Pharmacy Management Drug Policy

SUBJECT: Qalsody (tofersen)
POLICY NUMBER: PHARMACY-116
EFFECTIVE DATE: 04/2024
LAST REVIEW DATE: 04/12/2024

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/Essential/Medicaid 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 **experimental/investigational** 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 **experimental/investigational** 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 criteria:** 1. 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&ver=47

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.

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
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.