pan-Canadian Oncology Drug Review

Patient Advocacy Group Feedback on a pCODR Expert Review Committee Initial Recommendation

Vandetanib (Caprelsa) for Medullary Thyroid Cancer

Thyroid Cancer Canada

March 30, 2017

1 Feedback on pERC Initial Recommendation

Name of the drug indication(s): Caprelsa (Vandetanib) for Medullary Thyroid Cancer

Name of registered patient advocacy: Thyroid Cancer Canada

**pCODR may contact this person if comments require clarification. Contact information will not be included in any public posting of this document by pCODR.*

1.1 Comments on the Initial Recommendation

a) Please indicate if the patient advocacy group agrees or disagrees with the initial recommendation:

____ agrees ___X_ agrees in part ____ disagree

Please explain why the patient advocacy group agrees, agrees in part or disagrees with the initial recommendation.

Thyroid Cancer Canada (TCC) has interviewed numerous patients with Medullary Thyroid Cancer (MTC) who have had experience with vandetanib. While a small sample of patients were interviewed, it was clear there was **significant** clinical benefit from vandetanib for responders.

Further, pCODR evaluated vandetanib using the same criteria it uses to evaluate drugs for more common (higher incidence/prevalence) cancers. MTC is extremely rare, and accounts for approximately 1-2% of thyroid cancer cases. The PAG has recognized this indicating that "vandetanib fills a gap in therapy for a very small number of patients with symptomatic or progressive MTC." (p.9 of initial recommedations). TCC believes it is critically important for reviewers to recognize that conventional HTA methods are not well suited for reviewing Drugs for Rare Disorders (DRDs).

b) Notwithstanding the feedback provided in part a) above, please indicate if the patient advocacy group would support this initial recommendation proceeding to final pERC recommendation ("early conversion"), which would occur within 2(two) business days of the end of the consultation period.

___X_ Support conversion to final recommendation. Recommendation does not require reconsideration by pERC.

____ Do not support conversion to final recommendation. Recommendation should be reconsidered by pERC.

c) Please provide feedback on the initial recommendation. Is the initial recommendation or are the components of the recommendation (e.g., clinical and economic evidence) clearly worded? Is the intent clear? Are the reasons clear?

Page Number	Section Title	Paragraph, Line Number	Comments and Suggested Changes to Improve Clarity

1.2 Comments Related to Patient Advocacy Group Input

1.3 Additional Comments About the Initial Recommendation Document

Please provide any additional comments:

Page Number	Section Title	Paragraph, Line Number	Additional Comments
9	Adoption Feasibility	Para 5, Line 1	While the PAG has noted that the oral route of administration of vandetanib was an enabler to implementation, it noted that the PAG, and pERC, expressed concerns that the restricted distribution program (requiring that vandetanib be prescribed and dispensed only by registered and certified clinicians with the distribution program) would result in logistical limitations to access of vandetanib by patients. **What the Provincial Advisory Group has not recognized** is that in provinces where oral cancer drugs are not equally reimbursed on par with IV drugs (Ontario and Atlantic Canada specifically) it is the reimbursement policies themselves in those provinces that act as an impediment to equitable patient-centric implementation of pCODR's recommendations for reimbursement of vandetanib.
3	Summary of pERC Deliberations	Para 2, Line 5, 6 & 7	pERC noted the uncertainty of OS results due to the potential for confounding by crossover and discussing the appropriateness of PFS as a surrogate despite the CGP and clinician(s) who provided input identifying PFS as a likely surrogate. Ethical concerns are a key driver of treatment switching in that it may be unethical to

Page Number	Section Title	Paragraph, Line Number	Additional Comments
			disallow patients randomized to the inferior treatment access to the experimental drug – particularly if no other (non-palliative) treatments are approved for the disease state being studied, which is the case in the clinical trial commented upon by pERC. Clinical trials are designed to establish whether the active treatment arm is outperforming the control arm at the point of interim analysis, and if the active treatment demonstrates superiority, then typically patients are permitted to crossover to that treatment for ethical reasons. In oncology drug trials, disease progression may also be a reason to allow for switching. pCODR should be aware that recruiting and retaining patients in clinical trials will be increasingly difficult if treatment crossover is considered an insurmountable confounder in assessing the clinical value of a drug. TCC encourages pCODR to adopt new methods for interpreting the results of trials where treatment crossover has occurred. And, specifically as it applies to cancer drugs for rare cancer types. TCC encourages pCODR to accept PFS as a clinically meaningful surrogate and perhaps rely on post-market surveillance to address any uncertainties that remain.

