Pharmacy Management Drug Policy

SUBJECT: Qalsody (tofersen)
POLICY NUMBER: PHARMACY-127
EFFECTIVE DATE: 04/2024
LAST REVIEW DATE: 02/12/2026

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

This policy contains coverage requirements for Qalsody. For other ALS drugs, see the Amyotrophic Lateral Sclerosis (ALS) Policy (Pharmacy-111).

Qalsody (tofersen) is indicated for the treatment of ALS in adults who have a mutation in the SOD1 gene. This indication is approved under accelerated approval based on reduction in plasma neurofilament light chain (NfL) observed in patients treated with Qalsody. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).

The efficacy of Qalsody was evaluated in the 28-week, double-blind, placebo-controlled Phase 3 VALOR study involving patients with weakness attributable to ALS and confirmed SOD1 mutation. A total of 108 patients were randomized to receive treatment with either Qalsody 100 mg (n=72) or placebo (n=36) for 24 weeks (3 loading doses followed by 5 maintenance doses).[10]

The primary analysis population (n=60, mITT) had SVC $\geq$ 65% of predicted value and met criteria for rapid disease progression, defined by their pre-randomization ALSFRS-R decline slope and SOD1 mutation type.

The primary efficacy analysis was change from baseline to Week 28 in the ALSFRS-R total score. While Qalsody demonstrated less decline in the ALSFRS-R compared to placebo at Week 28, the results were not statistically significant.

The secondary endpoints of change from baseline at Week 28 in plasma NfL and cerebrospinal fluid (CSF) SOD1 protein were nominally statistically significant.

After the completion of the 28-week VALOR study, patients had the option to enroll in an open-label extension study. At the 52-week interim analysis, patients previously receiving placebo who initiated Qalsody saw reductions in Nfl, similar to those treated with Qalsody in the placebo-controlled period. Earlier initiation of Qalsody was associated with a trend for reduction in ALSFRS-R decline but was not statistically significant. In addition, earlier initiation of Qalsody was associated with a trend toward a reduction in the risk of death or permanent ventilation but was not statistically significant. It is noted in the Qalsody prescribing information that these results should be interpreted with caution given the limitations of data collected outside a controlled trial.

POLICY:

Qalsody-tofersen (Medical)

Qalsody coverage varies by line of business as below:
Commercial/Exchange/Essential/criteria:

1. Based upon our criteria and assessment of the peer-reviewed evidence, the use of Qalsody (tofersen) has not been medically proven to be effective and, therefore, is considered **not medically necessary** for the treatment of amyotrophic lateral sclerosis (ALS) in adults who have a mutation in the superoxide dismutase 1 (SOD1) gene.

The justification for Qalsody (tofersen) to be considered **not medically necessary** is as follows:

a. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug has a definite positive effect on health outcomes.
b. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug, over time, leads to improvement in health outcomes (e.g., the beneficial effects of the service outweigh any harmful effects).
c. Based upon our assessment of peer-reviewed medical literature, there is inconclusive evidence that the drug provides improvement in health outcomes in standard conditions of medical practice, outside the clinical investigatory settings.

Refer to Corporate Medical Policy #11.01.03 Experimental or Investigational Services

Medicare Part B, and D-SNP Plans:
Medicare reviews are to follow the Local Coverage Determination (LCD) for Drugs and Biologicals, Coverage of, for Label and Off-Label Uses (L33394). The LCD can be found on the CMS website at: https://www.cms.gov/medicare-coverage-database/view/lcd.aspx?lcdid=33394

Medicaid Managed Care (MMC) and Child Health Plus:

1. Must be prescribed by or in consultation with a neurologist, or provider that specializes in Amyotrophic Lateral Sclerosis (ALS) **AND**
2. Must be 18 years of age or older **AND**
3. Must have diagnosis of ALS with confirmed SOD1 mutation **AND**
4. Provider must attest that the patient has weakness attributable to ALS **AND**
5. Must not have tracheostomy (trach) **OR**
6. For patients who have a trach, the provider must attest that the trach is for prevention of aspiration only **AND**
7. Qalsody will not be covered for any other non-FDA approved indication
8. The recommended dosage is 100 mg (15 mL) of Qalsody per administration. Initiate Qalsody treatment with three (3) loading doses administered at 14-day intervals. Administer a maintenance dose every 28 days thereafter.
9. Initial and recertification will be for 6 months at a time. Recertification requires provider attestation for:
   a. The patient continues to benefit from therapy **AND**
   b. The patient is not dependent on invasive ventilation or tracheostomy (unless the trach is for prevention of aspiration only)
10. 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 GUIDELINES:

1. Approval will be granted for 6 months at a time if the above criteria are satisfied
2. Prior authorization is contract dependent
3. Not all contracts cover all Medical Infusible drugs. Refer to specific contract/benefit plan language for exclusions of Injectable Medications.
4. Qalsody is administered intrathecally and will be considered for coverage under the medical benefit.
5. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
6. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. Indications that have not been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan (which may include review per the Health Plan's Off-Label Use of FDA Approved Drugs policy). Step therapy requirements may be imposed in addition to LCD/NCD requirements.
7. Clinical documentation must be submitted for each request (initial and recertification) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments/treatment history, diagnostic testing, laboratory test results, genetic testing/biomarker results, imaging and other objective or subjective measures of benefit which support continued use of the requested product is medically necessary.
8. All requests will be reviewed to ensure they are being used for an appropriate indication and may be subject to an off-label review in accordance with our Off-Label Use of FDA Approved Drugs Policy (Pharmacy-32).
9. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
10. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.
CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:
Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).

HCPCS:

Drug Name	J-code (if assigned)	NDC
Qalsody	J1304	64406-0109-01

UPDATES:

Date	Revision
02/12/2026	P&T Committee Review & Approval
01/01/2026	Revised
11/19/2025	Revised
03/06/2025	Revised
02/06/2025	P&T Committee Review & Approval
01/16/2024	Policy # Changed to Pharmacy-127
12/15/2024	Revised
08/20/2024	Revised
04/12/2024	Created and Implemented
02/08/2024	P&T Committee Review/Approval

REFERENCES:

1. Qalsody [package insert]. Cambridge, MA: Biogen Inc.; April 2023.