Generating timely and meaningful Canadian real-world evidence to support an outcomes-based market access agreement

ASCOT: An RWE case study in oncology

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Roundtable Discussion

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ASCOT: A real-world evidence case study in oncology

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Panelists

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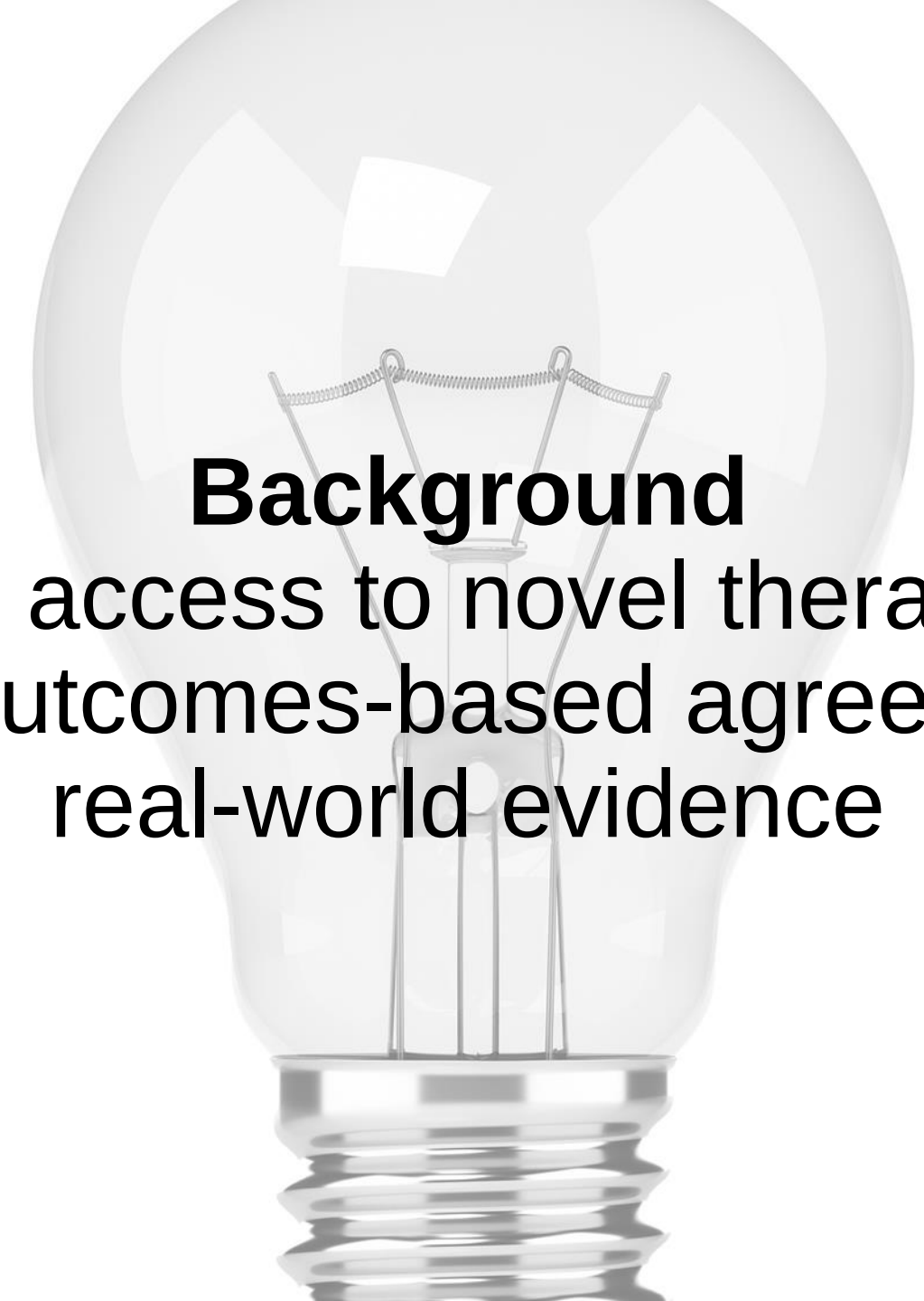

