Medical Policy

BlueCross BlueShield Kansas
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Blue Cross Blue Shield Association

Title: Genglycos

Professional / Institutional
Original Effective Date: 11-26-2026
Latest Review Date:
Current Effective Date: 11-26-2026

State and Federal mandates and health plan member contract language, including specific provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage. To verify a member's benefits, contact Blue Cross and Blue Shield of Kansas Customer Service.

The BCBSKS Medical Policies contained herein are for informational purposes and apply only to members who have health insurance through BCBSKS or who are covered by a self-insured group plan administered by BCBSKS. Medical Policy for FEP members is subject to FEP medical policy which may differ from BCBSKS Medical Policy.

The medical policies do not constitute medical advice or medical care. Treating health care providers are independent contractors and are neither employees nor agents of Blue Cross and Blue Shield of Kansas and are solely responsible for diagnosis, treatment and medical advice.

If your patient is covered under a different Blue Cross and Blue Shield plan, please refer to the Medical Policies of that plan.

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POLICY AGENT SUMMARY – MEDICAL PRIOR AUTHORIZATION

<table>
<tr><th>Indication</th><th>Dose</th></tr>
<tr><td>GSDIa</td><td>– The recommended dose of Genglycos is 1.0 × 10^{13} gc/kg body weight, administered as a single intravenous infusion.
<table>
<tr><th>Member body weight range (kg)</th><th>Total Dose Volume (mL)</th><th>Number of vials</th></tr>
<tr><td>15 to <21</td><td>6</td><td>3</td></tr>
<tr><td>21 to <27</td><td>8</td><td>4</td></tr>
<tr><td>27 to <33</td><td>10</td><td>5</td></tr>
<tr><td>33 to <39</td><td>12</td><td>6</td></tr>
<tr><td>39 to <45</td><td>14</td><td>7</td></tr>
<tr><td>45 to <51</td><td>16</td><td>8</td></tr>
<tr><td>51 to <57</td><td>18</td><td>9</td></tr>
<tr><td>57 to <63</td><td>20</td><td>10</td></tr>
<tr><td>63 to <69</td><td>22</td><td>11</td></tr>
<tr><td>69 to <75</td><td>24</td><td>12</td></tr>
<tr><td>75 to <81</td><td>26</td><td>13</td></tr>
<tr><td>81 to <87</td><td>28</td><td>14</td></tr>
<tr><td>≥87</td><td>30</td><td>15</td></tr>
<tr><td colspan="3">a. Dose volume is rounded up or down to the nearest 2 mL.
b. Members weighing >87 kg should be dosed at the 87 kg weight.</td></tr>
</table>
</td></tr>
<tr><td colspan="2">• Verify the required dose of Genglycos based on member's body weight, and confirm the kit contains enough vials to prepare the Genglycos infusion for the member.
• Vials should not be thawed or punctured until immediately prior to use. If the member is not expected to be ready for same-day administration, store Genglycos in a manner consistent with how it is outlined in the prescribing information.
• Pre-medicate with acetaminophen and non-sedating antihistamines 30 to 60 minutes prior to infusion of Genglycos.
• Administer Genglycos as a single-dose intravenous infusion through a peripheral venous catheter.
• Do not administer Genglycos as an intravenous push or bolus.</td></tr>
</table>

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for one dose.
- Renewal: Prior authorization validity may NOT be renewed.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 1 kit of 15 vials for a total of 30 mL (each vial contains 3.0 x 10^{13} genome copies-gc/mL – 2 mL)

III. Initial Approval Criteria

- Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of

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submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax. Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines.

Prior authorization validity is provided in the following conditions:

Glycogen Storage Disease type Ia (GSDIa) † Φ [1-5]

