CADTH Drug Portfolio Information Session

Monday, November 25, 2019

1:30 p.m. to 4:00 p.m.

CADTH

Overview of the Agenda

TOPIC	LEAD
Welcome and Strategic Overview	Brian O'Rourke
Pharmaceutical Reviews Portfolio Update a) Alignment b) Program Updates c) Updates on Consultations	Brent Fraser Trevor Richter
New Initiatives a) Plasma-Related Reviews b) Cell and Gene Therapy Reviews c) Biosimilar Consultation d) Deliberative Frameworks e) Real-World Evidence	Trevor Richter Heather Logan Nicole Mittmann
CADTH Scientific Advice	Nicole Mittmann
Patient Engagement at CADTH	Nicole Mittmann
Open Forum	CADTH Team
Wrap Up	Brian O'Rourke

Presenters

- **Brian O’Rourke,** President and CEO
- **Brent Fraser,** VP, Pharmaceutical Reviews
- **Trevor Richter,** Director, Pharmaceutical Reviews
- **Heather Logan,** Senior Advisor, Pharmaceutical Reviews
- **Nicole Mittmann,** Chief Scientist & VP, Evidence Standards

Questions

- Questions of clarification after each agenda item
- Open Forum at the end of the session
- In-person:
  - Please use a microphone for the benefit of on-line participants
- On-line:
  - Use the question feature on the livestream toolbar

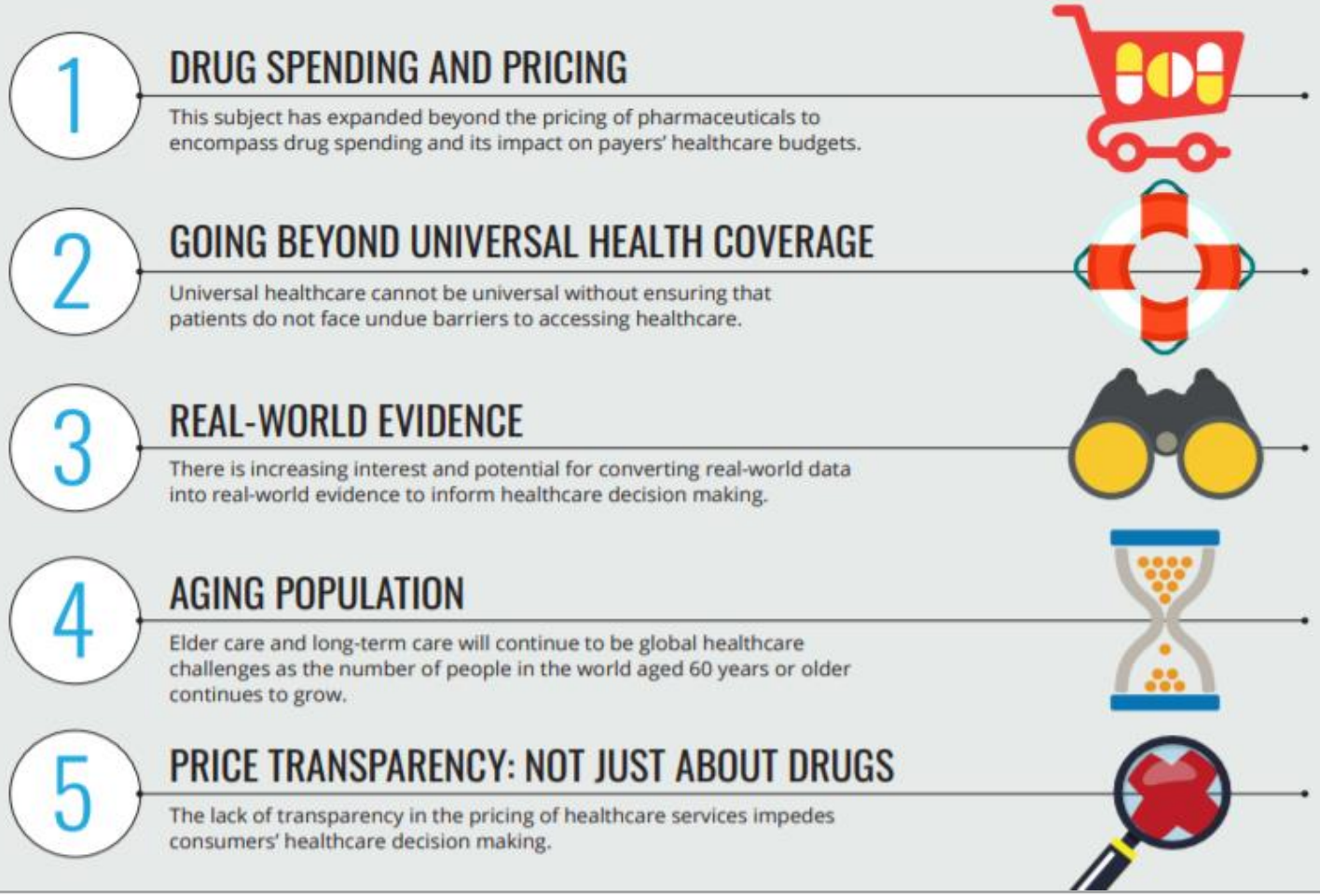
CADTH Succession Planning

- The CADTH Board of Directors is conducting a search for a new President and CEO.
- Contracted Boyden to support the search
- New President and CEO to be in place prior to March 31, 2020

Federal Government Priorities

- Cabinet announced on November 20, 2019
  - **Patty Hajdu**, Member of Parliament for Thunder Bay-Superior North, is the new Minister of Health
- Updated Health Minister mandate letter expected within two weeks.

ISPOR 2019 Top 10 HEOR



CADTH

ISPOR 2019 Top 10 HEOR

6

"BIG DATA" CONTINUE TO MAKE NOISE

The use of "big data" can assist clinicians in making better healthcare decisions for their patients.

7

VALUE ASSESSMENT FRAMEWORKS

Value assessment frameworks can be an important element in moving towards a more value-based care model.

8

HEALTHCARE DECISION MAKING IN LOW-INCOME COUNTRIES

The difference between health technology assessment use by high-income and low-income countries is notable.

9

PERSONALIZED/PRECISION MEDICINE

As researchers continue to determine the roles that genes play in diseases, HEOR will be needed to evaluate the diagnostics and drugs derived from their discoveries.

10

UNHEALTHY BEHAVIORS

The root causes of many chronic diseases include a host of unhealthy behaviors that lead to a variety of diseases responsible for the majority of all deaths worldwide.

