CADTH Patient Input Process Review

Findings and Recommendations

Prepared for CADTH
By SECOR
September 2012

April 2013

List of Abbreviations

BC	British Columbia
CADTH	Canadian Agency for Drugs and Technologies in Health
CDR	Common Drug Review
CEDAC	Canadian Expert Drug Advisory Committee
CFHI	Canadian Foundation for Healthcare Improvement
COI	Conflict of Interest
DBC	Drug Benefit Council (BC)
DPAC	Drug Policy Advisory Committee
FTE	Full-Time Equivalent
FWG	Formulary Working Group
HTA	Health Technology Assessment
INESSS	L'Institut national d'excellence en santé et en services sociaux
NICE	National Institute for Health and Clinical Excellence
PBAC	Pharmaceutical Benefits Advisory Committee (Australia)
pCODR	pan-Canadian Oncology Drug Review
SMC	Scottish Medicines Consortium

Contents

Context and Methodology

Analysis

- Current state analysis of the Common Drug Review (CDR) patient input process
- CDR stakeholder — survey data analysis
- Comparative analysis — local and international peers

Assessment

Recommendations

Context: Context and Key Messages

CADTH initiated a patient input program as part of the CDR process in May of 2010 in response to requests from several key stakeholders.

- *CADTH agreed to evaluate the program after 12 to18 months of having it in place; given that no standard patient input process exists across HTA agencies, it was expected that changes would need to be made after the evaluation.*

An initial high-level assessment of the program was undertaken and reported as part of the broader, external evaluation of CADTH conducted by SECOR at the end of 2011.

- *Key finding: Patients, committee members, stakeholders, manufacturers, and CADTH staff are not fully aligned on the objective of the patient input process, and are unclear as to what the impact has been.*

SECOR was engaged in April of 2012 to examine CADTH's Patient Input Program in more depth, and to compare CADTH's approach to international peer health technology assessment (HTA) agencies.

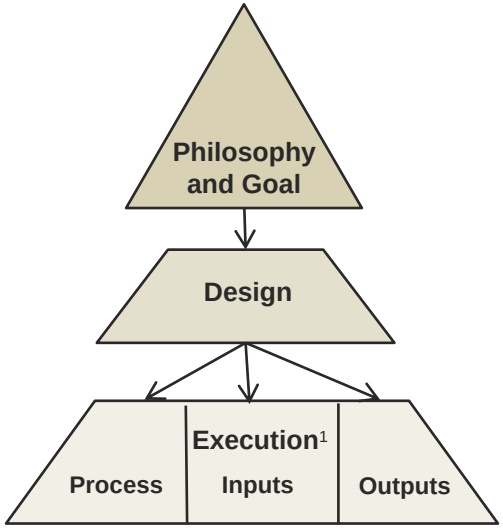
- *This report summarizes key findings and potential improvements for CADTH's consideration.*

Key messages from the analysis:

- CADTH's patient input program is roughly on par with, or more developed than, most of its peers. NICE and pCODR have significantly more evolved programs on several dimensions.
- CADTH could implement best practice learnings from international peers to address several patient input process design and execution gaps identified by key stakeholders.
- However, a more fundamental issue needs to be addressed first: whether stakeholders are aligned on the purpose, value, and credibility of soliciting patient input as evidence in making drug listing recommendations.

Context: Analytical Framework

SECOR evaluated CADTH's patient input process along three main dimensions and several sub-dimensions:



Evaluation Vectors	Underlying Questions
Philosophy and Goal	
▪ Importance to decision-making	*How is patient data weighted relative to clinical and economic evidence?* *How aligned are key drug review decision-makers and stakeholders regarding the role of patient input?*
Design	
▪ Stage of incorporation	*At what stage (s) of the review process is patient input incorporated?*
▪ Mechanisms of solicitation	*What types of patient entities are able to provide input? What form does the input comprise?*
Process	
▪ Flexibility of input	*How comprehensively can patients provide their data through the submission form?*
▪ Time allotment	*Does the time allotment meet the timelines of the review? Is the time allotment reasonable for patient groups to submit the data?*
▪ Utility	*How accessible and user-friendly is the process?*
Inputs	
▪ Breadth of data points	*Are there sufficient data points to inform decision-making?*
▪ Type/quality of data gathered	*How relevant/useful is the collected data in informing decision-making?*
▪ Quality control	*What types of support/communication mechanisms are available for patient groups (before, during, and after the process)?*
Outputs	
▪ Incorporation of evidence	*How is the evidence incorporated into decision-making?*
▪ Transparency of decision-making	*How are those who make a submission kept abreast of the process?* *How are key stakeholders (including patient groups who have submitted) made aware of how their information was used for decision-making?*

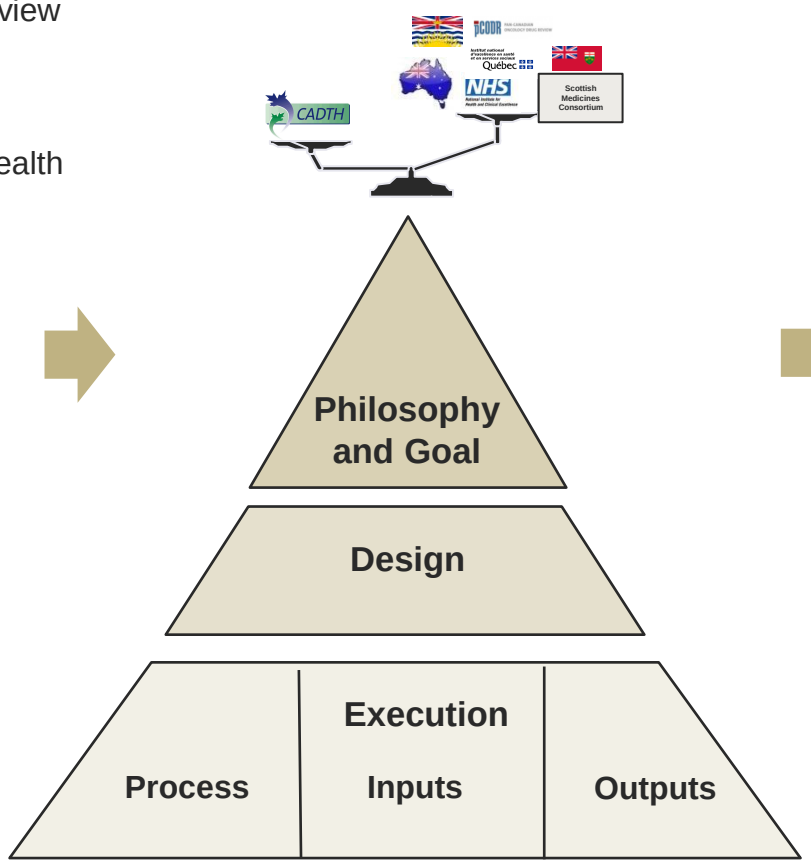
[1] *Written submission process only*

Methodology

Inputs

- **Data/document review**
  - including phase I evaluation interview notes and data
- **Key informant interviews**
  - Elaine MacPhail (CADTH)
  - Karen Facey (Evidence-based Health Policy Consultant, Scotland)
  - Judith Glennie (J.L. Glennie Consulting Inc.)
- **HTA Agency comparison**
  - pCODR (Mona Sabharwal)
  - INESSS (Lucie Robitaille)
  - Australia PBAC (Janet Wale)
  - NICE (Lizzie Amis)
  - Scotland SMC
  - British Columbia, Ontario
- **Survey data**
  - Patient group survey (29)
  - Industry survey (17)
  - CEDAC members survey (8)
  - CDR reviewer (13)
- **Literature** (grey, published)

Analysis



Transparency of the process

Outputs

Key Findings

Comparative Analysis

Recommendations for Improvement

The review was conducted over 5 weeks, from April 10 to May 4, 2012

Contents

Analysis: Overview of Current State of CDR Patient Input Process

Importance to decision-making

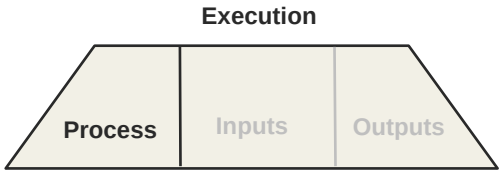
- Objectives
  - Seek information via Canadian patient groups
  - Respect existing review time frames for CDR
  - Systematically incorporate input throughout the process

Stage of incorporation

- Patient input is collected at the beginning of the CDR process and is used for protocol development, incorporated in the draft and final report, and presented by laymen representatives (CEDAC public members)
- The input is not provided/incorporated regarding the draft report