Background

Timely access to novel therapies in Canada, outcomes-based agreements and real-world evidence

Outcomes-based market access agreements are one tool in the “solutions toolbox” to support timely market access

An outcomes-based agreement is a market access agreement between a pharmaceutical manufacturer and a payer in which the manufacturer will issue a refund or rebate to the payer based on how well the therapy performs in a real-world patient population, measured against an agreed-upon, pre-defined set of benchmarks.

Sources: Early access for innovative oncology medicines: a different story in each nation, Journal of Medical Economics, 26:1, 944-953, 2023. https://doi.org/10.1080/13696998.2023.2237336; Managed access, NICE. https://www.nice.org.uk/about/what-we-do/our-programmes/managed-access

Research on RWD collection and RWE generation to support OBA implementation for Canada

Supportive Care in Cancer (2023) 31:5
https://doi.org/10.1007/s00520-022-07486-5

RESEARCH

Building infrastructure for outcomes-based agreements in Canada: can administrative health data be used to support an outcomes-based agreement in oncology?

Winson Y. Cheung[1] · Chris Cameron[2] · Arif Mitha[3] · Allison Wills[3]

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Abstract

Background Outcomes-based agreements (OBAs) have the potential to provide more timely patient access to novel therapies, although they are not suitable for every new medication or reimbursement scenario. The authors of this paper studied how to operationalize an OBA in oncology by leveraging existing real-world data (RWD) infrastructure in the province of Alberta.

Objective The main objectives were to (1) evaluate which health outcomes in oncology are suitable for OBAs and whether they can be tracked with existing infrastructure, and (2) determine how RWD in oncology can be used to implement an OBA and the expected timing for delivery.

Methods Using the Oncology Outcomes (O2) Group infrastructure and Alberta administrative data, a review of five key oncology outcomes was performed to determine suitability to support an OBA

Source: Cheung, W.Y., Cameron, C., Mitha, A., Wills, A. Supportive Care Cancer 31, 5 (2023). https://doi.org/10.1007/s00520-022-07486-5

OS and TTNT were identified as suitable health outcomes for OBAs from Alberta Health Services oncology data

Based on the evaluation of data readiness, data interpretation, and data timeframe, two health outcomes were identified as suitable for OBAs:

- Overall survival (OS)
- Time to next treatment (TTNT)

