genglycos™
pariglasgene
brecaparvovec-opnr
suspension for IV infusion

The first and only FDA-approved therapy
for GSDIa (glycogen storage disease type Ia)

Treatment with GENGLYCOS

Indication

GENGLYCOS (pariglasgene brecaparvovec-opnr) is indicated to reduce daily cornstarch intake as an adjunct to nutritional management in adult and pediatric patients 8 years of age and older with glycogen storage disease type Ia (GSDIa).

This indication is approved under accelerated approval based on reduction in daily cornstarch intake. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).

Important Safety Information

CONTRAINDICATIONS

GENGLYCOS is contraindicated in patients with known severe hepatic fibrosis or cirrhosis.

WARNINGS AND PRECAUTIONS

Hypersensitivity and Infusion Reactions (IRs)

- Hypersensitivity reactions including anaphylaxis and IRs have occurred with GENGLYCOS treatment. Severe reactions have been reported. Monitor for signs and symptoms of hypersensitivity and IRs, including urticaria, flushing, hypotension, bronchospasm, dyspnea, chest tightness, nausea, vomiting, headache, abdominal pain, lightheadedness, flu-like symptoms, shivering, rash, and hypertension.

Please see additional Important Safety Information throughout and the full Prescribing Information.

Getting started with GENGLYCOS

GENGLYCOS is administered as a single-dose intravenous infusion. The recommended dose of GENGLYCOS is 1.0 x 10^{13} genome copies per kilogram (gc/kg) body weight. GENGLYCOS is provided as a customized kit based on body weight.

Scan or click for Recommended Dosing in Prescribing Information.

The following tests and assessments should be performed prior to treatment with GENGLYCOS

Select patients without detectable anti-AAV8 antibodies for treatment with GENGLYCOS. Do not administer GENGLYCOS to patients with a detected result for antibodies to AAV8[a]

Assess adrenal function (perform clinical examination and laboratory testing including morning serum cortisol and ACTH)

Assess liver function (perform clinical examination and laboratory testing including ALT, AST, and total bilirubin)

[a]An FDA authorized test for detection of anti-AAV8 total binding antibodies is not currently available. For information regarding anti-AAV8 antibody testing, contact Ultragenyx Pharmaceutical Inc. at 1-888-756-8657. Currently available tests may vary in accuracy and design.

AAV8, adeno-associated virus serotype 8; ACTH, adrenocorticotropic hormone; ALT, alanine aminotransferase; AST, aspartate aminotransferase; FDA, US Food and Drug Administration.

Important Safety Information (Cont.)

WARNINGS AND PRECAUTIONS (CONT.)

Hypersensitivity and Infusion Reactions (IRs)(cont.)

- Premedicate with acetaminophen and non-sedating antihistamines and administer GENGLYCOS according to recommended infusion rates. Monitor patients during and after completion of GENGLYCOS infusion as clinically indicated. If anaphylaxis or severe IR occurs, pause GENGLYCOS infusion immediately and initiate medical treatment as clinically indicated, monitoring as needed. For mild to moderate IRs, consider slowing or temporarily interrupting the infusion, and administer symptomatic treatment as clinically indicated. The infusion may be restarted at half the prior rate upon resolution of symptoms.

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Timing of other medications

Timing of other medications before and after GENGLYCOS infusion

1 month before infusion:	**Finish administering any vaccinations** Vaccines should be avoided 1 month prior to GENGLYCOS treatment.
30 to 60 minutes before infusion:	**Administer premedication** To help reduce the risk of hypersensitivity and infusion reactions, premedicate with acetaminophen and non-sedating antihistamines 30 to 60 minutes prior to GENGLYCOS infusion.
2 weeks post-infusion:	**Start oral corticosteroids** To mitigate hepatic reactions, administer a course of oral corticosteroids (prednisone or equivalent) starting 2 weeks after GENGLYCOS treatment, and continue for a minimum of 8 weeks.

Timing and dosage regimen of corticosteroid after GENGLYCOS administration

	Corticosteroid (Prednisone or Equivalent) Dosage
Starting dose	For patients weighing: • Less than 60 kg: 1.0 mg/kg/day for 4 weeks • 60 kg or more: 60 mg/day for 4 weeks
Tapering gradually	• Taper corticosteroid slowly over a minimum of 4 weeks. Refer to page 9 for information on monitoring hepatic enzymes during and after the corticosteroid taper and assessing adrenal function after corticosteroid regimen.

Important Safety Information (Cont.)

WARNINGS AND PRECAUTIONS (CONT.)

