
**REAL-WORLD EVIDENCE AND
OUTCOMES-BASED AGREEMENTS
WORKING GROUP
2019 RESEARCH AND OUTPUTS
EXECUTIVE SUMMARY**

NOVEMBER 25, 2019



REAL-WORLD EVIDENCE AND OUTCOMES-BASED AGREEMENTS WORKING GROUP

2019 RESEARCH AND OUTPUTS EXECUTIVE SUMMARY

RWE AND OBA
WORKING GROUP

Background

The mission of the *Real-World Evidence and Outcomes-Based Agreements Working Group* is to advance the opportunity for the use of outcomes-based agreements in Canada.

The working group brings together organizations inspired by the opportunity for real-world evidence (RWE) generation to support outcomes-based agreements (OBAs) in Canada. The scope of the working group includes all therapeutic areas and both public and private payer markets. The working group values inclusion, knowledge sharing and collaboration, and

invites input and participation from all relevant parties, with the objective of advancing opportunities for OBAs to the benefit of all stakeholders in the Canadian healthcare system.

It is recognized that there are many challenges to overcome with the development and implementation of OBAs in Canada, and that the landscape is constantly evolving. The working group's method is to actively address these challenges and to find potential solutions and approaches that will provide value to all stakeholders.

Research Objectives

The working group's 2019 primary focus was on research and analysis to bring clarity on the opportunity for OBAs in Canada, and to develop a framework for OBA implementation in Canada, including:

1.

Developing clarity on when it is appropriate to use an OBA, the benefits of an OBA for payers and manufacturers, and how to evaluate when a drug is a good fit for an OBA.

2.

Developing a process for the monitoring and reporting on outcomes, and the creation of an implementation framework to support OBAs, through simple and complex drug case study exercises.

3.

Exploring the use of patient support program (PSP) and specialty pharmacy infrastructures to support outcomes data collection for OBAs.

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Outputs

Outputs produced by the working group include:

1.

Research document answering the questions *When is it appropriate to have an outcomes-based agreement? What are the benefits?*

2.

A checklist tool to help stakeholders evaluate if it is appropriate to pursue an OBA.

3.

A 7-step OBA implementation framework, detailing the end-to-end process elements to support the evaluation, implementation and application of an OBA.

A selection of the working group's output materials is included with this document.

Concluding Remarks and Key Action Areas for Consideration

The working group is committed to advancing the opportunity for the use of OBAs, to the benefit of all stakeholders in the Canadian healthcare system. From the work conducted in 2019, the working group identified the following key action areas for consideration:

1.

There is a need to further develop the details of OBA appropriateness and implementation processes to continue to remove barriers to OBAs, with input from all relevant stakeholders.

2.

There is a need for greater collaboration at the upstream decision-making level with HTA evaluators on adaptive pathways, and specifically to identify appropriate drugs for OBAs and to provide support to downstream parties (pCPA, payers) on their suitability. The potential to discuss OBA appropriateness for a drug during Early Parallel Scientific Advice (Health Canada, CADTH) has been identified as an area to investigate.

3.

Connecting with other related initiatives is required to ensure collaboration on work being conducted on RWE and OBAs. The working group will ensure that findings are shared appropriately with stakeholders to support the advancement of the opportunity for OBAs in Canada.

APPENDIX

OUTCOMES-BASED AGREEMENTS

WHEN IS IT APPROPRIATE TO PURSUE AN OUTCOMES-BASED AGREEMENT?

WHAT ARE THE BENEFITS?

Background

For managed entry strategies to be successful for all stakeholders, payers and pharmaceutical companies need to understand – and agree on – which types of agreements are suitable for which drugs. These agreements outline specific conditions related to drug plan's reimbursement of a drug and act as a way to mitigate risk by addressing concerns and reducing uncertainty around elements such as efficacy, real world effectiveness, safety risks, cost-effectiveness, budget impact and affordability. To date, most agreements have been financially-based managed

entry agreements (MEAs), focusing on cost effectiveness and budget impact, which are relatively simple to administer. Key to success with financially-based MEAs has been determining an agreed-upon price, or budget threshold. However, with the increasing trend of complex specialty drugs coming to market, agreeing on these terms has become increasingly difficult. In addition, while these types of agreements provide savings to drug plans, they may not address other important data gaps that may exist for the product.

Outcomes-based Agreements

Outcomes-based agreements (OBAs) provide an important additional option that can be used when a payer and a manufacturer cannot agree to a single, fixed price for a new drug; and when prolonged negotiations risk delaying the product launching or even limiting patient access. For an OBA to be successful, the outcome of interest must be measurable and agreed upon by both the manufacturer and the payer.