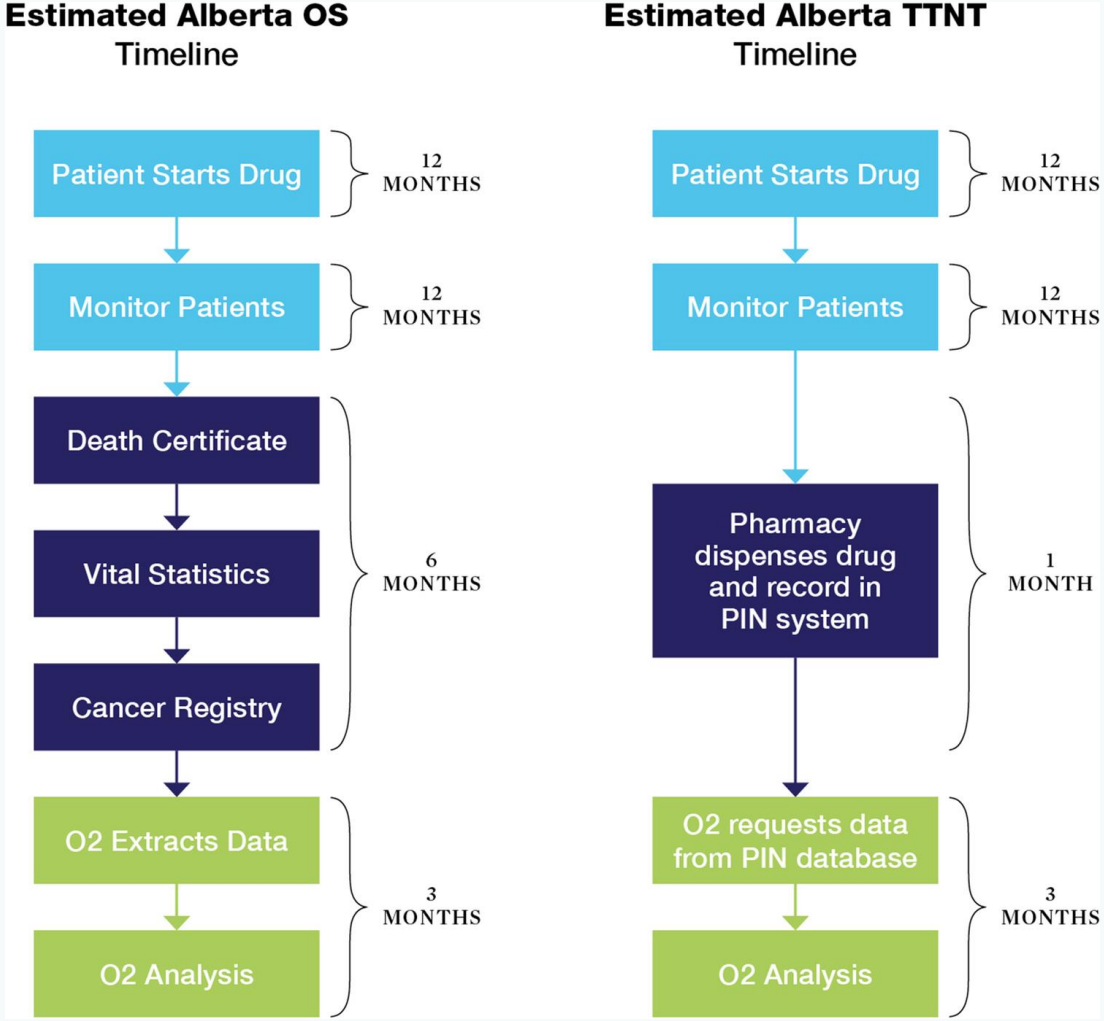
Table 1: Health outcomes data readiness, interpretation, and timeframes in Alberta

Health outcome	Suitable for an OBA?	Data readiness Accessible for an OBA, complete and accurate	Data interpretation Health outcome is clear and simple	Data timeframe Can be collected in a reasonable timeframe
1. Overall survival (OS)	Yes	• AB Cancer Registry and Vital Statistics • Data have been used in published studies	• Binary data point, easy to interpret	• 6-month time lag
2. Time to next treatment (TTNT)	Yes	• AB PIN Database contains all Rx's dispensed in AB (all payers) • Data have been used in published studies	• Algorithm required to ascertain specific treatment pattern	• 1-month time lag
3. Progression-free survival (PFS)	No	• AB administrative data • Incomplete: the timing of tests is not standardized	• Interpretation of results recorded in data is not standardized	N/A
4. Patient-reported outcomes (PROs)	No—future potential	• AB administrative data: ESAS, EQ5D Surveys • Incomplete: not consistently administered to all patients • No published studies	• EQ5D and ESAS are frequently included in HTA submissions	N/A
5. Return to work	No	• Data not available	• Patient may choose not to return to work	N/A