Hypersensitivity and Infusion Reactions (IRs) (cont.)

- Medical support measures, including cardiopulmonary resuscitation equipment and medications for the treatment of anaphylaxis (e.g., epinephrine, antihistamines, corticosteroids), should be available during GENGLYCOS administration.

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Supply, storage, and handling



GENGLYCOS is supplied as a sterile, clear to translucent suspension that may contain small white to off-white particles

GENGLYCOS is for intravenous infusion and comes in 8 mL vials, with an extractable volume of 2 mL

GENGLYCOS contains 3.0 x 10^{13} genome copies per mL

GENGLYCOS is provided as a kit to meet the dosing requirements for each patient. Each kit contains 3 to 15 vials of GENGLYCOS depending on the patient's weight-based dose

Storing GENGLYCOS

GENGLYCOS is shipped and stored frozen at ≤ -60 °C (-76 °F). Store GENGLYCOS upright at ≤ -60 °C (-76 °F) in the original carton until ready to use. Protect GENGLYCOS from light

Vials should not be thawed or punctured until immediately prior to use. Vials should be thawed for no more than 30 minutes. Once drawn from the vial and diluted into an infusion bag, GENGLYCOS should be administered immediately. Do not refreeze

If necessary, an intact vial (stopper not yet punctured) that has been thawed can be stored refrigerated 2 °C to 8 °C (36 °F to 46 °F) for up to 12 days, upright and protected from light (eg, in the original carton).

If necessary, diluted GENGLYCOS can be stored:

- At room temperature (20 °C to 25 °C [68 °F to 77 °F]) for up to 24 hours, including preparation and infusion times
- Refrigerated (2 °C to 8 °C [36 °F to 46 °F]) and administered within 48 hours, including preparation and infusion times. Do not freeze the solution

In case of spills of GENGLYCOS, work surfaces should be decontaminated, and materials should be removed according to local guidelines. Follow local guidelines on handling and disposal of biological waste

Important Safety Information (Cont.)

WARNINGS AND PRECAUTIONS (CONT.)

Hepatotoxicity

- Immune-mediated hepatotoxicity, with elevated alanine aminotransferase (ALT) and/or aspartate aminotransferase (AST) levels, has occurred with GENGLYCOS. Avoid use in patients with preexisting hepatic impairment or acute hepatic viral infection.

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suspension for IV infusion

Recommended dose

The recommended dose of GENGLYCOS is based on patient body weight

Patient Body Weight Range (kg)	Total Dose Volume[a] (mL)	Number of GENGLYCOS Vials
15 to <21	6	3
21 to <27	8	4
27 to <33	10	5
33 to <39	12	6
39 to <45	14	7
45 to <51	16	8
51 to <57	18	9
57 to <63	20	10
63 to <69	22	11
69 to <75	24	12
75 to <81	26	13
81 to <87	28	14
≥87[b]	30	15

[a]Dose volume is rounded up or down to the nearest 2 mL.
[b]Patients weighing >87 kg should be dosed at the 87 kg weight.

Important Safety Information (Cont.)

WARNINGS AND PRECAUTIONS (CONT.)

Hepatotoxicity (cont.)

- Prior to GENGLYCOS infusion, evaluate liver-related medical history and assess liver function by clinical examination and laboratory testing. Advise patients to immediately report signs and symptoms of hepatotoxicity, including fatigue, jaundice, dark urine, nausea, vomiting, and right upper quadrant pain. Administer corticosteroids to all patients after GENGLYCOS infusion in order to mitigate hepatic reactions. Elevated transaminases may require adjustment of the corticosteroid treatment regimen, including increased dose or prolongation of the corticosteroid taper.
- Monitor transaminase levels for the first 6 months after GENGLYCOS administration. Continue to monitor transaminases in all patients who develop transaminase elevations, until transaminases return to baseline or as clinically indicated.

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suspension for IV infusion

Preparing GENGLYCOS for infusion

General preparation precautions

- Dilute and prepare GENGLYCOS for intravenous infusion using aseptic technique
- Verify the required dose of GENGLYCOS based on patient's body weight, and confirm the kit contains enough vials to prepare the GENGLYCOS infusion for the patient
- Vials should not be thawed or punctured until immediately prior to use. If the patient is not expected to be ready for same-day administration, store GENGLYCOS in a manner consistent with the recommended storage and handling

Frozen GENGLYCOS vials must be thawed and inspected prior to preparation

- Remove and thaw vials at room temperature 20 °C to 25 °C (68 °F to 77 °F) for no more than 30 minutes
- Once thawed, gently swirl the vials for 5 to 10 seconds to ensure even mixing. Do not shake. Do not refreeze
- Visually inspect the thawed vials. GENGLYCOS is a clear to translucent suspension that may contain small white to off-white particles. Do not use the vial if the solution is discolored or contains matter other than small white to off-white particles

Important Safety Information (cont.)

