

Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy



Clinical Policy: Tofersen (BIIB067)

Reference Number: CP.PHAR.591

Effective Date: **FDA Approval Date**

Last Review Date: 11.22

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)

[Revision Log](#)

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

Description

Tofersen (BIIB067^{®/TM}) is an antisense oligonucleotide.

FDA Approved Indication(s) [Pending]

BIIB067 is proposed for the treatment of superoxide dismutase 1 (*SOD1*) amyotrophic lateral sclerosis (ALS).

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 BIIB067 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. 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 \geq 18 years;*
4. Percent predicted slowed vital capacity (SVC) \geq 45%;*
5. Prescribed concurrently with riluzole (at up to maximally indicated doses), unless contraindicated or clinically significant adverse effects are experienced;*
6. Dose does not exceed the FDA-approved maximum dose.*

Approval duration: 6 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) 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, and CP.PMN.255 for Medicaid; or
- b. For drugs NOT on the formulary (commercial, health insurance marketplace) 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, 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, and CP.PMN.53 for Medicaid.

II. Continued Therapy*

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

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.*
3. Prescribed concurrently with riluzole (at up to maximally indicated doses), unless contraindicated or clinically significant adverse effects are experienced.*
4. If request is for a dose increase, new dose does not exceed the FDA-approved maximum dose.*

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) 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, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) 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, 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, 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, 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

SOD1: superoxide dismutase 1

SVC: slowed vital capacity

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

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

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 2009 American Academy of Neurology ALS guideline for the care of the patient with ALS (reaffirmed January 2020) recommends that riluzole should be offered to slow disease progression (Level A).
- 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).

- Additionally, approximately 62% of patients in the phase 3 VALOR trial were receiving concomitant riluzole.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
<i>SOD1 ALS*</i>	Pending	Pending

VI. Product Availability [Pending]

Pending

VII. References

1. ClinicalTrials.gov. an efficacy, safety, tolerability, pharmacokinetics and pharmacodynamics study of BIIB067 in adults with inherited ALS (VALOR (Part C)). Last updated July 19, 2022. Available at: <https://clinicaltrials.gov/ct2/show/NCT02623699>. Accessed August 9, 2022.
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. Miller RG, Jackson CE, Kasarskis EJ, et al. Practice parameter update: the care of the patient with amyotrophic lateral sclerosis: drug, nutritional, and respiratory therapies (an evidence-based review): report of the Quality Standards Subcommittee of the American Academy of Neurology. *Neurology.* 2009 Oct 13;73(15):1218-26.
5. 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.
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 [Pending]

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
Pending	Pending

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

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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