

YOUR GUIDE TO TREATMENT WITH GENGLYCOS

Prepare for your experience with GENGLYCOS, the first and only FDA-approved gene therapy used along with nutritional management to help reduce daily cornstarch intake in individuals 8 years and older with glycogen storage disease type Ia (GSDIa).



GET STARTED

WHAT IS GENGLYCOS (pariglasgene brecaparvovec-opnr)?

GENGLYCOS is a prescription gene therapy used along with nutritional management to help reduce the amount of cornstarch taken daily in adults and children 8 years and older with Glycogen Storage Disease type Ia (GSDIa).

This use is approved under accelerated approval based on clinical study results that measured reduction in daily cornstarch intake. Verification of a clinical benefit may be needed for continued approval of GENGLYCOS.

IMPORTANT SAFETY INFORMATION

Do not use GENGLYCOS if you have severe liver scarring (fibrosis or cirrhosis).

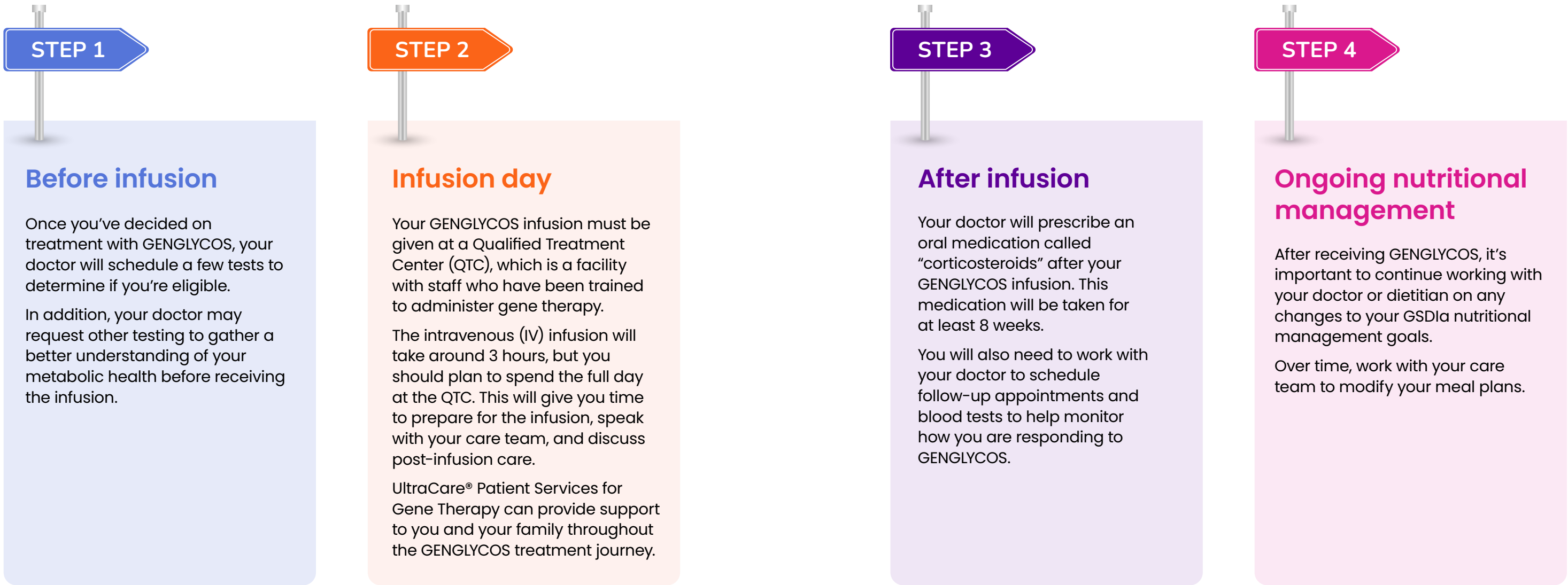
Please see pages 12-13 and full [Prescribing Information](#) for Important Safety Information.



genglycos[™]
pariglasgene
breCAParvovec-opnr
suspension for IV infusion

Know what to expect with GENGLYCOS

Talk with your doctor about each step in the GENGLYCOS treatment process. It's important to understand what's involved, so you can make an informed decision and develop a plan that best meets your needs.



STEP 1

Before infusion

Once you decide on treatment with GENGLYCOS, your doctor will schedule a few tests to determine if you're eligible.



Your doctor will perform a blood test to check for preexisting antibodies to adeno-associated virus serotype 8 (AAV8), the vector used to deliver GENGLYCOS into your liver cells. GENGLYCOS is not for use in individuals who have detectable AAV8 antibodies.

