RWD164

ASCOT: A case study in generating timely and meaningful Canadian real-world evidence to support the data infrastructure necessary for innovative patient access

Rana Qadeer[1], Iqra Syed[1], YongJin Kim[1], Amyn Sayani[1], Eon Ting[1], Allison Wills[2], Winson Cheung[3]

[1]AstraZeneca Canada, Mississauga, ON, Canada
[2] 20Sense, Toronto, ON, Canada
[3] University of Calgary, Oncology Outcomes, Calgary, AB, Canada

Objective

- In recent years, healthcare systems stakeholders have been investigating the use of outcomes-based agreements (OBAs) to accelerate timely access to innovative therapeutics. However, potential barriers (e.g., timely access to real-world data/evidence [RWD/E]) have prevented adoption.
- We provide a practical demonstration to show that RWD/E can be used to support OBAs based on a case study among patients with Extensive Stage-Small Cell Lung Cancer (ES-SCLC) receiving durvalumab in Alberta.

Results/Conclusions

- 63 patients diagnosed with ES-SCLC who received durvalumab were followed (median follow-up: 11.3 months). Real-world efficacy results supported the results of the clinical trial.
- **This practical case study demonstrates that RWD can provide timely and meaningful reports to support innovative access pathways, such as OBAs, for select drugs/indications in appropriate situations; however, there are challenges (e.g., early inclusion of payers/patients, need for appropriate infrastructure/process, administrative burden to generate ongoing reports).**

Plain language summary

Why did we perform this research?

- Outcomes-based agreements (OBAs) are a unique type of risk-sharing agreement between a medicine manufacturer and payer. Such agreements account for how well the medication performs in a real-world population.
- Healthcare systems stakeholders have been investigating the use of OBAs to accelerate timely access to innovative medications (e.g., for a rare disease).
- However, potential barriers have prevented the adoption of OBAs, including the availability of high-quality data, appropriate data infrastructure, and effective use of real-world data/evidence (RWD/E).
- We provide a practical demonstration to show that RWD/E can be used to support OBAs among patients with Extensive Stage-Small Cell Lung Cancer (ES-SCLC) in Alberta.

What were the findings of this research?

- Overall, this study showed that OBAs were feasible in Alberta; additionally, this case study showed that OBAs may be feasible in other provinces with accessible/robust RWD and appropriate infrastructure:
  - We successfully generated quarterly reports that included suitable data/outcomes for an OBA.
  - An OBA steering committee was successfully established. This committee provided key insights on the data process and both current/future state opportunities/challenges, and ensured that data collected was meaningful.
  - However, there are still a few pending challenges, including lack of availability and generation of outcomes data (e.g., time lags, resource burden), inclusion of appropriate parties for OBA steering committee (e.g., patients), and lack of a national data infrastructure.

What are the implications of this research?

- This practical case study demonstrates that RWD can provide timely and meaningful reports to support timely access, such as OBAs, for select drugs/indications; however, there are challenges (e.g., early inclusion of payers and patients, need for appropriate infrastructure/process, administrative burden to generate ongoing reports).

Poster presented at ISPOR 2025 (Montreal, Canada). Corresponding author email address: Rana Qadeer, rana.qadeer@astrazeneca.com

Disclosures

This study was funded by AstraZeneca Canada. RQ, IS, YJK, AS, and ET are employees of AstraZeneca Canada at the time of this research.

References

1. 20Sense. (2021, April 22). Outcomes-Based Agreements in Canada: An Overview. https://www.20sense.ca/articles/16-02
2. Cheung, W. Y., Cameron, C., Mitha, A., & Wills, A. (2023). Building infrastructure for outcomes-based agreements in Canada: can administrative health data be used to support an outcomes-based agreement in oncology?. Supportive Care in Cancer, 31(1), 5.

Introduction

- Outcomes-based agreements (OBAs) are a unique type of risk-sharing agreement between a medicine manufacturer and payer, which account for how well the medication performs in a real-world population.[1]
  - The benchmarks for performance are pre-defined and agreed-upon by both the manufacturer and payer.[1]
- In recent years, healthcare systems stakeholders have been investigating the use of OBAs to accelerate timely access to innovative therapeutics.
- However, potential barriers have prevented the adoption of OBAs, including availability of high-quality data, effective use of real-world data/evidence (RWD/E), alignment on relevant outcomes, and the necessary data infrastructure to report outcomes on a regular basis.
- Here, we provide a practical demonstration to show that RWD/E can be leveraged to support OBAs through the ASCOT (Alberta Small Cell Study on Clinical Outcomes and Treatment Patterns since the Introduction of Durvalumab) study.

Methods

Study Objectives

1. Understand the characteristics, real-world treatment patterns, and clinical outcomes for patients with Extensive Stage-Small Cell Lung Cancer (ES-SCLC) receiving durvalumab between July 2021 and July 2022.
2. Assess the feasibility of current data infrastructure in Alberta to support future OBAs, including identification of key learnings and challenges associated with data collection, analytics, and evidence-generation processes and infrastructure.

