

A Dialogue on Patient-Centered Value Assessment:

Overcoming Barriers to Amplify the Patient Voice

December 2018



NATIONAL HEALTH COUNCIL

National Health Council • 1730 M Street NW, Suite 500, Washington, DC 20036-4561 • 202-785-3910



Acknowledgements

This report was prepared by the National Health Council based on the notes from the group discussion and breakout sessions, as well as story boards constructed by the groups. It was reviewed by the participants who provided comments, edits, and additions.

National Health Council

- Eleanor M. Perfetto, PhD, MS
- Elisabeth Oehrlein, PhD, MS
- Jason Harris
- Eric Gascho

We acknowledge the following NHC staff for their technical assistance in preparing this report:

- Jennifer C. Schleman, MPS, APR
- Christina Howerton

We also thank the following graduate students from the University of Maryland, Baltimore who assisted with note-taking:

- Maya Hanna, MPH
- T. Rosie Love, MPH
- Bansri Desai, PharmD

How to cite this report:

National Health Council. A Dialogue on Patient-Centered Value Assessment: Overcoming Barriers to Amplify the Patient Voice. December 2018. Available from:
<https://www.nationalhealthcouncil.org/dialogue-patient-centered-value-assessment-overcoming-barriers-amplify-patient-voice>

Executive Summary

On July 31, 2018, the National Health Council (NHC) convened representatives from US organizations that have produced value frameworks/assessments over the last several years with participants from patient groups that have interacted with those organizations. The objectives of the meeting were to:

- Articulate a shared vision for what marks success in enhanced patient centrality in value assessment (VA) and
- Outline tangible, feasible actions that can be taken to achieve that success. The actions may be on the part of patient groups, value-assessment organizations (VA bodies), or both in collaboration.

Patient groups and VA bodies agreed, the ultimate goal of patient-centered VA is for patients to have access to treatments they need at prices they can afford. Patient-centered VA exists when patients have been engaged, heard, understood, and respected throughout the entire process, and their input is incorporated and guides decision-making. Participants developed seventeen recommendations to enhance patient centrality in VA. Recommendations include:

Examples of Recommended Actions for Improving Engagement

- Patient groups can develop a catalogue of data and other assets they have and provide this to VA bodies to help convey the role a patient group can play in value assessment.
- VA bodies can clearly define and effectively communicate opportunities regarding when and how patients can substantively contribute throughout an assessment, including during the scoping process.
- Patients and VA bodies can co-develop training to prepare patients to engage with VA bodies and VA bodies to engage with patients.

Examples of Recommended Actions for Collecting and Leveraging Patient-Provided Data

- Patient groups can engage VA bodies when the groups develop or update registries and/or surveys to ensure they include questions that provide data sought by VA bodies.
- VA bodies can articulate the data gaps in VA, which can assist patient organizations when developing standardized approaches to collecting and formatting needed data. Data needs can be communicated to relevant patient groups in advance of a VA (the earlier, the better).
- Following an appraisal, the VA body and patient groups can debrief about how data submitted by patient groups were/were not useful, and how data collection or presentation can be improved for future use.
- Learnings can be passed on to other organizations, for example through the NHC's Value Work Group.

The Dialogue concluded with participants reaffirming the importance of collaboration between patient groups and VA bodies moving forward. While they must partner to improve the patient centrality of VA, other stakeholder groups, such as employers and payers, are also critical and

their participation should be part of future dialogues. A dialogue with employers was recommended as a next step. Findings and recommendations from this Dialogue will be used by the NHC to inform future value initiative priorities.

NHC Dialogue and Objectives

Recently, a number of non-profit and professional organizations have publicly released value assessment (VA) frameworks intended to provide recommendations to payers, providers, patients, and/or others on the clinical and economic value of therapies. The National Health Council (NHC) is focused on ensuring the voice of the patient and patient groups are an integral part of VA. Patient engagement in the development of the frameworks and the resulting assessments has increased and evolved over time. Patient groups are eager to be involved to ensure the voices of patients they represent are heard and considered as part of VA. But, they are concerned about the resource burden these activities place on their organizations. Patient groups are cognizant that the resource burden on VA organizations related to patient engagement activities can also be a barrier, potentially discouraging assessors from fully engaging with the patient community.

