SmartPA Criteria Proposal

Drug/Drug Class:	Lambert-Eaton Myasthenic Syndrome (LEMS) Clinical Edit
First Implementation Date:	January 30, 2020
Proposed Date:	June 16, 2022
Prepared for:	MO HealthNet
Prepared by:	MO HealthNet/Conduent
Criteria Status:	☐Existing Criteria ☒Revision of Existing Criteria ☐New Criteria

Executive Summary

Purpose: Ensure appropriate utilization and control of agents for Lambert-Eaton myasthenic syndrome (LEMS)

Why Issue Selected: Lambert-Eaton myasthenic syndrome (LEMS) is a rare autoimmune disorder that affects the connection between nerves and muscles and causes weakness and other symptoms in affected patients. Current treatment strategies for LEMS include initial therapy to increase the amount of acetylcholine available at the post-synaptic membrane with agents such as pyridostigmine, amifampridine, and guanidine; since pyridostigmine is readily available and well-tolerated, it is usually the first step in therapy. Amifampridine is a broad spectrum potassium channel blocker; the exact mechanism in which it exerts its therapeutic effect in LEMS is unknown. On November 28, 2018, Firdapse® (amifampridine) was FDA approved for the treatment of LEMS in adults. It was the first FDA-approved drug for treatment of LEMS in adults, which comprise the majority of LEMS patients; however, the manufacturer of Firdapse, Catalyst, was widely criticized in the media for pricing Firdapse at $375,000 for one year of treatment (prior to this Jacobus Pharmaceutical was providing amifampridine free of charge to patients that were enrolled in a compassionate use protocol). On May 6, 2019, Jacobus Pharmaceutical's Ruzurgi® (amifampridine) was FDA approved for the treatment of LEMS in patients aged 6 to less than 17 years of age. In the same year, Catalyst filed a suit against the FDA regarding its approval of Ruzurgi. On February 3, 2022, Catalyst Pharmaceuticals announced that a summary judgement was entered in its favor in the company's litigation against the FDA. As a result of the court's decision, the FDA approval of Ruzurgi is no longer valid. Firdapse and Ruzurgi contain the same active drug and are considered therapeutically equivalent.

Due to high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of agents for LEMS Disease.

Program-Specific Information:

Date Range FFS 4-1-2021 to 3-31-2022			
Drug	**Claims**	**Cost per tab**	**Cost per month (based on 30mg/day)**
FIRDAPSE 10 MG TABLET	0	$197.28 MAC	$17,755.20 MAC
RUZURGI 10 MG TABLET	0	$79.60 MAC	$7,164.00 MAC

Type of Criteria:	☐ **Increased risk of ADE**	☐ **Preferred Drug List**
	☒ **Appropriate Indications**	☒ **Clinical Edit**
Data Sources:	☐ **Only Administrative Databases**	☒ **Databases + Prescriber-Supplied**

Setting & Population

- Drug class for review: agents for the treatment of Lambert-Eaton myasthenic syndrome (LEMS)
- Age range: All appropriate MO HealthNet participants aged 6 years or older

Approval Criteria

- Participant aged 6 years or older **AND**
- Diagnosis of LEMS consistent with 1 of the following:
  - Repetitive Nerve Stimulation (RNS) showing reproducible post-exercise increase in compound muscle action potential (CMAP) amplitude of at least 60% compared with pre-exercise baseline value or a similar increment on high-frequency repetitive nerve stimulation without exercise **OR**
  - Positive anti-P/Q type voltage-gated calcium channel antibody test **AND**
- Documentation of clinical symptoms suggestive of LEMS (proximal weakness affecting legs, eyes, face, throat) **AND**
- For first claim only:
  - Documented trial of pyridostigmine defined as 15 days of therapy in the past 30 days **AND**
  - Documentation of N-acetyltransferase 2 (NAT2) testing prior to initiation of therapy for dose determination **AND**
- ~~**For Firdapse:**~~
  - ~~**Documented trial of Ruzurgi (defined as 180 days out of 210 days) AND**~~
  - ~~**Clinical consultant review required**~~
- Renewal Criteria:
  - Initial approval of prior authorization is 3 months
  - Renewal of prior authorization may be up to 12 months following documentation of the following:
    - All approval criteria listed above
    - Lack of ADE/ADR to therapy
    - Documentation of clinical benefit of therapy (less than expected decline in functional ability and/or symptoms of disease)

Denial Criteria

- Therapy will be denied if all approval criteria are not met
- Documented history of a seizure disorder

Required Documentation

Laboratory Results:	X	Progress Notes:	X
MedWatch Form:		Other:	

Disposition of Edit

Denial: Exception code “0682” (Clinical Edit)
Ryle Type: CE

Default Approval Period

3 months

References

- FIRDAPSE® (amifampridine) [package insert]. Coral Gables, FL: Catalyst Pharmaceuticals, Inc; February 2021.
- RUZURGI® (amifampridine) [package insert]. Plainsboro, NJ: Jacobus Pharmaceutical Company, Inc; April 2020.
- Muscular Dystrophy Association. Lambert-Eaton Myasthenic Syndrome (LEMS). https://www.mda.org/disease/lambert-eaton-myasthenic-syndrome. Accessed May 16, 2022.
- IPD Analytics. New Drug Approval: Firdapse (amifampridine). December 2018.
- IPD Analytics. New Drug Approval: Ruzurgi (amifampridine). May 2019.
- IPD Analytics. Patients with LEMS Will Transition to Catalyst's Firdapse as Ruzurgi Approval Overturned. Trending Industry & Strategy Topics. February 7, 2022.