



Nonprofit corporations and independent licensees
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

Effective Date: 06/06/2024

Qalsody™ (tofersen)

HCPCS: J1304

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

- A. Coverage of the requested drug is provided when all the following are met:
 - a. FDA approved indication
 - b. FDA approved age
 - c. Confirmed superoxide dismutase 1 (SOD1) mutation
 - d. Vital capacity greater than 50% predicted
 - e. Submission of a baseline metrics from the ALSFRS-R
 - f. Currently receiving treatment and will continue to receive treatment with riluzole, if tolerated
 - g. Trial and failure, intolerance, or a contraindication to the preferred products as specified in the BCBSM/BCN medical utilization management drug list

- B. Quantity Limitations, Authorization Period and Renewal Criteria
 - a. Quantity Limits: Align with FDA recommended dosing
 - b. Authorization Period: One year at a time
 - c. Renewal Criteria: Continuation of coverage requires submission of patient assessments using the ALSFRS-R or other clinical documentation to determine if Qalsody is slowing the progression of ALS

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- Qalsody is indicated for the treatment of amyotrophic lateral sclerosis (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 observed in patients treated with Qalsody. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).
- ALS is the most common motor neuron disease in adults and is characterized by neurodegeneration of motor neurons in the brain and spinal cord. The incidence of ALS is approximately 1-2.6 cases per 100,000 persons annually, whereas the prevalence is approximately 6 cases per 100,000. The average age of onset of ALS is currently 58-60 years old and the average survival time from onset to death is 3-4 years. Mutations in the SOD1 gene account for approximately 2% of ALS cases (approximately 330 people in the US). In people with SOD1-ALS, mutations in their SOD1 gene cause their bodies to create a toxic misfolded form of SOD1 protein. This toxic protein causes motor neurons to degenerate, resulting in progressive muscle weakness, loss of function, and eventually, death.
- The American Academy of Neurology updated guidelines from 2009 (reaffirmed on January 11, 2020) titled "Update: The care of the patient with amyotrophic lateral sclerosis: drug, nutritional, and respiratory therapies" recommend that riluzole should be offered to slow the disease progression in patients with ALS. The European Federation of Neurological Sciences (EFNS) guidelines on clinical management of amyotrophic lateral sclerosis (MALS)-revised report of an EFNS task force, from 2012 also recommend riluzole in all patients. Neither set of guidelines have been updated to include the two most recent ALS drug approvals: Radicava® or Relyvrio™. There are no therapies listed specifically for ALS with SOD1 gene mutations
- The efficacy of Qalsody was assessed in the VALOR study, a Phase III, 28-week, randomized, double-blind, placebo-controlled clinical study in patients 23 to 78 years of age with weakness attributable to ALS and a SOD1 mutation confirmed by a central laboratory. One hundred eight (108) patients were randomized 2:1 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). Concomitant riluzole and/or edaravone use was permitted for patients, and at baseline 62% of patients were taking riluzole and 8% of patients were taking edaravone. The non-modified intent to treat population had a slow vital capacity $\geq 50\%$ of predicted value at baseline.
 - The trial showed a non-statistically significant 1.2-point difference in the Revised ALS Functional Rating Scale (ALSFRS-R) ($p = 0.97$).
 - Qalsody led to reductions in SOD1 by 35% compared to 2% for placebo and a 55% reduction in plasma neurofilament, a biomarker of neurodegeneration, compared to a 12% increase in the placebo-treated group.
 - Additional analysis of the Phase III data combined with an open-label extension (OLE) study showed that earlier initiation of Qalsody slowed decline across measures of clinical and respiratory function, strength, and quality of life.

References:

1. Qalsody [prescribing information]. Cambridge, MA; Biogen. April 2023
2. Talbott EO, Malek AM, Lacomis D. The epidemiology of amyotrophic lateral sclerosis. *Handb Clin Neurol*. 2016;138:225-38. doi: 10.1016/B978-0-12-802973-2.00013-6. PMID: 27637961.
3. R. G. Miller, C. E. Jackson, E. J. Kasarskis, J. D. England, D. Forshev, W. Johnston, S. Kalra, J. S. Katz, H. Mitsumoto, J. Rosenfeld, C. Shoesmith, M. J. Strong, S. C. Woolley 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* Oct 2009, 73 (15) 1218-1226; DOI: 10.1212/WNL.0b013e3181bc0141
4. Andersen, P.M., Abrahams, S., Borasio, G.D., de Carvalho, M., Chio, A., Van Damme, P., Hardiman, O., Kollewe, K., Morrison, K.E., Petri, S., Pradat, P.-F., Silani, V., Tomik, B., Wasner, M. and Weber, M. (2012), EFNS guidelines on the Clinical Management of Amyotrophic Lateral Sclerosis (MALS) – revised report of an EFNS task force. *European Journal of Neurology*, 19: 360-375. <https://doi.org/10.1111/j.1468-1331.2011.03501>.