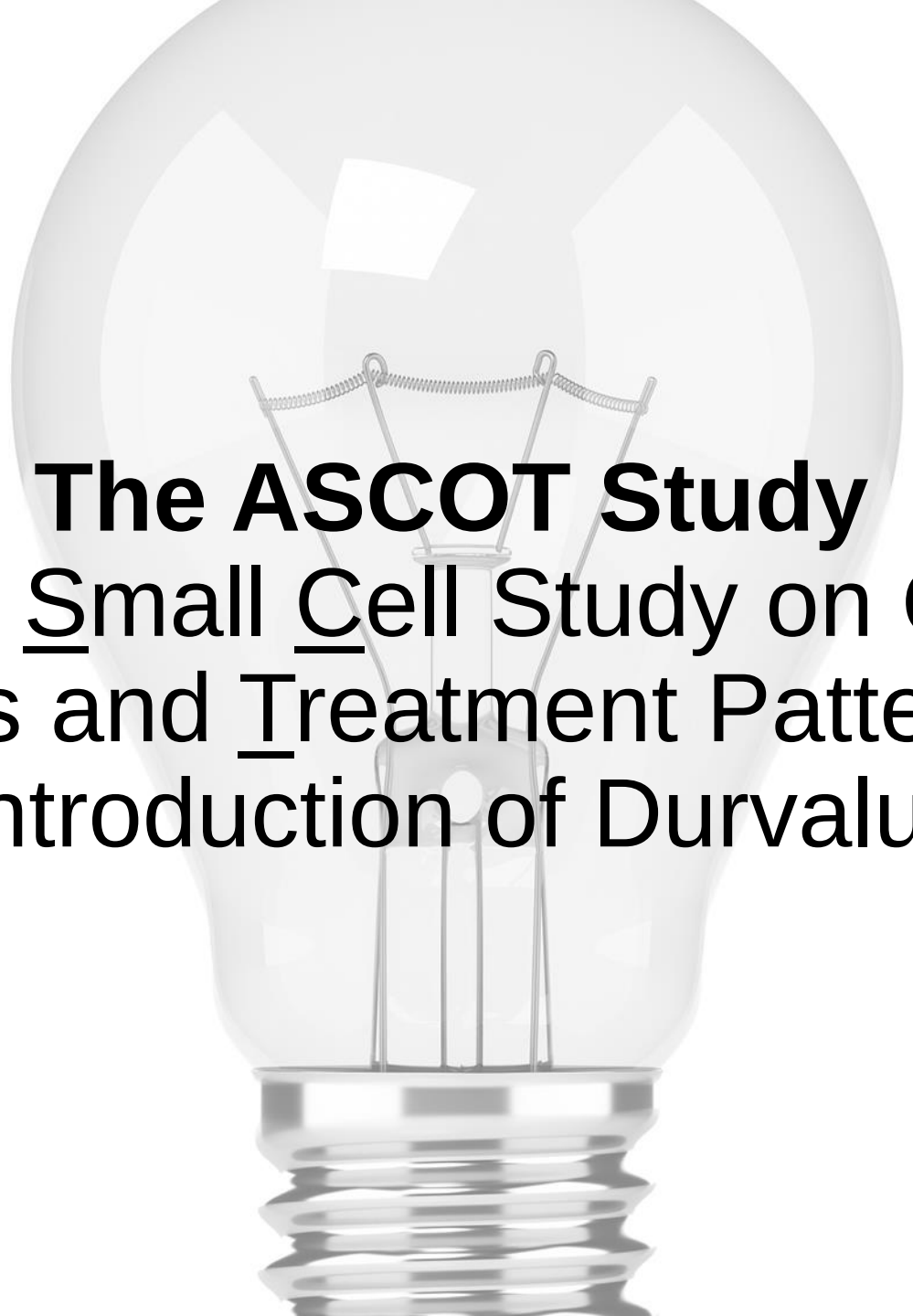
Source: Cheung, W.Y., Cameron, C., Mitha, A., Wills, A. Supportive Care Cancer 31, 5 (2023). https://doi.org/10.1007/s00520-022-07486-5

The total estimated timeframe required to complete the RWD component of an OBA is 2.5 to 3 years when using OS or TTNT in Alberta

- For both OS and TTNT, patients are recruited over a 12-month period and then monitored for an additional 12 months after initiation of therapy.
- For OS - 6 months for data collection logistics to receive information on survival status; 3 months for analysis and reporting by a team with data expertise. This resulted in a total approximate timeframe of 2 years and 9 months.
- For TTNT - 1 month for data collection logistics in the pharmacy PIN system; 3 months for analysis and reporting by a team with data expertise. This resulted in a total approximate timeframe of 2 years and 4 months.



