RWD25

# Use of Patient Support Program Data for Real-World Evidence Generation: Opportunities and Pitfalls Illustrated in a Case Study Assessing Trastuzumab Deruxtecan among Patients with Breast Cancer

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## Objective

- In Canada, patient support programs (PSPs) help patients access specialty medications prior to reimbursement. PSPs are also a rich source of real-world data (RWD), which can be used for real-world evidence (RWE).
- Here, we discuss opportunities and pitfalls associated with using Canadian PSP data for RWE, illustrated in a case study assessing trastuzumab deruxtecan (T-DXd) in metastatic breast cancer.

## Conclusions

- **It is feasible to generate robust RWE using PSP data with unique opportunities (e.g., national representation, first opportunity to assess the use of medications) to enhance decision making and improve patient outcomes.**
- However, RWE leveraging the PSP should be planned early; ensure transparency; employ strategies for methodological rigor, improved data collection, and robust analyses; and outline all known limitations.

## Plain language summary

**Why did we perform this research?**

- In Canada, patient support programs (PSPs) help patients access specialty medications (e.g., for cancer) prior to reimbursement.
- PSPs are also a rich source of real-world data (RWD), which can be used to generate real-world evidence (RWE).[1] However, use of PSP data for RWE has been limited.
- In this study, we assessed the opportunities and pitfalls associated with using Canadian PSP data for RWE using a case study in metastatic breast cancer.

**What were the findings of this research?**

- Overall, this case study showed that it is feasible to generate robust RWE using PSP data with some key opportunities and pitfalls:
  - Several strategies can be employed to ensure quality and rigor (e.g., registration to a public database, data quality checks, additional data collection).
  - PSP RWE leverages unique strengths of PSPs, including national representation and earliest opportunity to assess the use of specialty medications in the real-world.
  - However, RWE leveraging the PSP should be planned early, ensure transparency/methodological rigor, and outline all known limitations

**What are the implications of this research?**

- This study showed that RWE using PSP data is feasible and should be utilized routinely as it offers unique opportunities to enhance decision making and improve patient outcomes.

**Where can I access more information?**

More information can be found here: ClinicalTrials.gov, ID: NCT06386263, https://clinicaltrials.gov/study/NCT06386263?rank=1

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**Disclosures**

This study was funded by AstraZeneca Canada. RQ, SK, AN, SS, and AS are/were employees of AstraZeneca Canada at the time of this research.

**References**

1. Wills, A. (2023, October 19). Patient Support Program Data: A Uniquely Canadian Source Evidence — 20Sense. 20Sense. https://www.20sense.ca/articles/24-02
2. ClinicalTrials.gov. (2024). https://clinicaltrials.gov/study/NCT06386263?rank=1of

## Introduction

- In Canada, patient support programs (PSPs) help patients access specialty medications, approved by Health Canada, prior to reimbursement.
- PSPs are also a rich source of real-world data (RWD), which can be collected across the patient journey (i.e., at various touchpoints with the patient and the healthcare system).[1]
- These timely data can also be used for robust real-world evidence (RWE) that can generate early/first insights after the approval of the medication as these patients are often the first to receive a novel drug or an existing drug being used to treat a new indication.
  - However, use of PSP data for RWE has been limited in Canada, often due to data quality/collection issues and perceived acceptability.[1]
- Here, we discuss opportunities and pitfalls associated with using Canadian PSP data for RWE, illustrated in a case study assessing trastuzumab deruxtecan (T-DXd) in metastatic breast cancer.

## Feasibility of PSP Data for RWE: Opportunities and Pitfalls

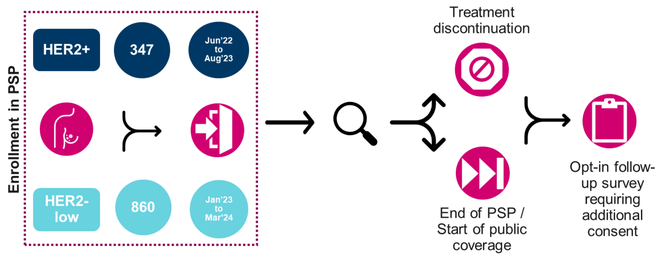
### Study Objectives

| | |
|---|---|
| Primary | • To estimate early discontinuation rates at 3-, 6-, and 9-months after initiating T-DXd<br>• To characterize T-DXd dose modifications over the course of treatment |
| Secondary | • Describe the demographic and clinical baseline characteristics<br>• Estimate real-world time to discontinuation (rwTTD) of T-DXd<br>• Characterize reasons for treatment discontinuation and treatment modifications<br>• Estimate real-world duration of treatment and dose intensity with T-DXd |
| Exploratory | • Assess real-world time to next treatment and characterize post-discontinuation therapies<br>• To assess drug wastage<br>• Describe the frequency and type of cardiotoxicity monitoring/CT scans<br>• Estimate the probability and association of early treatment discontinuation for demographic/clinical characteristics |