International HTA Activities

- **INAHTA**
  - New definition for HTA
  - Global collaborative on RWE
  - Harmonization of HTA and guidelines (G-I-N)
- **HTAi**
  - Annual conference in Beijing
  - Global Policy Forum – deliberative Frameworks
- **CIRS**
  - Managing uncertainty
- **ISPOR**
  - HTA roundtables
  - Cumulative budget impact
  - Value assessment of medical devices
- **Miscellaneous**
  - Paying for combination regimens in oncology
  - ICER-NICE-CADTH collaboration

Pharmaceutical Reviews Portfolio Update

CADTH

Alignment of CADTH Single Drug Review Processes

CADTH

CADTH Single Drug Reviews

- **Objective:** Establish aligned single drug review process for all eligible products
- **Approach:** Leverage best practices from existing processes based on the following considerations:
  - Transparency, efficiency, timeliness, sustainability
- **What's been the hold-up?**
  - Merger of two review processes is complex
  - Need for major migration of infrastructure
  - Challenging to change processes with high level of ongoing work (number one priority for CADTH is to ensure continuity of existing services)

Alignment: Patient Engagement

- Opportunities for **patient groups to comment** on draft recommendations for all reviews
- Patient engagement window to be **extended to 35 business days** for all single drug reviews
- Patient Engagement Team to provide **single co-ordinated contact point** for patient groups
- Opportunity for patient groups to liaise directly with CADTH staff (*Patient Engagement Team*)
- Harmonized process for transparent reporting and disclosure of COI information for patient groups

Alignment: Sponsor Engagement

- Sponsors will have an opportunity to review and comment on CADTH's draft review reports prior to the committee meeting
- Checkpoint meetings are likely to be replaced with more rapid communications between CADTH and the sponsor (e.g., immediate correspondence to address issues)
- Reconsideration meetings will be lengthened and offered for all review streams
  - Also considering opportunities to potentially include committee members in reconsideration engagement
- Potential opportunities to improve pre-submission meetings:
  - More structure (e.g., standardized agenda)
  - Participation from additional stakeholders

Alignment: Recommendations

- Aligned recommendation framework for CADTH drug review programs introduced in April 2016
- Historical differences in the general format and content of the recommendation documents
- CADTH is seeking to begin **posting draft recommendations** for all single drug reviews
- Considering allowing files to be discussed by the committees prior to NOC issuance
  - Potentially for those who elect to participate in HC/CADTH aligned review process

Alignment: Reconsideration Process

- Large disparity in number of reconsiderations:
  - Approximately 20% of files in CDR
  - Approximately 70-80% of files in pCODR
- **Allow second feedback period** in the event a reconsideration has led to substantial revisions to the recommendation
- Allow sponsor to engage with CADTH to discuss their request for reconsideration for all files
- Reconsideration format and template will be increased in pCODR from 3 pages to 10 pages
- *Should sponsors be permitted to request extensions to the feedback period for preparing a request for reconsideration?*

Alignment: Next Steps

- Alignment will proceed with CADTH's standard process for major procedural changes:
  - Internal consolidation of processes (being finalized)
  - Discussion with Pharmaceutical Advisory Committee
  - Consultation with external stakeholders (industry, patient groups, clinician groups)
  - Refinement and finalization of procedures
  - Launch of aligned single drug processes
- Timelines for the above will be communicated in a future update

Clinician Engagement

- Single drug review and OU programs currently have extensive engagement with Canadian clinical experts
- All CADTH review teams include ≥1 clinical specialist with expertise regarding the diagnosis and management of the condition for which the drug is indicated.
- Clinical experts are involved in all phases of the review process (e.g., protocol development; critical appraisal; interpreting outcomes; guidance on place in therapy during review and at expert committee meetings).
- Selecting experts: expertise, COI declaration, availability to commit to timelines, regional representation

Clinician Engagement

- CADTH will continue to seek opportunities to collaborate with INESSS for joint-engagement with clinical experts
- CADTH is **considering a new clinician engagement strategy** that would focus on input from groups and associations of health care professionals (e.g., tumour groups, guideline groups, professional associations)
- Similar to patient engagement: encourage individuals to work together on submissions to improve quality and reduce administrative burden on all participants
- Build on the lessons learned from the pCODR experience with open input from health care professionals

CADTH Drug Program Updates

CADTH

Expert Committee Updates

- Call for nominations to CADTH's expert review committees was issued in 2019
- Committee members are appointed for set terms and new members are rotated in as terms expire
- CADTH would like to thank outgoing committee members for their excellent work:
  - **CDEC:** Peter Jamieson
  - **pERC:** Matthew Cheung and Henry Conter
- CADTH welcomes new committee members:
  - **CDEC:** Danyaal Raza
  - **pERC:** Michael Crump

Expert Committee Updates

CDEC members

- James Silvius (Chair)
- Allen Lefebvre
- Ahmed Bayoumi
- Heather Neville
- Bruce Carleton
- Rakesh Patel
- Alun Edwards
- Danyaal Raza (New)
- Bob Gagne
- Emily Reynen
- Ran Goldman
- Yvonne Shevchuck
- Allan Grill
- Adil Virani

pERC members

- Maureen Trudeau (Chair)
- Catherine Moltzan (Vice-chair)
- Daryl Bell
- Kelvin Chan
- Flay Charbonneau
- Winson Cheung
- Michael Crump (New)
- Avram Denburg
- Leela John
- Anil Joy
- Christine Kennedy
- Christian Kollmannsberger
- Cameron Lane
- Christopher Longo
- Valerie McDonald
- Marianne Taylor
- Dominika Wranik

Advisory Committee Updates

New Advisory Committee:

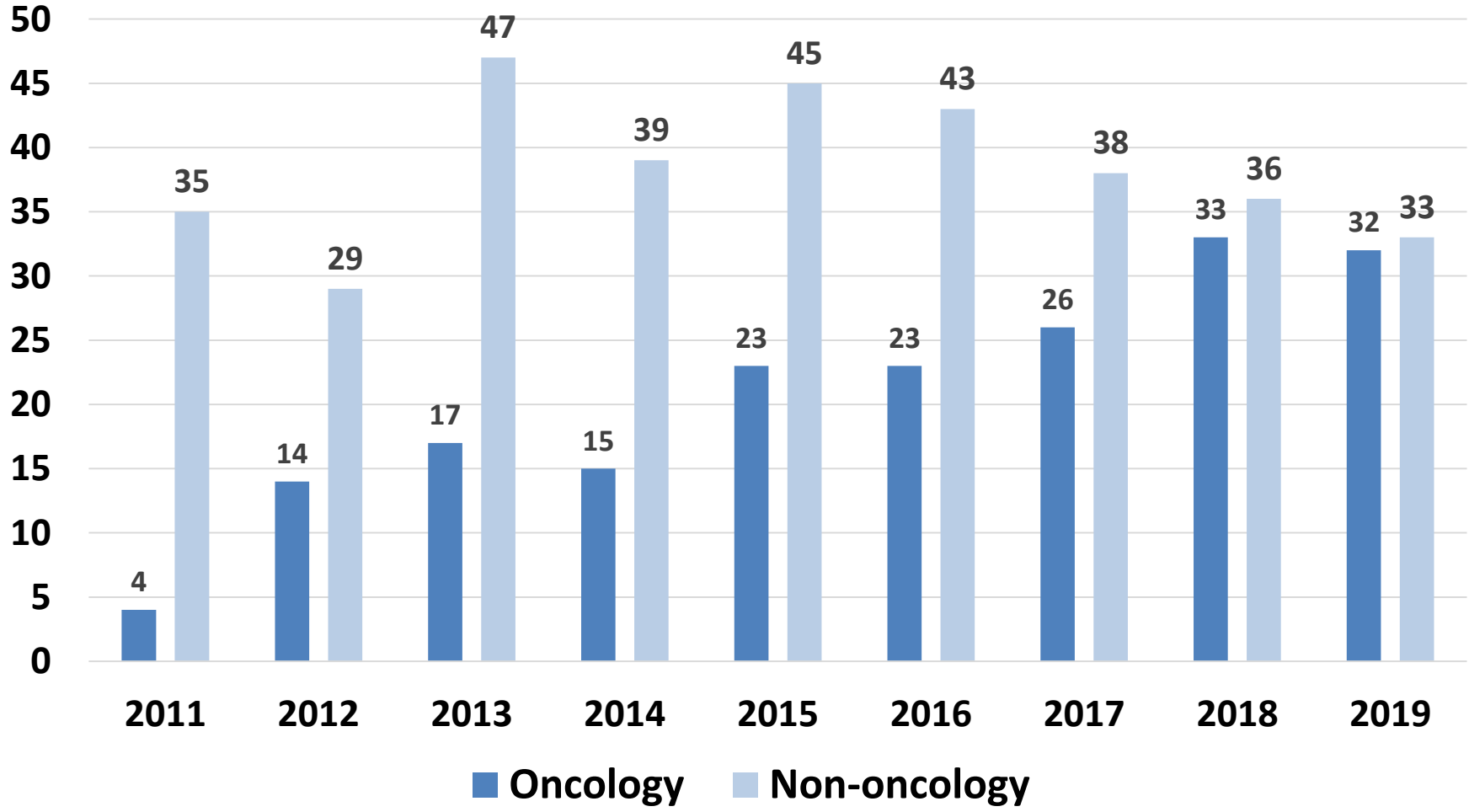
- CADTH Pharmaceutical Advisory Committee has been created to provide strategic advise on drug related issues and topics to CADTH
- Merger of two separate advisory committees for the oncology and non-oncology portfolios:
  - Drug Program Advisory Committee (DPAC)
  - pCODR Advisory Committee (PAC)
- Participation from drug programs, cancer agencies, federal departments, pCPA, and other stakeholders

Advisory Committee Updates

New Working Group:

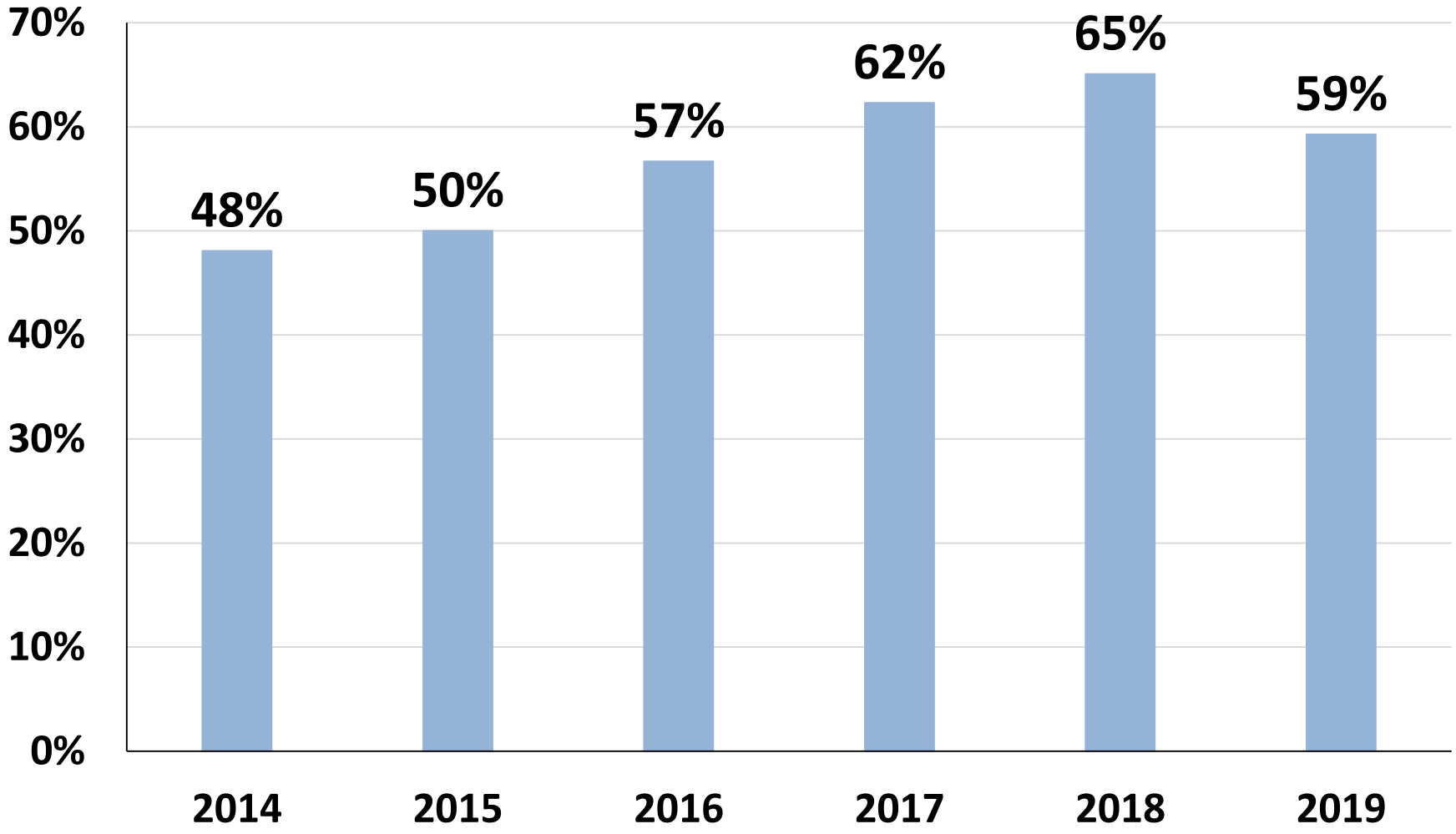
- CADTH conducted an internal review of OU program for drugs to identify efficiencies and opportunities to increase timeliness, relevance, and impact of OU projects.
- Included examining the role and mandate of CADTH's advisory committees in the OU process.
- CADTH established the DPAC Formulary Working Group for Health Technology Assessments (FWG-HTA)
- New advisory committee composed of jurisdictional representatives with a greater role in drug policy decision-making.

