

Clinical Policy: Venglustat (SAR402671)

Reference Number: CP.PHAR.795

Effective Date: **FDA Approval Date**

Last Review Date: 08.26

Line of Business: Commercial, HIM/ICHRA, Medicaid

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Venglustat (SAR402671) is a glucosylceramide synthase inhibitor.

FDA Approved Indication(s) **[Pending]**

SAR402671 is indicated for the treatment of type 3 Gaucher disease (GD3).

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Centene Corporation® that SAR402671 is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Type 3 Gaucher Disease (must meet all):

1. Diagnosis of GD3 confirmed by one of the following (a or b):*
 - a. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity;
 - b. DNA testing;
2. Age $\geq$ 12 years;*
3. Member exhibits neurologic symptoms with gaze palsy, predominantly horizontal, with slow or absent saccades;*
4. Member is currently receiving enzyme replacement therapy (e.g., Cerezyme®) with positive response as demonstrated by all of the following (a – e):*
 - a. Hemoglobin level of $\geq$ 11.0 g/dL for females and $\geq$ 12.0 g/dL for males;
 - b. Platelet count $\geq$ 100,000/mm³;
 - c. Spleen volume $<$ 10 multiples of normal (MN);
 - d. Liver volume $<$ 1.5 MN;
 - e. No bone crisis and free of symptomatic bone disease such as bone pain attributable to osteonecrosis and/or pathological fractures;
5. Provider attestation that current enzyme replacement therapy will be discontinued;*
6. SAR402671 is prescribed as monotherapy;*
7. Dose does not exceed 15 mg per day.*

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace/ICHRA, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace/ICHRA, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid.

II. Continued Therapy*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Type 3 Gaucher Disease (must meet all):

1. Member meets one of the following (a or b):
 - a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
 - b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B*);
2. Member is responding positively to therapy as evidenced by improvements or stabilization of neurological symptoms (e.g., motor coordination, ataxia, gait stabilization, limb coordination, speech fluidity, memory retention, visuomotor/special precision);*
3. SAR402671 is prescribed as monotherapy;*
4. If request is for a dose increase, new dose does not exceed 15 mg per day.*

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace/ICHRA, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the non-formulary policy for the

relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace/ICHRA, and CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration

GD1: type 1 Gaucher disease

GD3: type 3 Gaucher disease

MN: multiples of normal

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

- Contraindication(s): pending
- Boxed warning(s): pending

Appendix D: General Information

- GD3 is the chronic neuronopathic form of Gaucher disease. It is distinct from type 1 Gaucher disease (GD1) which lacks neurologic symptoms, but shares the systemic manifestations seen in GD1, such as liver and spleen enlargement, anemia, thrombocytopenia, or bone disease.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
GD3	15 mg PO QD	15 mg/day

VI. Product Availability [Pending]

Tablet: 15 mg

VII. References

1. NCT05222906 in ClinicalTrials.gov. Study to Evaluate the Efficacy and Safety of Venglustat in Adult and Pediatric Patients With Gaucher Disease Type 3 (LEAP2MONO). NIH U.S. National Library of Medicine. Available at: <https://clinicaltrials.gov/study/NCT05222906>. Accessed June 9, 2026.

2. Sanofi's venglustat met all primary endpoints in a phase 3 study of type 3 Gaucher disease. Press Release: Sanofi; February 2, 2026. Available at: <https://www.sanofi.com/en/media-room/press-releases/2026/2026-02-02-06-00-00-3229947>. Accessed June 9, 2026.
3. Hollak, CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. *Best Pract Res Clin Endocrinol Metab.* 2015;29:205-218.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. *Semin Hematol.* 2004;41(suppl 5):4-14.
5. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. *Genet Med.* 2005;7(2):105-110.
6. Gary SE, Ryan E, Steward AM, et al. Recent advances in the diagnosis and management of Gaucher disease. *Expert Rev Endocrinol Metab.* 2018 Mar;13(2):107-118.
7. Leonart LP, Fachi MM, Boger B, et al. A systematic review and meta-analyses of longitudinal studies on drug treatments for Gaucher disease. *Ann Pharmacother.* 2022;0(0). doi:10.1177/10600280221108443.
8. Kishnani PS, Al-Hertani W, Balwani M, et al. Screening, patient identification, evaluation, and treatment in patients with Gaucher disease: Results from a Delphi consensus. *Mol Genet Metab.* 2022 Feb;135(2):154-162. doi: 10.1016/j.ymgme.2021.12.009.
9. Vellodi A, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. *J Inherit Metab Dis.* 2009 Oct;32(5):660-664. doi: 10.1007/s10545-009-1164-2.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively.	07.14.26	08.26

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy,

contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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