



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

CDA-AMC REIMBURSEMENT REVIEW

Stakeholder Feedback on Draft Recommendation

avapritinib (Ayvakyt)
(Medison Pharma Canada Inc.)

Indication: For the treatment of adult patients with advanced systemic mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).

October 18, 2024

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CDA-AMC does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no identifying personal information or personal health information is included in the submission. The name of the submitting stakeholder group and all conflicts of interest information from individuals who contributed to the content are included in the posted submission.

CADTH Reimbursement Review Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	PC0335-000
Brand name (generic)	Ayvakyt (avapritinib)
Indication(s)	Advanced Systemic Mastocytosis
Organization	The Leukemia & Lymphoma Society of Canada (LLSC)
Contact information ^a	Name: Colleen McMillan, LLSC
Stakeholder agreement with the draft recommendation	
1. Does the stakeholder agree with the committee's recommendation.	Yes <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
We agree that there is currently a significant unmet need for additional treatment options for those affected by Advanced Systemic Mastocytosis. Given the poor prognosis and substantial morbidity of this disease and the possibility that avapritinib has the potential to reduce morbidity and mortality associated with AdvSM, this is a much-needed treatment option for patients. AdvSM has a poor prognosis and there is a need for effective therapies that may improve disease control.	
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
We thank the committee for this decision and for considering the input of LLSC and the patients we represent.	
Clarity of the draft recommendation	
3. Are the reasons for the recommendation clearly stated?	Yes <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
If not, please provide details regarding the information that requires clarification.	

^a CADTH may contact this person if comments require clarification.

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.

A. Patient Group Information				
Name	Colleen McMillan			
Position	Advocacy Lead, LLSC			
Date	17-10-2024			
<input checked="" type="checkbox"/>	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	<input checked="" type="checkbox"/>
			Yes	<input type="checkbox"/>
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	<input checked="" type="checkbox"/>
			Yes	<input type="checkbox"/>
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	<input type="checkbox"/>
			Yes	<input checked="" type="checkbox"/>
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

CADTH Reimbursement Review Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	PC0335-000
Brand name (generic)	Ayvakyt (avapritinib)
Indication(s)	For the treatment of adult patients with Advanced Systemic Mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).
Organization	Ontario Health (Cancer Care Ontario) Hematology Cancer Drug Advisory Committee
Contact information ^a	Name: Dr. Tom Kouroukis
Stakeholder agreement with the draft recommendation	
1. Does the stakeholder agree with the committee's recommendation.	Yes <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
Please explain why the stakeholder agrees or disagrees with the draft recommendation. Whenever possible, please identify the specific text from the recommendation and rationale.	
There are no approved and funded treatment for advanced systemic mastocytosis, even though effective options have been studied (cladribine, midostaurin, imatinib, interferon). Avapritinib is safe and effective and could be the first approved and funded treatment for these patients with a high unmet need. A reduction in price seems required to achieve an acceptable incremental cost-effectiveness ratio. It is recognized that the exact incremental effectiveness and utility of avapritinib over best available treatment is uncertain although it seems to be favouring avapritinib.	
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
If not, please provide details regarding the information that requires clarification.	

Appendix 2. Conflict of Interest Declarations for Clinician 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.
- For conflict of interest declarations:
 - Please 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.
 - Please note that declarations are required for each clinician that contributed to the input.
 - If your clinician group provided input at the outset of the review, only conflict of interest declarations that are new or require updating need to be reported in this form. For all others, please list the clinicians who provided input are unchanged
 - Please add more tables as needed (copy and paste).
 - All new and updated declarations must be included in a single document.

A. Assistance with Providing the Feedback		
2. Did you receive help from outside your clinician group to complete this submission?	No	<input type="checkbox"/>
	Yes	<input checked="" type="checkbox"/>
If yes, please detail the help and who provided it.		
OH-CCO provided secretariat support to the group in completing this submission.		
3. Did you receive help from outside your clinician group to collect or analyze any information used in this submission?	No	<input checked="" type="checkbox"/>
	Yes	<input type="checkbox"/>
If yes, please detail the help and who provided it.		
B. Previously Disclosed Conflict of Interest		
4. Were conflict of interest declarations provided in clinician group input that was submitted at the outset of the CADTH review and have those declarations remained unchanged? If no, please complete section C below.	No	<input type="checkbox"/>
	Yes	<input checked="" type="checkbox"/>
If yes, please list the clinicians who contributed input and whose declarations have not changed:		
<ul style="list-style-type: none"> Dr. Tom Kouroukis Clinician 2 Add additional (as required) 		

C. New or Updated Conflict of Interest Declarations

New or Updated Declaration for Clinician 1	
Name	Dr. Selay Lam
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member
Date	10-10-2024
<input checked="" type="checkbox"/>	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this clinician or clinician group with a company, organization, or entity that may place this clinician or clinician group in a real, potential, or perceived conflict of interest situation.