CADTH Drug Submissions



CADTH

CADTH Pre-NOC Submissions



Health Canada Aligned Reviews

Background:

- Process launched in late June 2018
- Sponsors have the option to consent to information sharing with Health Canada (signed consent form) and notify CADTH in their advance notification form
- Allows Health Canada to upload key documents directly to HTA agencies (e.g., Clarifaxes, reviewers reports)

Participation:

- A total of 47% (26/55) of all eligible submissions have opted into the information sharing process to date.
- Health Canada, CADTH, and INESSS would like to see these number increase in the future.

Health Canada Aligned Reviews

New temporary suspension process

- Sponsors that receive an NOD or NON may be permitted to remain in CADTH's process (rather than mandatory withdrawal).

Complete reports for sponsor comments

- CADTH receives HCRR directly from Health Canada and is able to incorporate details into the reports prior to manufacturer comments.
- Allows manufacturer to review and provide clarification if required during comment period.

Avoid potential delays

- Dialogue with Health Canada can help ensure that CADTH is fully aware of changes to the indications for drugs under review.
- Can help avoid delays by allowing CADTH to rapidly assess the impact of any revisions to the indication.

Program Updates (At a Glance)

• Call for clinical experts (CDR)	January
• Review phase clinical expert panels (CDR)	February
• Implementation phase panels	
• Updated application fee guidelines	March
• Additional embargo for revised recs (CDR)	April
• Discontinuation of biosimilar submissions	May
• Revised redaction process (CDR)	
• Updated clinical review templates (CDR)	
• Transfer of CDIAC functions (pCODR)	July
• Revised withdrawal process for NOD/NOC	
• Program consultations	August

Conflict of Interest

- CADTH will **cease redacting** any conflict of interest information from patient group input or registered clinician input submissions that are filed for drugs being reviewed through the pCODR process.
- Change will be effective for calls for patient input and registered clinical input issued **on or after January 2, 2020**.
- This change will increase transparency, promote alignment across our drug review processes, and offer efficiencies for CADTH by eliminating the need for redaction.

Update on Consultations

CADTH

CADTH Consultations

- Stakeholder consultations undertaken in Aug/Sept 2019
- Proposal to revise category 1 requirements (SLR, BIAs, CSRs, reimbursement status of comparators)
- Proposed reassessment framework (consolidation and streamlining of existing pathways)
- Enhance transparency of CADTH's reports (eliminating the need for redactions)
- 28 responses received from industry and government
- A detailed summary and proposed next steps will be communicated at a later date

CADTH Consultations

New Category 1 Requirements

- No major objections from industry
- Concerns regarding added expense and burden

Reassessment Framework

- No major objections from industry
- Concerns about increasing uncertainty for industry (e.g., having to renegotiate for products multiple times)
- Industry interested in potential for conditional listing recommendations based on RWE development

Enhancing Transparency

- Support from jurisdictions, but considerable opposition from industry respondents

Pending Revisions to Economics

- Revisions to economic requirements for all standard review submissions **filed on or after February 3, 2020 (TBC)**
- Base case must reflect the HC approved indication for all submissions (including those for oncology drugs).
- A pan-Canadian BIA will be required for all submissions.
- Economic models must be programmed in Microsoft Excel
- Economic models must have a run time of ≤ 8 hours
- Probabilistic analysis must be stable over multiple runs.
- CADTH is providing advance notification of these pending revisions to allow time for sponsors to plan accordingly.

Expansion of Tailored Reviews

- CADTH is expanding the tailored review process to include selected additional drug products, such as new formulations of existing drugs that are eligible for review by CADTH (e.g., those that are associated with a new route of administration).
- Revised tailored review application form
- New simplified submission template with improved instructions for sponsors
- The decision to conduct a tailored review will be made by CADTH on a case-by-case basis after reviewing the applicable considerations form filed by the sponsor.

Expansion of Therapeutic Reviews

- A therapeutic review is an evidence-based review of publicly available sources regarding a therapeutic category of drugs or a class of drugs in order to support drug reimbursement decisions and drug policy decisions, and to encourage the optimization of drug therapy.
- CADTH therapeutic review framework and process has been updated to include oncology products.
- First oncology therapeutic review announced last week:
  - *Optimal pharmacotherapy for transplant-ineligible multiple myeloma*

Reminders: Economics

- CADTH has noted an increase in issues with economic submissions in 2019
- Please ensure that the models are free of errors before they are filed with CADTH (administrative burden and can result in delays while they are fixed)
- Please ensure the technical economic report reflects the submitted model (inputs, methods, results)
- Please ensure that you are familiar with CADTH's economic requirements before filing the submission

Reminders

- Do not send samples of your product to the CADTH office
- Consultants must copy an official contact for the manufacturer on all email correspondence with CADTH.
- Please consider opting in to info-sharing initiative with Health Canada.
- Always use requests@cadth.ca for inquiries to ensure tracking, triage, and record keeping.
- Ensure templates are obtained exclusively from the CADTH website and are the latest versions posted.
- CADTH does not provide guidance on hypothetical scenarios (e.g., “What if we had a product that was…”)

New Initiatives

CADTH

Plasma-Related Reviews

CADTH

Plasma Protein Products

- New interim process for the review of plasma protein products while PTs complete a review of drug formulary processes in collaboration with CBS, CADTH, and other key stakeholders.