Study Design/Overview

- A series of quarterly retrospective analyses using real-world population-level data from Alberta, Canada were conducted among patients diagnosed with ES-SCLC between July 2021 and July 2022 who had received treatment with durvalumab (Figure 1).
  - Study period was Jul 2021 - Dec 2024. Patients were indexed on date of diagnosis of ES-SCLC.
  - Data were obtained from Alberta's integrated provincial administrative data, electronic health records, and lab and pathology results.
- Patient demographics and characteristics, treatment patterns, and clinical outcomes that may be suitable for OBAs[2] (i.e., overall survival [OS] from treatment initiation, time to next treatment/death [TTNT-D], duration of treatment, reasons for discontinuation) were analyzed.
  - Continuous variables were reported descriptively with means ± standard deviation and medians with interquartile range. Frequencies and percentages were used for categorical measures.
  - Time to event variables (e.g., OS) were summarized using Kaplan-Meier methods, including 95% confidence intervals (CIs).
- Additionally, a steering committee consisting of data experts, including programmers, analysts/statisticians, and chart abstractors (Oncology Outcomes, O2), clinical experts/medical oncologists, and study sponsor (AstraZeneca Canada) was established to support data evaluation, ensure relevance of elements in quarterly reports, and understand opportunities/challenges of OBA RWD implementation (Figure 2).

Figure 1. Study design

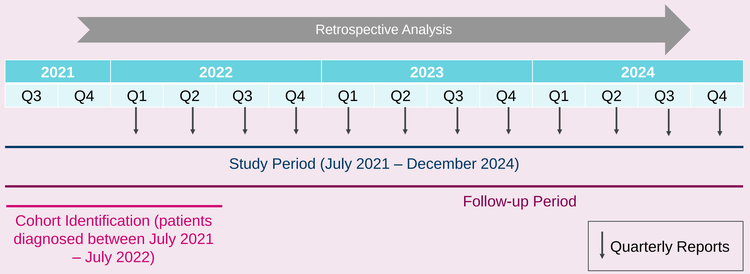
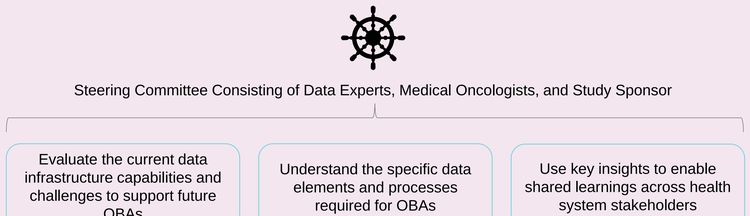


Figure 2. OBA steering committee



Results and Interpretation

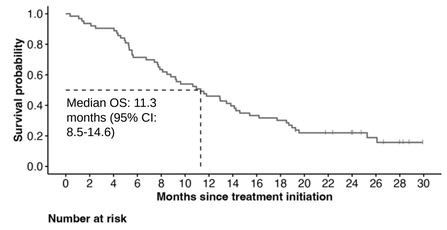
- 63 patients diagnosed with ES-SCLC who received durvalumab were followed (median follow-up: 11.3 months (95% confidence interval [CI]: 5.6-19.1). Baseline demographics and clinical characteristics are shown in Table 1.

Table 1. Demographics and clinical characteristics

Characteristic	N = 63
Age at treatment initiation, years	
Mean ± Standard Deviation	68 ± 7
Median (Interquartile Range)	67 (63, 73)
Male sex, n (%)	31 (49.2%)
Eastern Cooperative Oncology Group (ECOG) at initial diagnosis	
0, n (%)	<10 (suppressed)
1, n (%)	38 (65.5%)
2, n (%)	13 (22.4%)
3, n (%)	<10 (suppressed)
Centre type	
Urban, n (%)	51 (81.0%)
Sub-urban, n (%)	12 (19.0%)

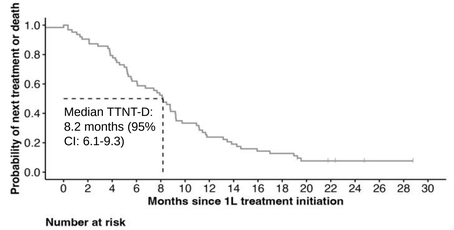
- Similar to the CASPIAN clinical trial, median OS was 11.3 months (95% CI: 8.5-14.6]) (Figure 3) and TTNT-D was 8.2 months (95% CI: 6.1-9.3) (Figure 4). The most common reason for treatment discontinuation was progression (n=35, 55.6%), followed by toxicity/death (n=17, 27.0%) or other (n=11, 17.5%).

Figure 3. OS for patients with ES-SCLC treated with durvalumab



CI: confidence interval, ES-SCLC: Extensive Stage-Small Cell Lung Cancer, OS: overall survival

Figure 4. TTNT-D for patients with ES-SCLC treated with durvalumab



CI: confidence interval, ES-SCLC: Extensive Stage-Small Cell Lung Cancer, TTNT-D: time to next treatment/death, 1L: first-line

- **OBAs were shown to be feasible in Alberta**:
  - Outcomes that could potentially be suitable for an OBA (e.g., OS, TTNT-D, reasons for discontinuation) were assessed.
  - Quarterly OBA reports were feasible to generate. Each report included a case flow diagram, demographic and clinical characteristics, survival outcomes, and TTNT-D information. Learnings from the quarterly reports were applied to future reports.
  - An OBA steering committee was successfully established, with committee insights gained on the data process, both current state and future state opportunities and challenges, and relevance of clinical data/outcomes collected and impact on patients.

Insights

- Feasible to obtain quarterly reports to support an OBA; reports can include data/outcomes that could be suitable for an OBA.
- Possible to address OBA data needs through creative solutions (e.g., integration of administrative and electronic health record data).
- OBA steering committee can be successfully established and can be an effective way to inform data needs for an OBA.

Challenges and Opportunities

- Lack of availability and generation of outcomes data for OBAs – source, timeliness, quality, and burden to generate continuous reports.
- Need to determine the appropriate parties for the OBA steering committee: payer, patients – when (ensuring early engagement) 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? Opportunity for the use of patient support program (PSP) data?

This practical case study demonstrates that **RWD can provide timely and meaningful reports to support innovative access pathways, such as OBAs, for select drugs/indications in appropriate situations.**