WARNINGS AND PRECAUTIONS (CONT.)

Adrenal Insufficiency

- Adrenal insufficiency, including serious events, has been reported in patients receiving GENGLYCOS during corticosteroid use and tapering.
- Signs and symptoms of adrenal insufficiency include fatigue, weakness, anorexia, nausea, vomiting, hypotension, hyponatremia, and hypoglycemia. Adrenal crisis may present as severe hypotension, acute abdominal pain, or loss of consciousness.
- Monitor patients for signs and symptoms of adrenal insufficiency and adrenal crisis after GENGLYCOS administration during and after corticosteroid therapy and tapering. Taper corticosteroid therapy gradually. Do not abruptly discontinue corticosteroid therapy.

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suspension for IV infusion

Preparing GENGLYCOS for infusion (cont.)

The total GENGLYCOS dose must be diluted in a 0.9% sodium chloride infusion bag prior to administration

- Withdraw 2 mL of GENGLYCOS from each vial using a polypropylene syringe and 18-gauge needle. Discard any unused portion left in the vials
  - For doses requiring multiple vials, change needles at least every third vial
- Remove and discard a volume of 0.9% sodium chloride from either a 100 mL infusion bag (for patients weighing 15 – 39 kg) or a 250 mL infusion bag (for patients weighing ≥40 kg) equal to the amount of GENGLYCOS that will be added to the bag. Use a polyvinyl chloride or polypropylene/polyethylene infusion bag. The volume to be withdrawn will vary based on the patient's body weight
- Add the contents of the syringes through a sterile 0.2-micron (µm) polyethersulfone (PES) or equivalent syringe filter into the infusion bag to bring the volume back up to either 100 mL (for patients weighing 15 – 39 kg) or 250 mL (for patients weighing ≥40 kg). Do not add GENGLYCOS into the airspace of the infusion bag to avoid foaming
- Gently invert the infusion bag 4 times to ensure adequate mixing. Do not shake the infusion bag to avoid foaming
- Connect the infusion bag to an infusion set equipped with a 0.2-µm filter and prime the tubing with diluted GENGLYCOS solution up to the hub of the catheter
- Diluted GENGLYCOS solution should be administered immediately. If not used immediately, store the diluted solution:
  - At room temperature (20 °C to 25 °C [68 °F to 77 °F]) for up to 24 hours, including infusion time
  - Refrigerated (2 °C to 8 °C [36 °F to 46 °F]) and administer within 48 hours, from time of dilution to end of infusion
  - Do not freeze the solution

Important Safety Information (Cont.)

WARNINGS AND PRECAUTIONS (CONT.)

AAV Vector Integration and Risk of Tumorigenicity

- There is a theoretical risk of tumorigenicity due to integration of AAV vector DNA into the genome.
- GENGLYCOS is composed of a recombinant, non-replicating AAV8 vector whose DNA persists largely in episomal form. Random integration of recombinant AAV-vector DNA into human DNA has been reported with AAV gene therapies. The clinical relevance of individual integration events is unknown, but it is acknowledged that individual integration events could potentially contribute to a risk of tumorigenicity. If a tumor develops in a patient receiving GENGLYCOS, health care providers should contact and report the tumor to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.

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suspension for IV infusion

Administering GENLYCOS

Administer GENGLYCOS over 3 hours as a single-dose intravenous infusion through a peripheral venous catheter

- Administer GENGLYCOS in a setting where personnel and equipment are immediately available to treat hypersensitivity and infusion reactions
- Administer GENGLYCOS as a single-dose intravenous infusion through a peripheral venous catheter
  - Do not administer GENGLYCOS as an intravenous push or bolus
- Follow the recommended intervals and rates of infusion (see Table below) to minimize the risk for infusion reactions

Recommended Infusion Rates for GENGLYCOS

Interval After Start of Infusion (minutes)	Infusion Rates (mL/hr)	
	Patient Body Weight <40 kg	Patient Body Weight ≥40 kg
0 to 60 minutes	3 mL/hr	6 mL/hr
61 to 80 minutes	8 mL/hr	18 mL/hr
81 to 100 minutes	15 mL/hr	38 mL/hr
101 to 120 minutes	30 mL/hr	75 mL/hr
121 to 180 minutes[a]	80 mL/hr	200 mL/hr

[a]Ensure all contents of the infusion bag are infused, even if the infusion time exceeds 180 minutes, and is within 24 hours of dose preparation.