- AAV8 is a common virus among the general population, with some studies estimating that approximately 20% to 30% of people may have naturally developed antibodies. If you have detectable AAV8 antibodies, it simply means that you were exposed to AAV8 at some point in your life



Your doctor will check your liver function before administering GENGLYCOS. Individuals with certain forms of liver damage, such as severe fibrosis or cirrhosis, are not eligible for treatment.



Your doctor will check your adrenal glands and certain hormone levels before administering GENGLYCOS.



Your doctor may request other tests to gather a better understanding of your metabolic health before administering GENGLYCOS.



Vaccines should be avoided 1 month prior to GENGLYCOS administration.

Find support with UltraCare

UltraCare from Ultragenyx is committed to supporting you and your family throughout the GENGLYCOS treatment journey. UltraCare professionals can help you understand insurance coverage, determine your eligibility, access resources, and more.

Work with your doctor to enroll in UltraCare Patient Services for Gene Therapy by completing the enrollment form together and then having your doctor submit it to UltraCare.

Learn more at [UltraCareSupport.com](https://ultracaresupport.com)



Questions to ask your care team

- 1. What should I expect with GENGLYCOS?
- 2. Why do I need to take tests for eligibility?
- 3. Can I retest to become eligible later?
- 4. If I decide to move forward with GENGLYCOS, how might it play a role in my GSDIa management goals?

Write down additional questions you have as you continue your treatment journey.





Infusion day

Once your doctor determines that you’re eligible, it’s time for the one-time IV infusion of GENGLYCOS.

GENGLYCOS must be administered at a Qualified Treatment Center (QTC). This is a healthcare institution with care teams that specialize in gene therapy.



Your care team will walk you through the process and give you acetaminophen and nondrowsy antihistamines 30 to 60 minutes before the infusion.



GENGLYCOS will be infused slowly into a vein (intravenously) for around 3 hours. As with any medical procedure, your care team will closely monitor you during your infusion for any side effects. If an infusion reaction occurs, your doctor may pause the treatment and care will be provided as appropriate. **Possible signs and symptoms of infusion reactions may include hives, flushing, low blood pressure, wheezing and coughing, shortness of breath, chest tightness, nausea, vomiting, headache, abdominal pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure.**



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

Questions to ask your care team

- 1. Do I have to travel for treatment with GENGLYCOS?
- 2. Can I bring family members or friends to the QTC?
- 3. What should I expect on infusion day?
- 4. How will any potential infusion reactions or side effects be managed?

Write down additional questions you have as you continue your treatment journey.



After infusion

After your GENGLYCOS infusion, your doctor will tell you about the medications and tests you may need to help monitor how your body is adjusting to treatment and for potential side effects.



Your doctor will prescribe a daily corticosteroid starting 2 weeks after infusion and lasting at least 8 weeks.

- Corticosteroids are given as immunosuppressive therapy with the goal of reducing potential side effects of treatment with GENGLYCOS
- Increased liver enzymes are a common immune system response and may be seen as your body adjusts to the gene therapy



Your doctor will perform regular liver function tests for the first few months after treatment:

- Weekly while you’re taking corticosteroids and for 2 weeks after you finish
- Every 2 to 4 weeks until month 6, and as needed after month 6



Your doctor will perform regular adrenal gland function tests:

- Before and after treatment with GENGLYCOS, and when requested by your doctor

Questions to ask your care team

1. Will my doctor provide me with an aftercare plan I can follow?
2. After the infusion, how long will I take corticosteroids, and how will my doctor make decisions about these medications?
3. Are there any side effects I should look out for following treatment with GENGLYCOS?
4. If I get sick or vomit in the weeks following the infusion, what should I do?

Write down additional questions you have as you continue your treatment journey.



Ongoing nutritional management

Continue following up with your care team to create a GSDIa nutritional management plan that fits your unique needs.



As your body adjusts to treatment with GENGLYCOS, your doctor may need to make changes to meet your nutritional goals, such as:

- Adjusting your daily cornstarch intake and/or frequency
- Making modifications to your meal plan
- Changing the vitamin or mineral supplements that you take



Continue monitoring your blood glucose levels via finger sticks and/or a continuous glucose monitor.

