



U.S. FOOD & DRUG
ADMINISTRATION

Memorandum

DATE: August 13, 2026

TO: Tolani Ishola, Pharm.D., RPM, CBER/OTP/ORMRR/RMSB1
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FROM: Benjamin S. Cyge, Ph.D.
Consumer Safety Officer
APLB/DCM/OCBQ

THROUGH: Kristine Khuc, PharmD
Branch Chief
APLB/DCM/OCBQ

SUBJECT: GENGLYCOS (pariglasgene brecaparvovec-opnr)
BLA: 125858/0
Sponsor: Ultragenyx Pharmaceutical Inc.

Background

The sponsor submitted:

☒ New Approval
☐ Changes Being Effectuated (CBE) supplement
☐ Prior Approval Supplement (PAS)
☐ Major Amendment

Submission contains:

☒ Prescribing Information (PI)
☐ Patient Package Insert (PPI)
☒ Package and/or container labels
☐ Other

Submission Date: December 23, 2025

PDUFA Action Date: August 21, 2026

APLB Comments/Recommendations

This is a labeling review for BLA 125858, submitted by Ultragenyx Pharmaceutical, Inc. for GENGLYCOS (pariglasgene brecaparvovec-opnr) on December 23, 2025. 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).

The GENGLYCOS Review Team iteratively reviewed the Prescribing Information (PI) through labeling review meetings. APLB received and reviewed the revised draft prescribing information (PI), package and container labels dated August 7, 2026. The following comments are from a promotional and comprehension perspective.

FULL PRESCRIBING INFORMATION

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- Consider revising the third bullet point in “Subsection 2.1 Patient Selection” to include a rationale for assessing liver function. For example, include a statement which explains that this is to determine baseline levels in order to adequately monitor the effects of treatment on liver health. Likewise, consider adding a rationale statement on assessing adrenal function.
 - Consider revising step 5 in “Subsection 2.3 Preparation” for clarity. Currently, the volume to remove and discard is confusing, as this is a very long and detailed sentence, and the volume is only defined at the very end of the sentence as the amount of GENGLYCOS to be added to the bag. For example, consider revising the sentence to “Remove and discard a volume of 0.9% sodium chloride equal to the volume of GENGLYCOS to be added from either the 100 mL...”
 - Consider revising the second bullet point in “Subsection 2.4 Administration.” In its current state, this direction can present comprehension issues. “Assess adrenal function with morning serum cortisol at baseline or other clinical signs and symptoms during the corticosteroid regimen and repeat morning serum cortisol testing thereafter as clinically indicated.” Since continuous monitoring of morning serum cortisol is a required step, including the option to either monitor morning serum cortisol *or* other clinical signs and symptoms is confusing. It is advised that monitoring of signs and symptoms of corticosteroid regimen be removed from this sentence and added as a separate sentence or bullet, if necessary.
 - Adding the sentence “Do not flush the tubing with sodium chloride injection as a bolus” can be misread and result in medication error. As the proper method of flushing the remaining medication is detailed above, this should be sufficient. If it is deemed
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necessary to include this sentence, it is advised that *DO NOT* is capitalized and either italicized or underlined.

PACKAGE AND CONTAINER LABELS

APLB has no comments on the package and container labels.

If you have any questions regarding this review, please contact Benjamin S. Cyge, Consumer Safety Officer at (301) 796-4212.