Source: Cheung, W.Y., Cameron, C., Mitha, A., Wills, A. Supportive Care Cancer 31, 5 (2023). https://doi.org/10.1007/s00520-022-07486-5



The ASCOT Study

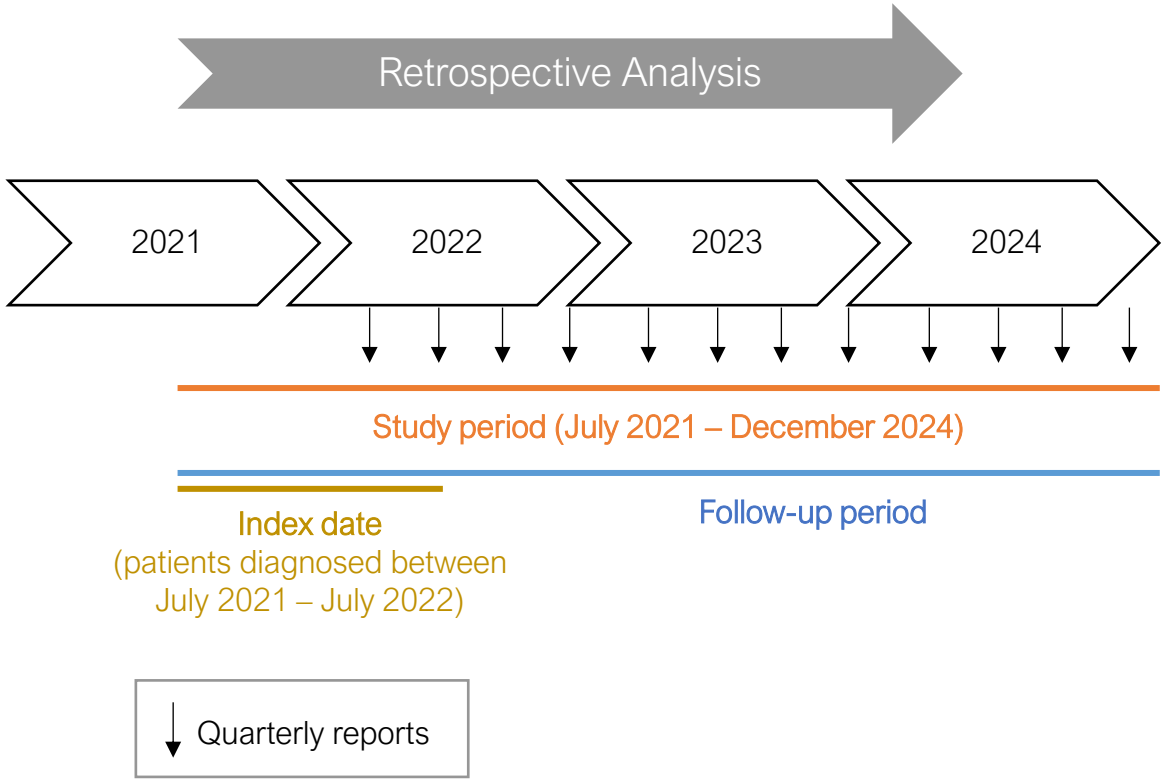
Alberta Small Cell Study on Clinical Outcomes and Treatment Patterns since the Introduction of Durvalumab

What were the objectives of the Alberta Small Cell Study on Clinical Outcomes and Treatment Patterns since the Introduction of Durvalumab Study?

