Protocol for Qalsody® (tofersen)

Approved July 2023

Background: *Amyotrophic lateral sclerosis (ALS) is a relentlessly progressive, presently incurable neurodegenerative disorder that causes muscle weakness, disability, and eventually death.*

Qalsody *is an antisense oligonucleotide indicated for the treatment of ALS in adults who have a mutation in the superoxide dismutase 1 (SOD1) gene.*

Note: This indication is approved under accelerated approval based on reduction in plasma neurofilament light (NfL) chain observed in patients treated with Qalsody. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).

Criteria for approval:

1. Patient is 18 years of age or older; **AND**
2. Patient has a diagnosis of ALS; **AND**
3. Patient has weakness attributable to ALS and has a confirmed mutation in the superoxide dismutase 1 (SOD1) gene **AND**
4. Other motor disorders have been ruled out [e.g., dementia, schizophrenia, and other neurodegenerative diseases such as primary lateral sclerosis (PLS)]
5. Medication is prescribed by or in consultation with a neurologist, neuromuscular specialist, or a physician who specializes in the treatment of ALS **AND**
6. Baseline plasma neurofilament light chain (NfL) levels have been measured **AND**
7. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Continuation of therapy:

1. Compared to baseline, there are reductions in plasma neurofilament light chains (NfL) **AND**
2. Prescriber attests to continued monitoring of NfL **AND**
3. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 6 months

Renewal Approval: 6 months

References:

1. Qalsody [prescribing information]. Biogen MA Inc. Cambridge, MA 02142. April 2023
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
3. Miller TM, Cudkowicz ME, Genge A, et al; VALOR and OLE Working Group. Trial of antisense oligonucleotide tofersen for SOD1 ALS. N Engl J Med. 2022;387(12):1099-1110. doi:10.1056/NEJMoa2204705
4. National Organization for Rare Disorders. Amyotrophic Lateral Sclerosis. Accessed May 5, 2023 at https://rarediseases.org/rare-diseases/amyotrophic-lateral-sclerosis/