FAYUVI™
rebisufligene
etisparvovec-hopf
suspension for IV infusion

Your guide to important *moments* along your child's treatment journey

Prepare for your child's experience with FAYUVI prescription gene therapy 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 pages 12-13 and full Prescribing Information for Important Safety Information.

Know what to expect at every step

Once a treatment decision has been made, you and your child's doctor will develop a plan that best meets their needs. Continue reading to learn key details about each step.

Before Treatment

STEP 1

LAB TESTS

Confirmatory and screening tests will be ordered by your child's doctor. These may include genetic tests, baseline blood tests, a test for adeno-associated virus-9 (AAV9) antibodies, and other assessments.

MEDICATIONS AND BLOOD TESTS

Your child's doctor will prescribe corticosteroids to help control the body's immune response and reduce the risk of side effects of treatment with FAYUVI. Your child's doctor will also monitor your child's liver function while taking the medications.

VACCINES

Vaccines should be avoided for 30 days prior to FAYUVI, and before starting and while on corticosteroids. Discuss your child's vaccination schedule with their doctor for more specific guidance.

Treatment Day

STEP 2

INFUSION

The infusion lasts about 1 hour.

Talk with your care team about how long you may expect to spend at the Qualified Treatment Center (QTC) both before and after your infusion.

Your child's FAYUVI infusion must be administered at a Qualified Treatment Center (QTC).

UltraCare® Patient Services for Gene Therapy will work with enrolled individuals and families to identify a potential QTC for the infusion.

After Treatment

STEP 3

MEDICATIONS AND BLOOD TESTS

Continue to follow instructions from your child's doctor, including giving your child **the prescribed daily oral corticosteroid** following their FAYUVI infusion and taking your child in for lab work. Be sure to ask your care team if your child will need any additional follow-up tests.

DISPOSAL OF WASTE

Temporary shedding of the vector that delivers FAYUVI may be found in your child's bodily fluids and waste. Please follow precautions for 3 months after infusion. Seal and dispose of materials that contain your child's bodily waste (such as diapers) with your regular trash. Always wash your hands after coming into contact with your child's bodily fluids or waste.

STEP 4

ONGOING MONITORING

Monitoring will be a collaborative effort between you and your child's care team. Watch for any side effects that your child may experience and inform your child's doctor immediately if they occur.

As you move forward on your child's FAYUVI treatment journey, **contact their care team with any questions.**

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

Prepare for your child's treatment

Your child's doctor will guide you on **specific tests** and **medications** needed leading up to treatment day.

STEP 1

Tests

Before administering FAYUVI, your child's doctor will:

Check their liver function due to the risk of elevated liver enzymes.

Perform a platelet count, as well as check to see how long it takes blood clots to form.

Test for any conditions that may increase the risks associated with corticosteroid therapy.

Perform a blood test to check for preexisting antibodies to adeno-associated virus-9 (AAV9) vector, the vehicle used to deliver FAYUVI into cells.

- FAYUVI is not recommended for use in individuals who have elevated levels of AAV9 antibodies
- AAV9 is a common virus among the general population, and some people may have naturally developed antibodies. If you have detectable antibodies, it simply means that you were exposed to AAV9 at some point in your life

Medications

Corticosteroids are given with the goal of reducing potential side effects of treatment with FAYUVI and to help control the body's immune response.

Starting **1 day** before treatment, give your child the prescribed daily **oral corticosteroid**.

- **Follow the dose and duration** of corticosteroid prescribed by your child's doctor

All medications should be taken as prescribed by your child's doctor.

Questions to ask your child's care team

Before Treatment

1. Are there any additional recommendations for how to prepare my child for treatment?
2. Are there any special precautions we need to follow with corticosteroids?
3. What can we expect at the treatment center?
4. How should we share information with the doctor if my child becomes ill prior to infusion day?

Write down additional questions you have as your child prepares for treatment.

Before treatment day, be sure to discuss **all medications** that your child is taking. As an extra step of precaution, your child's doctor will check for interactions between FAYUVI and any medications that your child may be taking (including prescription and over-the-counter medications as well as vitamins and herbal supplements).

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

On your child's *treatment day*

For the **one-time** FAYUVI infusion, talk with your care team about how long you may expect to spend at the **Qualified Treatment Center**.

STEP 2

Infusion

The FAYUVI treatment will be infused slowly into a vein (intravenously) for **about 1 hour** at a Qualified Treatment Center. This is a healthcare institution with care teams that specialize in gene therapy.

Your care team will closely monitor your child during their infusion for any infusion-related reactions or side effects. This means they will closely observe your child to look for anything unusual. If an infusion reaction occurs, your child's care team will slow or pause the treatment and care will be provided as appropriate. Possible signs and symptoms of infusion reactions may include **low blood pressure, fever, rapid, strong, or irregular heartbeat, nausea, vomiting, chills, or headache**.

