PREFERRED SPECIALTY MANAGEMENT POLICY

POLICY: Amifampridine Products Preferred Specialty Management Policy
- Firdapse® (amifampridine tablets – Catalyst Pharmaceuticals)
- Ruzurgi® (amifampridine tablets – Jacobus Pharmaceutical)

REVIEW DATE: 06/23/2021

OVERVIEW

Amifampridine, a broad spectrum potassium channel blocker, is approved for the **treatment of Lambert-Eaton myasthenic syndrome** (LEMS).[1,2]
- Firdapse is indicated in **adults**.[1]
- Ruzurgi is indicated in **patients 6 years to < 17 years of age**.[2]

Although Ruzurgi is indicated for use in children, the efficacy of Ruzurgi for the treatment of LEMS was established in one randomized, double-blind, placebo-controlled, withdrawal study in adults with an established diagnosis of LEMS (n = 32).[2] The efficacy of Ruzurgi in patients 6 to < 17 years of age is supported by evidence from adequate and well-controlled studies of Ruzurgi in adults with LEMS, pharmacokinetic data in adult patients, pharmacokinetic modeling and simulation to identify the dosing regimen in pediatric patients, and safety data from pediatric patients 6 to < 17 years of age. Firdapse and Ruzurgi are available as scored 10 mg tablets.[1,2] Firdapse labeling does not include instructions for making an oral suspension from the tablets.[1] Ruzurgi labeling includes instructions for preparing a 1 mg/mL suspension using three 10 mg tablets and mixing with 30 mL of sterile water.[2]

Disease Overview

LEMS is a rare autoimmune disorder affecting the connection between nerves and muscles and causing proximal muscle weakness, autonomic dysfunction, and areflexia.[3,4] LEMS can occur at any age. The prevalence of LEMS specifically in pediatric patients is not known, but the overall prevalence of LEMS is estimated to be three per million individuals worldwide.[3] 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.[4] More than half of LEMS cases are associated with small cell lung carcinoma, which expresses functional VGCC. The diagnosis of LEMS is confirmed by electrodiagnostic studies, including repetitive nerve stimulation, or anti-P/Q-type VGCC antibody testing to confirm the diagnosis.

Safety

Firdapse and Ruzurgi are contraindicated in patients with a history of seizures.[1,2] There is also a Warning/Precaution in the prescribing information for these medications because seizures have been observed in patients with and without a history of seizures taking amifampridine at the recommended doses. Many of these patients were taking medications or had comorbidities that may have lowered their seizure threshold. Seizures may be dose-dependent. The discontinuation or dose-reduction of amifampridine should be considered in patients who have a seizure while on treatment.

POLICY STATEMENT

This Preferred Specialty Management program has been developed to encourage the use of the Preferred Product. For all medications (Preferred and Non-Preferred), the patient is required to meet the standard *Prior Authorization Policy* criteria. The program also directs the patient to try one Preferred Product prior

to the approval of a Non-Preferred Product. Requests for Non-Preferred Products will also be reviewed using the exception criteria (below). All approvals are provided for the duration noted below. If the patient meets the standard *Prior Authorization Policy* criteria but has not tried a Preferred Product, approval for a Preferred Product will be authorized.

Automation: None.

Preferred Product: Ruzurgi
Non-Preferred Product: Firdapse

RECOMMENDED EXCEPTION CRITERIA

Trade Name	Exception
Firdapse	**1.** Approve for 1 year if the patient meets the following criteria (A and B): **A)** Patient meets the standard *Amifampridine Prior Authorization Policy* criteria; AND **B)** Patient meets both of the criteria below (i and ii): **i.** Patient has tried the Preferred Product (Ruzurgi) [see Note below]; AND **ii.** Patient has demonstrated inadequate efficacy or unacceptable safety or tolerability to Ruzurgi, according to the prescriber. **2.** If the patient has met the standard *Amifampridine Prior Authorization Policy* criteria (1A), but has not met exception criteria (1B) above for Firdapse: approve Ruzurgi.

Note: The efficacy of Ruzurgi for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) was established in one pivotal study in adults with an established diagnosis of LEMS (n = 32). The use of Ruzurgi in patients 6 years to < 17 years of age is supported by studies of Ruzurgi in adults with LEMS, pharmacokinetic data in adults, and pharmacokinetic modeling to identify the dosing regimen in pediatric patients.

REFERENCES

1. Firdapse® tablets [prescribing information]. Coral Gables, FL: Catalyst Pharmaceuticals; February 2021.
2. Ruzurgi® tablets [prescribing information]. Princeton, NJ: Jacobus Pharmaceutical Company; May 2019.
3. FDA news release. FDA approves first treatment for children with Lambert-Eaton myasthenic syndrome, a rare autoimmune disorder. Issued on: May 6, 2019. Available at: https://www.fda.gov/news-events/press-announcements/fda-approves-first-treatment-children-lambert-eaton-myasthenic-syndrome-rare-autoimmune-disorder. Accessed on June 17, 2021.
4. Kesner VG, Oh SJ, Dimachkie MM, et al. Lambert-Eaton Myasthenic Syndrome. *Neurol Clin*. 2018;36(2):379-394.
5. 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.