

FDA Approves First Gene Therapy for Pediatric Patients with Sanfilippo Syndrome Type A

The U.S. Food and Drug Administration has approved **Fayuvi** (rebisufligene etisparvovec-hopf), the first treatment for pediatric patients with mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo syndrome type A. MPS IIIA is a rare inherited disease that progressively damages the brain and nervous system, causing children to lose cognitive, language and other developmental abilities over time. Until today, treatment was limited to managing symptoms; there was no FDA-approved therapy designed to change the underlying course of the disease.

"The Trump Administration is committed to bringing safe and effective treatments to patients with the most urgent and unmet needs. The approval of **Fayuvi** marks a historic moment for children and families living with MPS IIIA, which is a disease that has, until now, offered no approved treatment to alter its devastating course," said Acting **FDA** Commissioner **Kyle Diamantas, J.D.** "**Gene therapy** holds tremendous promise for rare diseases like Sanfilippo syndrome type A, and this milestone reflects the FDA's commitment to action."

Fayuvi is a one-time, intravenous (given directly into a vein) gene therapy that uses a modified, non-infectious virus called an adeno-associated virus serotype 9 (AAV9) to deliver a working copy of the **SGSH** gene into the patient's cells. This enables the body's cells to produce sulfamidase-the enzyme that is missing or deficient in MPS IIIA -allowing heparan sulfate to be properly broken down in lysosomes and reducing its harmful buildup throughout the body and brain.

"For families living with Sanfilippo syndrome type A, the trajectory of this disease is heartbreaking-children who develop normally in their earliest years facing a relentless regression with no approved treatment to slow it. Parents and clinicians have been waiting far too long for an option," said **Karim Mikhail, B. Pharm., M.S.,** Director of the Center for Biologics Evaluation and Research. "Today's approval of **Fayuvi** is a meaningful step forward-not only for these children and their families, but for the promise of gene therapy to address rare and devastating diseases where the need for safe and effective treatment is the most urgent."

The safety and effectiveness of **Fayuvi** was evaluated in an open-label, single-arm, multicenter clinical study in pediatric patients with MPS IIIA. The study measured mean changes in cognitive scores in patients between the ages of 2 and 5 years. **Fayuvi**-treated patients maintained or improved cognitive function compared to an untreated historical control cohort — a meaningful divergence from the expected natural disease course of plateau and decline during this critical developmental window.

"Achieving meaningful neurodevelopmental benefit through a single intravenous administration represents a significant scientific milestone — demonstrating that systemic AAV9-mediated gene delivery can reach the central nervous system at therapeutically relevant levels in pediatric patients. This approval underscore **OTP's** and **FDA's** commitment to applying rigorous evidentiary standards as the field of gene therapy continues to advance," said **Megha Kaushal, M.D., M.Sc.,** Acting Deputy Director of the Office of Therapeutic Products.

The safety of Fayuvi was evaluated in pediatric patients who received a single intravenous infusion across clinical studies. The most commonly adverse reactions reported in more than 5% of patients were increases in liver enzymes (AST), nausea and vomiting, fever, decreased appetite, decreased white blood cell and platelet counts and increased amylase. Important safety warnings include the risk of thrombotic microangiopathy (TMA). As with other AAV-based gene therapies, there is a potential long-term risk that the inserted genetic material could integrate into the genome and potentially lead to tumor development.

Fayuvi is administered in a healthcare setting equipped to manage infusion reactions. All patients receive corticosteroid treatment beginning one day before the infusion and continuing for a minimum of eight weeks afterward.

Fayuvi was granted Orphan Drug and Fast Track, and Breakthrough Therapy designations. The FDA granted Fayuvi approval to Ultragenyx Pharmaceutical, Inc.

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