Embrace the moments that matter



FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

Not an actual patient or caregiver.

The **first and only treatment** for mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo syndrome type A

WHAT IS FAYUVI™ (rebisufligene etisparvovec-hopf)?

FAYUVI is a prescription gene therapy used to treat the neurological (nervous system) symptoms of mucopolysaccharidosis type IIIA (MPS IIIA, Sanfilippo syndrome type A), in pediatric patients with preserved neurodevelopmental function.

Please see Important Safety Information throughout and full Prescribing Information.



Not an actual patient or caregiver.

MPS IIIA is a rare *genetic* condition

Mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo syndrome type A, is a severe progressive disorder that affects the central nervous system (brain and spinal cord).

Signs and symptoms in children with MPS IIIA can vary widely—from difficulty communicating to having trouble feeding themselves.

Typically, signs and symptoms become noticeable after 2 years of age.

IMPORTANT SAFETY INFORMATION

What is the most important information to know about FAYUVI?

- FAYUVI can **increase certain liver enzyme levels**. Liver function and medical history will be assessed prior to FAYUVI infusion. Patients will receive oral corticosteroid medication before and after infusion with FAYUVI and will undergo regular blood tests to monitor liver function. Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish or if the patient misses a dose of corticosteroid or vomits it up. Do not use any medications, herbal products, or supplements without first confirming with the patient's doctor that they are not harmful to the liver.

Understanding what *causes* MPS IIIA

In MPS IIIA, one gene can change everything

MPS IIIA is caused by a change in a specific gene called ***SGSH***. This change in the *SGSH* gene may result in the cell not making enough of an enzyme called **sulfamidase**.

Without enough working sulfamidase, **heparan sulfate**, a complex sugar in the body, isn't broken down. Instead, it builds up in the cells, which can cause damage.

As a result, many of the **cells in the nervous system** can't work properly. That's why your child experiences signs and symptoms of MPS IIIA that can **progress**.

IMPORTANT SAFETY INFORMATION (continued)

- **Thrombocytopenia (low platelet count that can lead to an increased risk of bruising and bleeding)** was observed typically in the first 2 weeks after FAYUVI infusion. Blood tests will be performed weekly for the first 4 weeks after the FAYUVI infusion then monthly for 6 months. Seek medical attention if unexpected bruising or bleeding occurs.

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The *first* and *only* treatment for MPS IIIA

What is FAYUVI™ (rebisufligene etisparvovec-hopf)?

FAYUVI (pronounced fay-yoo-vee) is a one-time prescription gene therapy infusion that's approved to treat the neurological (nervous system) symptoms of MPS IIIA in pediatric patients with preserved neurodevelopmental function.

The efficacy of FAYUVI was studied in **17 children with MPS IIIA** aged ≤2 years or >2 years with a cognitive development quotient score of ≥60 on the Bayley Scales of Infant and Toddler Development®—Third Edition (Bayley-III) or the Mullen Scales of Early Learning.

- The primary goal of the 2 studies was to see how FAYUVI affected children's development compared with the natural history of MPS IIIA (27 children who did not receive FAYUVI). Skills were measured using Bayley-III cognitive raw score. **Scores range from 0 to 91**, with higher scores indicating higher skill level
- Children had a median duration of follow-up of **4.2 years (range: 2.9 to 7.8 years),** with **14 of 17 children reaching the age of 5 years (60 months)** during the studies

Please speak with your doctor to determine if FAYUVI may be an appropriate treatment for your child.

IMPORTANT SAFETY INFORMATION (continued)

What is the most important information to know about FAYUVI?

- **Thrombotic Microangiopathy (TMA) (decreased platelets and red blood cells, acute kidney injury, and a higher risk of bruising or bleeding)** has been reported with the use of AAV gene therapies. While there have been no cases of TMA associated with FAYUVI in clinical studies, patients should be monitored for signs and symptoms of TMA, with blood tests performed to assess platelet counts during the first 4 weeks after the FAYUVI infusion. Seek immediate medical attention if the patient experiences unexpected bruising or bleeding, seizures, or decreased urine output.