Conflict of Interest Declaration				
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

New or Updated Declaration for Clinician 2	
Name	Dr. Jordan Herst
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member
Date	10-10-2024
<input checked="" type="checkbox"/>	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this clinician or clinician group with a company, organization, or entity that may place this clinician or clinician group in a real, potential, or perceived conflict of interest situation.

Conflict of Interest Declaration				
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

New or Updated Declaration for Clinician 3	
Name	Dr. Joanna Graczyk
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member
Date	10-10-2024
<input checked="" type="checkbox"/>	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this clinician or clinician group with a company, organization, or entity that may place this clinician or clinician group in a real, potential, or perceived conflict of interest situation.

Conflict of Interest Declaration				
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
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New or Updated Declaration for Clinician 4				
Name	Dr. Lee Mozessohn			
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member			
Date	10-10-2024			
<input checked="" type="checkbox"/>	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this clinician or clinician group with a company, organization, or entity that may place this clinician or clinician group in a real, potential, or perceived conflict of interest situation.			
Conflict of Interest Declaration				
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

New or Updated Declaration for Clinician 5				
Name	Dr. Christopher Cipkar			
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member			
Date	10-10-2024			
<input checked="" type="checkbox"/>	I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this clinician or clinician group with a company, organization, or entity that may place this clinician or clinician group in a real, potential, or perceived conflict of interest situation.			
Conflict of Interest Declaration				
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	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add company name	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Add or remove rows as required	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

New or Updated Declaration for Clinician 6	
Name	Dr. Guillaume Richard-Carpentier
Position	OH (CCO) Hematology Cancer Drug Advisory Committee member
Date	18-10-2024

CADTH Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information		
CADTH project number	PC0335	
Name of the drug and Indication(s)	Avapritinib For the treatment of adult patients with Advanced Systemic Mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).	
Organization Providing Feedback	PAG	
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	<input type="checkbox"/>
	Minor revisions: A change in reimbursement conditions is requested	<input type="checkbox"/>
No Request for Reconsideration	Editorial revisions: Clarifications in recommendation text are requested	<input type="checkbox"/>
	No requested revisions	X
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		
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 Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	PC0335
Brand name (generic)	AYVAKYT (avapritinib)
Indication(s)	For the treatment of adult patients with advanced systemic mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).
Organization	Medison Pharma Canada Inc.
Contact information ^a	<div>██████████</div> <div>██████████████████</div> <div>██████████████████████</div> <div>██████████████████████████████</div> <div>██████████████████████</div> <div>██</div> <div> </div> <div>██████████████████</div> <div>██████████████████████</div> <div>██████████████████████</div> <div>██████████████████████████████</div> <div>██████████████████████</div> <div>██</div>
Stakeholder agreement with the draft recommendation	
1. Does the stakeholder agree with the committee's recommendation.	Yes <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
Yes, Medison Pharma Canada Inc. (Medison), agrees with the committee's recommendation to reimburse AYVAKYT (avapritinib) for the treatment of adult patients with advanced systemic mastocytosis (AdvSM).	
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 <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
Yes, the recommendation demonstrates that the committee has considered the stakeholder input provided to CADTH.	
Clarity of the draft recommendation	
3. Are the reasons for the recommendation clearly stated?	Yes <input checked="" type="checkbox"/>
	No <input type="checkbox"/>
Medison agrees that the reasons for the recommendation are clearly stated.	

4. Have the implementation issues been clearly articulated and adequately addressed in the recommendation?	Yes	<input checked="" type="checkbox"/>
	No	<input type="checkbox"/>
Medison agrees that the implementation issues have been clearly articulated and adequately addressed in the recommendation.		
5. If applicable, are the reimbursement conditions clearly stated and the rationale for the conditions provided in the recommendation?	Yes	<input checked="" type="checkbox"/>
	No	<input type="checkbox"/>
Medison agrees that the reimbursement conditions are clearly stated that the rationale for the conditions are provided in the recommendation.		

^a CADTH may contact this person if comments require clarification.