Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines:
Medical Benefit Drugs
Qalsody™ (tofersen)

Effective: July 1, 2025

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Applies to:
Commercial Products
☐ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☐ Tufts Health Plan Commercial products; Fax 617-673-0988
CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Public Plans Products
☐ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans; Fax 617-673-0939
☐ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☒ Tufts Health One Care* – A Medicare-Medicaid Plan (a dual eligible product); Fax 617-673-0956
*The MNG applies to Tufts Health One Care members unless a less restrictive LCD or NCD exists.

Senior Products
☒ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☒ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☒ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Accelerated approval of Qalsody was based on a reduction in plasma neurofilament light (NfL), a biomarker of axonal injury and neurodegeneration that the Food and Drug Administration believes is reasonably likely to predict a clinical benefit in patients with SOD1-ALS. This is the first time a biomarker has been used as a surrogate endpoint in ALS. In the Phase 3 VALOR trial, Qalsody failed to demonstrate a statistically significant benefit over placebo on the primary efficacy endpoint of change from baseline to Week 28 in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) in the prespecified analysis population. Qalsody showed improvements in multiple secondary and exploratory endpoints. In the overall intent-to-treat population (n=108), Qalsody-treated patients experienced a 55% reduction in plasma NfL compared to a 12% increase in placebo-treated patients (nominal P-value <0.0001). Additionally, levels of cerebrospinal fluid SOD1 protein, which is an indirect measure of target engagement, were reduced by 35% in Qalsody-treated patients compared to 2% in placebo-treated patients (nominal P-value <0.0001).

Food and Drug Administration (FDA) Approved Indications

Qalsody (tofersen) is an antisense oligonucleotide 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 (tofersen). Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trials.

Clinical Guideline Coverage Criteria

The plan may authorize coverage of Qalsody for Members when **ALL** of the following criteria are met:

1. Documented diagnosis of amyotrophic lateral sclerosis

AND

2. Documentation of superoxide dismutase 1 (SOD1) gene mutation

AND

3. Prescribed by or in consultation with a neurologist

Limitations

None

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J1304	Injection, tofersen, 1 mg

References:

1. Miller RG, 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).
2. Qalsody (tofersen) [package insert]. Cambridge, MA: Biogen MA Inc.; April 2023.
3. Benatar M, et al. Design of a randomized, placebo-controlled, Phase 3 trial of tofersen initiated in clinically presymptomatic SOD1 variant carriers: the ATLAS study [published correction appears in Neurotherapeutics. 2022 Sep 29]. Neurotherapeutics. 2022;19(4):1248–1258.
4. Miller T, et al. Phase 1–2 trial of antisense oligonucleotide tofersen for SOD1 ALS. N Engl J Med. 2020;383(2):109–119.
5. Miller T, et al. Trial of antisense oligonucleotide tofersen for SOD1 ALS. N Engl J Med.2022;387(12):1099–1110.

Approval And Revision History

July 11, 2023: Reviewed by the Pharmacy & Therapeutics Committee (P&T).

Subsequent endorsement date(s) and changes made:

- August 2023: Administrative update to rebrand Tufts Health Unify to Tufts Health One Care for 2024.
- November 2023: Administrative Update in support of calendar year 2024 Medicare Advantage and PDP Final Rule.
- May 14, 2024: No changes. Administrative update to add HCPCS Code (eff 7/1/24).
- June 10, 2025: No changes. Administrative update to remove Harvard Pilgrim Health Care Stride Medicare Advantage from the Medical Necessity Guideline template (eff 7/1/25).
- June 2025: Joint Medical Policy and Health Care Services UM Committee review (eff 7/1/25).

Background, Product and Disclaimer Information

Point32Health prior authorization criteria to be applied to Medicare Advantage plan members is based on guidance from Medicare laws, National Coverage Determinations (NCDs) or Local Coverage Determinations (LCDs). When no guidance is provided, Point32Health uses clinical practice guidance published by relevant medical societies, relevant medical literature, Food and Drug Administration (FDA)-approved package labeling, and drug compendia to develop prior authorization criteria to apply to Medicare Advantage plan members. Medications that require prior authorization generally meet one or more of the following criteria: Drug product has the potential to be used for cosmetic purposes; drug product is not considered as first-line treatment by medically accepted practice guidelines, evidence to support the safety and efficacy of a drug product is poor, or drug product has the potential to be used for indications outside of the indications approved by the FDA. Prior authorization and use of the coverage criteria within this Medical Necessity Guideline will ensure drug therapy is medically necessary, clinically appropriate, and aligns with evidence-based guidelines. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guidelines not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.