Efficacy in children with MPS IIIA

FAYUVI improved cognitive development scores vs natural history

Children who received FAYUVI scored an **average of 23.5 points higher** from 2 to 5 years of age compared with the similarly aged natural history population.

Average change in Bayley-III cognitive score

FAYUVI treatment (N = 17)	**Natural history** (N = 27)	**FAYUVI vs natural history** (point difference)
+16	-7.6	+23.5

Children who received FAYUVI showed improvements in cognitive skills such as reacting to sensory input, play activities, building with blocks, problem solving, and memory.

FAYUVI helped children **gain or maintain developmental skills** while the natural history group showed a loss of developmental skills.

IMPORTANT SAFETY INFORMATION (continued)

- **Infusion reactions, including hypersensitivity reactions and anaphylaxis,** may occur during and after infusion with FAYUVI. Patients will be monitored during and after FAYUVI infusion. If an infusion-related reaction occurs, the FAYUVI infusion may be paused, and additional medical treatment provided as needed. Symptoms may include low blood pressure, fever, rapid, strong, or irregular heartbeat, nausea, vomiting, chills, or headache. Immediately inform medical staff if the patient experiences these symptoms.

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Designed to address MPS IIIA at the *genetic level*

How does FAYUVI work?

FAYUVI consists of a working copy of the ***SGSH* gene** and a vehicle to deliver that gene. The vehicle is called **adeno-associated virus-9**, or **AAV9**. It is a **noninfectious virus** that was chosen for its ability to target the brain and central nervous system.



Once inside the cell, AAV9 releases the **working copy of the *SGSH* gene**. The gene is designed to help the body produce an enzyme called sulfamidase.



By producing more sulfamidase, the body may be able to **properly break down heparan sulfate**.



Lowering levels of heparan sulfate **helps to reduce its harmful buildup** throughout the body and brain.

IMPORTANT SAFETY INFORMATION (continued)

What is the most important information to know about FAYUVI?

- **Cancer** may occur after AAV gene therapies like FAYUVI. If cancer develops, contact the patient's doctor and Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.

Observations in the body from treatment with FAYUVI

FAYUVI showed an effect on levels of heparan sulfate in cerebrospinal fluid (CSF), a biomarker of MPS IIIA

Compared with the start of the study*:

1 month after FAYUVI

59%

average reduction of heparan sulfate in CSF (n = 16)

24 months after FAYUVI

57%

average reduction of heparan sulfate in CSF (n = 16)

*Average reductions in heparan sulfate in CSF were also captured at 6 months (74%) and 12 months (67%).

94% of children (15 out of 16) maintained a ≥50% reduction in levels of heparan sulfate in CSF after 24 months.

IMPORTANT SAFETY INFORMATION (continued)

Are there any considerations for vaccination schedules and FAYUVI?

- Vaccines should be avoided 30 days before the FAYUVI infusion, before starting corticosteroids, and while on corticosteroid therapy.

What is important to know about pregnancy?

- FAYUVI should not be given during pregnancy. If a patient can become pregnant, a pregnancy test must show that the patient is not pregnant before treatment with FAYUVI.

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Safety observed in FAYUVI clinical trials

The **safety of FAYUVI** was studied in 33 children with MPS IIIA in 3 clinical studies, where 27 patients received the recommended dose.

The most common (≥5%) side effects among children who received the recommended dose of FAYUVI (N = 27) in the studies are shown in the table below.