- Regular monitoring helps you to understand your body's response to adjustments after GENGLYCOS
- Your doctor may recommend certain blood tests to assess your overall metabolic health

Questions to ask your care team

1. Who should I work with to manage my GSDIa after infusion with GENGLYCOS?
2. After the GENGLYCOS infusion, how might my nutritional needs change?
3. What kinds of changes might I expect to see to my diet following treatment with GENGLYCOS?
4. How might GENGLYCOS impact my daily activities (e.g., will I still be able to travel or exercise)?

Write down additional questions you have as you continue your treatment journey.

IMPORTANT SAFETY INFORMATION

Do not use GENGLYCOS if you have severe liver scarring (fibrosis or cirrhosis).



What is the most important information I should know about GENGLYCOS?

- Allergic and infusion reactions, including anaphylaxis, occurred with GENGLYCOS treatment. Some of these reactions have been severe. You will be closely monitored during and after infusion for signs and symptoms including hives, flushing, low blood pressure, wheezing, trouble breathing, chest tightness, nausea, vomiting, headache, stomach pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure. If you think you're having a reaction to the infusion, contact your healthcare provider immediately.
- GENGLYCOS can cause certain liver enzymes to increase. You will receive oral corticosteroids with or without other medications after your infusion and will receive regular blood tests to monitor your liver function. Contact your healthcare provider right away if you experience unusual tiredness, yellowing of your skin and/or eyes, dark urine, nausea, vomiting, or pain in the upper right side of your stomach area, or if you miss a dose of your corticosteroid or vomit after taking it.
- Do not take any medicines, including herbal products or nutritional supplements, before confirming with your healthcare provider that they are not harmful to the liver.
- Talk to your healthcare provider about the possible risks of drinking alcohol following GENGLYCOS infusion.
- Problems with adrenal glands, including serious events, have occurred in patients receiving GENGLYCOS during corticosteroid treatment. Signs of adrenal gland problems can include unusual tiredness, weakness, loss of appetite, nausea, vomiting, low blood pressure, low sodium levels in the blood, and low blood sugar. A severe form called adrenal crisis can cause very low blood pressure, sudden severe stomach pain, or loss of consciousness. You will be monitored for signs and symptoms of adrenal gland problems. Do not stop taking your corticosteroid suddenly—follow your provider's instructions.
- There is a theoretical risk of tumor growth with gene therapies like GENGLYCOS; no cases have been reported in GENGLYCOS clinical studies. Contact your healthcare provider and Ultragenyx Pharmaceutical Inc. at 1-888-756-8657 if a tumor develops.



Are there any considerations for vaccination schedules and GENGLYCOS?

- Talk to your healthcare provider to determine if your vaccination schedule needs to be adjusted to accommodate corticosteroid and other medication administration prior to and after GENGLYCOS infusion.
- Vaccines should be avoided 1 month prior to GENGLYCOS administration.



What do I need to know about pregnancy and breastfeeding with GENGLYCOS?

- It is not known if GENGLYCOS can harm your baby or affect your ability to become pregnant. GENGLYCOS should not be used during pregnancy.
- It is not known if GENGLYCOS passes into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take GENGLYCOS.



What do I need to know about contraception and sperm donation with GENGLYCOS?

- Women of childbearing potential should use an effective method of contraception for at least 12 months after administration of GENGLYCOS.
- For 6 months after GENGLYCOS administration, men should not donate sperm, and men capable of fathering a child and their female partner should avoid pregnancy and use an effective form of birth control.



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

- Small amounts of GENGLYCOS can travel through the patient's blood and be temporarily passed through urine, stool, and saliva. Talk to your healthcare provider about proper hygiene when handling patient body waste. Precautions should be followed for 3 months after GENGLYCOS infusion.



What are the possible or likely side effects of GENGLYCOS?

Seven serious side effects were observed in a GENGLYCOS study, including anaphylaxis/infusion reaction (2), adrenal gland problems (2), high lactate level (2), and low blood sugar (1).

The most common side effects that occurred in individuals treated with GENGLYCOS in a study were:

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| • Increased liver enzymes | • Constipation |
| • Nausea | • High blood sugar |
| • Headache | • Acne or acne-like skin rash |
| • High triglycerides (high levels of a type of fat in the blood) | • Cushingoid features (changes caused by high steroid levels such as rounded face, weight gain, or increased body fat) |
| • Low adrenal hormone levels or adrenal gland problems | • Anaphylaxis (severe allergic reaction) |



These are not all the possible side effects of GENGLYCOS. Talk to your healthcare provider about any side effects that bother you or don't go away.

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.



Notes

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Scan the QR code to learn more about
GENGLYCOS, including efficacy and safety
information, at **GENGLYCOS.com**.

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

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