoms present alone or in combination
- Confirmation of diagnosis can be made when at least two biochemical or genetic markers of the disorder are present
- Markers of disease include:
 - Increased urine heparin sulfate
 - Decreased heparan-N-sulfatase enzymatic activity via enzymatic analysis using blood leukocytes or cultured fibroblasts is considered the gold standard and first-line for diagnosis
 - Patient has homozygous pathogenic mutations or is heterozygous for two known pathogenic mutations of the SGSH gene
- The primary goal of management is to optimize the quality of life for patients and their families
- Supportive care with orthopedics, physical therapy, stimulants, mood stabilizers, antipsychotics, antianxiety medications, anti-epileptic drugs, and/or melatonin is the current mainstay of therapy

References:

1. Ultragenyx. UX111 (rebisulfogene etispavovec and formerly ABO-102) gene therapy for the potential treatment of Sanfilippo syndrome (MPS IIIA). Available at: <https://www.ultragenyx.com/our-research/pipeline/ux111-for-mps-iiia/>. Accessed on March 20, 2025.
2. Clinicaltrials.gov. Phase I/II/III gene transfer clinical trial of ScAAV9.U1a.hSGSH for mucopolysaccharidosis (MPS) IIIA. Available at: <https://clinicaltrials.gov/study/NCT02716246>. Accessed on March 20, 2025.
3. Biospace. Ultragenyx announces data demonstrating treatment with UX111. 2024 Feb 6. Available at: <https://www.biospace.com/ultragenyx-announces-data-demonstrating-treatment-with-ux111>. Accessed on March 20, 2025.
4. Muschol N, Giugliani R, Jones SA, et al. Sanfilippo syndrome: consensus guidelines for clinical care. Orphanet J Rare Dis. 2022 Oct 27; 17 (391): <https://doi.org/10.1186/s13023-022-02484-6>.
5. National Organization for Rare Disorders. Mucopolysaccharidosis type III. 2024 Jan 23. Available at: <https://rarediseases.org/rare-diseases/mucopolysaccharidosis-type-iii/>. Accessed on March 21, 2025.

Policy History												
#	Date	Change Description										
1.2	Effective Date: 10/01/2026	10/01/2026 - UM medical management system update for MAPPO and BCNA <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>No</td></tr><tr><td>BCN</td><td>No</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	No	BCN	No	MAPPO	Yes	BCNA	Yes
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1.1	Effective Date: 06/11/2026	Annual review of criteria was performed, no changes were made										
1.0	Effective Date: 06/05/2025	Preliminary Drug Review <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>No</td></tr><tr><td>BCN</td><td>No</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	No	BCN	No	MAPPO	No	BCNA	No
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* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.