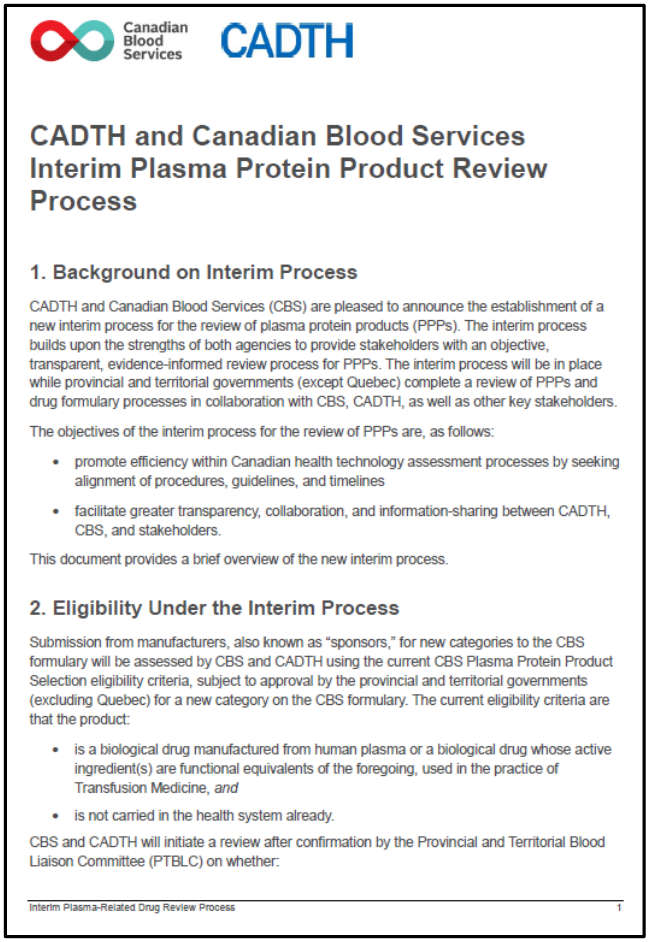
- **Objectives:**
  1. Promote efficiency by seeking alignment of procedures, guidelines, and timelines
  2. Facilitate greater transparency, collaboration, and information-sharing between CADTH, CBS, and stakeholders.

Plasma Protein Products

- Product eligibility will be determined by PTs:
  - **In scope:** A new category is unlike the products currently distributed by CBS, is a new therapeutic product type which may or may not replace demand for other products distributed by CBS
  - **Out of scope:** A new brand is a product similar to an existing product already distributed by CBS, which would replace demand for another brand, and which would not add to the overall portfolio budget.
- Sponsors should contact CADTH for information regarding product eligibility (CADTH will work with CBS and PTs to confirm the review pathway in a timely manner)

Plasma Protein Products

- Initial guidance documentation posted (process in brief)
- Interim process will be similar to existing CDR process with the additional inclusion of BIA submission and review
- New subcommittee (CPEC) will issue recommendations for plasma protein products
  - Inclusion of experts in blood disorders



Canadian Blood Services CADTH

CADTH and Canadian Blood Services Interim Plasma Protein Product Review Process

1. Background on Interim Process

CADTH and Canadian Blood Services (CBS) are pleased to announce the establishment of a new interim process for the review of plasma protein products (PPPs). The interim process builds upon the strengths of both agencies to provide stakeholders with an objective, transparent, evidence-informed review process for PPPs. The interim process will be in place while provincial and territorial governments (except Quebec) complete a review of PPPs and drug formulary processes in collaboration with CBS, CADTH, as well as other key stakeholders.

The objectives of the interim process for the review of PPPs are, as follows:

- promote efficiency within Canadian health technology assessment processes by seeking alignment of procedures, guidelines, and timelines
- facilitate greater transparency, collaboration, and information-sharing between CADTH, CBS, and stakeholders.

This document provides a brief overview of the new interim process.

2. Eligibility Under the Interim Process

Submission from manufacturers, also known as "sponsors," for new categories to the CBS formulary will be assessed by CBS and CADTH using the current CBS Plasma Protein Product Selection eligibility criteria, subject to approval by the provincial and territorial governments (excluding Quebec) for a new category on the CBS formulary. The current eligibility criteria are that the product:

- is a biological drug manufactured from human plasma or a biological drug whose active ingredient(s) are functional equivalents of the foregoing, used in the practice of Transfusion Medicine, *and*
- is not carried in the health system already.

CBS and CADTH will initiate a review after confirmation by the Provincial and Territorial Blood Liaison Committee (PTBLC) on whether:

Interim Plasma-Related Drug Review Process 1

Cell and Gene Therapy Reviews

CADTH

Experience to date

Two chimeric Antigen Receptor T-cell Therapy submissions:

- tisagenlecleucel (Kymriah™)
- axicabtagene ciloleucel (axi-cel; Yescarta ™)

Reviewed through medical devices and clinical interventions with recommendations from HTERP

Recommendations released simultaneously with INESSS

Large volume of cell and gene therapies – dedicated interim process being established

Determining Eligibility

- Anticipated that the majority of cell and gene therapies will be reviewed through the single drug review processes
- In some selected instances, a product may be reviewed through the HTA process if it has characteristics that are not necessarily well-suited to the existing single drug processes
  - *e.g., novel advanced therapeutics with the potential to require broad system changes for adoption*
- CADTH will make the determination based on an improved eligibility request form that will offer better insight into the complexity of a product prior to the submission being filed

Optimizing the Process

- CADTH has undertaken an internal review of our processes for drugs and devices and established a novel process for the review of cell and gene therapies.
- New process leverages strengths of both programs to ensure that cell and gene therapies are reviewed in the optimal manner for all stakeholders:
  - **Drugs:** Process has firm performance targets and well-established methods for conducting reviews and issuing recommendations
  - **Devices:** Process includes additional ethical and implementation considerations

What's new: Implementation Plan

- New addition to the submission requirements and review process could help facilitate faster access for patients following the completion of CADTH's review.
- Products can be associated with implementation challenges for the public health system, **sponsors will be required to complete a template** that describes key aspects of their plans for implementing the product in Canada.
- Appraisal and discussion by CADTH, drug plans, and pCPA to ensure recommendations can be readily implemented
- Information would be **kept confidential** by CADTH

What's new: Fee Guidelines

- Due to the added complexity of reviews for cell and gene therapies, CADTH will be implementing a new fee schedule for the these drugs
- **Schedule E:** $108,000

A	Standard review	$72,920
B	Standard review (subsequent indication)	$58,340
	Resubmission	
C	Tailored review	$36,460
D	Request for reconsideration	$7,090
E	Cell or gene therapy	$108,000

CADTH

Next Steps

- The following documentation for the new cell and gene therapy review processes will be posted in the coming weeks:
  - Process documentation
  - Revised fee schedule for CADTH
  - New eligibility inquiry form
- Any sponsors with cell or gene therapies that could be filed in the near future can contact CADTH for guidance on next steps

Biosimilar Consultation

CADTH

Background

Concept of a national consultation initiated by pCPA summer 2018

Agreement to proceed provided to CADTH July 2019

Objective is to provide a mechanism for stakeholders to provide input to jurisdictions that may be exploring policy options to:

- ensure a competitive and sustainable market for both biosimilar and innovator drugs
- increase appropriate use of biosimilar treatments, and
- to reduce the overall cost burden to enable savings to be redirected into the healthcare system.