Policy History												
#	Date	Change Description										
1.4	Effective Date: 06/06/2024	Annual review of policy. No changes to criteria were made.										
1.3	Effective Date: 08/01/2023	UM medical management system update for MAPPO and BCNA <table border="1" style="margin-left: 20px;"> <thead> <tr> <th>Line of Business</th> <th>PA Required in Medical Management System (Yes/No)</th> </tr> </thead> <tbody> <tr> <td>BCBS</td> <td>Yes</td> </tr> <tr> <td>BCN</td> <td>Yes</td> </tr> <tr> <td>MAPPO</td> <td>Yes</td> </tr> <tr> <td>BCNA</td> <td>Yes</td> </tr> </tbody> </table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	Yes	BCNA	Yes
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	Yes											
BCNA	Yes											
1.2	Effective Date: 06/08/2023	New policy										
1.1	Effective Date: 05/18/2023	UM medical management system update for BCBS and BCN <table border="1" style="margin-left: 20px;"> <thead> <tr> <th>Line of Business</th> <th>PA Required in Medical Management System (Yes/No)</th> </tr> </thead> <tbody> <tr> <td>BCBS</td> <td>Yes</td> </tr> <tr> <td>BCN</td> <td>Yes</td> </tr> <tr> <td>MAPPO</td> <td>No</td> </tr> <tr> <td>BCNA</td> <td>No</td> </tr> </tbody> </table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	No	BCNA	No
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	No											
BCNA	No											
1.0	Effective Date: 02/02/2023	Preliminary drug review										

* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.

**Blue Cross Blue Shield/Blue Care Network of Michigan
Medication Authorization Request Form
Qalsody (tofersen)
HCPCS CODE: J1304**



This form is to be used by participating physicians to obtain coverage for Qalsody. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

Nonprofit corporations and independent licensees of the Blue Cross and Blue Shield Association

PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	Name
ID Number	Specialty
D.O.B. <input type="checkbox"/> Male <input type="checkbox"/> Female	Address
Diagnosis	City /State/Zip
Drug Name <input type="checkbox"/> Qalsody	Phone/Fax: P: () - F: () -
Dose and Quantity	NPI
Directions	Contact Person
Date of Service(s)	Contact Person Phone / Ext.

STEP 1: DISEASE STATE INFORMATION

1. Is this request for: Initiation Continuation of therapy *Date patient started therapy:* _____

2. Please provide the NPI number for the place of administration: _____

3. Please specify the location of administration (e.g. name of facility): _____

4. Initiation AND Continuation of therapy:

a. What is the patient's diagnosis?

- Amyotrophic lateral sclerosis (ALS)
 Other – *please specify diagnosis:* _____

b. Does the patient have a confirmed superoxide dismutase 1 (SOD1) mutation? ***(Please attach any tests confirming diagnosis)***

- Yes
 No, please specify: _____

c. What is the patient's current predicted vital capacity (%)? _____

d. What is the patient's baseline score from ALS Functional Rating scale(ALSFRS-R)? _____

e. Is the patient receiving treatment with riluzole and will the patient continue to receive treatment with riluzole?

- Yes No *Comment* _____

5. Continuation of therapy

a. If the patient is continuing therapy, please give the patient's current disease status since beginning treatment:

- Improved: Please describe: _____
 Stable; Please describe: _____
 Worsened; Please describe: _____
 Other; Please describe: _____

6. *Please add any other supporting medical information necessary for our review*

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name	Physician Signature	Date
Step 2: Checklist	<input type="checkbox"/> Form Completely Filled Out <input type="checkbox"/> Attached necessary chart notes	<input type="checkbox"/> Important laboratory results
Step 3: Submit	By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979	By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320

Confidentiality notice: This transmission contains confidential information belonging to the sender that is legally privileged. This information is intended only for use of the individual or entity named above. The authorized recipient of this information is prohibited from disclosing this information to any other party. If you are not the intended recipient, you are hereby notified that any disclosure, copying, distribution or action taken in reliance on the contents of this document is strictly prohibited. If you have received this in error, please notify the sender to arrange for the return of this document.