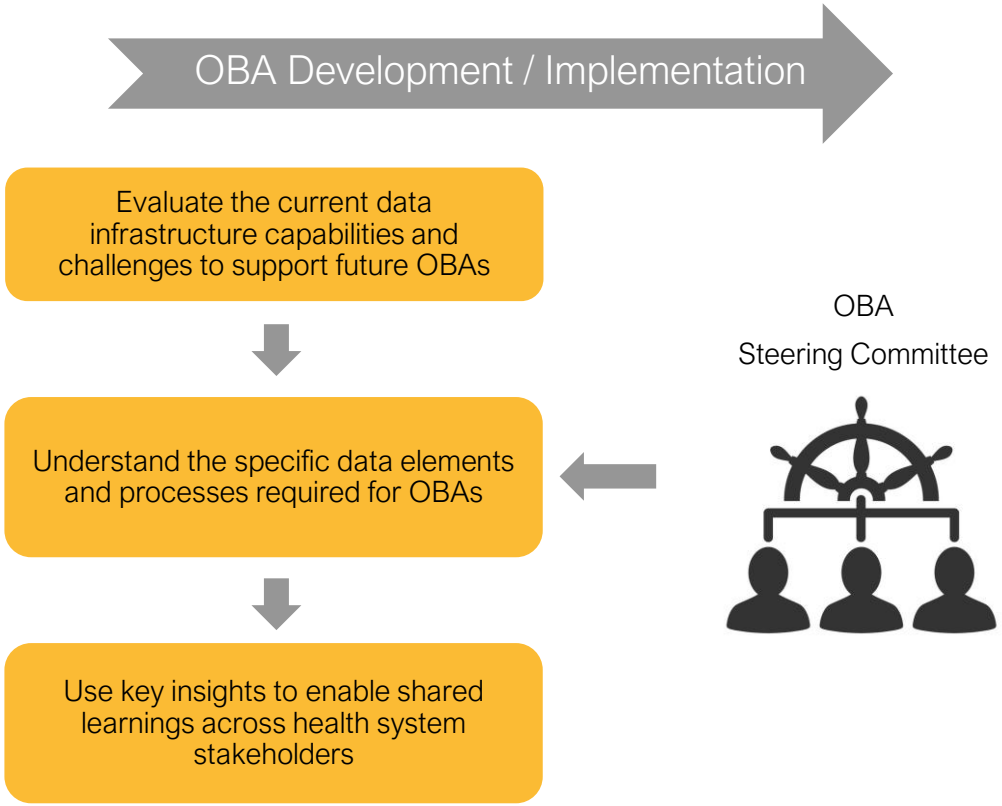
- Understand the characteristics, real-world treatment patterns, and clinical outcomes for patients with Extensive Stage-Small Cell Lung Cancer (ES-SCLC).
- Encourage discussion around the feasibility, development and implementation of outcomes-based agreements (OBAs) in Canada for oncology therapies.
- Through a "mock OBA", identify key OBA learnings and challenges with data collection, analytics, and evidence-generation processes and infrastructure.
- Gain experience with the implementation components of OBAs to enable shared learnings across health system stakeholders – the study may generate valuable insights into the logistics of conducting and implementing a true OBA.

The ASCOT Study
Overview

To understand the patient characteristics, treatment patterns, and clinical outcomes among patients with ES-SCLC in real-world clinical practice in Canada since the introduction of durvalumab.



To assess the feasibility of current data infrastructure in Alberta to support future outcomes-based agreements.