There are three main types of situations where OBAs can be used:

1.

Variable response rates:

Drugs for which clinical trials have shown a specified health outcome is reached for only a percentage of patients (i.e. for 50% of patients). OBAs can be used to reduce a payer's risk for these drugs by only reimbursing drug when patients on therapy meet specified health outcome targets.

2.

Limited clinical trial data:

In a situation where there is incomplete clinical trial data, there is increased uncertainty about the drug's performance and appropriate price (for example: a situation where phase III clinical trial data has not been completed and therefore the benefits of the drug to patients is uncertain, but promising). By employing this type of OBA, patients can access treatment earlier, and the payer only pays for drug in instances where patients meet specified outcome targets. Ideally, new innovative drugs will reach patients quickly.

3.

Drug differentiation, under certain circumstances:

In this situation, an OBA can be used to differentiate drugs within the same therapeutic area by tracking real world health outcomes and applying a pay-for-performance model. The payer would then only be paying for drug when patients achieve the agreed-upon health outcome(s) target(s), thus generating savings in instances when the outcomes are not achieved.

OUTCOMES-BASED AGREEMENTS

WHEN IS IT APPROPRIATE TO PURSUE AN OUTCOMES-BASED AGREEMENT?

WHAT ARE THE BENEFITS?

Continued

Benefits of OBAs for Manufacturers and Payers

For Manufacturers

1. Option if an agreement on price cannot be reached.
2. Earlier access to drug for patients, especially for therapeutic areas where limited or no alternatives are available and/or drug may have a significant benefit over existing therapies.
3. RWD can be collected and used to determine the appropriate value of the drug (vs. RCT data, or instances where data is incomplete).
4. Option to differentiate drugs under certain circumstances, and secure access.

For Payers

1. Option if an agreement on price cannot be reached.
2. Access to drug for patients, especially for therapeutic areas where limited or no alternatives are available and/or drug may have a significant benefit over existing therapies.
3. Certainty of paying only for drug when the agreed-upon outcomes (i.e. health, clinical, quality of life, resources utilisation and adherence outcomes) have been achieved, and not paying when outcomes have not been achieved.
4. Potential budget savings or justification of cost in must-fund scenarios.
5. RWD can be collected and used to help with uncertainty in the drug's performance and appropriate value of the drug (vs. RCT data, or instances where data is incomplete).

OBA Payment Schemes

There are 3 payment scheme categories that can be applied at the patient or population level:

1.

Full rebate to payers for patients that do not meet the agreed-upon health outcomes.

2.

Partial rebate to payers for patients that do not meet the agreed-upon health outcomes.

3.

Free initial treatment cycle is provided by the manufacturer, after which it is determined if patient is responding as per agreed-upon health outcomes. Responders continue on drug, fully funded by payer.

Other considerations for OBA payment scheme models/options to add-on to the agreement, based on the situation:

1. Layering on traditional financially-based schemes into the OBA. These may alleviate payer concerns of budget certainty.

For example:

- Maximum \$ amount per patient per year
- Volume discount
- Budget cap

2. Renegotiation and moving to financially-based schemes after OBA data has been collected.

3. Conditional treatment continuation: payment for the continued use of a drug is based on intermediate endpoints.

4. Partial achievement of health outcomes has a sliding scale for which partial pricing is determined.

OUTCOMES-BASED AGREEMENTS

WHEN IS IT APPROPRIATE TO PURSUE AN OUTCOMES-BASED AGREEMENT?

WHAT ARE THE BENEFITS?

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Continued

Real World Data Collection to Optimize Drug Utilization

There is increasing recognition that evidence of a drug's effectiveness from clinical trials – while essential to prove a drug's safety and efficacy – may not always reflect a medicine's benefit to patients in a real-world setting. This may lead to a greater emphasis on using real world data (RWD) of patients' treatment outcomes to enable a payer and a manufacturer to agree on a price that better reflects the drug's true benefit to patients. Complementing clinical trial data with real world evidence (RWE) could help achieve quicker patient access and optimize the drug's long-term utilization and benefits.

Challenges in Implementing Outcomes-based Agreements

With new innovation comes many challenges, and OBAs are no exception: they are relatively new for pharmaceuticals, and particularly so in Canada. The idea of monitoring drug performance in the real world and basing compensation (for a manufacturer) or payment (by a payer) on the results – which could differ from clinical trials – is revolutionary, and needs to be socialized for all stakeholders.