About Completing This Template

pCODR invites those registered patient advocacy groups that provided input on the drug under review **prior** to deliberation by the pCODR Expert Review Committee (pERC), to also provide feedback and comments on the initial recommendation made by pERC. (See www.cadth.ca/pcodr for information regarding review status and feedback deadlines.)

As part of the pCODR review process, the pCODR Expert Review Committee makes an initial recommendation based on its review of the clinical, economic and patient evidence for a drug. (See www.cadth.ca/pcodr for a description of the pCODR process.) The initial recommendation is then posted for feedback and comments from various stakeholders. The pCODR Expert Review Committee welcomes comments and feedback that will help the members understand why the patient advocacy groups agree or disagree with the initial recommendation. In addition, the members of pERC would like to know if there is any lack of clarity in the document and if so, what could be done to improve the clarity of the information in the initial recommendation. Other comments are welcome as well.

All stakeholders have 10 (ten) business days within which to provide their feedback on the initial recommendation and rationale. If all invited stakeholders, including registered patient advocacy groups, agree with the recommended clinical population described in the initial recommendation, it will proceed to a final pERC recommendation by 2 (two) business days after the end of the consultation (feedback) period. This is called an "early conversion" of an initial recommendation to a final recommendation.

If any one of the invited stakeholders does not support the initial recommendation proceeding to final pERC recommendation, pERC will review all feedback and comments received at the next possible pERC meeting. Based on the feedback received, pERC will consider revising the recommendation document as appropriate. It should be noted that the initial recommendation and rationale for it may or may not change following consultation with stakeholders.

The final pERC recommendation will be made available to the participating provincial and territorial ministries of health and cancer agencies for their use in guiding their funding decisions and will also be made publicly available once it has been finalized.

Instructions for Providing Feedback

a) Only registered patient advocacy groups that provided input at the beginning of the review of the drug can provide feedback on the initial recommendation.

- Please note that only one submission per patient advocacy group is permitted. This applies to those groups with both national and provincial / territorial offices; only one submission for the entire patient advocacy group will be accepted. If more than one submission is made, only the first submission will be considered.
- Individual patients should contact a patient advocacy group that is representative of their condition to have their input added to that of the group. If there is no patient advocacy group for the particular tumour, patients should contact pCODR for direction at www.cadth.ca/pcodr.

b) Feedback or comments must be based on the evidence that was considered by pERC in making the initial recommendation. No new evidence will be considered during this part of the review process; however, it may be eligible for a Resubmission.

c) The template for providing *pCODR Patient Advocacy Group Feedback on a pERC Initial Recommendation* can be downloaded from the pCODR website. (See www.cadth.ca/pcodr for a description of the pCODR process and supporting materials and templates.)

d) At this time, the template must be completed in English. Patient advocacy groups should complete those sections of the template where they have substantive comments and should not feel obligated to complete every section, if that section does not apply to their group. Similarly, groups should not feel restricted by the space allotted on the form and can expand the tables in the template as required.

e) Feedback on the initial pERC recommendations **should not exceed three (3) pages in length**, using a minimum 11 point font on 8 ½″ by 11″ paper. If comments submitted exceed three pages, only the first three pages of feedback will be forwarded to the pERC.

f) Feedback should be presented clearly and succinctly in point form, whenever possible. The issue(s) should be clearly stated and specific reference must be made to the section of the recommendation document under discussion (i.e., page number, section title, and paragraph). Opinions from experts and testimonials should not be provided. Comments should be restricted to the content of the initial recommendation.

g) References to support comments may be provided separately; however, these cannot be new references. New evidence is not considered during this part of the review process, however, it may be eligible for a Resubmission. If you are unclear as to whether the information you are considering to provide is eligible for a Resubmission, please contact the pCODR Secretariat.

h) The comments must be submitted via a Microsoft Word (not PDF) document by logging into www.cadth.ca/pcodr and selecting "Submit Feedback" by the posted deadline date.

i) Patient advocacy group feedback must be submitted to pCODR by **5 P.M. Eastern Time** on the day of the posted deadline.

j) If you have any questions about the feedback process, please e-mail pcodrinfo@cadth.ca. For more information regarding patient input into the pCODR drug review process, see the *pCODR Patient Engagement Guide*. Should you have any questions about completing this form, please email pcodrinfo@cadth.ca

Note: Submitted feedback is publicly posted and also may be used in other documents available to the public. The confidentiality of any submitted information at this stage of the review cannot be guaranteed.