Advisory Committee

- Gastrointestinal Society
- Crohn's and Colitis Canada
- Arthritis Society
- Arthritis Consumer Experts
- Canadian Arthritis Consumer Association
- Canadian Council of the Blind
- Psoriasis Network
- Canadian Diabetes Association
- Canadian Digestive Health Forum
- Canadian Association of Gastroenterology
- Canadian Rheumatology Association
- Canadian Ophthalmological Society
- Canadian Dermatology Association
- Canadian Association of Endocrinology and Metabolism
- Pan-Canadian Oncology Biosimilar Initiative

Consultation Components

CADTH

Next Steps

- In-Person Consultation Report
- Online consultation: December 9 – January 8
- Consolidation of stakeholder input
- Submission to pCPA and to the jurisdictions by January 31, 2020

Review of Deliberative Frameworks

CADTH

What is a deliberative process?

- “the critical examination of an issue involving the weighing of reasons for and against a course of action.”

- This definition implies a series of coordinated activities allowing a group of people to receive and exchange information, to critically examine an issue, and to come to an overall group judgement that will inform decision making.

Gauvin F-P (2009) *Factsheet. What is a deliberative process? Publication no. 1193*. 838 Ottawa, ON: The National Collaborating Centre for Healthy Public Policy. Available 839 at: http://www.ncchpp.ca/docs/DeliberativeDoc1_EN_pdf.pdf.

Why examine Deliberative Frameworks?

- Improved consistency across decisions
- Transparency to stakeholders and patients
- Decision accountability
- Public trust

Cole, A., Marsden, G., Devlin, N., Grainger, D., Lee, E. K., and Oortwijn, W. (2016). *New Age Decision Making in HTA: Is It Applicable in Asia?* Report of the HTAi 2016 Panel Session, Tokyo, 10–14 May. [online] Available at: https://www.ohe.org/publications/%E2%80%9Cnew-age%E2%80%9D-decision-making-hta-it-applicable-asia [Accessed 7 May 2018].

CADTH Expert Review Committees

- CADTH has three expert review committees.
- Committees develop reimbursement recommendations for the optimal use of drug and non-drug interventions in Canada.
- Deliberations that occur during these meetings are a key step in the development of recommendations for the optimal use of drug and non-drug interventions in Canada.
- Deliberations are guided by deliberative processes and frameworks.

CADTH Expert Review Committees

- The three CADTH committees share many similarities in their deliberative processes and frameworks with some differences.
- Objective
  - Assess the deliberative processes and frameworks that are in place for the three CADTH committees to identify opportunities for improvement or alignment.

CADTH Expert Review Committees

To inform this work, CADTH is:

- Undertaking an internal review and assessment of our advisory committees' deliberative processes and frameworks;
- Reviewing the literature and liaising with external organizations and experts to discuss best practices and practices globally;
- Participating in the Center for Innovation in Regulatory Science (CIRS) – Quality of Decision Making study;
- Participating in the HTAi 2020 Global Policy Forum Meeting which will discuss principles for deliberative processes in HTA.

Assessing the Quality of the Deliberation

Centre for Innovation in Regulatory Sciences (CIRS)

Mission
To maintain a thought leadership role in identifying and applying scientific principles for the purpose of advancing regulatory and HTA policies and processes

CIRS provides a **neutral, independent, international** forum for industry, regulators, HTA and other healthcare stakeholders to meet, debate and develop regulatory and reimbursement policy through the innovative application of regulatory science

Key International Stakeholders
23 member companies (Top-20 international, research-based):
9 (USA), 11 (EU), 3 (Japan)
47 Medicine Regulatory Agencies
22 HTA/Payer Agencies

Self-supporting operated as a nonprofit. Financed by:
Member Company annual membership fees
In-kind support by Agencies
Special projects
Grants (e.g., from HTA and regulatory agencies, BMGF, APEC etc)

HTA and decision making – gap analysis

Key results from a questionnaire with 11 HTA agencies undertaken in 2017: (Australia, Belgium, Brazil, Canada (national and Quebec), England, Netherlands, Poland, Scotland, Spain (Basque region) and Sweden)

Decision Making Frameworks	Measuring quality of decision making		Improving quality of decision making
64% agencies had a formally defined and codified framework	55% agencies undertake formal assessments of decision making quality	64% agencies believe that there are ways of doing this	82% of agencies believe their decision making could be improved

Definition: A decision framework is a structured, flexible systematic and scientific approach to organizing, evaluating, quality assuring and summarizing information and re-assessing over time. It will include various aspects of the quality decision-making practices such as the subjective values and judgments that formed the basis of the decision.

Bujar M, McAuslane N, Walker SR and Salek S. Quality Decision Making in Health Technology Assessment: Issues Facing Companies and Agencies. Ther Inn Reg Sci. 2019a; DOI: 10.1177/2168479019833660.

CADTH

HTAi

HTAi Global Policy Forum Meeting January 2020

- Meeting scheduled for January 2020 in New Orleans.
- Objective of the meeting is to discuss principles for deliberative processes in Health Technology Assessment.
- Backgrounder was open for consultation until November 18th 2019 (https://htai.org/blog/2019/11/05/2020-htai-global-policy-forum-background-paper-consultation/)

Anticipated Timelines

- Current: Review of frameworks at CADTH, other HTAs and Quality work
- January 2020: HTAi Global Policy Forum Meeting
- February/March 2020: CIRS Quality of Decision Making Study Results
- February/March 2020: Completion of literature review and discussions with external organizations and experts
- April 2020: Recommendations

Real-World Evidence

CADTH

What is RWD and RWE?

Real World Data (**RWD**) are **the data relating to patient health status** and/or the delivery of health care routinely collected from a variety of sources. RWD can come from a number of sources, for example: Electronic health records (EHRs) Claims and billing activities

Real World Evidence (**RWE**) is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD. RWE can be generated by different study designs or analyses, including but not limited to, randomized trials, including large simple trials, pragmatic trials, and observational studies (prospective and/or retrospective).

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

Factors to consider

Barriers to use of RWD/E by HTA and Payers:

- managing uncertainty
  - RCT vs. RWE
  - regulatory/decision grade
  - acceptable levels of uncertainty
- illustrating unmet need
- lack of consensus on guidelines or principles
- trustworthiness / transparency
- lack of knowledge and skill

Ongoing activities

Partnership between HTA and Regulator

- collaboration between CADTH, HC and INESSS
- produce strategy for use of RWE across the product lifecycle *(fall 2019)*
- guidance documents for use of RWE for drugs:
  - *principles and expectations of RWD*
  - *appropriate approaches for RWE*

Ongoing activities

Formation of RWE Drug Core Action Team (CAT)

- collaboration between HTA and regulator
- contribution from payers, data holders/producers, academics, and industry
- strategic-thinking and address common barriers
- improved transparency and awareness

Ongoing activities

Involvement in CanREValue Collaboration:

- multi-year grant led by Dr. Kelvin Chan

Goal:

  - develop a framework for Canadian provinces to generate and use RWE for cancer drug funding decisions

Ongoing activities

Potential Impact:

- **reassessment** of cancer drugs by recommendation-makers
- **refinement** of funding decisions or
- re-negotiations/re-investment

Ongoing activities

ISPOR RWE Transparency Initiative

- multi-stakeholder participation including HTA
- identify practical implementation steps to facilitate routine registration of RWE studies
- includes posting of protocol, with date-stamp
- white paper currently available

Next steps

- continued collaboration between stakeholders to provide guidance and framework
- continued collaboration with industry and data stewards to produce “appropriate” RWD
- improve capacity and skill across HTA and payers
- “dive-in” with pilots

CADTH Scientific Advice

CADTH

CADTH Scientific Advice Program

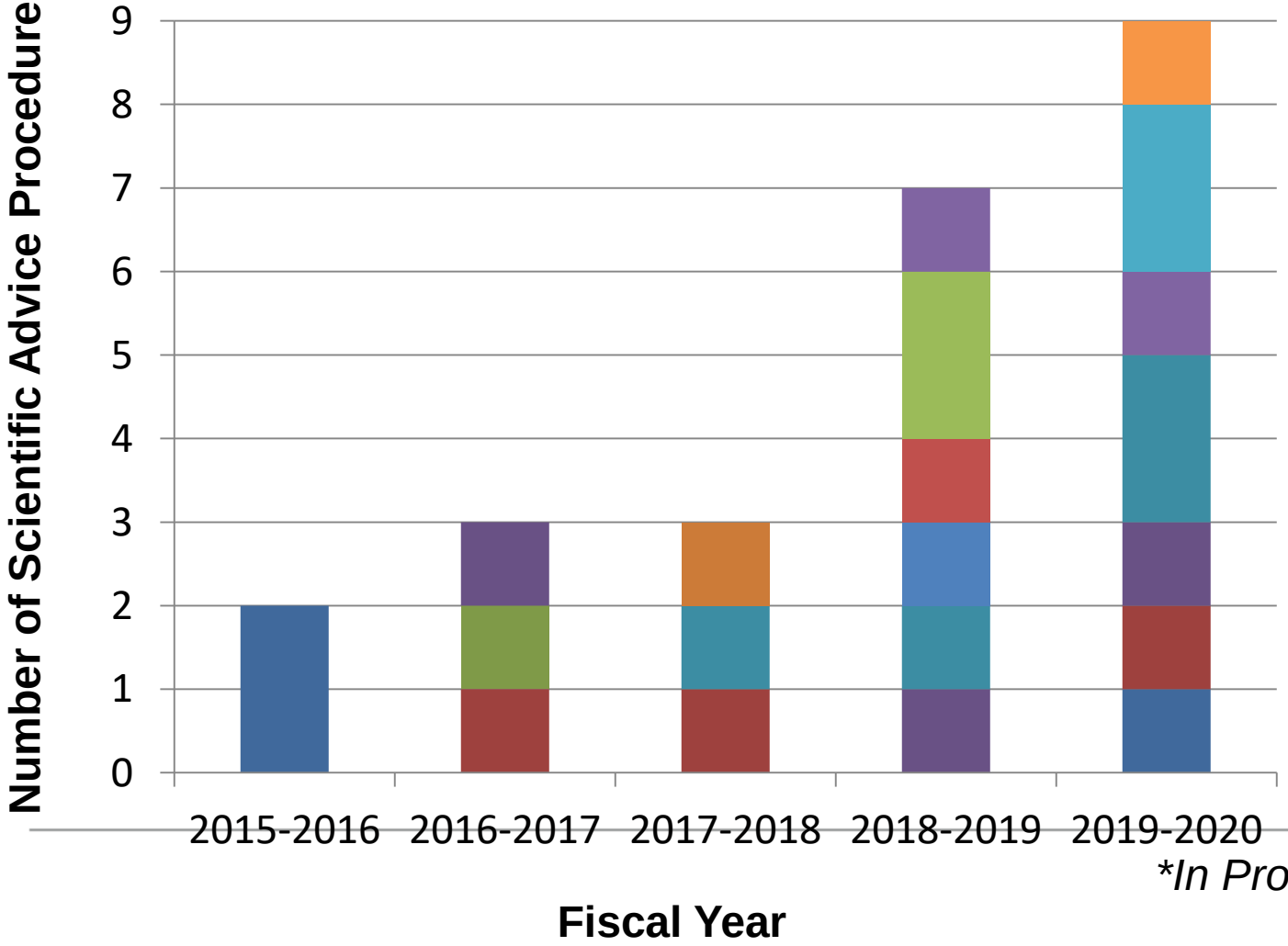
- CADTH provides advice to pharmaceutical companies on clinical trial design and economic evaluation plans a number of years (typically 3 to 7 years) before market authorization
- Eligibility:
  - Prior to initiation of pivotal trials (Phase II or Phase III)
  - Oncology, non-oncology, rare disease
- Voluntary, non-binding, confidential, fee for service, cost-recovery

CADTH Scientific Advice Program

- Standard Offerings:
  - Parallel Scientific Advice with CADTH and Health Canada (including INESSS initially in an observer role)
  - Parallel Scientific Advice with CADTH and NICE (U.K.)
  - Standard Scientific Advice from CADTH only

- **Applications being accepted for 2020 meetings**

CADTH Scientific Advice: Experience To Date



24 SA Procedures

7 companies each returned between 2 to 4 times

**In Progress*

CADTH

Parallel Scientific Advice

- CADTH / NICE Parallel Scientific Advice
  - 3 procedures completed or in progress
  - F2F Meetings: Sept 2018; Oct 2019; Dec 2019

- CADTH / Health Canada Parallel Scientific Advice
  - 2 procedures in progress
  - F2F Meetings: Nov 2019; Jan 2020

CADTH Scientific Advice

Of 24 Procedures:

- 14 non-oncology
- 10 oncology

Of note:

- 2 CAR T-cell products
- 1 Patient Preference Study

Categories (n):

Neurologic (4)

Hematologic cancer (3)

Genitourinary cancer (3)

Lung cancer (3)

Cardiovascular (2)

Dermatologic (2)

Others (7)

Learn About CADTH's Scientific Advice Program

- www.cadth.ca/scientificadvice
- scientificadvice@cadth.ca

Patient Engagement at CADTH

CADTH

2018 Listening Exercise for Future Direction

Patient Community Liaison Forum

How can we greater involve patients, patient groups and communities in our work?