As OBAs are a new innovation, at present the infrastructure is not fully in place to operationalize such agreements – however progress is being made. Resources are needed to create and manage the agreement, as well as to collect and analyze the health outcomes data. Agreeing to the health outcomes to be measured and targets for complex drugs will be challenging and not always possible. The data sources and analytics must be accurate and free of bias. Methodology options – for example, tracking patient-level outcomes or aggregating outcomes data at a population level – must be evaluated and selected on a case-by-case basis.

These are a few of the challenges with implementing OBAs. Overcoming these challenges will be critical to establishing OBAs as a viable option for the Canadian market – especially to provide an additional option for the growing number of complex drugs being developed.

Progress on OBAs is being made in other countries, and Canada should continue to explore OBA opportunities or risk falling behind.

OUTCOMES-BASED AGREEMENTS USING THE PATIENT SUPPORT PROGRAM INFRASTRUCTURE TO COLLECT OUTCOMES DATA FOR OBA

Continued

Why do patient support programs (PSPs) exist?

With complex pharmaceutical drugs, patients often require support to initiate and continue treatment. In many instances, the healthcare system either does not provide services (i.e. infusion facilities, patient training), or is complex to navigate. PSPs are programs supported by drug manufacturers that provide services and fill the gaps left in the healthcare system to meet patient needs.

A PSP may perform some or all of the following services, among others:

- **Educate the patient** on the disease and the drug – including how and when to take the medication.
 - **Provide financial assistance** and/or compassionate drug until reimbursement is in place.
 - **Liaise** with the patient's private or public insurer to obtain reimbursement, often a complex process.
 - **Set up** a treatment schedule and remind patients of appointments.
 - **Provide treatment updates** to physicians.
 - **Deliver medication** to its destination (e.g. pharmacy, clinic, hospital, or patient's home).
- In Canada, it is now standard for new specialty drugs to have associated patient support programs. Physicians, payers and patients are accustomed to these services. It is estimated that hundreds of products have been launched on the Canadian market with associated PSPs, and this trend continues today.

PSP capabilities and infrastructure

- National in reach, covering all provinces and payers.
- A PSP in effect is a patient registry for patients enrolled in the PSP, including consent considerations.
- Follow-up with patients and physicians: adherence, appointment reminders, treatment schedule, treatment updates, etc.
- Patient services including patient education and training, pharmacy services, at home delivery, at home nursing, infusions, etc.
- Capabilities have grown and continue to evolve, including data capture.
 - Data capture from pharmacy systems, connection to EHR and labs, pre-authorization and continued use information, lab tests and physician-provided information.
 - Examples of health outcomes data that is already being collected today via EHR, physician or patient: lipid assessment (cholesterol), sustained viral response (SVR), fecal calprotectin test, MS 25 step test, psoriasis PASI score, weight change.

Why use the PSP infrastructure to collect outcomes data for OBAs?

- The capabilities and infrastructure (people / processes / systems) to support OBA outcomes data collection are already in place today within PSPs, including pharmacists, nurses, IT, reimbursement specialists and robust data capabilities.
- Manufacturers invest in PSP at the launch of each new specialty drug. It is cost effective to leverage this infrastructure to facilitate OBAs.
- Patients and doctors are already accustomed to PSP, and the PSP often retains a significant volume of patients throughout a drug's lifecycle. Adding an OBA element to a PSP may create minimal change for these stakeholders.
- Health outcomes data is already being collected by many PSP. If a drug's health outcome measurement can be captured by its PSP, it may provide an elegant solution for OBA data collection purposes.

Conclusions

The patient support program infrastructure is increasingly recognized as a valid source to collect outcomes data for RWE generation, and the existing PSP infrastructure contains many of the necessary requirements to enable the OBA data collection process.

Through the case studies conducted by the working group, it was found that an OBA-type program can be designed and executed in a similar manner to a PSP, as a stand-alone program or in parallel with a traditional PSP, to support outcomes data collection for OBA. The opportunity for drug manufacturers who are already running PSPs is to leverage the existing infrastructure to support OBA programs.

OUTCOMES-BASED AGREEMENTS

7-STEP OBA IMPLEMENTATION FRAMEWORK

Through the application of simple and complex drug case study exercises, literature reviews and research, the working group developed a 7-step OBA implementation framework, which outlines the end-to-end process elements to support the evaluation, implementation and application of an OBA.

The 7 steps of the OBA implementation framework include sections on each of the following:

