CADTH

CADTH REIMBURSEMENT REVIEW

Stakeholder Feedback on Draft Recommendation

Givosiran
(Givlaari)
(Alnylam Netherlands B.V.)

Indication : Acute hepatic porphyria (AHP) in adults

August 26, 2021

CADTH

CADTH Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0679
Name of the drug and Indication(s)	Givlaari (givosiran) for the treatment of acute hepatic porphyria (AHP) 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
N/A

3. Clarity of the recommendation Complete this section if editorial revisions are requested for the following elements
a) Recommendation rationale
N/A
b) Reimbursement conditions and related reasons
N/A
c) Implementation guidance

FWG suggests rewording and moving the Implementation Guidance point on case-by-case criteria (bullet #4) to a discussion point since it may not be possible for drug programs to implement such criteria consistently and systematically due to lack of evidence.

August 25, 2021

RE: Feedback on Draft Reimbursement Recommendations for Givosiran

Dear CADTH,

Thank you for the opportunity to review and comment on the draft reimbursement recommendations for Givosiran (SR0679). The recommendation for reimbursement of Givosiran for acute hepatic porphyria (AHP) patients is a very positive step to giving AHP patients treatment options. The Canadian Association for Porphyria (CAP) is concerned, however, that some of the reimbursement conditions may be open to interpretation in a way that could limit patient access, and would like more clarity into how these conditions should be applied. A summary of our concerns is below:

Reimbursement Condition	CAP/ACP Interpretation Concerns	Reason
Reimbursement Condition #1 (p. 4)- Restricted to patients with 4 or more attacks requiring either hospitalization, an urgent care visit, or intravenous hemin in the year prior to the prescribing date.	• Does establishing the annual rate require that AHP patients who are newly diagnosed need to wait a year prior to being prescribed givosiran in order to establish the baseline? Or can they begin treatment once they've had 4 attacks? • If the AHP patient has frequent, recurrent attacks (3 attacks in three months, for example), would they need to wait until they've had a fourth attack? Or would the frequency be extrapolated?	• We are concerned that AHP patients will suffer needlessly if they are required to wait for a year before they can access treatment in order to establish a baseline.
Reimbursement Condition 2 (p. 4)- A reduction in the annualized attack rate after twelve months of therapy compared to baseline.	• If a newly diagnosed AHP patient can begin treatment with givosiran after four attacks in less than a year, would the baseline attack rate be extrapolated for the full year? For example: would four attacks in four months translate to an	• We are concerned that newly diagnosed AHP patients will not be able to renew their prescription if the rate is not annualized.

	annualized baseline of 12 attacks?	
Reimbursement Condition #3 (p.4) - Prescription should be restricted to clinicians experienced in the management of AHP.	● How would an experienced clinician be determined?	● We are concerned about access. There are only a handful of clinicians in Canada who have multiple porphyria patients, and some provinces do not yet have any clinicians managing AHP patients. Will an AHP patient's doctor or specialist be able to prescribe?
Implementation Guidance #4 (p. 4)- CDEC noted that the participating drug plans and the clinical expert community may need to establish case-by-case evaluation criteria for the initiation and continuation of givosiran in certain patients.	Is this case-by-case determination meant to be an alternate way of assessing need (for patients who have severe chronic pain, for example) or does it mean that additional criteria could be applied (beyond the 4 attacks/year) for eligibility? What would the process be for determining the additional criteria? Who would be involved and how would patients access treatment?	● Although we feel that case-by-case evaluation could benefit AHP patients, we are concerned that this guidance may be implemented in a way that limits access for AHP patients.

We would be happy to provide further information or clarifications. Please direct any further questions to [redacted] at [redacted] or by phone at [redacted]

Yours sincerely,

Wendy Sauvé
Past-President (CAP)

Jeannie Reimer
Treasurer (CAP)