Clinical Policy: Tofersen (Qalsody)

Reference Number: CP.PHAR.591
Effective Date: 04.25.23
Last Review Date: 11.26
Line of Business: Commercial, HIM/ICHRA, Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description

Tofersen (Qalsody®) is an antisense oligonucleotide.

FDA Approved Indication(s)

Qalsody is indicated for the treatment of amyotrophic lateral sclerosis (ALS) in adults who have a mutation in the superoxide dismutase 1 (*SOD1*) gene.

This indication is approved under accelerated approval based on reduction in plasma neurofilament light chain observed in patients treated with Qalsody. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).

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 Qalsody is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Amyotrophic Lateral Sclerosis (must meet all)**:**

1. Diagnosis of ALS with both of the following (a and b):
   a. Muscle weakness attributed to ALS;
   b. Documentation of *SOD1* mutation;
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 18 years;
4. Percent predicted slowed vital capacity (SVC) ≥ 50%;
5. Prescribed concurrently with riluzole (at up to maximally indicated doses), unless contraindicated or clinically significant adverse effects are experienced;
6. Member does not have presence of tracheostomy or permanent ventilation;
7. Dose does not exceed 100 mg (1 vial) on days 1, 15, and 29, followed by maintenance dose of 100 mg (1 vial) every 28 days.

Approval duration:
Commercial – 6 months or to the member's renewal date, whichever is longer
Medicaid/HIM/ICHRA – 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

A. Amyotrophic Lateral Sclerosis (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 (e.g., slowing of ALSFRS-R slope decline compared to baseline);
3. Member does not require tracheostomy or permanent ventilation;
4. Prescribed concurrently with riluzole (at up to maximally indicated doses), unless contraindicated or clinically significant adverse effects are experienced;
5. If request is for a dose increase, new dose does not exceed 100 mg (1 vial) every 28 days.

Approval duration:
Commercial – 6 months or to the member's renewal date, whichever is longer
Medicaid/HIM/ICHRA – 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

ALS: amyotrophic lateral sclerosis
FDA: Food and Drug Administration
LMN: lower motor neuron
SOD1: superoxide dismutase 1
SVC: slowed vital capacity
UMN: upper motor neuron

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

None reported

Appendix D: General Information

- Revised El Escorial diagnostic criteria for ALS requires the presence of:
  1. Signs of lower motor neuron (LMN) degeneration by clinical, electrophysiological or neuropathologic examination;
  2. Signs of upper motor neuron (UMN) degeneration by clinical examination, and
  3. Progressive spread of signs within a region or to other regions, together with the absence of:
     a. Electrophysiological evidence of other disease processes that might explain the signs of LMN and/or UMN degenerations; and
     b. Neuroimaging evidence of other disease processes that might explain the observed clinical and electrophysiological signs.
- Gold Coast consensus diagnostic criteria for ALS requires the presence of:
  1. Progressive motor impairment documented by history or repeated clinical assessment, preceded by normal motor function; and
  2. Presence of upper and lower motor neuron dysfunction in at least 1 body region, (with upper and lower motor neuron dysfunction noted in the same body region if only one body region is involved) or lower motor neuron dysfunction in at least 2 body regions, and

3. Investigations excluding other disease processes.

Appendix E: Riluzole Co-administration
Guidelines support the co-administration of riluzole in ALS:

- The 2020 Canadian best practice recommendations for the management of ALS state the following: riluzole has demonstrated efficacy in improving survival in ALS (level A), there is evidence that riluzole prolongs survival by a median duration of 3 months (level A), and riluzole should be started soon after the diagnosis of ALS (expert consensus).
- The 2024 European Academy of Neurology guideline on the management of ALS recommends offering lifelong riluzole to all people with ALS at diagnosis (strong recommendation in favor).
- Additionally, approximately 62% of patients in the phase 3 VALOR trial were receiving concomitant riluzole.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
SOD1 ALS	Initiate recommended dose of 100 mg with 3 loading doses administered intrathecally at 14-day intervals. Maintenance dose of 100 mg should be administered intrathecally once every 28 days thereafter.	100 mg/dose/day

VI. Product Availability

Single-dose vial for injection: 100 mg/15 mL

VII. References

1. Qalsody Prescribing Information. Cambridge, MA: Biogen; April 2023. Available at: https://www.biogencdn.com/us/pdfs/qalsody-prescribing-information.pdf. Accessed July 8, 2026.
2. Brooks BR, Miller RG, Swash M, et al. El Escorial revisited: revised criteria for the diagnosis of amyotrophic lateral sclerosis. Amyotroph Lateral Scler Other Motor Neuron Disord. 2000 Dec;1(5):293-9.
3. Shefner JM, Al-Chalabi A, Baker MR, et al. A proposal for new diagnostic criteria for ALS. Clin Neurophysiol. 2020;131(8):1975-1978.
4. Shoesmith C, Abrahao A, Benstead T, et al. Canadian best practice recommendations for the management of amyotrophic lateral sclerosis. CMAJ. 2020 Nov;192(46):E1453-E1468.
5. Van Damme P, Al-Chalabi A, Andersen PM, et al. European Academy of Neurology (EAN) guideline on the management of amyotrophic lateral sclerosis in collaboration with European Reference Network for Neuromuscular Diseases (ERN EURO-NMD). Eur J Neurol. 2024;31(6):e16264.
6. Miller T, Cudkowicz M, Shaw PJ, et al. Phase 1-2 Trial of Antisense Oligonucleotide Tofersen for *SOD1* ALS. N Engl J Med. 2020;383(2):109-119.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1304	Injection, tofersen, 1 mg

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively. Template changes applied to other diagnoses/indications and continued therapy section.	08.30.22	11.22
Drug is now FDA approved – criteria updated per FDA labeling: updated SVC ≥ 50% to reflect SVC eligibility criteria from VALOR part C trial, added no tracheostomy or permanent assisted ventilation for initial approval criteria and positive response continuation criteria, and updated maximum dose criteria; references reviewed and updated.	05.23.23	06.23
4Q 2023 annual review: no significant changes; references reviewed and updated. Added HCPCS code [J1304].	07.10.23	11.23
4Q 2024 annual review: revised Commercial approval durations to be for "6 months or to the member's renewal date, whichever is longer;" references reviewed and updated.	07.19.24	11.24
4Q 2025 annual review: for continued therapy, added requirement for no tracheostomy or permanent ventilation and a positive response example of slowing of ALSFRS-R slope decline compared to baseline; references reviewed and updated.	07.14.25	11.25
4Q 2026 annual review: no significant changes; added ICHRA line of business; references reviewed and updated.	08.17.26	11.26

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.

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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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