Adverse reaction	Number of children who experienced side effect
Increased liver enzymes*	23 (85%)
Vomiting	18 (67%)
Abnormal behavior*	15 (56%)
Diarrhea	13 (48%)
Fever	11 (41%)
Decreased white blood cell count*	8 (30%)
Cushingoid features†	8 (30%)
Decreased appetite	6 (22%)
Decreased platelet‡ count*	5 (19%)
Low red blood cell levels	5 (19%)
Constipation	4 (15%)
Nausea	3 (11%)
Increased digestive enzyme	3 (11%)
Increased alkaline phosphatase enzymes	3 (11%)
Seizure*	3 (11%)
Enlarged liver	3 (11%)
Muscle stiffness or spasms	2 (7%)
Low potassium levels	2 (7%)
Difficulty walking or changes in balance	2 (7%)
Low adrenal hormone levels or adrenal gland problems	2 (7%)

*This side effect may include several related conditions.
†Changes caused by high steroid levels, such as rounded face, weight gain, or increased body fat.
‡A type of blood cell that helps with clotting.

Safety observed in FAYUVI clinical trials

Increased liver enzymes



In clinical trials, 85% of children (23 out of 27) receiving the recommended dose of FAYUVI experienced side effects associated with liver enzyme elevations. These were mostly mild or moderate except for one severe case, which got better on its own within 8 days. Increased liver enzymes most commonly first appeared within 2 weeks of treatment with FAYUVI (range: 2 days to 30.3 months). All liver enzyme elevations resolved within 6 days to 40.6 months of onset except for 2 children who still had slightly elevated enzyme levels.

Low white blood cells



In clinical trials, 22% of children (6 out of 27) receiving the recommended dose of FAYUVI experienced neutropenia, or low levels of a type of white blood cell called neutrophils. One child experienced severe neutropenia, and the remaining events were mild or moderate.

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Navigate your child's path to treatment

Once enrolled, UltraCare can help you throughout your child's treatment journey

UltraCare®

Understand your **insurance coverage**.

Help determine your eligibility for **financial assistance and travel assistance support**.

Get to know the steps **before and after treatment** with FAYUVI.

Our UltraCare Gene Therapy Guides are experienced professionals who can help you and your family along the gene therapy journey. They can directly answer your questions regarding treatment access, help you navigate logistical challenges you may encounter while preparing for treatment, and provide post-infusion educational support.

Learn more at **UltraCareSupport.com**.

IMPORTANT SAFETY INFORMATION (continued)

Are there any precautions that need to be considered when handling a patient's bodily fluids or waste?

- Temporary shedding of the vector that delivers FAYUVI may be found in patient bodily fluids or waste. Family members and caregivers should use proper hand hygiene, such as hand washing when coming into direct contact with patient bodily fluids or waste. To properly dispose of materials that may be contaminated with the patient's bodily fluids or waste, place them in a sealed bag before discarding them in the regular trash. Precautions should be followed for 3 months after FAYUVI infusion.

Questions to ask your child's doctor

Consider these conversation starters for your child's next appointment

- Is FAYUVI prescription gene therapy an appropriate treatment option for my child?
- What kind of efficacy may we expect with FAYUVI?
- What risks do we need to consider with FAYUVI?
- What should we know about the infusion process, both before and after undergoing treatment?

IMPORTANT SAFETY INFORMATION (continued)

What are the possible or likely side effects of FAYUVI?

The most common side effects that occurred in patients treated with FAYUVI were:

- Increased liver enzymes
- Vomiting
- Abnormal behavior
- Diarrhea
- Fever
- Decreased white blood cell count
- Cushingoid features (changes caused by high steroid levels such as rounded face, weight gain, or increased body fat)
- Decreased appetite
- Decreased platelet count
- Low red blood cell levels
- Constipation
- Nausea
- Increased digestive enzyme
- Increased alkaline phosphatase enzymes
- Seizure
- Enlarged liver
- Muscle stiffness or spasms
- Low potassium levels
- Difficulty walking or changes in balance
- Low adrenal hormone levels or adrenal gland problems

These are not all the possible side effects of FAYUVI. Contact the patient's doctor right away if the patient experiences any side effects.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.

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If your child has MPS IIIA, *ask their doctor* about FAYUVI

Learn more at FAYUVI.com.

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Notes:

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