

Specialty Guideline Management

Genglycos

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Genglycos	pariglasgene breca-parvovec-opnr

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications¹

Genglycos is an adeno-associated virus (AAV) vector-based gene therapy 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).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Reference number(s)
7594-A

- Genetic testing results demonstrating pathogenic variants in the G6PC gene or deficient enzyme activity level on liver biopsy.
- Medical records (e.g., chart notes and/or laboratory reports) documenting baseline liver function and adrenal function.

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or glycogen storage disorders.

Coverage Criteria

Glycogen Storage Disease Type Ia¹⁻³

Authorization of 3 months for one dose total may be granted for treatment of glycogen storage disease type Ia (GSDIa) when all of the following criteria are met:

- Member is at least 8 years of age but less than 38 years of age.
- Member has documented genetic testing demonstrating pathogenic variants in the G6PC gene or documented deficient enzyme activity level on liver biopsy.
- Member is receiving a therapeutic regimen of cornstarch (or equivalent), following international guidance/recommendations with stable nutrition, glycemic, and clinical status.
- Member's adrenal function (e.g., morning serum cortisol, adrenocorticotrophic hormone (ACTH)) and liver function (e.g., alanine aminotransferase (ALT), aspartate aminotransferase (AST), total bilirubin) have been assessed at baseline and will be monitored after Genglycos administration as clinically appropriate.
- Member does not have history of liver disease.
- Member does not have preexisting hepatic impairment or acute hepatic viral infection.
- Member does not have significant hepatic inflammation, liver fibrosis, or cirrhosis as evidenced by imaging or laboratory abnormalities (e.g., ALT or AST > upper limit of normal (ULN), total bilirubin > 1.5 x ULN, or alkaline phosphatase > 2.5 x ULN).
- Member does not have a history of liver transplant, including hepatocyte cell therapy or transplant.
- Member does not have either of the following:
 - Liver adenoma greater than 5 centimeters in size; or
 - Liver adenoma greater than 3 centimeters in size but less than or equal to 5 centimeters in size with a documented annual growth rate of at least 0.5 centimeter per year.
- Member has non-fasting triglyceride level < 1,000 mg/dL.
- Member's dose will not exceed 1.0×10^{13} genome copies per kilogram (gc/kg) body weight.
- Member's vaccination status will be up to date prior to Genglycos administration.

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- Member does not have detectable pre-existing antibodies to the adeno-associated virus serotype 8 (AAV8) capsid.
- Member does not have a history of illicit drug use within 60 days prior to Genglycos administration or does not have positive results from an 8-panel urine drug screen conducted at 2 time points at least 4 weeks apart prior to Genglycos administration.
- Member has not received Genglycos or other gene therapy previously.

Note: Members weighing >87 kg should be dosed at the 87 kg body weight recommended dosing.

References

1. Genglycos [package insert]. Novato, CA. Ultragenyx Pharmaceutical, Inc; August 2026.
2. Kishnani PS, Austin SL, Abdenur JE, et al. Diagnosis and management of glycogen storage disease type I: a practice guideline of the American College of Medical Genetics and Genomics. *Genet Med*. 2014;16(11):e1. doi:10.1038/gim.2014.128.
3. ClinicalTrials.gov. A Study of Adeno-Associated Virus Serotype 8-Mediated Gene Transfer of Glucose-6-Phosphatase in Patients With Glycogen Storage Disease Type Ia (GSDIa) (NCT05139316). Updated March 25, 2026. Accessed August 24, 2026. <https://clinicaltrials.gov/study/NCT05139316>.