- **In the event of an anaphylactic reaction/infusion reaction, pause the infusion and administer supportive care as clinically indicated to manage the anaphylactic reaction/infusion reaction. If the infusion is paused, restart at half the prior rate after the infusion reaction has resolved. If the infusion needs to be paused and restarted, GENGLYCOS infusion should be completed within 24 hours of dose preparation**
- After all contents of the bag are infused, flush the tubing with 50 mL of 0.9% sodium chloride (NaCl) injection using the same infusion rate that was used for the last part of the infusion to ensure all GENGLYCOS is delivered. Do not flush the tubing with sodium chloride injection as a bolus
- Dispose unused GENGLYCOS and disposable materials that may have come in contact with GENGLYCOS in accordance with the local biosafety guidelines

Important Safety Information (Cont.)

ADVERSE REACTIONS

- Seven serious adverse events were observed in the Primary Efficacy Analysis Period (PEAP) of Study 1 (Weeks 1-48), including anaphylaxis/infusion reaction (2), adrenal insufficiency (2), high lactate level (2) and hypoglycemia (1).

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Patient monitoring

Liver function

- Assess liver function (AST, ALT, total bilirubin) after GENGLYCOS administration weekly until 2 weeks after completion of the corticosteroid taper period. If the patient is clinically stable with unremarkable findings (normal clinical exam, total bilirubin, and ALT and AST levels [below 1.5x ULN or below 1.5x baseline]) at the end of the corticosteroid taper period, continue to monitor liver function every 2 to 4 weeks until month 6, and as clinically indicated.
  - If hepatic enzyme (ALT or AST) elevations (≥1.5x ULN and ≥1.5x baseline) occur, the corticosteroid regimen may need to be initiated or adjusted followed by gradual taper as clinically indicated.
  - If hepatic enzyme elevations do not respond to corticosteroid adjustment, consult a gastroenterologist or hepatologist.

Adrenal function

- Assess adrenal function with morning serum cortisol and ACTH before the corticosteroid regimen, closely monitor adrenal function after GENGLYCOS administration, and repeat testing as clinically indicated.
- Consult with an endocrinologist in patients with abnormal baseline or persistent low morning cortisol levels. If adrenal insufficiency is suspected, increase the corticosteroid dose to the last tolerated dose and consult an endocrinologist.

ACTH, adrenocorticotropic hormone; ALT, alanine aminotransferase; AST, aspartate aminotransferase; ULN, upper limit of normal.

Important Safety Information (Cont.)

ADVERSE REACTIONS (CONT.)

- The most common adverse reactions during the PEAP of Study 1 (occurring in ≥10% of patients) with higher frequency in GENGLYCOS compared to placebo were ALT/AST enzyme elevated (71%), nausea (38%), headache (24%), hypertriglyceridemia (29%), adrenal insufficiency (24%), constipation (19%), hyperglycemia (14%), acne/dermatitis acneiform (19%), Cushingoid features (14%), and anaphylaxis (10%).

DRUG INTERACTIONS

Vaccinations

- Vaccine schedules may need to be adjusted for immunosuppressive therapy, and vaccines should be avoided 1 month prior to GENGLYCOS administration.

Please see additional Important Safety Information throughout and the full Prescribing Information.

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Patient and caregiver education

It is important to educate patients and caregivers on the symptoms associated with possible reactions the patient may experience during and after GENGLYCOS treatment.

Infusion reactions

- Educate patients and caregivers on the possible symptoms of infusion reactions during and after infusion and advise them to immediately inform medical staff if the patient experiences a reaction

Hepatotoxicity

- Advise patients and caregivers to adhere to the corticosteroid and/or immunomodulatory therapy regimen and about potential adverse reactions and necessary precautions. Advise patients and caregivers to immediately contact their healthcare provider if the patient's skin and/or whites of their eyes appear yellowish or if the patient misses a dose of corticosteroid or vomits it up. Advise patients and caregivers not to use any medication, herbal product, or supplement without first confirming with a healthcare provider that they are not hepatotoxic. Inform patients on the potential risks of alcohol consumption following GENGLYCOS infusion

Remind patients and caregivers about the importance of continuing corticosteroid treatment for a minimum of 8 weeks starting 2 weeks after GENGLYCOS infusion. Ensure they are also aware of the post-infusion monitoring schedule for assessing liver and adrenal function

Important Safety Information (Cont.)

