Effective Date: 3/1/2023
Reviewed: 12/22/22
Scope: Medicaid

RELYVRIO (sodium phenylbutyrate and taurursodiol)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Relyvrio is indicated for the treatment of amyotrophic lateral sclerosis (ALS) in adults.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
Chart notes or medical record documentation supporting use as applicable in section IV and V.

A. Initial Requests:
   1. Diagnosis of probable or definite ALS
   2. Slow Vital Capacity (SVC) >60% of predicted value for gender, height, and age
   3. Date of symptom onset

B. Continuation Requests:
   1. Documentation of clinical benefit from Relyvrio therapy

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a neurologist, neuromuscular specialist or physician specializing in the treatment of amyotrophic lateral sclerosis (ALS).

IV. CRITERIA FOR INITIAL APPROVAL

Authorization of 6 months may be granted for treatment of ALS when all of the following criteria are met:
A. Diagnosis of probable or definite ALS
B. Member is 18 years of age or older
C. Slow Vital Capacity (SVC) >60% of predicted value for gender, height, and age
D. Member does not have a tracheostomy
E. Member's symptom onset within the past 18 months

V. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for members continuing with Relyvrio therapy for the treatment of ALS when the following criteria are met:

A. Diagnosis of probable or definite ALS
B. Member is tolerating therapy without significant adverse effects and there is a clinical benefit demonstrated from Relyvrio therapy
C. Patient's respiratory status does not require invasive ventilation or tracheostomy

VI. QUANTITY LIMIT

Relyvrio has a quantity limit of 2 packets per day.

Indication	Dose
ALS	1 packet (3 g sodium phenylbutyrate and 1 g taurursodiol) daily for the first 3 weeks, then 1 packet twice daily thereafter.

VII. REFERENCES

1. Relyvrio [package insert]. Cambridge, MA: Amylyx Pharmaceuticals, Inc.; September 2022.
2. EFNS Task Force on Diagnosis and Management of Amyotrophic Lateral Sclerosis; Andersen PM, et al. EFNS guidelines on the Clinical Management of Amyotrophic Lateral Sclerosis (MALS) – revised report of an EFNS task force. *Eur J Neurol.* 2012;19(3):360-75.
3. Paganoni S, Macklin EA, Hendrix S, et al. Trial of sodium phenylbutyrate-taurursodiol for amyotrophic lateral sclerosis. N Engl J Med 2020; 383:919-30.
4. Paganoni S, Hendrix S, Dickson SP, et al. Long-term survival of participants in the CENTAUR trial of sodium phenylbutyrate-taurursodiol in amyotrophic lateral sclerosis. Muscle Nerve 2021; 63:31-9.
5. Paganoni S, Hendrix S, Dickson SP, et al. Effect of sodium phenylbutyrate/taurursodiol on tracheostomy/ventilation-free survival and hospitalization in amyotrophic lateral sclerosis: long-term results from the CENTAUR trial. J Neurol Neurosurg Psychiatry 2022.