

Pharmacy Focus:
Fayuvi™ for Sanfilippo Syndrome Type A (MPS Type IIIA)

Key Takeaways[1-3]

Mucopolysaccharidosis Type IIIA (MPS Type IIIA) is an ultra-rare, progressive genetic disorder primarily affecting the brain.

Historically, the standard of care for MPS Type IIIA has focused on managing symptoms and complications through a multidisciplinary team of specialists; there have been no FDA-approved potentially curative or disease-modifying treatments until now.

Fayuvi™ is a new gene therapy for MPS Type IIIA that was approved for pediatric patients with preserved neurodevelopmental function. The estimated cost of Fayuvi is $3,950,000 for the one-time infusion, not including ancillary costs.

Disease Overview[1-5]

Description:

- Mucopolysaccharidosis Type IIIA (MPS Type IIIA), also known as Sanfilippo syndrome, is a rare inherited condition that primarily affects the central nervous system.
- The disease is characterized by rapid developmental regression and progressive cognitive and motor function loss.

Characteristics:

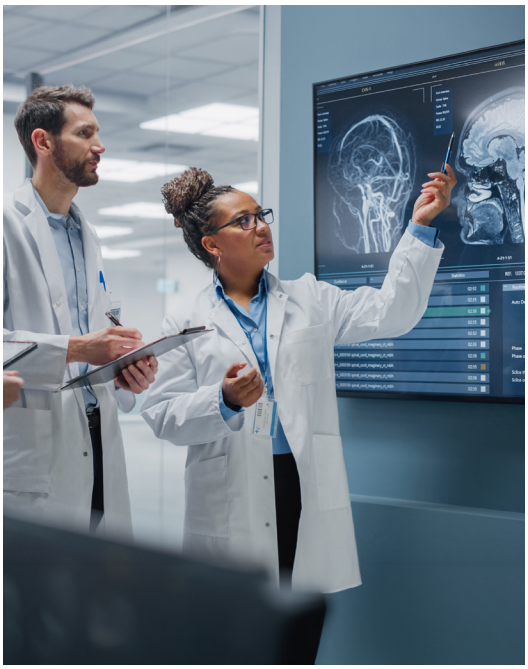
- Disease onset typically occurs in early childhood and appears as developmental delays, hyperactivity, and aggression.
- Symptoms include rapid developmental regression, sleep disturbances, severe behavioral problems, and eventual motor function loss.

Subtypes & Causes:

- There are four subtypes of MPS Type III (IIIA, IIIB, IIIC, IIID).
- On a molecular level, MPS Type IIIA is caused by a deficiency in the heparan N-sulfatase enzyme due to mutations in the *SGSH* gene, resulting in the build-up of heparan sulfate in cells and damage to the central nervous system.

Severity:

- MPS Type IIIA often appears earlier in life and progresses more rapidly than the other subtypes.
- Life expectancy for patients with MPS Type IIIA is about 15 to 20 years.



Occurrence:

- MPS Type III occurs in approximately one in every 70,000 live births worldwide, with the most common types being MPS Types IIIA and IIIB.
- Males and females are affected equally.
- It is estimated that there are between 130 and 150 patients with MPS Type IIIA in the U.S.

Continued...

Treatment[2-5]

- Historically, there have been no disease-modifying treatments for MPS Type IIIA.
- The standard of care has been supportive and focused on managing symptoms and complications through a multidisciplinary team of specialists. Therapies include medications for behavioral symptoms, physical therapy, and interventions for feeding and breathing challenges.
- Fayuvi is the first FDA-approved treatment for MPS Type IIIA. This one-time intravenous gene therapy is estimated to cost $3,950,000 for the drug alone.
- FDA approval of Fayuvi was based on results from the *Transpher A* clinical trial. Treatment with Fayuvi resulted in rapid and sustained reductions in heparan sulfate (HS) in the cerebrospinal fluid, a key biomarker of the disease across all age groups. In patients treated at a young age (≤30 months) who did not already have severe cognitive delay, the therapy showed a preservation of neurocognitive development that remained within the normal range.

Fayuvi™	
Generic Name	Rebisufligene etisparvovec-hopf
Manufacturer	Ultragenyx Pharmaceutical Inc.
Approval	September 17, 2026
Use	Pediatric patients with preserved neurodevelopmental function
How It Works	Delivers a working copy of the deficient gene to help restore the enzyme needed to break down heparan sulfate
Procedure Location	Provider-administered at a Qualified Treatment Center (QTC)
Timeframe	Available through QTCs within 30 to 60 days following approval
Efficacy	In patients treated at a young age (≤30 months), who did not already have severe cognitive delay, the therapy showed a preservation of neurocognitive development that remained within the normal range. A reduction in heparan sulfate levels in the cerebral spinal fluid (CSF) was also demonstrated across all age groups treated.
Estimated Cost	$3,950,000 for the one-time administration
Associated Benefit	Medical

HMConnects™

As part of the HMConnects cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing, and collaborating on the content of current policies, monitoring trends, and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies, and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com.**

References: [1]"Mucopolysaccharidoses," National Organization for Rare Disorders, rarediseases.org/rare-diseases/mucopolysaccharoidoses/, accessed September 21, 2026; [2]"Mucopolysaccharidoses: Clinical Features, Diagnosis, Treatment, and Care Management," Emerging Therapy Solutions, 2025; [3]IPD Analytics. (2026). *UX111 for Mucopolysaccharidosis Type IIIA.*; [4]Ultragenyx Pharmaceutical Inc. (2026, September 17). *Ultragenyx announces approval of FAYUVI™ gene therapy, the first-ever FDA-approved treatment for Sanfilippo syndrome type A (MPS IIIA)* [Press release].; [5]*Fierce Pharma. (2026, September 17). FDA approves Ultragenyx's gene therapy Fayuvi for rare neurodegenerative disorder.*

Products are underwritten or reinsured by HM Life Insurance Company, Pittsburgh PA; HM Life Insurance Company of New York, New York, NY; or Bridge City Insurance Company, Pittsburgh, PA.

This is an informational document only and is not intended to provide legal advice, tax advice or advice on your health plan's content and design. This document is not meant to address federal or other applicable laws for health plans. You should consult with your legal counsel and/or a qualified plan design professional.

HM Insurance Group

800.328.5433 | hmig.com

MTG-3712 (10/26)