Mechanisms of solicitation

- Patient input currently comes from patient group written submissions to CADTH



Flexibility of input

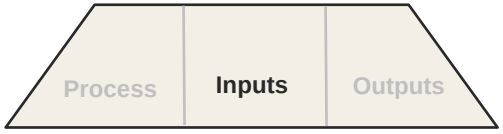
- Patient groups can submit information up to 6 pages; there is no word limit to questions included in the template

Time allotment

- Current patient input process respects the existing CDR timeline

Utility

- A section on a CADTH web page is dedicated for patient input
- The CDR review process is published on the CADTH website
- The submission document is to be downloaded by patient groups, completed, and submitted online or faxed



Breadth of data points

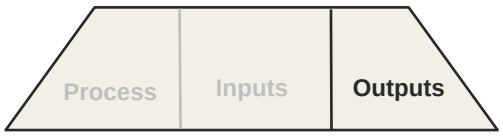
- Number of patient submissions range from 0 to 9 per review with an average of 1.8 submission and a median of 1 submission
- CADTH does not accept individual patient input

Type/quality of data gathered

- Patient input currently comes from patient group written submissions to CADTH
- Submission form has questions in a similar structure as some other HTA agencies

Quality control

- A guidance document is available on the CADTH website
- CADTH organized one patient group training session in 2011
- Elaine has participated in ~5-6 initiatives where she spoke about CADTH patient input process
- No formal feedback mechanism in place
- No dedicated FTE to patient initiative
- Contact information is posted on the CADTH website should patient groups have any questions



Incorporation of evidence

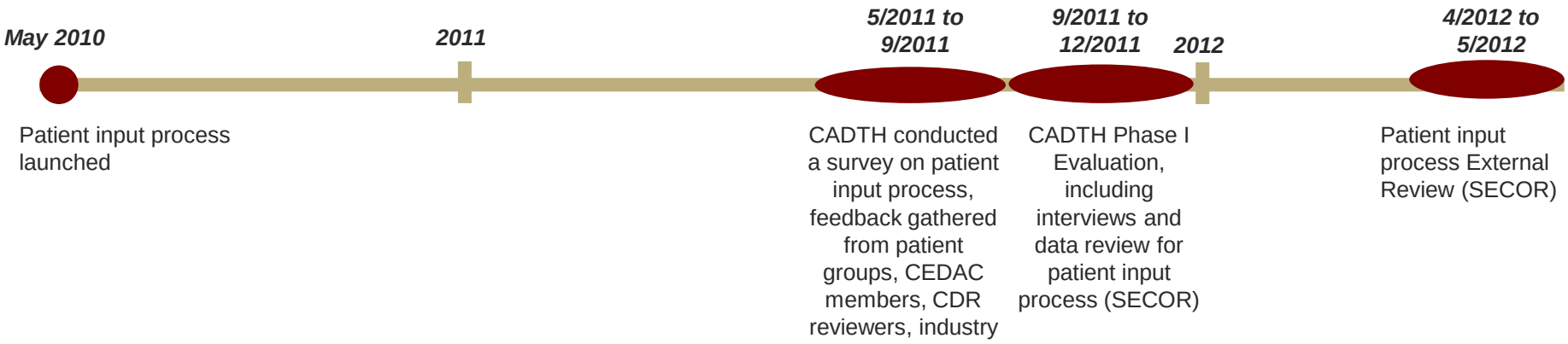
- Information such as issues or outcomes of importance is used by CDR reviewers to develop protocol
- Patient input information is summarized in its own section in the clinical report
- Patient input is included in the CEDAC Brief, is presented by laymen representatives at the CEDAC meeting, and the committee considers the patient input with other evidence to make a decision

Transparency of decision-making

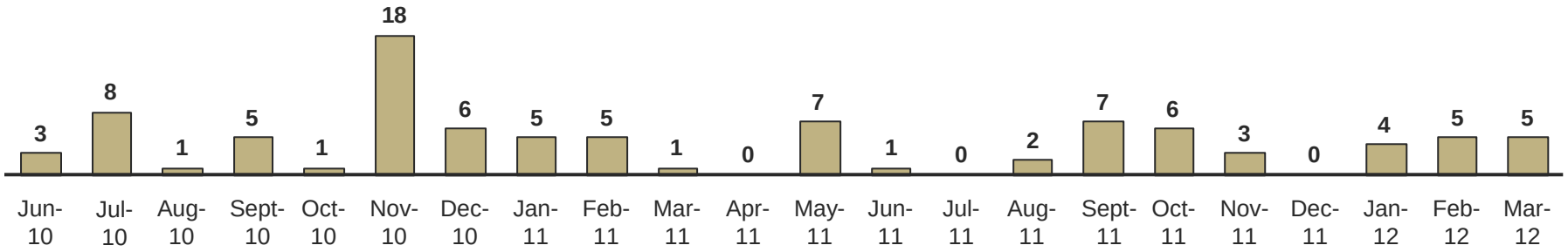
- Patient input is summarized in a section of the final recommendation document, and may be included in the recommendation and reasons for recommendation section. The final recommendation is posted online

Source: CADTH website, interview with Elaine MacPhail, SECOR Analysis

Analysis: Historical View of CDR Patient Input Process



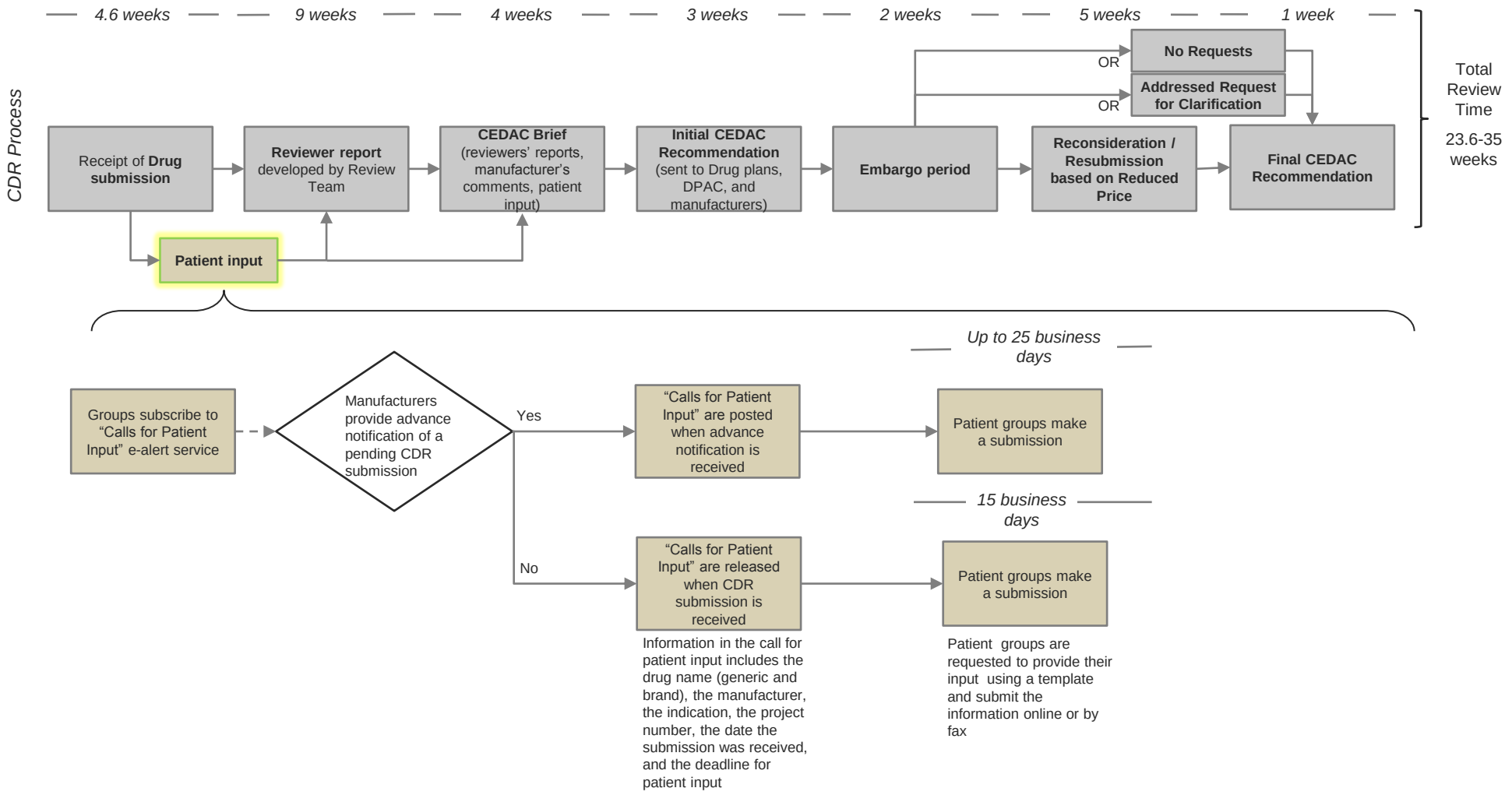
Number of patient input submissions by month from June 2010 to March 2012*



