CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Qalsody (tofersen)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Qalsody, approved by the FDA in 2023, 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 (NfL) observed in patients treated with Qalsody. There is literature to support the use of NfL as a prognostic marker of ALS disease progression. It is a biomarker of nerve injury and neurodegeneration, reasonably believed to predict clinical benefit in SOD1-ALS.
ALS, also known as Lou Gehrig's disease, is a fatal, progressive neurodegenerative disorder in which motor neuron loss leads to muscle weakness, with most patients succumbing to respiratory failure. SOD1-ALS is a rare genetic form of ALS, accounting for approximately 2% of ALS cases.
Qalsody binds to SOD1 mRNA to cause its degradation, resulting in a reduction of SOD1 protein synthesis. Efficacy was assessed in the Phase 3 VALOR study in which Qalsody did not meet the primary endpoint for clinical function (ALSFRS-R score change over 24 weeks). Confirmatory clinical trials are pending.

Qalsody (tofersen) will be considered for coverage when the following criteria are met:

Amyotrophic Lateral Sclerosis (ALS)

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist or neuromuscular specialist; AND
3. Member has a documented diagnosis of ALS with signs/symptoms of weakness; AND
4. Member has genetic test results showing mutation in the SOD1 gene; AND
5. Documentation of baseline ALSFRS-R score.
6. **Dosage allowed/Quantity limit:** 100 mg (15 mL) intrathecally every 14 days for 3 loading doses, then every 28 days thereafter for maintenance. (QL: 1 vial per 28 days maintenance)

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must document positive clinical response, such as reduced plasma NfL level or reduced rate of disease progression/functional decline.

If all the above requirements are met, the medication will be approved for an additional 6 months.

CareSource considers Qalsody (tofersen) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/08/2023	New policy for Qalsody created.
06/16/2025	Added supporting references (Van Damme; Hamad).

References:

1. Qalsody [prescribing information]. Biogen MA Inc.; 2023.
2. Miller TM, Cudkowicz ME, Genge A, et al. Trial of Antisense Oligonucleotide Tofersen for *SOD1* ALS. *N Engl J Med*. 2022;387(12):1099-1110. doi:10.1056/NEJMoa2204705
3. van den Berg LH, Sorenson E, Gronseth G, et al. Revised Airlie House consensus guidelines for design and implementation of ALS clinical trials. *Neurology*. 2019;92(14):e1610-e1623. doi:10.1212/WNL.0000000000007242
4. ALS Functional Rating Scale. Available at: http://www.outcomes-umassmed.org/als/alsscale.aspx.
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. doi:10.1111/ene.16264
6. Hamad AA, Alkhawaldeh IM, Nashwan AJ, Meshref M, Imam Y. Tofersen for SOD1 amyotrophic lateral sclerosis: a systematic review and meta-analysis. *Neurol Sci*. 2025;46(5):1977-1985. doi:10.1007/s10072-025-07994-2

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