- Member is at least 8 years of age; **AND**
- Member does NOT have any FDA labeled contraindications to the requested agent (e.g., severe hepatic fibrosis or cirrhosis); **AND**
- Member has a diagnosis of glycogen storage disease type Ia (also known as von Gierke disease) as established by the following:
    - Member has biallelic pathogenic variants in the *G6PC1* gene identified by molecular genetic testing; **OR**
    - Member has deficient hepatic enzyme activity (i.e., glucose-6-phosphatase catalytic activity) as evidenced from a liver biopsy specimen; **AND**
- Member is currently receiving a therapeutic regimen of cornstarch (or equivalent), following international guidelines/recommendations with stable nutrition, glycemic, and clinical status and will use therapy to reduce daily cornstarch intake as an adjunct to nutritional management; **AND**
- Member does not have detectable anti-adeno-associated virus, serotype 8 (anti-AAV8) antibody titers at baseline; **AND**
- Member will have adrenal function assessed including performing a clinical examination and laboratory testing (i.e., morning serum cortisol and ACTH) prior to start of therapy and as clinically indicated; **AND**
- Member will have liver function assessed including performing a clinical examination and laboratory testing (i.e., alanine aminotransferase (ALT), aspartate aminotransferase (AST), and total bilirubin) prior to start of therapy and then every 2 to 4 weeks until month 6, and as clinically indicated**; AND**
- Member will use immunosuppressive corticosteroid therapy, following Genglycos, and will be monitored for signs and symptoms of hepatotoxicity and/or adrenal insufficiency and adrenal crisis; **AND**
- Member does not have pre-existing hepatic impairment or an acute hepatic viral infection; **AND**
- Member has not received prior adeno-associated viral (AAV) vector-based gene therapy; **AND**
- Member has not received a liver transplant

† FDA Approved Indication(s); **‡** Compendia Recommended Indication(s); **Φ** Orphan Drug

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IV. Renewal Criteria [1]

Duration of authorization has not been exceeded *(refer to Section I)*

Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

CLINICAL RATIONALE

See package insert for FDA preshttps://dailymed.nlm.nih.gov/dailymed/index.cfm

CODING

The following codes for treatment and procedures applicable to this policy are included below for informational purposes. This may not be a comprehensive list of procedure codes applicable to this policy.

Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

The code(s) listed below are medically necessary ONLY if the procedure is performed according to the "Policy" section of this document.

HCPCS code(s):

- J3590 – Unclassified biologics
- C9399 – Unclassified drugs or biologicals *(Hospital Outpatient Use Only)*

NDC:

- Genglycos 3.0 × 10^{13} genome copies (gc) per mL – 2 mL single dose vial

Member body weight range (kg)	Total Dose Volume (mL)	Number of vials	NDC
15 to <21	6	3	69794-0333-03
21 to <27	8	4	69794-0334-04
27 to <33	10	5	69794-0335-05
33 to <39	12	6	69794-0336-06
39 to <45	14	7	69794-0337-07
45 to <51	16	8	69794-0338-08
51 to <57	18	9	69794-0339-09
57 to <63	20	10	69794-0340-10
63 to <69	22	11	69794-0341-11
69 to <75	24	12	69794-0342-12
75 to <81	26	13	69794-0343-13
81 to <87	28	14	69794-0344-14
≥87	30	15	69794-0345-15

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REVISIONS	
Posted: 10-27-2026 Effective: 11-26-2026	New medical policy added to the bcbsks.com web site. Policy is maintained by Prime Therapeutics LLC.

REFERENCES

1. Genglycos [package insert]. Novato, CA; Ultragenyx Pharm., Inc.; August 2026. Accessed September 2026.
2. ClinicalTrials.gov. NCT01395641. A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Adeno-Associated Virus Serotype 8-Mediated Gene Transfer of Glucose-6-Phosphatase in Patients With Glycogen Storage Disease Type Ia. Search ClinicalTrials.gov for: NCT05139316 | Card Results | ClinicalTrials.gov.
3. Bali DS, El-Gharbawy A, Austin S, et al. Glycogen Storage Disease Type I. 2006 Apr 19 [Updated 2021 Oct 14]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1312/.
4. Glycogen storage disease type 1A. National Institutes of Health: Genetic and Rare Diseases Information Center Website. https://rarediseases.info.nih.gov/diseases/7864/glycogen-storage-disease-type-1a. Updated March 1, 2019. Accessed September 3, 2026.
5. Mitchell J, Abdenur J, de Boer F, et al. Efficacy and safety results from a pivotal phase 3 trial of DTX401, an AAV8-mediated liver-directed gene therapy, in individuals with glycogen storage disease type Ia (GSDIa). *Endocrine Abstracts* (2025) **110** RC3.6. DOI: 10.1530/endoabs.110.RC3.6

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