Source: SECOR Analysis, CADTH internal data

**Note: The number of patient submissions is influenced by the number of CDR submissions, and number of patient groups that may be impacted by the drug.*

Analysis: Overview of the CDR Patient Input Process



Source: CADTH website; interviews with internal CADTH staff and external stakeholder groups

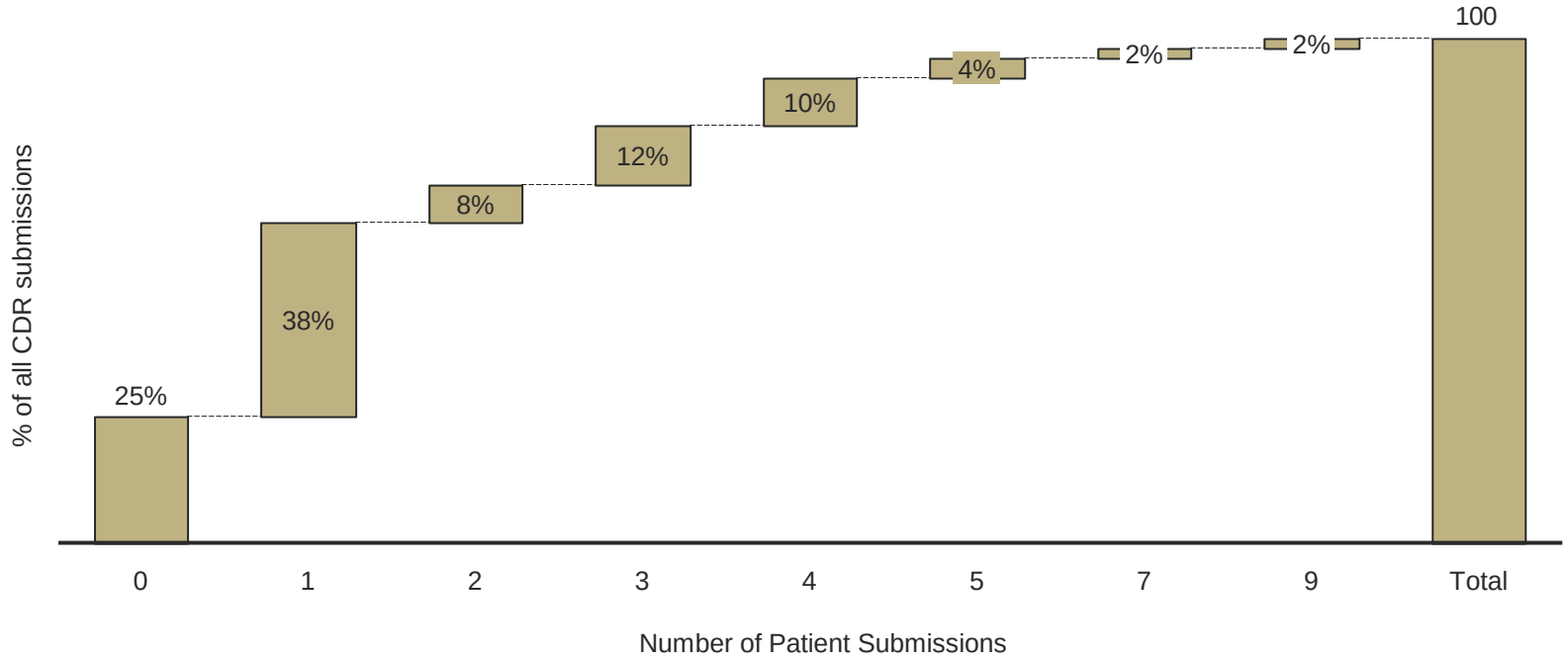
Analysis: 70% of CDR Drug Submissions Receive 0 to 4 Patient Submissions

Across 52 drug reviews from May 2010 to March 2012:

- 25% did not receive a patient submission
- 38% had 1 patient submission
- 30% had 2 to 4 patient submissions

Number of patient input submissions received across all drug reviews to date

Review number – all patient input submissions from 21 May 2010 – 31 March 2012



Source: CADTH Internal Data, SECOR Analysis

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Analysis: Comprehensive Survey Data was Analyzed

In September 2011, CADTH surveyed four groups of stakeholders involved in the patient input process (patient groups, industry, CEDAC members, and CDR reviewers).

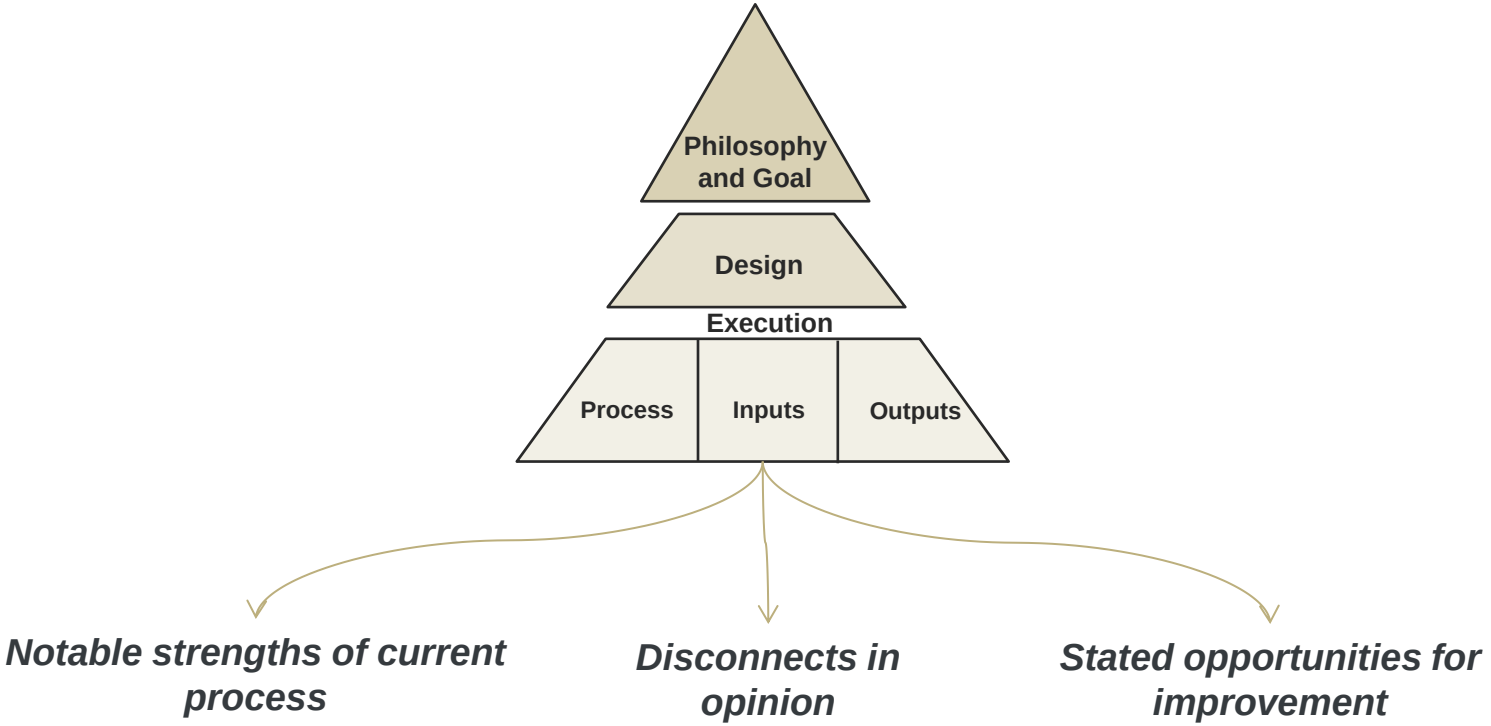
The questions were tailored based on touch points the stakeholders have in the process.

Surveys contained multiple choice questions, as well as open-ended questions.

