Canada's Drug Agency
L'Agence des médicaments du Canada

CDA-AMC REIMBURSEMENT REVIEW

Stakeholder Feedback on Draft Recommendation

epontersen (Wainua)

(AstraZeneca Canada Inc.)

Indication: Wainua (eplontersen injection) is indicated for the treatment of polyneuropathy associated with stage 1 or stage 2 hereditary transthyretin-mediated amyloidosis (hATTR) in adults.

October 4, 2024

CADTH Reimbursement Review
Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0826-000
Brand name (generic)	Eplontersen aka WAINUA for hATTR-PN
Indication(s)	Amyloidosis Neuropathy
Organization	Hereditary Amyloidosis Canada
Contact information[a]	Name: Marie Carr

Stakeholder agreement with the draft recommendation		
1. Does the stakeholder agree with the committee's recommendation.	Yes	x
	No	☐
Please explain why the stakeholder agrees or disagrees with the draft recommendation. Whenever possible, please identify the specific text from the recommendation and rationale.		

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	x
	No	☐
If not, what aspects are missing from the draft recommendation?		

Clarity of the draft recommendation		
3. Are the reasons for the recommendation clearly stated?	Yes	X
	No	☐
If not, please provide details regarding the information that requires clarification.		
4. Have the implementation issues been clearly articulated and adequately addressed in the recommendation?	Yes	x
	No	☐
If not, please provide details regarding the information that requires clarification.		
5. If applicable, are the reimbursement conditions clearly stated and the rationale for the conditions provided in the recommendation?	Yes	x
	No	☐
If not, please provide details regarding the information that requires clarification.		

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

[redacted]

Appendix 1. Conflict of Interest Declarations for Patient Groups

- To maintain the objectivity and credibility of the CADTH drug review programs, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest.
- This conflict of interest declaration is required for participation. Declarations made do not negate or preclude the use of the feedback from patient groups and clinician groups.
- CADTH may contact your group with further questions, as needed.
- Please see the *Procedures for CADTH Drug Reimbursement Reviews* for further details.

info@madhattr.ca

A. Patient Group Information		
Name	*Please state full name*	Anne Marie Carr
Position	*Please state currently held position*	Founder & Executive Director
Date	*Please add the date form was completed (DD-MM-YYYY)*	03-10-2024
☒	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.	

B. Assistance with Providing Feedback		
1. Did you receive help from outside your patient group to complete your feedback?	No	X ☐
	Yes	☐
If yes, please detail the help and who provided it.		
2. Did you receive help from outside your patient group to collect or analyze any information used in your feedback?	No	X ☐
	Yes	☐
If yes, please detail the help and who provided it.		

C. Previously Disclosed Conflict of Interest		
1. Were conflict of interest declarations provided in patient group input that was submitted at the outset of the CADTH review and have those declarations remained unchanged? If no, please complete section D below.	No	☒
	Yes	☐

D. New or Updated Conflict of Interest Declaration

3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

Company	Check Appropriate Dollar Range			
	$0 to 5,000	**$5,001 to 10,000**	**$10,001 to 50,000**	**In Excess of $50,000**
Add company name Pfizer	☐	☐	☐	X ☐
Add company name Alnylam	☐	☐	☐	X ☐
Add or remove rows as required Astra Zeneca	☐	☐	☐	X ☐

Bridge Bio $10 001 to $50 000 X

SOBI $10 001 to $50 000 X

[redacted]

CADTH Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0826
Name of the drug and Indication(s)	Eplontersen (Wainua)
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	X
No Request for Reconsideration	**Editorial revisions:** Clarifications in recommendation **text** are requested	☐
	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.

The current recommendation for eplontersen specifies:

"Eligibility for eplontersen should be based on the criteria used by each of the public drug plans for initiation, renewal, and prescribing of vutrisiran for hATTR-PN".

While the CDEC recommended criteria for vutrisiran, patisiran, and inotersen are the same, it is not clear why CDEC chose to specifically reference vutrisiran. As vutrisiran is currently undergoing negotiations at pCPA, B.C's preference would be to specify the criteria rather than referencing another agent.