The NHC convened representatives from US organizations that have produced value frameworks/assessments (VA bodies) over the last several years with participants from patient groups that have interacted with those organizations. The goal of the dialogue was for patient groups and VA bodies to collaboratively articulate specific strategies and recommendations that further enhance the patient centricity of VA.

The objectives of the meeting were to:

- Articulate a shared vision for what marks success in enhanced patient centricity in VA and
- Outline tangible, feasible actions toward achieving that success. The actions may be on the part of patient groups, VA developers, or both in collaboration.

Format and Methods

An NHC-convened Dialogue met on July 31, 2018, in Washington, DC. The meeting followed Chatham House Rules to increase openness in dialogue. All participant identities and organizations, except our invited speaker, are anonymized. Two sessions offered prepared remarks, a keynote featuring Dr. Brian O'Rourke of the Canadian Agency for Drugs and Technologies in Health (CADTH), discussing "Meaningful Engagement of Patients in Health Technology Assessment," and a panel discussion on "Understanding One Another's Perspectives: What Does Success Look Like to You."

In remaining sessions, Dialogue participants took part in semi-structured discussions captured by scribes assigned to roundtables. The morning session included a large group discussion on defining success. Since the overall goal was to collaboratively articulate strategies to enhance the patient centricity of value assessment, the group was asked to describe a vision for success and to complete the sentence: "We have enhanced patient-centered value assessment when..."

In the afternoon, participants were organized in small roundtable work groups, pre-assigned to ensure balanced representation of VA bodies and patient groups. The topics were:

- **Engagement: Charting a Path Forward** – In this session, small groups were asked to discuss what opportunities exist to enhance patient engagement in VA and to answer the questions: What are the opportunities for how patient experiences can inform VA? What can patient groups do to be prepared for engaging with value assessors? What can value assessors do to support patient/patient group engagement in their processes? What can patient groups and assessors do together?
- **Data: Charting a Path Forward** – In this session, small groups were asked to discuss how patient-centered data sources can be leveraged in VA and to answer the questions: What data are needed? How might patient groups be able to collect and provide those data? What characteristics do patient-generated data sources and evidence need to have to be useful in VA? What can value assessors do to leverage patient/patient group data? What novel and emerging methods can best be used to leverage patient input?

The notes from the group discussion and breakout sessions, as well as story boards constructed by the groups, were used by the NHC staff to develop a draft of this white paper, which was reviewed by the participants who provided comments, edits, and additions.

Keynote: Meaningful Engagement of Patients in Health Technology Assessment

In his keynote, Dr. O'Rourke described the evolution of patient engagement at CADTH over the past decade. He highlighted the many opportunities for patients to participate in CADTH reviews, including the Patient Liaison Forum, providing formal input during drug and device reviews, participating as voting members of the oncology expert panel, providing input during program/process consultations, and conducting interviews as part of the Scientific Advice program. To facilitate patient engagement, CADTH has developed tools, such as the Patient Input Template,¹ and undertaken qualitative research to understand patient perspectives on meaningful patient engagement and how patient-provided information is integrated into CADTH appraisals.^{2,3} Dr. O'Rourke pointed out that patient engagement in CADTH assessments is considered a patient-group core function in Canada; as such, groups are not typically compensated for their work. While this is the norm in Canada, it is not the norm in the US.

Setting the Stage: Defining What Success Looks Like

The patient-group and VA-body participants offered insights on what success in patient engagement in value assessment looks like.

Patient community stakeholders described a number of characteristics of what success looks like from their point-of-view. In general, patients believe success has been achieved when individual patients sit down with a final VA report and it reflects the multi-dimensionality of their experiences, the burden of disease, and recognizes the different ways a disease manifests. In patient-centered VA, patient groups would be seen as curators for patient-provided data.

That is, they would also be consulted on whether data published in the peer-reviewed literature has face validity, is representative of all important patient subgroups, etc. Patient groups would have a clear understanding of the points at which during a VA they can contribute data on patient experiences. Importantly, the impact of their submitted experience data would be clearly identifiable in final VA reports.

For **VA bodies**, patient-centered VA success is achieved when patients respect final VA reports - even if they disagree with the recommendations - because they agree that methods were transparent, data used were patient centered, and they can see that patients were engaged throughout the process. Assessments will be clear and replicable and focus not only on pharmaceuticals but will examine value across the health care ecosystem.

Patients and VAs agreed that the ultimate goal should be for patients to have access to treatments they need at prices they can afford. They agree that there is nothing worse than an effective treatment existing, but patients not having equitable access to it due to cost or other related barriers. Patient-centered VA should be relevant to and inform decision-making on the part of patients and providers. Reports should be used to improve access to care and not as justification for not reimbursing for a therapy or procedure.

Patient-centered VA exists when patients have been engaged, heard, understood, and respected throughout the entire process, and their input incorporated. Patient-centered VA should be transparent and reflects input throughout a VA final report – not isolated only to a “patient-centered” section, which conveys a token gesture. Success means that VA includes high-quality data on aspects of care that are important to patients whenever such data exist.

The group also discussed important barriers to achieving patient-centered VA, which include:

- When clinical-trial data are the foundation of VA and the outcomes important to patients were not measured as part of the included trials, it hinders the VA from being patient-centered.
- Similarly, some trials do capture patient-reported outcome (PRO) data. However, studies often use older, legacy PRO tools that may not be patient centered, as they do not capture experiences and outcomes important to patients. The inclusion of a PRO can provide the potentially misleading impression that a study is patient centric, when it, in fact, is not.
- Patient-provided data may not be fully utilized, not because of specific concerns over data quality, but as a result of funding-source concerns, possibly due to lack of funding transparency or concerns over industry funding even though transparent. This might prevent good-quality, patient-provided information from being used, decreasing patient centrality.
- Clinical practice guidelines inform VA. However, many guidelines are out-of-date, have had no patient input, and do not reflect appropriate comparators, all subpopulations, or patient-prioritized outcomes. If the guideline is not patient-centered, it hampers patient-centered VA.

- Many VA frameworks do not allow for incorporation of evolving evidence sources to support the value of a given therapy or sequence of treatment. For example, many do not accept real-world data sources.

The discussions on defining success and identifying potential barriers led to an afternoon of small-group discussions that resulted in the following set of recommended actions:

Recommended Actions for Achieving a Shared Vision of Success:

1. Patient-focused drug development will yield clinical-trial data that is more patient centered, focusing on experiences of and outcomes important to patients. This will improve data sources for patient-centered VA in the future. Early dialogue between VA bodies, patient groups, and industry should encourage patient-centered clinical development plans. Patient groups should continue to advocate for patient engagement in medical-product development.
2. The PICOTS framework (i.e., population, intervention, comparator, outcome, time, and setting) should be used to ensure that VA relies on an agreed-upon set of facts, information, or assumptions (e.g., subgroups). Patients and patient groups can become familiar with the PICOTS framework and VA bodies can seek input from patients on PICOTS framework elements. Thus, when stakeholders engage on VA - using the PICOTS framework – they are using the same language and misunderstandings can be avoided with clearer communications.
3. VA bodies should establish opportunities for patients to participate throughout VA. Example opportunities include submitting data, participating on committees, and presenting oral comments. Patient groups should ensure that their members are prepared to effectively participate in engagement opportunities.
4. Patient groups can seek to partner with professional societies to achieve meaningful patient engagement in patient-centered, clinical-practice guideline development, which can help to inform more patient-centered VA.
5. Patient groups can lead or seek to partner with other stakeholders to develop patient-centered, patient-reported outcome (PRO) and experience measures. When used in trials and other research, they will help to ensure that the patient-reported data used in VA focuses on experiences of and outcomes important to patients.

Recommendations for Patients to Be Prepared for Engaging with VAs

6. Patient groups can add health economists to their scientific advisory boards, just as clinicians are currently included on these boards.
7. Patient groups can develop a catalogue of assets to share with VA bodies to help them understand the role a patient group can play, especially in conveying the wholistic experience of the patient and family.