The ASCOT Study
Methods

- The population included adult patients diagnosed with ES-SCLC between July 2021 and July 2022.
- A series of quarterly retrospective analyses were conducted using real-world data from Alberta.
- A steering committee was established to assess current data infrastructure and elements to support the “mock OBA” process.
  - The committee consisted of:
    - Data experts (O2)
    - Clinician experts (Dr. Don Morris; Dr. Randeep Sangha, Dr. Daniel Heng, Dr. Winson Cheung)
    - The study sponsor (AstraZeneca Canada)
  - Key questions reviewed by the committee included:
    - What data and outcomes were available?
    - Would it be possible to generate quarterly reports?

The ASCOT Study
Key outcomes

1. Real-world efficacy results validated the results of the clinical trial.
2. OBAs were shown to be feasible in Alberta, of note:
    1. Outcomes were assessed that could potentially be suitable for an OBA, including:
        - Overall survival (OS)
        - Time to next treatment (TTNT)
        - Duration of treatment
        - Reasons for discontinuation
    2. Quarterly OBA reports were feasible to generate. Each report included a case flow diagram, demographic and clinical characteristics, survival outcomes, and time to next treatment or death information.
    3. An OBA steering committee was successfully established, with committee insights gained on the data process, both current state and future state opportunities and challenges.

The ASCOT Study
Excerpt from quarterly OBA Reports

Demographic and clinical characteristics

Characteristics of patients who initiated first-line (1L) durvalumab, summarized by descriptive statistics. Categorical variables are presented as n (%) and continuous variables as mean ± standard deviation and median (interquartile range).

Due to data privacy legislation, cell counts less than 10 are suppressed and denoted by "<10".

Characteristic	N	N = 63
Age at treatment initiation, y	63	
Mean ± SD		68 ± 7
Median (IQR)		67 (63, 73)
Sex	63	
Female		32 (50.8%)
Male		31 (49.2%)
Stage at initial diagnosis	63	
Extensive Stage		suppressed
Limited Stage		<10
ECOG PS at initial diagnosis	58	
0		<10
1		38 (65.5%)
2		13 (22.4%)
3		<10
Centre type	63	
Urban		51 (81.0%)
Sub-Urban		12 (19.0%)
1L treatment duration, mo	60	
Mean ± SD		6.1 ± 4.3
Median (IQR)		5.4 (3.2, 8.5)
1L dose reduction	63	<10
Reason for 1L treatment discontinuation	63	
Progression		35 (55.6%)
Toxicity/death		17 (27.0%)
Other		11 (17.5%)
2L treatment initiation	63	28 (44.4%)
Time to 2L treatment, mo	28	
Mean ± SD		1.5 ± 2.6
Median (IQR)		0.4 (0.2, 1.1)
Follow-up time, mo	63	
Mean ± SD		12.8 ± 8.2
Median (IQR)		11.3 (5.6, 19.1)

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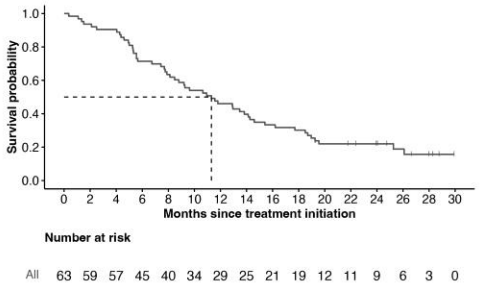
Survival outcomes

Overall survival (OS) of patients who initiated first-line durvalumab, from treatment initiation.



Median OS, mo (95% confidence interval [CI])[a]: 11.3 (8.5 , 14.6)

Median OS, mo (95% CI)[b]: 11.3 (8.1 , 14.3)

OS estimates (95% CI)[a]

6-month OS	12-month OS	18-month OS	24-month OS
0.71 (0.61, 0.84)	0.46 (0.35, 0.60)	0.30 (0.21, 0.44)	0.22 (0.14, 0.35)

OS estimates (95% CI)[b]

6-month OS	12-month OS	18-month OS	24-month OS
0.71 (0.60, 0.83)	0.46 (0.34, 0.58)	0.30 (0.19, 0.41)	0.22 (0.12, 0.32)