USE IN SPECIFIC POPULATIONS

Pregnancy

- GENGLYCOS should not be used during pregnancy. There are no data on the use of GENGLYCOS in pregnant women. It is unknown whether GENGLYCOS can cause fetal harm when administered to a pregnant woman or can affect reproductive capacity.

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Patient and caregiver education (cont.)

It is also important to inform patients and caregivers on the potential risks associated with GENGLYCOS treatment.

AAV vector integration and risk of tumorigenicity

- There is a theoretical risk of tumorigenicity with AAV therapies like GENGLYCOS. Advise patients and caregivers to contact their healthcare provider and Ultragenyx Pharmaceutical Inc. (1-888-756-8657) if the patient develops a tumor

Vaccinations

- Advise patients and caregivers to consult with their healthcare provider to determine if adjustments to the patient's vaccination schedule are necessary during corticosteroid and other immunosuppressive therapy use. Vaccines should be avoided 1 month prior to GENGLYCOS administration

Contraception and semen donation

Males

- Advise patients that after a male patient has been treated with GENGLYCOS, the male patient and/or his female partner must avoid pregnancy for 6 months using an effective form of contraception. Male patients must not donate semen for 6 months following GENGLYCOS administration

Females

- Women of childbearing potential should use effective contraception for at least 12 months after administration of GENGLYCOS

Vector shedding

- Inform patients/caregivers that vector distribution in blood and vector shedding in urine, stool, and saliva may occur after GENGLYCOS infusion. Advise patients/caregivers on proper hygiene when handling patient body waste. These precautions should be followed for 3 months after GENGLYCOS infusion

AAV, adeno-associated virus.

Important Safety Information (Cont.)

USE IN SPECIFIC POPULATIONS (CONT.)

Contraception

- Women of childbearing potential should use effective contraception for at least 12 months after administration of GENGLYCOS.
- For 6 months after administration of GENGLYCOS, men must not donate semen, and men of reproductive potential and their female partners must prevent or postpone pregnancy using an effective form of contraception.

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IMPORTANT POINTS TO REMEMBER

GENGLYCOS treatment checklist

PREMEDICATION (See page 3)

- ✓ Acetaminophen and non-sedating antihistamines should be administered 30 to 60 minutes prior to infusion

GENGLYCOS INFUSION (See page 8)

- ✓ Administer GENGLYCOS as a single-dose intravenous infusion over 3 hours. Follow the recommended intervals and rates of infusion to minimize the risk for infusion reactions
- ✓ In the event of an anaphylactic reaction/infusion reaction during administration, pause the infusion and administer supportive care as necessary to manage the reaction
- ✓ If the infusion is paused, restart at half the prior rate after symptoms resolve

CORTICOSTEROID REGIMEN (See page 3)

- ✓ Starting 2 weeks after GENGLYCOS treatment, administer a course of oral corticosteroids for a minimum of 8 weeks
- ✓ Refer to the Monitoring Post-Administration section in the Prescribing Information for monitoring hepatic enzymes during and after the corticosteroid taper and assessing adrenal function after corticosteroid regimen

UltraCare® Gene Therapy Guides are available to help support your patient along their GENGLYCOS treatment journey

Scan or click to access UltraCare Patient Services for Gene Therapy

Scan or click to learn more about GENGLYCOS

POST-INFUSION MONITORING (See page 9)

- ✓ **Liver function:** monitor liver function (AST, ALT, total bilirubin) weekly after GENGLYCOS administration until 2 weeks after completion of the corticosteroid taper period. If the patient is clinically stable with unremarkable findings at the end of the corticosteroid taper period, continue to monitor liver function every 2 to 4 weeks until month 6 and as clinically indicated
- ✓ **Adrenal function:** assess adrenal function with morning serum cortisol and ACTH before the corticosteroid regimen, closely monitor adrenal function after GENGLYCOS administration, and repeat testing as clinically indicated

ACTH, adrenocorticotropic hormone; ALT, alanine aminotransferase; AST, aspartate aminotransferase.

Important Safety Information (Cont.)

ADDITIONAL PATIENT COUNSELING INFORMATION

Vector Shedding

- Inform patients/caregivers that vector distribution in blood and vector shedding in urine, stool, and saliva can occur after GENGLYCOS infusion. Advise patients/caregivers on proper hygiene when handling patient body waste. These precautions should be followed for 3 months after GENGLYCOS infusion.

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 the full Prescribing Information for GENGLYCOS.

Reference: GENGLYCOS [Prescribing Information]. Novato, CA: Ultragenyx Pharmaceutical Inc; August 2026.

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