CADTH

CADTH REIMBURSEMENT REVIEW

Stakeholder Feedback on Draft Recommendation

PALOVAROTENE (Sohonos)

(Ipsen Biopharmaceuticals Canada, Inc.)

Indication: Sohonos (palovarotene capsules) is indicated to reduce the formation of heterotopic ossification in adults and children aged 8 years and above for females and 10 years and above for males with Fibrodysplasia (myositis) Ossificans Progressiva.

April 27, 2023

CADTH

CADTH Reimbursement Review

Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0761
Name of the drug and Indication(s)	Palovarotene (Sohonos) for Fibrodysplasia (myositis) Ossificans Progressiva
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	☐
	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.

CADTH

CADTH Reimbursement Review
Feedback on Draft Recommendation

Stakeholder information	
CADTH project number	SR0761-000
Brand name (generic)	SOHONOS (palovarotene)
Indication(s)	Fibrodysplasia Ossificans Progressiva
Organization	The Canadian FOP Network (CFOPN) & The Canadian Organization for Rare Disorders (CORD)
Contact information[a]	Name: Durhane Wong-Rieger, President & CEO, CORD e: [redacted]

Stakeholder agreement with the draft recommendation		
1. Does the stakeholder agree with the committee's recommendation.	Yes	☒
	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.

We agree with the overall recommendation and appreciate the highly nuanced approach of CDEC to the assessment of the available information to provide criteria-based access (diagnosis and mutation) and not ankylosis of the whole body), recognizing the difficulties for assessing benefits and risks in growing children. This recommendation does indeed give appropriate discretion to the clinician specialist to assess and also to inform the patient and family.
While we appreciate that the committee has provided wide discretion to the treating clinician to prescribe and monitor, it is important that guidelines be articulated, with a breadth to accommodate all patients. These guidelines should be clear to both the clinicians and the patients in language that is also accessible to the patient community, so there is general agreement on who should or should not be offered the treatment, patients have sufficient understand to participate in informed choice discussion and, importantly, the conditions for continuation or discontinuation are clear enough that clinicians and patients would be in agreement.
It is equally important that the clinical experts and the patient community remain updated about emerging evidence by sharing across treaters and referencing the international community. Indeed, a committee of experts should be formed to jointly assess the patients but also to continue to gather real world data and to update the benefits, risks, and outcomes.

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?

The committee has indeed balanced the unknowns against the potential benefits and risks and has appropriately provided the patients with the opportunity to try, which is most important. There is clear understanding of the high unmet need and the lack of other options, with not only the hope but the ability to monitor on effectiveness and safety.

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. The draft recommendations are clear enough based on the limits of the information available.		
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. The committee has addressed the overall implementation issues, relying on the clinical experts. It could be improved to acknowledge or recommend a committee of experts to assess, monitor, and re-evaluate findings to update best practices, as discussed in point #1.		
5. If applicable, are the reimbursement conditions clearly stated and the rationale for the conditions provided in the recommendation?	Yes	☐
	No	☒
If not, please provide details regarding the information that requires clarification. We recognize the challenge of calculating an ICER and feel the value generated is total nonsense. It does not speak to the therapy and disease but really to the lack of appropriateness of even attempting to generate this value. Obviously, the price will be negotiated but the ICER based on the vagaries of the calculation process are not realistic or helpful. And, of course, the inclusion of the arbitrary and decades old $50k/QALY adds further to the absurdity of the exercise and results. It is VERY VERY important that the public plans do not delay implementation subject to a deliberation on price but that the plans and the company enter an agreement for immediate access with a price that can be adjusted (with paybacks or increases) pending further negotiation and the collection of additional usage and impact information. Provide access now; negotiate going forward. The company should be encouraged to enter a risk-sharing agreement with adjustments pending.		

[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	*Canadian FOP Network*
Position	*President*
Date	*25-04-2023*
☒	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	☒
	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	☒
	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	☐	☐	☐	☐
Add company name	☐	☐	☐	☐
Add or remove rows as required	☐	☐	☐	☐

A. Patient Group Information	
Name	*Canadian Organization for Rare Disorders*
Position	*Durhane Wong-Rieger, President & CEO*
Date	*25-04-2023*
☒	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		
4. Did you receive help from outside your patient group to complete your feedback?	No	☒
	Yes	☐
If yes, please detail the help and who provided it.		
5. Did you receive help from outside your patient group to collect or analyze any information used in your feedback?	No	☒
	Yes	☐
If yes, please detail the help and who provided it.		

C. Previously Disclosed Conflict of Interest		
2. 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

6. 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**
Ipsen	☐	☐	☒	☐
Add company name	☐	☐	☐	☐
Add or remove rows as required	☐	☐	☐	☐