REAL-WORLD EVIDENCE AND OUTCOMES-BASED AGREEMENTS WORKING GROUP

2022 RESEARCH AND OUTPUTS

EXECUTIVE SUMMARY

DECEMBER 5, 2022

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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 real-world evidence (RWE) and outcomes-based agreements (OBAs) to the benefit of all stakeholders in the Canadian healthcare system.

Established in 2019, the working group conducts research on RWE and OBAs to support shared learnings on OBA implementation for Canadian stakeholders, including HTA, the pCPA, payers, patients, physicians, academics, and industry. 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 interested parties.

20Sense chairs the working group. 2022 members include AbbVie, AstraZeneca, Bayer, BioScript Solutions, Janssen, and Pfizer.

For further information, resources, and past publications from the *RWE & OBA Working Group*, please consult **https://www.20sense.ca/obas**. Inquiries may be directed to **info@20sense.ca**.

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BACKGROUND ON OBAS IN CANADA

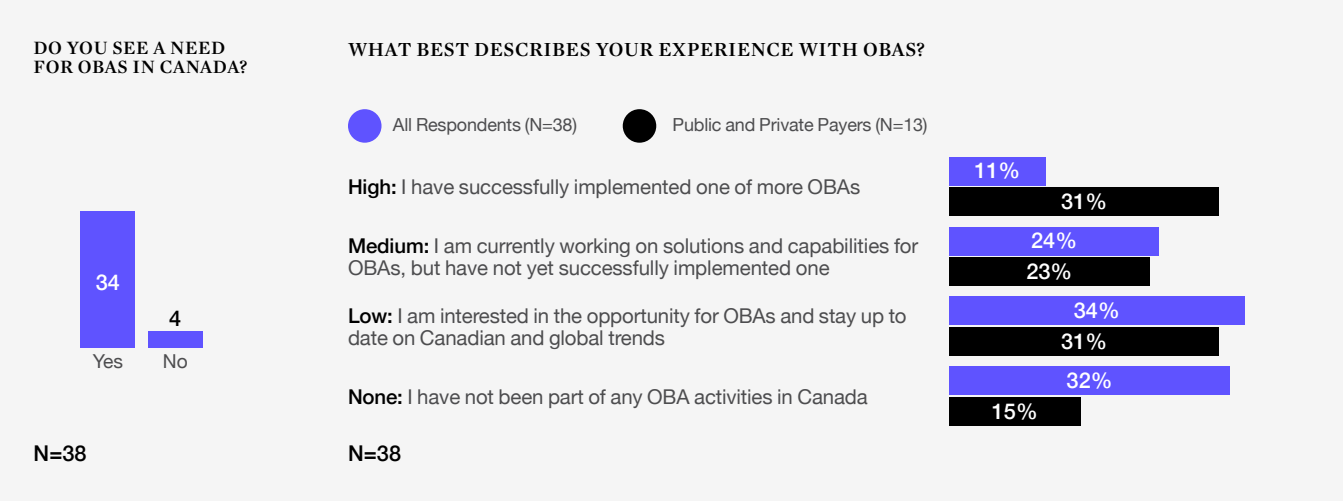
With the number of promising therapies with imperfect data increasing, particularly in rare disease and precision oncology, coupled with long reimbursement timelines, timely access for patients to novel therapies has become increasingly challenging. OBAs are a potential solution to provide timely access for patients to therapies with associated uncertainties, while mitigating the risk for payers of non-performance in the real world.

OBAs have gained ground internationally as a strategy for managing access and risk but are still in their infancy in Canada. Barriers to OBAs that have been identified include a lack of resources, infrastructure, and know-how for OBAs; a lack of appropriate RWD; and a lack of trust between stakeholders.

Current state of OBAs in Canada

Recent research with Canadian payers and other stakeholders with an interest in OBAs (i.e., HTAs, the pCPA, academics, patients, HCPs and industry) has shown that there is a need for OBAs in Canada, and that leading payers have successfully implemented one or more of these innovative agreements.