Recommendations for VA Bodies to Help Support Patient/Patient Group Engagement in their Processes

8. VA bodies can clearly define and communicate about opportunities regarding when and how patients can substantively contribute throughout an assessment, including during the scoping process. VA bodies can partner with patient groups to ensure that communications are effective and are reaching the patient community.
9. VA bodies can establish their own, standing patient-engagement advisory committees or collaborate with existing committees, such as the NHC's Value Work Group.

Recommendations for How Patients and Value Assessors Can Collaborate to Improve Engagement

10. Patients and VA bodies should seek to improve bi-directional communication and continue to engage and learn from one another even after an appraisal is completed.
11. Patients and VA bodies can co-develop training to prepare patients to engage with VA bodies and VA bodies to engage with patients.

Recommendations for Patients on Collecting and Providing Patient Data

12. Patient groups can develop a patient-provided information (e.g., experiences, preferences, PROs) dossier to facilitate working with VA bodies/payers/PBMs/employers.
13. Patient groups can engage VA bodies when the patient groups are developing or updating patient registries and/or surveys to ensure they include questions that provide information sought by VA bodies.

Recommendations for VA Bodies to Support Patients Collecting and Providing Patient Data

14. VA bodies can develop a catalogue of patient-provided information they wish they had or templates from data collection tools that were useful to inform their appraisals (e.g., copy of a successful survey). The catalogue should rank the importance and necessary characteristics of data elements to improve patient group information-gathering efficiency. For example, letting groups know if submitted survey data need to have been published in a peer-reviewed journal would help patient groups begin collecting and publishing data in advance of a VA (the earlier, the better).

Recommendation for How Patients and VAs Can Collaborate to Support Patient-Centered Data Collection

15. Following an appraisal, the VA body and patient group can debrief on how submitted data were useful and not useful, and how data collection or presentation can be improved for the future.

In addition to collecting and providing data, it is critical that these data are leveraged by VAs in their assessments. Patients believe that patient-submitted data is currently a footnote rather

than a priority in assessments today. Participants identified the following recommendations for VAs to leverage submitted data so that it is incorporated in decision-making:

Recommendations on Leveraging Patient/Patient Group Data

16. VA bodies can open a VA report by leading with patient-experience input to provide context and set the stage for interpretation of the assessment. Patient groups can work with VA bodies to develop this section.
17. VA bodies can develop a VA-report section describing how patient input guided VA decision-making. Rationale for why patient input was not used in a report should also be described to help improve data in the future.

The group also recommend that any learnings produced from implementing these recommendations be passed on to other organizations, for example through the NHC's Value Work Group and through wider dissemination.

The Dialogue concluded with a brief summary of the day's findings. Participants reaffirmed the importance of moving forward with collaboration between patient groups and VA bodies. While patient groups and VA bodies must partner to improve the patient-centricity of VA, participation on the part of other stakeholder groups is also critical. For example, participants described the critical role of the Food and Drug Administration's Patient-Focused Drug Development initiative. This initiative can help drive biopharmaceutical companies to incorporate patient-centered outcomes into clinical trials – resulting in more patient-centered evidence for VA. Partnership with clinical-practice guideline developers is also important since guidelines must include up-to-date and comprehensive information on subpopulations and standards of care. Participants suggested that future dialogues or roundtable discussions also include representatives of large and small employers and/or employer groups, pharmacy benefit managers, clinical societies, and public payers. Emphasis was placed on engaging employers and employer groups in the short term.

Next Steps

Patients and VA bodies agree that the goal of patient-centered VA is for patients to have access to the treatments that they need at prices they can afford. Both agree that communication is key. Participants identified 17 recommendations on what patient groups alone, VA bodies alone, or both in collaboration can do to improve the patient centricity of VA. Some of these recommendations are simple structural changes that can be achieved quickly, while others require greater investment of resources in the longer term.

The findings and recommendations from this Dialogue will be used by the NHC to inform future priorities for its value initiative. The NHC will work with its membership and VA bodies to operationalize recommendations and organize dialogues with additional stakeholders.