[a] log(survival function)

[b] Greenwood formula

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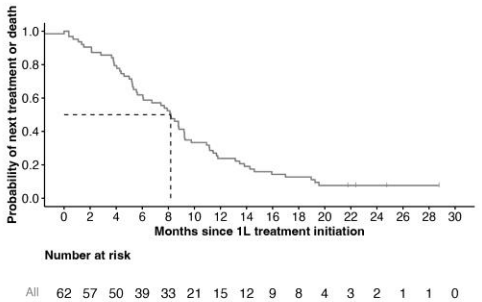
Time to next treatment or death

Time to next treatment or death (TTNT-D) of patients who received first-line durvalumab, from first-line treatment initiation.



Median TTNT-D, mo (95% CI)[a]: 8.2 (6 .1, 9.3)

Median TTNT-D, mo (95% CI)[b]: 8.2 (5.6 .1, 9.2)

TTNT-D estimates (95% CI)[a]

6-month TTNT-D	12-month TTNT-D	18-month TTNT-D	24-month TTNT-D
0.62 (0.51, 0.75)	0.24 (0.15, 0.37)	0.13 (0.07, 0.24)	0.08 (0.03, 0.18)

TTNT-D estimates (95% CI)[b]

6-month TTNT-D	12-month TTNT-D	18-month TTNT-D	24-month TTNT-D
0.62 (0.50, 0.74)	0.24 (0.13, 0.34)	0.13 (0.04, 0.21)	0.08 (0.01, 0.14)

[a] log(survival function)

[b] Greenwood formula

5

The ASCOT Study
Discussion

Insights

- ✓ It is feasible to obtain quarterly reports to support an OBA.
- ✓ It is possible to address OBA data needs through creative solutions – for example, through the integration of administrative and EMR data as seen in this mock OBA.
- ✓ An OBA steering committee is an effective way to inform data needs for an OBA.

Challenges and opportunities

- ➢ Lack of availability of suitable outcomes data for OBAs – source, timeliness, quality.
- ➢ There is a need to determine the appropriate parties for the OBA steering committee: payer, patients – when and how, and who will lead the committee.
- ➢ Lack of a national data infrastructure for OBAs; how can provincial data support equitable access across Canada? Is there an opportunity for the use of PSP data to support OBAs?

Roundtable discussion

What can we learn from the ASCOT study about generating timely and meaningful Canadian real-world evidence to support an outcomes-based market access agreement?

Roundtable Discussion

Generating timely and meaningful Canadian real-world evidence to support an outcomes-based market access agreement

Dr. Winson Cheung

Medical Oncologist, Professor of Medicine, and Principal Director Oncology Outcomes,
University of Calgary

Jessica Arias

Manager, Provincial Drug Reimbursement Programs
Ontario Health

Graham Statt

Former Vice-Chair of the **pCPA** & ADM of the Pharmaceutical and Supplementary Benefits Division at
Alberta Health

Dr. Amyn Sayani

Head, Medical Evidence
AstraZeneca Canada

Audience Q&A

Generating timely and meaningful Canadian RWE to support an OBA
The last word goes to…

On OBA-type agreements:

"Innovative agreements like OBAs definitely play a role. When a drug shows great promise but there is uncertainty around efficacy or price, it makes sense to provide the drug first, track the data, and then review and adjust access criteria and price accordingly. It's a commonsense approach for people who need quick access."

Stephanie Stavros, Founder and co-Director CF Get Loud

Stephanie has been fighting CF since birth. She made news in 2020 when she became the first person in Canada to be approved for compassionate care for Trikafta, after tirelessly lobbying to government and to the manufacturer of the drug.

Source: https://static1.squarespace.com/static/61d708f9587415184afa9452/t/61e5ae6a15030f1b545d6d33/1645544244267/Issue_17_EN_The_20Sense_Report.pdf

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