MEDICAL MUTUAL®

Drug Policy

Policy:	Firdapse (amifampridine) tablets Ruzurgi (amifampridine) tablets	Annual Review Date: 01/18/2024 Last Revised Date: 01/18/2024

OVERVIEW

Amifampridine is a broad-spectrum potassium channel blocker. Firdapse is indicated for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults. Ruzurgi is indicated for the treatment of LEMS in in patients 6 years to < 17 years of age. The mechanism by which amifampridine exerts its therapeutic effect in patients with LEMS has not been fully elucidated.

LEMS is a rare autoimmune disorder affecting the connection between nerves and muscles and causing proximal muscle weakness, autonomic dysfunction, and areflexia. The characteristic weakness is thought to be caused by antibodies generated against the P/Q-type voltage-gated calcium channels (VGCC) present on presynaptic nerve terminals and by diminished release of acetylcholine (ACh). More than half of LEMS cases are associated with small cell lung carcinoma (SCLC), which expresses functional VGCC. The diagnosis of LEMS is confirmed by electrodiagnostic studies, including repetitive nerve stimulation (RNS), or anti-P/Q-type VGCC antibody testing to confirm the diagnosis.

POLICY STATEMENT

This policy involves the use of Firdapse and Ruzurgi. Prior authorization is recommended for pharmacy benefit coverage of Firdapse and Ruzurgi. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Firdapse and Ruzurgi as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Firdapse and Ruzurgi be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Firdapse and Ruzurgi is recommended in those who meet the following criteria:

1. Lambert-Eaton Myasthenic Syndrome (LEMS).

A) Initial therapy: Approve if the patient meets the following criteria:

i. Patient is 18 years of age or older if requesting Firdapse, or 6 years of age or older if requesting Ruzurgi; AND
ii. Patient has confirmed LEMS based on at least one electrodiagnostic study (e.g., repetitive nerve stimulation) or anti-P/Q-type voltage-gated calcium channels antibody testing, according to the prescribing physician; AND
iii. Patient has moderate to severe weakness that interferes with daily functions; AND
iv. Patient does not have a history of seizures; AND
v. The requested agent is being prescribed by or in consultation with a neurologist or a neuromuscular specialist

B) Patients continuing therapy with Firdapse or Ruzurgi: Approve if the patient has had a clinical response to therapy, as determined by the prescriber.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 3 months
B) *Extended Approval:* 1 year

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Firdapse and Ruzurgi have not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Concomitant use with another amifampridine or dalfampridine product (e.g. Ampyra, Firdapse, Ruzurgi).**

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Firdapse® tablets [prescribing information]. Coral Gables, FL: Catalyst Pharmaceuticals, Inc.; November 2018.
2. FDA news release. FDA approves first treatment for Lambert-Eaton myasthenic syndrome, a rare autoimmune disorder. Issued on: November 28, 2018. Available at: https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm627093.htm. Accessed on November 30, 2018.

3. Kesner VG, Oh SJ, Dimachkie MM, et al. Lambert-Eaton Myasthenic Syndrome. *Neurol Clin*. 2018;36(2):379-394.
4. Oh S, Shcherbakova N, Kostera-Pruszczyk A, et al. Amifampridine phosphate (Firdapse®) is effective and safe in a phase 3 clinical trial in LEMS. *Muscle Nerve*. 2016;53(5):717-25.
5. Weinberg, DH. Lambert-Eaton myasthenic syndrome: Treatment and prognosis. Updated: January 07, 2019. Available at: https://www.uptodate.com. Accessed on January 16, 2019
6. Amifampridine. In: DRUGDEX [online database]. Truven Health Analytics; Greenwood Village, CO. Last updated 6 December 2019. Accessed on 21 January 2020.
7. Ruzurgi [prescribing information]. Jacobus Pharmaceutical Company Inc. Plainsboro, NJ. May 2019.