Figure 1: Need for and experience with OBAs in Canada



SOURCE: CANADIAN OUTCOMES-BASED AGREEMENTS EXPERIENCE AND PERCEPTIONS: SURVEY RESULTS, RWE & OBA WORKING GROUP, OCT. 2021. HTTPS://BIT.LY/3TAJ33M

However, in Canada, OBAs are confidential and there are no publicly available examples for stakeholders to learn from. Providing examples of how an OBA can be done in Canada is critical to increase shared learnings for all stakeholders, and to help advance the opportunity for OBAs as potential solution to provide timely access for patients to therapies with associated uncertainties. This has been the focus of the *RWE & OBA Working Group*.

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2022 RESEARCH AREAS

By building on the tools developed and research conducted to support OBA implementation by the working group in past years, in 2022 there was a **continued focus on research and analysis to bring clarity on the opportunity for RWE and OBAs in Canada, while developing expertise and knowledge in key areas to ensure readiness for OBA implementation**.

The working group had two primary areas of research in 2022:

1.

Investigating the feasibility and current infrastructure readiness to collect real-world data to support an outcomes-based agreement in Canada, focusing on the existing prior authorization processes used by private payers.

2.

Investigating the needs of payers and manufacturers regarding the financial adjudication of OBAs.

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RESEARCH AREA 1: RWD & PRIVATE PAYERS

Investigating the feasibility and current infrastructure readiness to collect real-world data to support an outcomes-based agreement in Canada, focusing on the **existing prior authorization processes and infrastructure used by private payers**.

Research Objectives

To address the concerns of RWD availability, research is required to evaluate if existing RWD sources and infrastructure could be leveraged to support OBAs and to provide stakeholders with examples of potential RWD solutions to support OBAs in Canada.

To this end, in 2022 the *RWE & OBA Working Group* continued research on RWD availability focusing on the existing prior authorization processes used by private payers, with the following research question: **Could RWD from the private payer prior authorization (PA) process be used to operationalize an outcomes-based agreement?**

The project had two objectives:

1. To determine which health outcomes are currently tracked or could be tracked using the current private payer PA process.
2. To evaluate if the health outcomes and data from the private payer PA process are suitable to support RWE generation for an OBA.

Research Status

Research interviews were conducted from June-October 2022 with 8 private payers and PBMs. There were 20 interview questions across 4 themes:

1. Prior Authorization (PA)
2. Outcome-based Agreements (OBAs)
3. OBAs and PA
4. OBA Financial Models

Research findings and results are in the process of being compiled. Publication of full results and findings is anticipated for early 2023.

RESEARCH AREA 1: RWD & PRIVATE PAYERS

continued

Preliminary Findings

A sample of PA forms from the websites of the top 10 insurers and 2 PBMs was conducted to confirm the types of health outcomes data collected in the current state PA process. A selection of outcomes are show in the below table (Figure 1).

Figure 1: Selection of outcomes data collected and reviewed in private payer prior authorization forms

CATEGORY	OUTCOME MEASURES
1. Clinical event or measurement	• Number of relapses, attacks or hospitalizations within last 12 months • Reduction in the number and/or severity of attacks and/or relapses • Body mass index (BMI) • Weight
2. Lab tests and scans	• LDL-C • Liver function tests (ALT, AST, bilirubin) • T2 Gandolinium-enhancing lesions (MRI) • Lesions (MRI) • C-reactive protein (CRP) value
3. Performance scale	• ECOG Performance Status Scale • Tiffeneau-Pinelli Index (FEV1) • CFQ-R Respiratory Domain Score • Expanded Disability Status Scale (EDSS) • Hammersmith Functional Motor Scale Expanded (HFMSE) Score • Crohn's Disease Activity Index (CDAI) • Harvey-Bradshaw Index (HBI) • Mayo score (Endoscopic sub-score; Rectal bleeding sub-score) • ALSFRS-R Score • Spirometry report • Dermatology Life Quality Index (DLQI) • Psoriasis Area Severity Index (PASI)
4. Other	• Return to work

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RESEARCH AREA 2: OBA FINANCIAL MODELS

Investigating the **needs of payers and manufacturers regarding the financial adjudication of OBAs**.

Research Objectives

As a relatively new type of agreement, OBAs require new processes and resources to be developed to support their financial adjudication. There are multiple issues (i.e., fiscal year budget alignment, risk liability carried over multiple years, revenue recognition, etc.) that need to be overcome to enable successful OBA implementation.

Thus, in 2022 the *RWE & OBA Working Group* investigated the needs of payers and manufacturers regarding the financial adjudication of OBAs by evaluating OBA financial models, with the following research question:

Which OBA financial models are best suited to meet the needs of both payers and manufacturers?

The project had two objectives:

1. Determine payer and manufacturer needs regarding the financial adjudication of OBAs (i.e., resources, budget alignment, rebates, budget risk, revenue recognition, etc.).
2. Compile a list of OBA financial model options and evaluate each based on the needs of both payers and manufacturers.

Research Status

Research interviews were conducted from June-October 2022 with 24 stakeholder groups, attended by 40 individuals including:

- 10 public payers, HTA, and the pCPA – former and current.
- 8 private payers and PBMs – current.
- 6 pharmaceutical manufacturers.

There were 10 interview questions on various aspects of financial model considerations to support OBAs.

Research findings and results are in the process of being compiled. Publication of full results and findings is anticipated for early 2023.

RESEARCH AREA 2: OBA FINANCIAL MODELS

continued

Preliminary Findings

Research was conducted on publicly available examples of OBA financial adjudication models, with a focus on understanding the relevant factors impacting the financial model of the agreements (i.e., timeframe of financial uncertainty, health outcomes selected, data sources, resources, etc.). As Canadian OBAs are confidential, 7 international OBAs were identified with varying characteristics (Figure 2).

Figure 2: OBA financial adjudication models with timeframes of financial uncertainty

	OBA FINANCIAL MODEL	TIMEFRAME OF FINANCIAL UNCERTAINTY
1	**KYMRIAH (tisagenlecleucel) / oncology (CAR-T) / USA** CMS will only pay for Kymriah if patients respond within one month of initiating treatment.	< 1 year
2	**XALKORI (crizotinib) / oncology / USA** Refund up to 3 months if the patient discontinues therapy within 3 months.	< 1 year
3	**IRESSA (gefitinib) / oncology / USA** Manufacturer will reimburse costs of gefitinib if a patient discontinues treatment before third prescription fill.	< 1 year
4	**HARVONI (ledipasvi, sofosbuvir) / Hep C / USA** Rebate paid from the manufacturer to the payer based on the patient's sustained virologic response 12 weeks after the completion of treatment. If cured, no rebate. If not cured, a rebate is paid.	< 1 year
5	**KEYTRUDA (pembrolizumab) / oncology / Australia** Initial price aligned with current standard of care therapy pricing (net, including maximum cap), with provision for future clinical trial evidence to support a potential price increase based on the OBA results. OBA data source is the clinical trial data.	2 years
6	X**ALKORI (crizotinib) / oncology / Australia** Manufacturer to track first 50 real-world patients on drug to determine overall survival rate at 12 months. Manufacturer to provide payer rebate if less than clinical trial results achieved. Estimated timeframe to complete is 3 years.	3 years
7	**ZOLGENSMA (onasemnogene abeparvovec-xioi) / oncology (gene therapy) / USA** Rebate if not deliver the clinical health outcomes for a five-year period.	5 years

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CONCLUDING THOUGHTS

Research findings suggest that there is a need for OBAs in Canada as a potential solution to provide timely access for patients to therapies with associated uncertainties, while mitigating the risk for payers of non-performance in the real world. Many stakeholders are exploring opportunities to leverage OBAs when appropriate, and some leading manufacturers and payers have implemented OBAs.

For OBAs to be available as an option to a broader set of stakeholders, there is a need to increase overall understanding and expertise of how to do an OBA in Canada. Transparency and sharing of learnings from OBAs implemented in Canada, as well as increasing knowledge about RWE generation for OBAs, can help support this. The willingness and openness of most stakeholders is currently medium-high; however, implementation readiness is low – but increasing.

The rate of adoption and use of OBAs to support timely market access for patients in Canada is only expected to increase in the coming years. This includes the further development of capabilities and expertise to support OBAs.

FOR FURTHER INFORMATION, RESOURCES, AND PAST PUBLICATIONS FROM THE RWE & OBA WORKING GROUP, PLEASE CONSULT WWW.20SENSE.CA/OBAS. INQUIRIES MAY BE DIRECTED TO INFO@20SENSE.CA.