Response rates were as follows:

- Patient (29)
- Industry (17)
- CEDAC members (8)
- CDR reviewers (13)

The answers are synthesized and mapped to the framework below.



Analysis: Survey Results — Philosophy and Goal

Although most CEDAC members and CDR reviewers agree with the rationale for a patient input process, there continues to be skepticism about the objectivity of the patient voice

Notable Strengths of Current Process

- Most members believe that the concept of having patient submissions is important
  - "I believe that patient-related outcomes are important and should be highlighted"
- Most believe that patient input provides information that is not otherwise available

- The majority said patient input has enhanced CDR review
  - "Seems to be adequately described during CEDAC meetings"
  - "The patient input information simply allows us to be more confident in stating that a given outcome is of importance to patients, rather than speculating that it is"
  - "We are interested to know the patients' opinions, experiences, etc…we cannot critically appraise that in the same way"
- Most believe patient input helps reviewers gain more understanding on how treatments work in real life: logistically and in terms of efficacy
- Most said patient input is fairly or very relevant for CDR reports
- ~70% feel the information from patient submissions is not otherwise obtainable
- The majority of reviewers are comfortable with using qualitative data from patient submissions. Reviewers:
  - Believe that expert opinion and evidence-based information can compliment patient input
  - Understand the context of patient input and will not mistake for data obtained from clinical trials
  - Can use patient input for guiding context

Patient | CEDAC members | Key informants | Industry | CDR reviewers

Disconnects in Opinion

- 5/8 members feel patient submissions play an adequately or fairly meaningful role in decision-making, while 3/8 felt patient submissions do not play a very meaningful role
- 3/7 are not very or not at all comfortable using the qualitative patient submissions to inform recommendation

- Mixed opinions on integration of patient input:
  - "I'm not sure trying to integrate patient values, which are subjective, into a very objective and structured systematic review…is the best approach; perhaps a separate document that deals only with patient input could be part of committee's briefing materials…"
  - "It is very challenging to integrate patient values when often industry has not captured this type of information in their clinical trials"

- 3/7 CEDAC members are not at all or not very comfortable in using qualitative patient submissions
  - "Integration of patient view should be limited to providing context to clinical and cost evidence… recommendations should continue to be based on objective clinical and cost-effectiveness data"

- 8/15 do not feel patient-important outcomes identified through the patient input process will influence clinical trial design, 3 reported it will substantially influence the design
  - "Manufactures really need to receive clearer signals from CDR on the impact that the inclusion of such outcomes will have on their recommendations"

Stated Opportunities for Improvement

- Most do not believe that patient input has enhanced CDR
- Most do not think patient input has increased transparency, fairness, and objectivity of the drug review process

- Most say patient submissions have little influence on their final voting decision

- Most believe that there is a innate subjectivity of inputs from patient groups
  - "I'm not sure how well or how consistently they truly represent the individual issues or concerns"

- Most do not think patient submissions represent the majority view of patients and caregivers, and the minority perspective of important subgroups
  - "The information via the public members has not struck me as true patient input but rather as another selling avenue from companies"
  - "Not sure that patient evidence should be given much weight versus objective clinical cost data. It is inherently subjective and biased: no patient or patient group will ever not want access to a new drug"

- Patients also question the objectivity of the reviewer
  - "I would like to see full disclosure of all possible biases for stakeholders in the CADTH process…e.g., salary from cancer agency, public drug program, government…that should be equally declared"
- "The guidance document places far too much emphasis on the bias of pharmaceutical funding. Perhaps this concern…can be approached in a different way that does not immediately come off as an assault on the integrity of patient organizations"

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Design of Patient Input Process

CDR reviewers and CEDAC members believe that patient information has impacted various stages of the CDR review. Most agree that additional stakeholders such as individual patients/ caregivers/professionals could/should provide input; however, there is no clear inclination toward any particular group(s)

Notable Strengths of Current Process

- 70% feel patient information is relevant for protocol-building
- 76% feel patient information is relevant for contextualizing clinical and economic data

- 86% agreed patient input contributed to protocol development
- 71% agreed that presentation by public members at CEDAC meeting contributed to CDR review
- 86% agreed that reference to patient input in the CEDAC recommendations and reasons document contributed to CDR review

Disconnects in Opinion

- High proportion agree that groups/individuals (such as individual patients/caregivers/professionals and health charities) could make submissions, while some noted that they feel patient groups are the best sources of input

- No majority agreement on whether alternative formats such as survey, patient preference ranking, target questionnaire, or testimonials would be the most conducive to gathering valuable/objective patient information
  - "I think the use of specific and standard questions which are aimed at views of the entire patient population would be good"
  - "I would avoid individual patient input (presumably, their information is captured overall by querying patient groups)"
  - "A target questionnaire would introduce potential for bias"
  - "I think that surveying patient groups will capture information most relevant to patients"
  - "My preference is to incorporate a survey"

- 5/7 agree that CADTH should allow other stakeholders to provide patient input, and the most relevant, alternative sources of information include individual patients, caregivers, and health care professionals, but no clear inclination toward any particular group

- 4/7 do not think patient input contributes to contextualization of data

Stated Opportunities for Improvement

- Some would like to have a patient advisory committee that reports to CADTH executives

- Some feel it may be valuable to have health care professionals who have hands-on expertise provide input
- "We need more patient input versus input from patient groups"

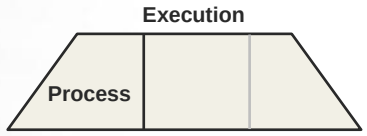
- HTAs should conduct their own research to reduce the tendency of bias

- Most do not think patient submissions represent the majority view of patients and caregivers and the minority perspective of important subgroups

Patient | CEDAC members | Key informants | Industry | CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Execution of Submission Process (I)



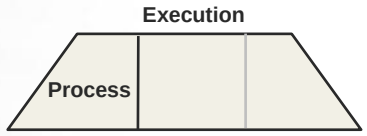
Most are satisfied with the utility of the submission form and with the CADTH website; however, patient groups noted some opportunities for improvement. Opinions are mixed (more say “no”) on whether groups with conflict of interest should be managed differently compared with declarations by other persons involved in the CDR process.

Utility and Flexibility of Input	Notable Strengths of Current Process	Disconnects in Opinion	Stated Opportunities for Improvement
Submission Form	▪ Most felt the submission template allowed the patient group to describe the issues/outcomes that are important to the majority of patients in the group ○ “We are able to fit all of the information we wish to include into the template”		▪ Some said the submission form is not long enough ▪ “Some of the questions are more geared to physical impairments than mental conditions” ▪ Some said the submission form is not long enough
Website	▪ Most groups are fairly or very satisfied with the accessibility of the website		▪ Some would like to see a better navigation structure on the CADTH website ▪ Some would like CADTH to send a receipt when a submission is received ▪ Some would like CADTH to send disease-specific e-alerts
Management of Conflict of Interest		▪ Mixed opinions (with slightly more numbers say “no”) on whether groups with conflict of interest should be managed differently compared with declarations by other persons involved in the CDR review process	▪ “Move conflict of interest statements to the top of the section”

Patient · CEDAC members · Key informants · Industry · CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Execution of Submission Process (II)



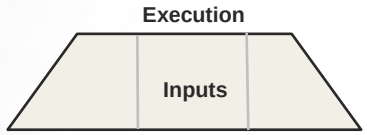
Patient groups have commented that there is not enough time to complete the submission. Industry groups suggest it is possible to provide more advanced notice to patient groups

	Stated Opportunities for Improvement
Time Allotment	▪ Large proportion indicated that they do not have enough time to complete a submission ▪ One group noted that lack of resources is a major reason to not make submissions in a timely manner
	▪ Some suggested that it is possible to provide more advanced notice to patient groups ▪ Some respondents are aware that the timeline for submission is short and suggested that longer advance notice would give patient groups more time to complete the submission
	▪ Some commented that there is perhaps not enough time for patient groups to collect and submit input

Patient | CEDAC members | Key informants
Industry | CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Data Inputs by Patient Groups (I)



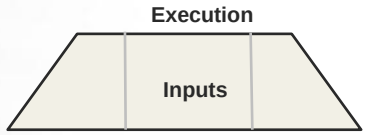
The quality of submissions are variable: 70% of CDR reviewers feel the information from patient submission is not otherwise obtainable; 5/7 CEDAC members feel the information collected is adequately or fairly relevant for making a recommendation. Patient groups see value in having forums for in-person discussion such as presentations, focus groups, and direct conversations.

	Notable Strengths of Current Process	Disconnects in Opinion	Stated Opportunities for Improvement
Quality/Type of Data Gathered	▪ 70% feel the information from patient submissions is not otherwise obtainable ▪ 5/7 feel the information collected is adequately or fairly relevant for making a recommendation ▪ Most agree that patient information elicits the most relevant patient values and preferences	▪ Mixed opinions whether CADTH should ask specific questions for each review ▪ Some information may not be relevant ○ "Testimonials are not useful"	▪ "The quality is highly variable" ▪ Separate the questions from the specific technology ○ "Make sure input focuses on outcomes of importance to the patient rather than be specific to a given drug" ▪ 6/7 agreed patient preference ranking and 5/7 agreed targeted question based on specific disease/drug would be conducive to gathering valuable and objective patient information ▪ Should have sections for individual examples, as well as group summary ▪ Would like to add questions that address the broader impact of drug on patients' and caregivers' lives ▪ Most supported various forms of in-person discussion such as presentation, focus groups, and direct conversations with CADTH and patient/public members on CEDAC ▪ Respondents commented CADTH could consider more diverse form/media of submission

Patient · CEDAC members · Key informants · Industry · CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Data Inputs by Patient Groups (II)



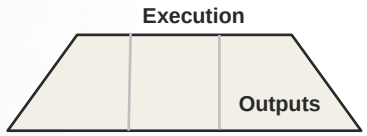
Patients are satisfied with the guidance document and viewed training sessions to be helpful. CDR reviewers and CEDAC members noted more support could be provided to lay members.

	Notable Strengths of Current Process	Disconnects in Opinion	Stated Opportunities for Improvement
Quality Control			▪ Would like CADTH to give more support to lay representatives
Communication/ Support Mechanisms	▪ "Training sessions are helpful" ▪ Majority respondents are satisfied with guidance document	▪ Respondents have informed patient groups of the submission; however, they have a different approach to support other aspects of the process ○ "We've been trying to inform patient groups of this opportunity and to give them some insights (where we can) re the process…" ○ "Other than making a patient group aware of a CDR submission, we do not engage with patient groups in any way regarding the submission"	▪ The guidance document could be more patient-friendly ○ "Could be more patient-friendly, including FAQs" ○ "Write from the patients' perspectives; e.g., advice from patients to patients on how to prepare submissions ▪ "Training on preparing submissions would provide value" ▪ "The challenge for the public members, of course, is that they don't necessarily have the expertise to be critical of what they are receiving and presenting, and this impacts their ability to meaningfully participate in the discussion. One of them has actually observed this to me, incidentally" ▪ Would like to have an opportunity to comment on the draft summary before submitting to CEDAC ▪ Meeting with lay representatives would be helpful to ensure accurate information is presented
Patient's understanding of the technology under evaluation and the process		▪ Most groups feel they have adequate information about the drugs; however, no information is available for those who do not have adequate information ▪ 67% have a "fairly well " or "very well" understanding of the process	▪ Patient groups would like to have better understanding of information needed

Patient | CEDAC members | Key informants | Industry | CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and stakeholders, SECOR Analysis

Analysis: Survey Results — Outputs of Patient Input Process



Industry and patient respondents seek more clarity on how information is used in the decision-making process

	Notable Strengths of Current Process	Disconnects in Opinion	Stated Opportunities for Improvement
Incorporation of Evidence	▪ Most indicated using patient-important outcomes/issues identified in the submission contributed to protocol development and contextualizing clinical and economic data	▪ Half do not feel the information is useful for contextualizing clinical data	▪ Most say patient submissions have little influence on their final voting decision
	▪ Most indicated using patient-important outcomes/issues identified in the submission contributed to protocol development		▪ 4/7 felt that inclusion of patient input information throughout the body of CDR reports does not contribute to CDR review
Transparency of Decision-Making	▪ 10/14 have adequate understanding of CDR patient input process		▪ Large proportion identified that lack of understanding of patient input's impact is a key gap, and would like to see CADTH demonstrate more accountability for the use of patient input
	▪ 67% have a "fairly well" or "very well" understanding of the process		▪ Would like more information on how the submission is being used in the process ○ "The perception among organizations is that our submissions are all but DISMISSED by some reviewers if they see any pharmaceutical funding whatsoever…this is incredibly unfair to the work we do"
			▪ "Would like to see patient submissions transparent (e.g., posted online)"
			▪ Would like clearer disclaimer on how the "conflict of interest" is used

Patient | CEDAC members | Key informants
Industry | CDR reviewers

Source: CADTH Survey, interviews with subject matter experts and and stakeholders, SECOR Analysis

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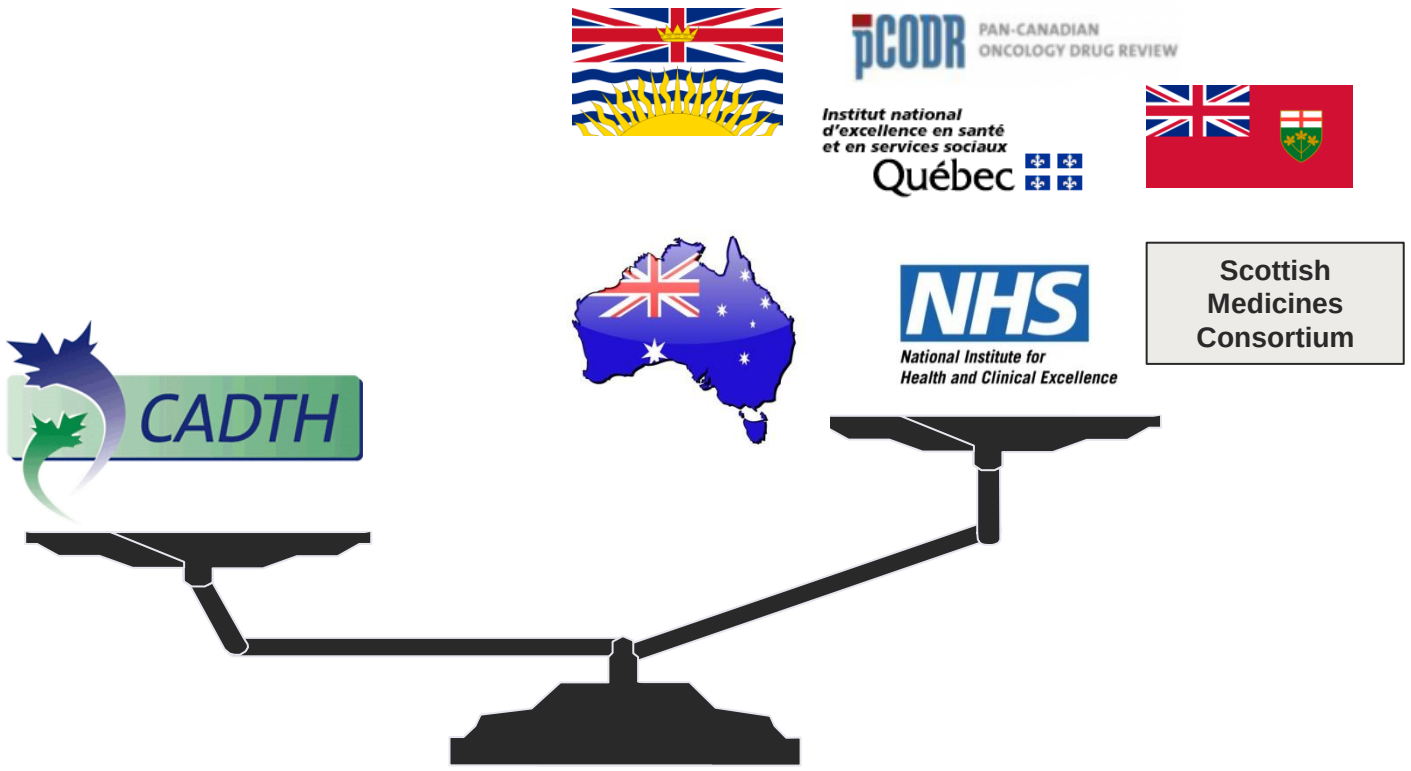
Analysis: CADTH was Compared to 3 International HTA Agencies and 4 Canadian HTA Agencies



Analysis: Summary Agencies Map Spectrum of Models

	New and Evolving	*Range of HTA Agency Patient Input Models*	*Mature, Highly Resourced*
Philosophy and Goal			
▪ Importance to decision-making	*Minor consideration*		*Equal weighting with other evidence*
Design			
▪ Stage of incorporation	*One stage*		*Multiple stages*
▪ Mechanisms of solicitation	*Limited, one-dimensional*		*Broad, varied*
Process			
▪ Flexibility of input	*Limiting*		*Adaptable to given context*
▪ Time allotment	*Conflict with review timeline*		*In line with review timeline*
▪ Utility	*Manual, complex*		*Automated, user-friendly*
Inputs			
▪ Breadth of data points	*0*		*> 5*
▪ Type/quality of data gathered	*Generic*		*Relevant*
▪ Quality control	*Minimal*		*Multi-faceted*
Outputs			
▪ Incorporation of evidence	*Anecdotal*		*Systematic, proactive*
▪ Transparency of decision-making	*Transparency of decision-making*		*Timely, detailed, active*

Note: Based on initial data-gathering from secondary sources, as well as limited interviews with select organizations; only where sufficient data was available to assess.

Analysis: Overview of Agencies Compared to CADTH (I)

CADTH is among few HTA agencies that consider patient input as a piece of evidence

	Canada	United Kingdom	Scotland	Australia	Quebec	Ontario	BC	Canada
Agency	CADTH	NHS National Institute for Health and Clinical Excellence	Scottish Medicines Consortium (SMC)	Pharmaceutical Benefits Advisory Committee (PBAC)	Institut national d'excellence en santé et en services sociaux Québec	Ontario	British Columbia Ministry of Health	pCODR Pan-Canadian Oncology Drug Review
Philosophy and Goal	Launched in 2010 "Systematically incorporate input throughout the process"	Launched in 1999 "We endeavour to treat patient input as an equal evidence as clinical and cost-effectiveness data"	Launched in 2003 "Patient/carer perspective is taken into consideration by the SMC"	Launched in ~2007 "Support HTA agencies to make decisions that will work for patients and clinicians"	2007 (allow patient groups/individuals to intervene) 2010 (public representatives) "The data are definitely used"	Launched in 2010 "Formal framework to systematically incorporate patient evidence into the drug review and funding process"	Launched in 2010	Started in 2011 "Patient is regarded on the same level as manufacturers as a relevant and impacted stakeholder. Goal is to have patient groups have more access to review information and be more active participants in review process"
Design — Stage of incorporation (type of technology, incorporate patient input, stage of incorporation for drug appraisal)	Drugs Use submission for protocol development, integrated in the CDR review reports, and presented at the CEDAC discussion	Overarching issues, drug and non-drug appraisals, and other work programs (clinical guidelines ,etc.) Submit ideas for topic selection, comment on scoping document and draft guidance, presented at appraisal discussion, may choose to appeal	Overarching issues, Drugs Presented at decision making discussion	Drugs Consumer could submit comments on PBAC agenda Presented at decision - making discussion	Drugs During the appraisal process (can ask for a meeting with the evaluation committee)	Overarching issues, drugs Summarized by patient member and presented to the committee during funding deliberation stage	Drugs Presented by public members during funding deliberation stage	Drugs Used for protocol development, the draft report, and decision-making discussion
Design — Mechanisms of solicitation	Patient group written submissions 2 public members on CEDAC (present submission information)	3 public members (present submission information) on appraisal committee and 2 patient experts also attend Patient group written submissions Comments on draft documents (scope, reports, and guidance) Appeal Citizens Council (overarching ethical principles)	The Patient and Public Involvement Group (PAPIG, provide overarching recommendations) Public members (present submission information) Patient group written submissions	1 patient representative on the committee speaks to the submission information (does not present the information) Patient group/individual patient written submissions (filtered by internal staff member) Consumer impact statements — produced by Consumer Health Forum at the request of PBAC secretariat to inform decision-making	Patient group intervention Remarks and suggestions from the public/patient Public representative on standing committee	Patient group written submission 2 patient representatives on the committee (present submission information)	Patient group /individual patient and caregiver submission 3 public members on the Council (present submission information)	Patient representation on pCODR Expert Review Committee Patient written submission Comments on the draft recommendation

Source: Agency websites, public documents, limited interviews with select organizations

Analysis: Overview of Agencies Compared to CADTH (II)

	Canada	United Kingdom	Scotland	Australia	Quebec	Ontario	BC	Canada
Agency	CADTH	NHS National Institute for Health and Clinical Excellence	**Scottish Medicines Consortium (SMC)**	**Pharmaceutical Benefits Advisory Committee (PBAC)**	Institut national d'excellence en santé et en services sociaux Québec	Ontario	British Columbia Ministry of Health	pCODR Pan-Canadian Oncology Drug Review
Process — Flexibility of input	Patient groups can submit information up to 6 pages	No limit on number of pages (summary requested if > 10)	No limit on number of pages	200 words for each answer	N/A	2-page limit (do provide flexibility if required)	No limit on length of answers	8-page limit
Process — Time allotment (entire review process)	Up to 25 business days with advance notice (if provided by manufacturer) 15 business days without advance notice (CDR process is ~6 months)	8 weeks for single technology appraisal and 14 weeks for multiple technology appraisal (appraisal process is ~1 year)	Up to 2 months (appraisal process is ~4.5 months)	14 business days (appraisal process 17 weeks)	N/A	1-3 months, shorter timeline if the drug is undergoing a "rapid review" (Timeline information not readily available for normal review, rapid review is 30 days)	4 weeks (Target timeline for standard review is 9 months; for complex review,12 months)	Up to 1 month advance notice (if provided by submitter) plus 10 business days 10 business days without advance notice (Review process is 5-8 months)
Process — Utility	A section on web page dedicated for patient input; the review process posted on website; the submission document to be completed and submitted online or faxed	A section on web page dedicated for patient involvement; each topic has web page; review process, and large amount of education material is made available |	A section on web page dedicated for patient input	Little information on patient engagement; no process map posted; patient submission can be submitted online	No dedicated section for patient input in English on website	A section on web page dedicated for patient input; the review process posted on website; the submission document to be completed and submitted via email/fax/mail	A section on webpage dedicated for patient input; the review process posted on website; the submission document can be completed online	A section on web page dedicated for patient input; the review process is posted on website; input is entered on a submission form , which can be submitted online or emailed

Source: Agency websites, public documents, limited interviews with select organizations

Analysis: Overview of Agencies Compared to CADTH (III)

	Canada	United Kingdom	Scotland	Australia	Quebec	Ontario	BC	Canada
Agency Name	CADTH	NHS National Institute for Health and Clinical Excellence	**Scottish Medicines Consortium (SMC)**	**Pharmaceutical Benefits Advisory Committee (PBAC)**	Institut national d'excellence en santé et en services sociaux Québec	Ontario	British Columbia Ministry of Health	pCODR Pan-Canadian Oncology Drug Review
Inputs: Breadth of data points	Range from 0 to 9 per submission, with average of 1.8 submissions and median of 1 submission	"A couple, sometimes 3 or 4"	Information not readily available	"Major submissions (0 to a couple), less for minor submissions"	N/A	April 2010 to June 2011, average of 0.63 submission/review	Average 16 submissions/drug. For the 16, 8.8 from individual patients, 5.5 from caregivers, and 1.7 from patient groups	Information not readily available
Inputs: Type/quality of data gathered	"The quality is variable" Generic questions on submission form	Quality is variable Generic questions on submission form	Generic questions on submission form	Generic questions on submission form	N/A	Generic questions on submission form	Generic questions on submission form	"The quality is variable" Generic questions on submission form
Inputs: Quality control	Has organized 1 training session Guidance document No formal feedback	Sophisticated Patient and Public Involvement Programme (1 dedicated FTE) that supports patient groups/representatives and lay members through formal and informal formats Section in draft guidance consultation document sets out each input	Sample patient input posted online Has organized a training day in 2011 Employed a part-time individual to support and provide feedbacks to patient groups	No formal feedback No training session by HTA, some support from Consumer Health Forum (an independent organization not supported by PBAC)	N/A	Guidance document Executive Officer has regular meetings with patient groups No formal feedback on submission No information on training for patient groups	A review was completed 12 months after input was solicited from general public from manufacturers, DBC members, and ministry staff Training — one info/training session with patient groups prior to official start of patient input process	Guidance document ~Quarterly webinars No formal feedback
Outputs: Incorporation of evidence	Input is considered at each designated stage Minor framework on input incorporation	Input is considered at each designated stage Some framework on input incorporation (formal methods and process guides)	Input is considered at decision-making stage (presented by one of three public members after clinical and cost-effectiveness evidence) Minor framework on input incorporation	Input is considered at each designed stage Minor framework on input incorporation	Input considered when patient groups intervene No systematic framework on input incorporation	Input considered at funding deliberation stage	No information on how evidence is incorporated	Patient input incorporated into clinical and economic reports A deliberative framework is used to form recommendation
Outputs: Transparency of decision-making	A section that includes summaries of patient input information in final recommendation, no specifics about how information is used	Section in draft guidance consultation document sets out each input. No detailed specifics about how information was used	No public information about how patient information impacted the decision-making	PBAC produces a public summary document of decisions and reasons	Patient groups participate in the intervention meetings	Committee discussions relating to patient input summarized in "transparency bulletins" outlining committee recommendation and its rationale	No public information about how information impacted the decision-making	Patient group submission is integrated into various parts of the report (clinical report) (posted online) Patient feedback on recommendation is posted online

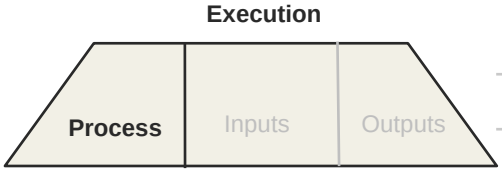
Source: Agency websites, public documents, limited interviews with select organizations

Analysis: Key Findings From Comparative Analysis (I)

	Relevant findings from comparative analysis
Importance to decision-making	• NICE — Endeavours to have patient input as "an equal leg of the evidence stool," along with clinical and cost-effectiveness evidence • NICE — Patient group presentations required to state conflicts up front • pCODR – Patients, as a relevant and impacted stakeholder group, are regarded on the same level as manufacturers • pCODR — Only allows patient groups who receive funding from more than one funder; no single funder provides more than 50% of the group's operating funds to submit evidence; COI of patient group also posted online
Stage of incorporation	• NICE & PBAC — Incorporate patient input for all topics (including non-drug and clinical guidelines) • NICE — Incorporates patient input at every stage of drug appraisal process in different forms; patient groups can appeal on certain grounds • pCODR — Allows patient groups to comment on draft recommendations
Mechanisms of solicitation	• NICE — Standing lay committee members present at committee meetings, patient experts attend and answer questions • PBAC — Patient representatives are well connected within the patient community and have a strong voice at the decision-making table • PBAC, BC, Quebec — Accept individual patient input • NICE — Only accepts input from national patient organizations (but anyone may comment on draft guidance via the website) • PCODR — Individual patients not allowed to submit input but can contact pCODR if there is no patient advocacy group for a particular cancer type • Denmark HTA agencies conduct their own systematic literature research on patient information

Source: Agency websites, public documents, limited interviews with select organizations

Analysis: Key Findings From Comparative Analysis (II)



	Relevant findings from comparative analysis
Flexibility of input	• NICE — No limit on number of pages for patient groups, but asks patient groups to present a 1-page summary if submission is > 10 pages
Time allotment	• Timeline allotment varies by agency depending on the overall review process
Utility	• BC formulary — Allows patients/caregivers/patient groups to fill out the submission form directly online
Breadth of data points	• BC formulary — Had higher number of data points than other HTA agencies because it accepts submissions from individual patients
Type/quality of data gathered	• BC formulary — BC had disease-/drug-specific questions previously but only generic questions now • SMC — Recently updated its submission form based on the feedback from Public Involvement Officer who supports patient groups to make submissions
Quality control	• SMC — Has posted a sample patient submission online, supports a part-time Public Involvement Officer who gives advice/feedback to patient groups on submissions • NICE — Sophisticated Patient and Public Involvement Programme, support patient groups and lay representatives through various informal (e.g., email, telephone) and formal formats (e.g., training sessions)
Incorporation of evidence	• pCODR — Has established a deliberate framework to incorporate patient input • NICE — Reviewers receive patient input data verbatim • NICE — One of the initiatives this year is to improve the “methods guide” for reviewers/committee members, including the section on patient input data integration
Transparency of decision-making	• NICE — Sends draft guidance verbatim to patient groups
	• NICE — Provides resource support (e.g., a meeting room) to Patients Involved in NICE, an independent forum that exists to provide organizations who engage with NICE with a system of mutual support and information-sharing, and to act as a “critical friend” to NICE • PBAC — Recuperates its review cost by charging industry members by submission • Australia — Consumer Health Forum (not supported by PBAC) occasionally provides support to patient groups

Source: Agency websites, public documents, limited interviews with select organizations

Contents

Assessment: Initial Mapping of CADTH Relative to Peers

	New and Evolving	*Range of HTA Agency Patient Input Models*	*Mature, Highly Resourced*
Philosophy and Goal			
▪ Importance to decision-making	*Minor consideration*		*Equal weighting with other evidence*
Design			
▪ Stage of incorporation	*One stage*		*Multiple stages*
▪ Mechanisms of solicitation	*Limited, one-dimensional*		*Broad, varied*
Process			
▪ Flexibility of input	*Limiting*		*Adaptable to given context*
▪ Time allotment	*Conflict with review timeline*		*In line with review timeline*
▪ Utility	*Manual, complex*		*Automated, user-friendly*
Inputs			
▪ Breadth of data points	*0*		*> 5*
▪ Type/quality of data gathered	*Generic*		*Relevant*
▪ Quality control	*Minimal*		*Multi-faceted*
Outputs			
▪ Incorporation of evidence	*Anecdotal*		*Systematic, proactive*
▪ Transparency of decision-making	*Transparency of decision-making*		*Timely, detailed, active*

Assessment: CADTH Assessment — Philosophy/Goal and Design

Philosophy and Goal

Importance to decision-making

- CADTH — Patient input is not given the same weighting as clinical and cost-effectiveness data due to perceived lack of objectivity of the data sensed by CEDAC members and CDR reviewers
  - Most CDR reviewers and CEDAC members feel the information collected is relevant for CDR; however, not all are comfortable with using information for decision-making due to perceived conflicts/lack of objectivity/lack of representation
  - Industry respondents do not believe that patient input has enhanced CDR
- At NICE, patient evidence is given equal weighting with clinical and cost-effectiveness evidence

Design

Stage of incorporation

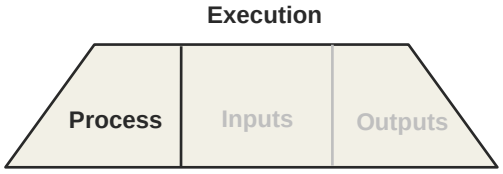
- CADTH — Patient input is collected at the beginning of the process and is designed to be used for protocol development, incorporated in the CDR review reports, and presented by lay representatives
- Patient groups are not consulted at the draft report stage and reconsideration stage (this may be constrained by factors such as confidentiality agreement); comparatively, agencies such as NICE and pCODR allow patients to comment on draft reports, with valid grounds (patient groups can also ask for an appeal at NICE or a procedural review at pCODR)
- A few other agencies incorporate input at select stages only — PBAC and SMC do not consider patient input at the protocol stage, SMC considers patient evidence at the committee discussion
- CDR reviewers and CEDAC members believe that patient information has contributed to various stages of the CDR review

Mechanisms of solicitation

- Patient input currently received via written submissions to CADTH via patient groups; patient input is presented by public members at the CEDAC deliberations
- Most CDR reviewers, CEDAC members, and patient groups agree that other stakeholders such as individual patients/caregivers/professionals could provide input; however, there is no clear inclination toward any particular group(s)
- Comparatively, PBAC and NICE have patient experts present at committee meetings; NICE and pCODR also allow patient groups to make comments on draft reports (with valid grounds, patient groups can also ask for appeal at NICE or a procedural review at pCODR)

Source: SECOR Analysis

Assessment: CADTH Assessment — Patient Submission Process



Flexibility of input

- Most patient groups felt the CADTH submission template allowed patient groups to describe the issues/outcomes that are important to the group, although some expressed that the submission form is not long enough
- Comparatively, NICE, SMC, and BC do not have a limit on the length of the submissions, while organizations such as PBAC, Ontario, and pCODR have more stringent limits

Utility

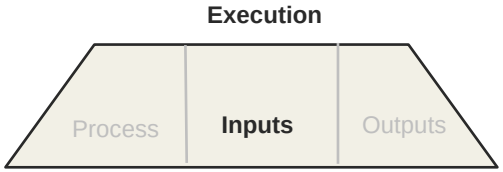
- There is a website page dedicated to patient submission; a guidance document and the CDR process is posted on website; patients can make submissions online or via fax; and contact information is available should patient groups have any questions
- Most groups are “fairly” or “very satisfied” with the accessibility of the website and the submission form
- Some noted opportunities for improvement:
  - Better navigation structure of the website
  - CADTH could send disease-specific e-alerts
- CADTH sends “Calls for Patient Input” emails to notify patient groups; PBAC only pastes the information on its website; NICE actively searches the database for relevant patient groups and invites them to participate in a targeted way (i.e., not all groups in the database are alerted for every review)