9 CADTH directors

4 patient and public committee members from pERC, CDEC and HTERP

28 patient groups involved with CADTH

We Heard

Greater Engagement:

- Meaningful, respectful engagement
- Need for greater diversity of voices
- Greater interaction with expert committees and CADTH researchers
- Involvement in CADTH governance
- Input and engagement measured to demonstrate impact

To Be Supported:

- Travel awards to CADTH symposium much appreciated
- Clear guidance on what is helpful or seen as biased
- Awareness raising of CADTH and role for patient perspectives in assessments
- Help preparing / refining patient input

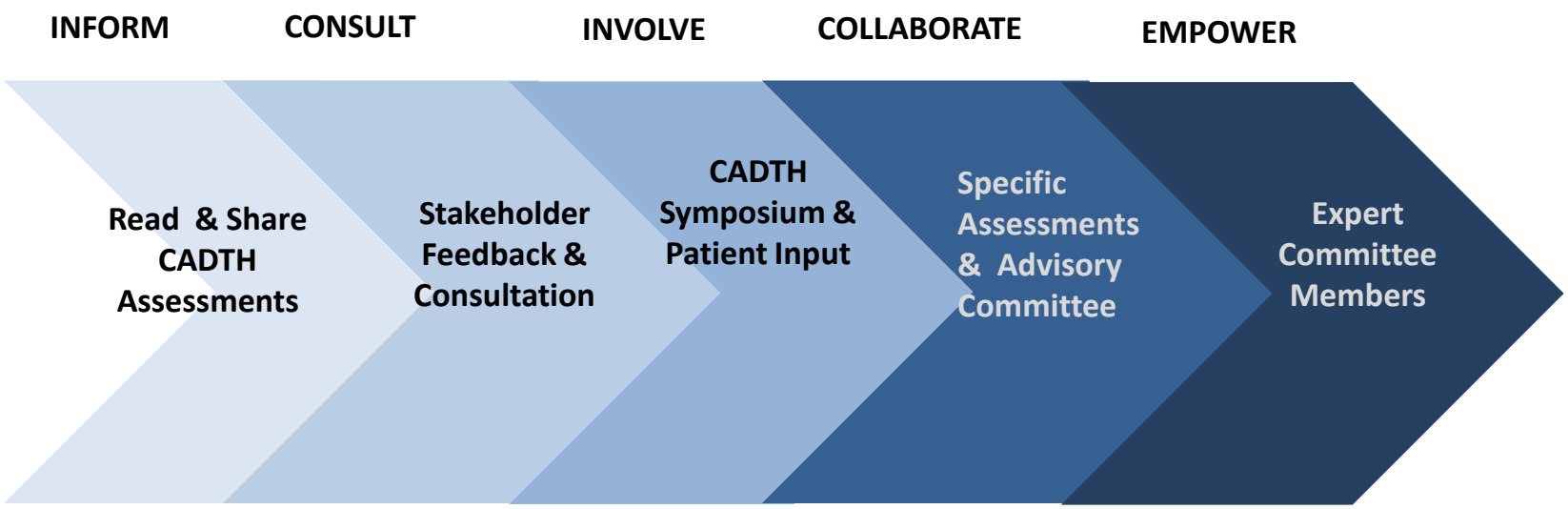
CADTH Patient and Community Advisory Committee

- The Committee will advise CADTH across all programs:
  - Help CADTH to explore the voices we're hearing from and not hearing from
  - Help us explore approaches to strengthen how we currently engage and how we could engage differently
  - Provide advice on approaches to enhance the transparency of CADTH processes
  - Provide patient and public perspectives to CADTH in the development of initiatives to improve the appropriate use of drugs and devices across the life cycle of health technologies

CADTH Patient and Community Advisory Committee

- Advisory Committee adds to CADTH's existing approaches to hear from patients and patient groups
  - We still have patient and public members on our expert committees
  - We continue to rely on patient groups to provide patient input
  - We continue to rely on patient groups to comment in stakeholder consultation
  - We continue to rely on individual patients to contribute to Scientific Advice and individual projects

Become Involved with CADTH



Based on the Spectrum of Public Participation, the International Association for Public Participation (IAP2)

Participate in CADTH Symposium

- Attend in person or watch live sessions on CADTH
- Question or comment #CADTHSymp
- Present a poster, give a presentation, or join a panel
- Planning committee
- 25 abstracts submitted by members of the patient community for 2019 Symposium

Travel Award Application by
December 10, 2019
for 2020 CADTH Symposium
April 19 – 21 in Toronto

CADTH

Contribute to CADTH Assessments

Individuals and/or groups **work with CADTH teams** for:

- HTA/OU Projects (medical devices and procedures)
- Horizon and Environmental Scans
- Scientific Advice

Groups provide **Patient Input** on specific drugs for:

- Common Drug Review
- pan-Canadian Oncology Drug Review
- CAR T-cell projects

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What does the evidence say?
When Canada's health care decision-makers need to know, they ask CADTH. We're a trusted source for evidence on drugs and medical devices.
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CADTH Evidence Driven.

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Find the information you need SEARCH

Home ▸ Patient and Community Engagement

Patient and Community

- Become Involved
- Common Acronyms
- Framework for Engagement
- Patient Involvement in Organizational Governance

CADTH seeks patient perspectives to improve the quality of our assessments of medical procedures, devices, and drugs. Patients, families, and communities can offer insight on the diversity of individual needs and health care settings across Canada. CADTH's recommendations on publicly funded devices, procedures, and drugs impact Canadian patients. So, it makes sense that patients and the public be aware of, and involved in, our work.

We have many opportunities for individuals, patients, families, and caregivers — and for patient groups and Canadian communities — to read, contribute to, and shape our work. We explain in our engagement framework why we engage with patients, families, and communities across our different programs and processes. We're aware that patient and community engagement practices are rapidly developing and, as we learn from our own and others' experiences, CADTH approaches may change as a result.

- Subscribe to E-Alerts
- Stakeholder Feedback
- CADTH Symposium
- Patient Input and Feedback to CADTH Pan-Canadian Oncology Drug Review
- Patient Input to CADTH Common Drug Review
- Patient and Community Advisory Committee

Open Forum

CADTH

Wrap Up

CADTH

2020 CADTH SYMPOSIUM

decision- making in an age of uncertainty

april 19 to 21, 2020
sheraton centre toronto

Subscribe

Sign up at cadth.ca/subscribe to get updates sent directly to your inbox.

CADTH E-Alerts

Calls for stakeholder feedback and patient group input, plus other time-sensitive announcements.

New at CADTH

Monthly newsletter including a summary of new reports plus corporate news, announcements of upcoming events, and more.

CADTH Symposium and Events

Updates about our flagship annual Symposium, workshops, webinars and other events.

Stay Connected

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