1. Canadian Agency for Drugs and Technologies in Health. Common Drug Review Patient Input. Available from: <https://www.cadth.ca/about-cadth/what-we-do/products-services/cdr/patient-input>
2. Rozmovits L., Mai H., Chambers A., Chan K. What does meaningful look like? A qualitative study of patient engagement at the Pan-Canadian Oncology Drug Review: perspectives of reviewers and payers. J Health Serv Res Pol 2018 Apr;23(2):72-79.
3. Berglas S., Jutai L., MacKean G., Weeks L. Patients' perspectives can be integrated in health technology assessments: an exploratory analysis of CADTH Common Drug Review. Research Involvement and Engagement (2016) 2:21.



Table 1. Summary of Recommended Actions

Patient Groups	VA Bodies	Both
Patient groups can seek to partner with professional societies to achieve meaningful patient engagement in patient-centered, clinical-practice guideline development, which can help to inform more patient-centered VA.	VA bodies should establish opportunities for patients to participate throughout VA. Example opportunities include submitting data, participating on committees, and presenting oral comments. Patient groups should ensure that their members are prepared to effectively participate in engagement opportunities.	Patient-focused drug development will yield clinical-trial data that is more patient centered, focusing on experiences of and outcomes important to patients. This will improve data sources for patient-centered VA in the future. Early dialogue between VA bodies, patient groups, and industry should encourage patient-centered clinical development plans. Patient groups should continue to advocate for patient engagement in medical-product development.
Patient groups can lead or seek to partner with other stakeholders to develop patient-centered, patient-reported outcome (PRO) and experience measures. When used in trials and other research, they will help to ensure that the patient-reported data used in VA focuses on experiences of and outcomes important to patients.	VA bodies can clearly define and communicate about opportunities regarding when and how patients can substantively contribute throughout an assessment, including during the scoping process. VA bodies can partner with patient groups to ensure that communications are effective and are reaching the patient community.	The PICOTS framework (i.e., population, intervention, comparator, outcome, time, and setting) should be used to ensure that VA relies on an agreed-upon set of facts, information, or assumptions (e.g., subgroups). Patients and patient groups can become familiar with the PICOTS framework and VA bodies can seek input from patients on PICOTS framework elements. Thus, when stakeholders engage on VA – using the PICOTS framework – they are using the same language and misunderstandings can be avoided with clearer communications.

Patient groups can add health economists to their scientific advisory boards, just as clinicians are currently included on these boards.	VA bodies can establish their own, standing patient-engagement advisory committees or collaborate with existing committees, such as the NHC's Value Work Group.	Patients and VA bodies should seek to improve bi-directional communication and continue to engage and learn from one another even after an appraisal is completed.
Patient groups can develop a catalogue of assets to share with VA bodies to help them understand the role a patient group can play, especially in conveying the wholistic experience of the patient and family.	VA bodies can develop a catalogue of patient-provided information they wish they had or templates from data collection tools that were useful to inform their appraisals (e.g., copy of successful survey). The catalogue should rank the importance and necessary characteristics of data elements to improve patient group information-gathering efficiency. For example, letting groups know if submitted survey data need to have been published in a peer-reviewed journal would help patient groups begin collecting and publishing data in advance of a VA (the earlier, the better).	Patients and VA bodies can co-develop training to prepare patients to engage with VA bodies and VA bodies to engage with patients.
Patient groups can develop a patient-provided information (e.g., experiences, preferences, PROs) dossier to facilitate working with VA bodies/payers/PBMs/employers.	VA bodies can open a VA report by leading with patient-experience input to provide context and set the stage for interpretation of the assessment. Patient groups can work with VA bodies to develop this section.	Following an appraisal, the VA body and patient group can debrief on how submitted data were useful and not useful, and how data collection or presentation can be improved for the future.
Patient groups can engage VA bodies when the patient groups are developing or updating patient registries and/or surveys to ensure they include questions that provide information sought by VA bodies.	VA bodies can develop a VA-report section describing how patient input guided VA decision-making. Rationale for why patient input was not used in a report should also be described to help improve data in the future.	