Lambert-Eaton Myasthenic Syndrome (LEMS) Products
Approved January 2020

Firdapse® (amifampridine)
Ruzurgi® (amifampridine)

Background:

Lambert-Eaton myasthenic syndrome (LEMS) *is a rare autoimmune disorder of the neuromuscular junction. It is a miscommunication between the nerve cell and the muscles that lead to the gradual onset of muscle weakness.*

Firdapse *is a potassium channel blocker indicated for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults. It blocks voltage-dependent potassium channels, thereby prolonging presynaptic cell membrane depolarization, which enhances calcium transport into nerve endings. The increased intracellular calcium concentrations facilitate exocytosis of acetylcholine-containing vesicles, which, in turn, enhances neuromuscular transmission.*

Ruzurgi *is also a potassium channel blocker indicated for the treatment of LEMS in patients 6 to less than 17 years of age.*

Criteria for approval:

1. Patient is 6 to 16 years old (for Ruzurgi); OR
2. Patient is an adult, 17 years or older (for Firdapse); AND
3. Patient has a documented diagnosis of LEMS; AND
4. Diagnosis has been confirmed by one electrodiagnostic study (e.g., repetitive nerve stimulation) OR anti-P/Q-type voltage-gated calcium channels antibody testing; AND
5. Patient does not have a history of seizures; AND
6. Medication is being prescribed by or in consultation with a neurologist, pediatric neurologist, or a neuromuscular specialist
7. Patient is not receiving medication in combination with similar potassium channel blockers [for example, Ampyra (dalfampridine)]
8. Patient does not have end-stage renal disease (CrCl less than 15 mL/minute, or on dialysis, or post renal transplant)
9. Documentation of patient's baseline clinical muscle strength assessment for at least one of the following:
    i. Quantitative Myasthenia Gravis (QMG) score
    ii. Triple-Timed Up-and-Go test (3TUG)
    iii. Timed 25-foot Walk test (T25FW)
10. Weight must be received for drugs that have weight-based dosing. Height and weight must be received for drugs that have dosing based on body surface area.
11. 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, or national guidelines.

Initial Approval Duration: 6 months

Continuation of therapy:

1. Documentation that patient experienced a positive clinical response to therapy as evidenced by one of the following clinical muscle strength assessments:
   i. Quantitative Myasthenia Gravis (QMG) score
   ii. Triple-Timed Up-and-Go test (3TUG)
   iii. Timed 25-foot Walk test (T25FW)
2. Patient is not receiving medication in combination with similar potassium channel blockers [for example, Ampyra (dalfampridine)]
3. Patient does not have end-stage renal disease (CrCl less than 15 mL/minute, or on dialysis, or post renal transplant)
4. 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, or national guidelines

Renewal Approval Duration: 12 months

References:

1. Firdapse [prescribing information]. Coral Gables, FL; Catalyst Pharmaceuticals, Inc; November 2018
2. Ruzurgi [prescribing information]. Plainsboro, NJ; Jacobus Pharmaceuticals, Inc; May 2019
3. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2018. Updated periodically
4. Weinberg, D.H., Lambert-Eaton myasthenic syndrome: Treatment and prognosis. (2019). UpToDate. From: https://www.uptodate.com/contents/lambert-eaton-myasthenic-syndrome-treatment-and-prognosis/print. Accessed: 10.30.19