Time allotment

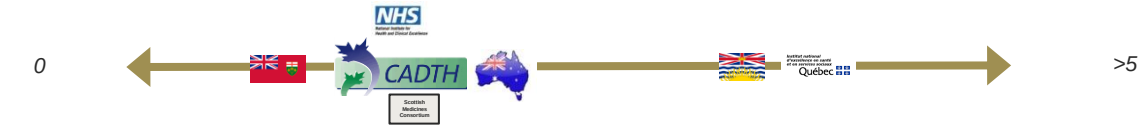
- Current patient input process respects the existing CDR timelines
- However, a large proportion of patient groups indicated that they do not have enough time to complete submission; the CDR reviewers have also commented that perhaps more time is needed for patient groups
- Industry members have suggested that it is possible to provide more advance notice to the patient groups
- While NICE’s time allotment is significantly longer, its appraisal process is approximately 1 year; SMC, while giving patient groups up to 2 months, does not consider the patient information until the decision-making stage

Source: SECOR Analysis

Assessment: CADTH Assessment — Data Inputs



Breadth of data points



- Number of patient submissions range from 0 to 9 per review, with average of 1.8 submissions and median of 1 submission; CADTH does not accept individual patient input
- From the 8 HTA agencies examined, BC, Quebec, and PBAC are the only ones that accept individual patient submissions;
- CADTH's number of data points is in line with most HTA agencies that accept patient group submissions; the number varies depending on the drug under review
- BC and Quebec have higher data points mainly due to the acceptance of individual patient and caregiver input

Type/quality of data gathered

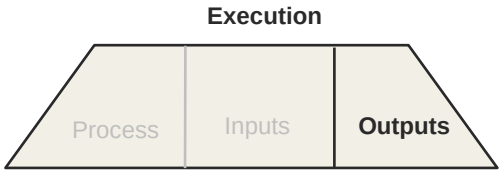
- Most CDR reviewers and CEDAC members feel the information is valuable, not otherwise obtainable. More CDR reviewers and CEDAC members feel the requested information elicits the most relevant patient values
- CDR reviewers commented the quality of submissions is highly variable, which is a comparable situation with a few other agencies such as pCODR and PBAC
- Most CDR reviewers, CEDAC members, and patient groups agree that other stakeholders such as individual patients/ caregivers/professionals could make inputs; however, there is no clear inclination toward any particular group(s) nor type of data (e.g., survey), and 6/7 CEDAC members agreed patient preference ranking would be helpful to gather valuable and objective patient information
- Most HTAs have generic questions (not disease-specific) on submission forms
- Most patient groups agree that they welcome some form of in-person discussion (focus groups, presentation, etc.)

Quality control

- A guidance document is available on CADTH website; CADTH has organized one patient group training session in 2011
- Patients support more training sessions and better support mechanisms, and would like an opportunity to comment on the draft summary before submitting to CEDAC discussion; industry groups has commented that more support from CADTH is needed
- CDR reviewers and CEDAC members have commented that providing training for lay members would be valuable
- SMC has dedicated resources to support patient groups in making submissions; NICE has organized multiple training sessions for lay members and has full-time staff dedicated to all patient initiatives in drug appraisal; SMC has also posted sample submission documents on website

Source: SECOR Analysis

Assessment: CADTH Assessment — Outputs of Submission Process



Incorporation of evidence

Anecdotal ← CADTH | NHS pCODR → *Systematic, proactive*

- Most CDR reviewers and CEDAC members indicated patient-important outcomes/issues have contributed to protocol development; CDR reviewers also noted patient information is useful for contextualizing clinical and economic data
- However, most CEDAC members say patient submissions have little influence on their final voting decisions
- While most agencies do not have a systematic framework to incorporate patient data, comparatively pCODR has published a "deliberative process" framework for patient evidence incorporation

Transparency of decision-making

Unclear, passive, opaque ← CADTH | pCODR | NHS → *Timely, detailed, active*

- Patient groups and industry have a fairly good understanding of the patient input process
- There is a section in the final recommendation that summarizes the patient input information; however, patient groups and industry are unclear about how patient information is used during decision-making
- Most HTA agencies do not have public information about how patient information impacted decision-making; NICE, which is the most transparent agency, sends draft guidance verbatim to patient groups

Source: SECOR Analysis

Contents

Recommendations: Philosophy and Goals

G1 Clearly define the objective of patient input and align internal and external stakeholders, accordingly

G2 Increase transparency by communicating how patient information is used in decision-making process — during and after the review is published

G3 Further increase awareness of program among patient groups, and broader patient community in order to broaden reach of intake

For further consideration

G4 Reduce duplication of patient input process in BC and Ontario and continue to forward the patient input information to jurisdictions

Recommendations: Design

Strategic ⟷ **Tactical**

D1 Establish a framework to more objectively and systematically incorporate patient input into the decision-making process

D2 Establish strategic relationships with research agencies such as CFHI and academia to diversify sources of patient-based evidence

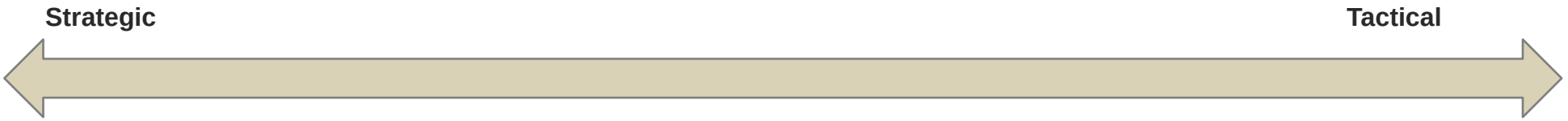
D3 Create opportunities for individual patients/caregivers to be engaged in the process without necessarily accepting individual patient submissions (e.g., have patient experts at decision-making table, provide links to patient groups should an individual patient want to make a submission, post patient preference-ranking of outcomes of importance online)

D4 Continue to share and exchange patient group email lists with Ontario and BC, include patient groups currently not subscribed to the mailing list

For further consideration

D5 Sign a non-disclosure agreement with manufacturers so draft report can be released to public for comments

Recommendations: Submission Process



P1 Encourage industry to give even more advanced notice to CADTH, when possible

P2 Increase flexibility of input by removing or increasing the page limit; ask patient groups to submit a summary if the information is > 10 pages

P3 Send disease-specific alerts

P4 Send patient group an email receipt when submission has been received

Recommendations: Data Inputs

Strategic → **Tactical**

- **I1** Schedule periodic formal communication opportunities with patient groups to understand needs and incorporate feedback into improvements for the overall process
- **I2** Devote a half-time/full-time employee to patient engagement initiatives to support patient groups on making submissions (e.g., provide advice and feedback to patient groups)
- **I3** Demonstrate what a “good” submission is by posting examples online
- **I4** Allow patient groups to review draft summary of patient input before CDEC discussion
- **I5** Organize and deliver quarterly training sessions to public members on CDEC committee
- **I5** Organize and deliver quarterly training sessions to patient groups

Recommendations: Outputs

Strategic ⟷ **Tactical**

O1 Establish a framework to systematically and objectively incorporate patient input as an evidence for decision-making (same as D1 in slide #35)

O2 Increase transparency by communicating how patient information is used in decision-making process; e.g., distribute verbatim comments, explicitly summarize how data contributed to decision-making (same as G3 in slide #34)

Recommendations: Vision for Future Patient Input Initiative

Overall Patient Input Initiative

Stakeholder alignment and engagement

- Internal and external stakeholders aligned on the objectives of patient input process (G1, slide #34)
- Increased awareness of patient input initiative among patient groups and broader patient community (G3, slide #34)
- Strategic relationship established with research agencies such as CFHI and academia, broader source of patient information is considered (D2, slide #35)
- Periodic formal communication opportunities exist for patient groups to give feedback (I1, slide #37)

Incorporation of evidence

- A framework established to incorporate patient input objectively and systematically (D1, slide #35; O1, slide #38)
- Opportunities available for individual patients engaged in the process (D3, slide #35)

Education

- A full-time/half-time employee available to support groups on making submissions (I2, slide #37)
- Quarterly training sessions for patient groups and public members on CEDAC (I5, I6, slides #37)

Written Submission Process