After infusion

Once the FAYUVI infusion is complete, your child's care team will tell you about potential side effects to look out for. At home, you should continue to monitor your child for any unusual signs and symptoms, and notify your child's doctor immediately if they occur.

Find support with UltraCare

UltraCare from Ultragenyx is committed to supporting enrolled individuals and families throughout the FAYUVI treatment journey. UltraCare professionals can help you understand insurance coverage, determine your eligibility, access resources, and provide educational information. Work with your child's doctor to enroll in UltraCare Patient Services for Gene Therapy by completing the enrollment form together and then having your child's doctor submit it to UltraCare.

Learn more at UltraCareSupport.com

Questions to ask your child's care team

Treatment Day

1. What should I pack for my child to be comfortable during treatment?
2. How many family members may accompany my child on infusion day?
3. How will you manage any side effects that may happen during the infusion?

Write down additional questions you have as your child continues their treatment journey.

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

After treatment

Plan for your child to have regular **follow-up appointments** with their doctor to track their progress.

STEP 3

Daily medication

After the infusion, continue to follow instructions from your child's doctor, including **the prescribed daily oral corticosteroid** for 8 weeks followed by a tapering period of at least 4 weeks.

Testing schedule

Lab work is suggested for at least 6 months after the infusion. Confirm the testing schedule and medications with your child's doctor. Based on lab results, additional lab work may be required.

Liver function testing will take place weekly for the **first 2 weeks,** then **every other week** until 2 weeks after completing corticosteroids, or as directed by the doctor.

Platelet count testing will take place weekly for the **first 4 weeks**, then monthly for 6 months after the infusion, and as needed after that.

For 3 months after treatment

Even though the vector that delivers the gene therapy does not cause disease, it passes through your child's body after it breaks down and is eliminated in their urine and feces (called vector shedding).

Seal and dispose of any materials that may contain your child's bodily waste (e.g., diapers) with your regular trash.

Always wash your hands after you come into direct contact with your child's bodily fluids or waste.

Questions to ask your child's care team

After Treatment

1. What possible side effects should I look out for, and who should I inform if they occur?
2. Have the suggested blood tests already been ordered for my child, and where will these orders be sent?
3. After treatment, how long will my child take corticosteroids?
4. How should we share information with the doctor or emergency team if my child becomes ill with something else?
5. What considerations should I have around vector shedding?

Write down additional questions you have as your child continues their treatment journey.

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

Ongoing *monitoring*

Monitor your child for any **possible side effects** and report them to your child's doctor immediately.



STEP 4

Monitoring your child after the infusion is a team effort between you and your child's care team. Your child's doctor will educate you on some of the possible side effects to be aware of.
Contact your child's doctor immediately if any of the following occur:



Your child experiences any infusion-related reactions, including hypersensitivity and anaphylaxis.



Your child urinates less than normal.



Your child misses a dose of corticosteroids or vomits the dose.



Your child has unusual or unexplained bleeding.



You see bruises without you or your child knowing how they got there.



Your child has yellowish discoloration of the skin and/or the whites of the eyes.



Your child experiences a seizure.



You see any other concerning side effects.

In AAV gene therapies like FAYUVI, cancer may occur. If cancer develops, contact your child's doctor and Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.

Questions to ask your child's care team

1. After the FAYUVI infusion, will my child's care transition to a different healthcare provider for long-term monitoring?
2. Will other family members need to take special precautions around my child who has received the infusion? If so, for how long?
3. What does aftercare look like beyond the first 6 months of follow-up?

Write down additional questions you have as you monitor your child after the infusion.

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

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.
- 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.
- 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.
- 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.
- 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.

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.

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.

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.

Please see full Prescribing Information for FAYUVI.

FAYUVI™
rebisufligene
etisparvovec-hopf
suspension for IV infusion

Notes

Please see pages 12-13 and full Prescribing Information for Important Safety Information.

FAYUVI™
rebisufligene
etisparvovec-hopf
suspension for IV infusion

Scan code for more information about ***study results*** seen with FAYUVI prescription gene therapy, or visit **FAYUVI.com**.

Please see pages 12-13 and full Prescribing Information for Important Safety Information.



FAYUVI™
rebisufligene etisparvovec-hopf
suspension for IV infusion

ultragenyx

© 2026 Ultragenyx Pharmaceutical Inc. All rights reserved.
FAYUVI is a trademark and UltraCare is a registered trademark of Ultragenyx Pharmaceutical Inc. MRCP-UX111-00064 10/2026