For example:
"Treatment with eplontersen should be reimbursed in adult patients with stage 1 or stage 2 genetically confirmed hATTR-PN who are symptomatic with early-stage neuropathy, defined as:
1.1. PND stage I to ≤ IIIB, or FAP stage I or II
1.2. no severe heart failure symptoms (defined as NYHA class III or IV)
1.3. no previous liver transplant"

The renewal and discontinuation criteria can also be copied from vutrisiran's recommended criteria.

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
Please provide details regarding the information that requires clarification.
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.

Outstanding Implementation Issues

In the event of a positive draft recommendation, drug programs can request further implementation support from CADTH on topics that cannot be addressed in the reimbursement review (e.g., concerning other drugs, without sufficient evidence to support a recommendation, etc.). Note that outstanding implementation questions can also be posed to the expert committee in Feedback section 4c.

Algorithm and implementation questions
1. Please specify sequencing questions or issues that should be addressed by CADTH (oncology only)
1. 2.
2. Please specify other implementation questions or issues that should be addressed by CADTH
1. 2.
Support strategy
3. Do you have any preferences or suggestions on how CADTH should address these issues?
May include implementation advice panel, evidence review, provisional algorithm (oncology), etc.

CADTH

CADTH Reimbursement Review
Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0826
Brand name (generic)	WAINUA™ (eplontersen)
Indication(s)	For the treatment of polyneuropathy associated with stage 1 or stage 2 hereditary transthyretin amyloidosis (hATTR) in adults
Organization	AstraZeneca Canada
Contact information[a]	[redacted]

Stakeholder agreement with the draft recommendation		
1. Does the stakeholder agree with the committee's recommendation.	Yes	☒
	No	☐
WAINUA (eplontersen) fulfills an important care gap in Canada for patients whose quality of life is significantly impacted by hereditary transthyretin-associated amyloidosis (hATTR-PN). WAINUA offers hATTR-PN patients with the choice of an alternative treatment option that slows disease progression, limits AEs, and offers greater independence and convenience through its at-home administration. WAINUA will also provide clinicians with an additional mechanism of action to help address hATTR-PN disease heterogeneity. This will help ensure that patients can have access to tailored therapies, regardless of their phenotypic or genotypic differences. Hence, WAINUA expands patient choice, and brings value to patients, their caregivers and clinicians.		
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	☐
If not, what aspects are missing from the draft recommendation?		
Clarity of the draft recommendation		
3. Are the reasons for the recommendation clearly stated?	Yes	☒
	No	☐
If not, please provide details regarding the information that requires clarification.		
4. Have the implementation issues been clearly articulated and adequately addressed in the recommendation?	Yes	☒
	No	☐
If not, please provide details regarding the information that requires clarification.		
5. If applicable, are the reimbursement conditions clearly stated and the rationale for the conditions provided in the recommendation?	Yes	☐
	No	☒
In table 1 titled "Reimbursement conditions and reasons", CDA states the Initiation, Renewal, Discontinuation and Prescribing conditions of WAINUA (eplontersen) to be based on the criteria used by each of the public drug plans for vutrisiran. AstraZeneca requests CDA to outline the details of the reimbursement criteria for WAINUA instead of referring to vutrisiran's for clarity and transparency. More specifically:		

- CDA references the reimbursement criteria of a product that is not listed in jurisdictions across Canada.
    - While vutrisiran is undergoing pCPA negotiations, as of October 4th, 2024, its reimbursement status remains unknown.
    - Referencing WAINUA's clinical criteria to a product that has yet to be listed implies that this product is guaranteed to be listed across the country. It is requested that the WAINUA CDA recommendation includes the full description of the clinical/reimbursement criteria to ensure clarity and allow for the recommendation to stand on its own.
- It is requested that the WAINUA CDA recommendation outlines the reimbursement criteria in full to increase clarity and ensure ease of information access for all readers.
    - As mentioned above, the listing criteria for vutrisiran across the country do not currently exist, which could lead to reader confusion (patients, caregivers, clinicians, etc.) of its reimbursement status.
- Lastly, even should vutrisiran be successful at securing reimbursement, access to reimbursement criteria across jurisdictions can be challenging and often not available to all readers. To ensure that the WAINUA clinical criteria is easily accessible to all, regardless of the reader's knowledge of the Canadian reimbursement landscape, AZC requests CDA to outline the clinical criteria in full for WAINUA in the recommendation (again, allowing for the recommendation to stand on its own).

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