tofersen (Qalsody)

Medicare Part B Drug Policy

- Medicare coverage is limited to items and services that are reasonable and necessary for the diagnosis or treatment of an illness or injury (and within the scope of a Medicare benefit category).
- Medicare Benefit Policy Manual - Pub. 100-02, Chapter 15, Section 50, describes national policy regarding Medicare guidelines for coverage of drugs and biologicals.
- Blue Shield of California (BSC) follows Medicare statutes, regulations, National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), and policy articles for determining coverage for Part B drug requests when applicable.
- BSC Medicare Part B Drug Policies will be used when coverage criteria are not fully established or there is an absence of any applicable Medicare statutes, regulations, NCDs or LCDs.

Drug Details

USP Category: CENTRAL NERVOUS SYSTEM AGENTS
Mechanism of Action: Antisense oligonucleotide specific for SOD1 mRNA
HCPCS:
J1304:Injection, tofersen, 1 mg
How Supplied:
100 mg/15 mL (6.7 mg/mL) 1 single-dose vial

Condition(s) listed in policy *(see coverage criteria for details)*

- Amyotrophic Lateral Sclerosis (ALS) with SOD1 Mutation

Any request for a condition not listed in policy must meet the definition of a medically accepted indication. Section 1861(t)(2)(B) of the Act defines "medically-accepted indication," as any use of a prescription drug or biological product which is approved under the Federal Food, Drug, and Cosmetic Act, or the use of which is supported by one or more citations included (or approved for inclusion) in one or more of the CMS approved compendia.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

Coverage Criteria
The following condition(s) require Prior Authorization/Preservice.

Amyotrophic Lateral Sclerosis (ALS) with SOD1 Mutation
Meets medical necessity if all the following are met:
Initial
1. Being prescribed by or in consultation with a neurologist
2. Presence of superoxide dismutase 1 gene mutation
3. Patient has received concurrent or prior treatment with riluzole or has medical reason why riluzole cannot be used

Reauthorization
1. Patient has not progressed to become dependent on a ventilator

2. Patient is responding to therapy

Covered Doses:
Up to 100 mg as an intrathecal injection every 14 days for 3 doses, followed by maintenance of 100 mg every 28 days thereafter

Coverage Period:
6 months

ICD-10:
G12.21

Additional Information

Summary of Evidence

The contents of this policy were created after examining the following resources:

1. The prescribing information for Qalsody
2. CMS approved compendium in accordance with the accepted compendia ratings listed:
   a. Micromedex DrugDex - Class I, Class IIa, of Class IIb
   b. American Hospital Formulary Service-Drug Information (AHFS-DI) - supportive narrative text
   c. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium - Category 1 or 2A
   d. Lexi-Drugs – "Use: Off-Label" and rated as "Evidence Level A"
   e. Clinical Pharmacology - supportive narrative text
3. American Academy of Neurology (AAN): Guideline on the care of the patient with amyotrophic lateral sclerosis – Drug, nutritional, and respiratory therapies, update (2009, reaffirmed 2023)

Explanation of Rationale:

- Support for FDA-approved indications can be found in the manufacturer's prescribing information.
- Beginning January 1, 2019, the Centers for Medicare & Medicaid Services (CMS) provided Medicare Advantage (MA) plans the option of applying step therapy for physician-administered and other Part B drugs to lower costs and improve the quality of care for Medicare beneficiaries.
- Riluzole is FDA-approved for the treatment of patients with amyotrophic lateral sclerosis (ALS). Support for using riluzole as pharmacotherapy treatment for ALS to slow disease progression is found in the American Academy of Neurology guideline on the Care of the Patient with Amyotrophic Lateral Sclerosis: Drug, Nutritional, and Respiratory Therapies. .

References

1. CMS Benefit Policy Manual. Chapter 15; § 50 Drugs and Biologicals
2. Medicare Coverage Database. Available at https://www.cms.gov/Medicare-Coverage-Database/search.aspx
3. Social Security Act (Title XVIII) Standard References, Sections: 1862(a)(1)(A) Medically Reasonable & Necessary; 1862(a)(1)(D) Investigational or Experimental; 1833(e) Incomplete Claim; 1861(t) (1) Drugs and Biologicals
4. AHFS. Available by subscription at http://www.lexi.com
5. DrugDex. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
6. Qalsody (tofersen) [prescribing information]. Cambridge, MA: Biogen MA Inc; April 2023.

7. Miller RG, Jackson CE, Kasarskis EJ, et al. Quality Standards Subcommittee of the American Academy of Neurology. Practice parameter update: the care of the patient with amyotrophic lateral sclerosis: multidisciplinary care, symptom management, and cognitive/behavioral impairment (an evidence-based review): report of the Quality Standards Subcommittee of the American Academy of Neurology. Neurology. 2009 Oct 13;73(15):1227-33. Reaffirmed on February 21, 2026. doi: 10.1212/WNL.0b013e3181bc01a4.
8. 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. Available at: https://onlinelibrary.wiley.com/doi/10.1111/ene.16264.

Review History

Date of Last Annual Review: 3Q2026
Changes from previous policy version:

- No clinical changes following annual review.

Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee