CADTH

CADTH REIMBURSEMENT REVIEW

Stakeholder Feedback on Draft Recommendation

amifampridine phosphate (Firdapse)

(KYE Pharmaceuticals Inc.)

Indication: Lambert-Eaton Myasthenic Syndrome, adults

June 3, 2022

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CADTH Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0664
Name of the drug and Indication(s)	Amifampridine phosphate (Firdapse) for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults
Organization Providing Feedback	FWG

1. Recommendation revisions Please indicate if the stakeholder requires the expert review committee to reconsider or clarify its recommendation.		
Request for Reconsideration	**Major revisions:** A change in recommendation **category** or patient **population** is requested	☐
	Minor revisions: A change in reimbursement **conditions** is requested	☐
No Request for Reconsideration	**Editorial revisions:** Clarifications in recommendation **text** are requested	x
	No requested revisions	☐

2. Change in recommendation category or conditions Complete this section if major or minor revisions are requested
Please identify the specific text from the recommendation and provide a rationale for requesting a change in recommendation.

3. Clarity of the recommendation Complete this section if editorial revisions are requested for the following elements
a) Recommendation rationale
Please provide details regarding the information that requires clarification.
b) Reimbursement conditions and related reasons
1. In the renewal criteria, it would be helpful to clarify that 'alternative measurements' refer to non-ambulatory patients. 2. In the discussion points, clarification should be added that the less specific scoring system refers to the QMG. 3. In Table 2: Summary of Drug Plan Input and Clinical Expert Response: ▪ the reference to Ruzurgi's Health Canada approved indication should be removed. Ruzurgi no longer has Health Canada approval.

- Suggest removal of the following sentence to help with recommendation clarity:
  Considering the variability of resources across Canada, a combination of assessments as determined by the treating physician would be reasonable.

c) Implementation guidance

Please provide high-level details regarding the information that requires clarification. You can provide specific comments in the draft recommendation found in the next section. Additional implementation questions can be raised here.

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CADTH Reimbursement Review
Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0664
Brand name (generic)	FIRDAPSE (amifampridine phosphate)
Indication(s)	For the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS) in adults.
Organization	KYE Pharmaceuticals Inc.
Contact information[a]	Name: Eryn Corriveau

Stakeholder agreement with the draft recommendation		
1. Does the stakeholder agree with the committee's recommendation.	Yes	☒
	No	☐

KYE agrees with the committee's recommendation for the following reasons:

- Given "*no other effective alternative treatment options are currently available,*" it is important to fill the unmet need and provide Canadian patients with an effective treatment option for the symptomatic treatment of LEMS.
- Results of LMS-002 and LMS-003 demonstrated that "*continuous treatment of patients with amifampridine phosphate was associated with a reduction in disability progression compared with patients for whom amifampridine phosphate was replaced with placebo*." KYE agrees with the committee that FIRDAPSE demonstrates a clinical benefit for the symptomatic treatment of LEMS patients.
- The committee noted that "*amifampridine phosphate was less costly compared with amifampridine base and considered similarly effective*." Based on the results of DAPSEL, the efficacy of amifampridine phosphate and amifampridine base were not expected to differ. Thus, KYE agrees with the committee that FIRDAPSE represents the least costly formulation of amifampridine, an effective treatment option for LEMS.

Expert committee consideration of the stakeholder input		
2. Does the recommendation demonstrate that the committee has considered the stakeholder input that your organization provided to CADTH?	Yes	☐
	No	☐

Not Applicable.

Clarity of the draft recommendation		
3. Are the reasons for the recommendation clearly stated?	Yes	☒
	No	☐

n/a

4. Have the implementation issues been clearly articulated and adequately addressed in the recommendation?	Yes	☒
	No	☐

n/a

5. If applicable, are the reimbursement conditions clearly stated and the rationale for the conditions provided in the recommendation?	Yes	☒
	No	☐

n/a

[a] CADTH may contact this person if comments require clarification.