### Study Design

- A hybrid longitudinal cohort study (Figure 1), consisting of primary and secondary data (Figure 2) collected from Canadian patients with human epidermal growth factor receptor 2 (HER2)-positive and HER2-low metastatic breast cancer receiving treatment with T-DXd who enrolled in the PSP, was conducted.

Figure 1. Study design




HER: human epidermal growth factor receptor, PSP: patient support program

### Study Design Elements Implemented to Improve Rigor/Quality

Registered the study on ClinicalTrials.gov to ensure the quality and rigor of PSP RWE and improve acceptability among stakeholders (**study registration:** NCT06386263).

Employed additional strategies that improved the quality and rigor of PSP RWE, including strict data **governance/quality processes** (e.g., automated data quality checks), **third-party verification** of analyses, and a **robust study design** to meet RWE guidelines.

Leveraged the opportunity to **collect additional data** not captured in the PSP through optional patient questionnaire (requires an additional consent).

Leveraged unique strengths of PSP RWE, including **national representation** of patients across Canada and the **earliest opportunity to assess** T-DXd in the real-world.

Figure 2. Primary and secondary data collected

HER2+ and HER2-Low Cohorts

**Prior to T-DXd Initiation**

Secondary data:
- Clinical/demographic characteristics (i.e., age, gender, ECOG PS, IHC/ISH score, weight, number of prior lines of therapy, presence/absence of stable brain metastases, HR status*, disease recurrence*, progressed on prior anti-HER2**, sites of metastases**)

**On T-DXd Treatment**

Secondary data:
- Visit/cycle numbers
- Date of each infusion
- Dose at each infusion
- Weight at each infusion

**PSP End or T-DXd Discontinuation**

Secondary data:
- Date of discontinuation
- Reason for discontinuation

Primary data:
- Self-reported treatment discontinuation information collected every 3 months for up to 12 months after PSP closure (treatment discontinuation date, reason for discontinuation, subsequent treatment type and start date, frequency of monitoring/toxicity)

Primary data:
- Self-reported clinical/demographic characteristics (i.e., height, HR status for HER2+ cohort, race/ethnicity, prior therapies for metastatic disease, month/year diagnosis of metastatic disease, major comorbidities)
- Self-reported treatment information (i.e., frequency of cardiotoxicity monitoring/CT scans collected every 3 months for up to 12 months)

* Only collected for the HER2-low cohort
** Only collected for the HER2+ cohort
Secondary data: Patients who enrolled in the PSP consented for the use of their routinely collected data for research purposes.
Primary data: To supplement the secondary data, patients could provide an additional consent for optional data collection.
CT: computed tomography, ECOG PS: Eastern Cooperative Oncology Group Performance Status, HER: human epidermal growth factor receptor, HR: hormone receptor, IHC: immunohistochemistry, ISH: in situ hybridization, PSP: patient support program, T-DXd: trastuzumab deruxtecan

### Statistical Analyses

- Continuous variables reported as means with standard deviations (SDs) and medians with interquartile ranges (IQR)/ranges. Categorical variables reported as counts and proportions.
- Kaplan-Meier (KM) analyses used to evaluate time to event outcomes such as treatment discontinuation.
- Occurrence of dose modification or interruptions reported as the number and percentage of patients experiencing at least one event, while the rate of event was assessed using a binomial model and a complementary log-log link.
- Exploratory objectives involving assessment of statistical associations between treatment discontinuation and baseline demographic and clinical factors assessed using multivariable Cox proportional hazards models, if feasible.
- Trial registration: ClinicalTrials.gov, ID: NCT06386263. Retrospectively registered on April 25th, 2024.[2]

### Pitfalls/Future Directions

Due to reliance on secondary data from the PSP**, all potential prognostic/confounding variables were not captured**. Additional data collection was only feasible for a subset of the population and was self-reported (i.e., subject to bias).

Despite strategies highlighted above, there are **some methodological/data rigor challenges** (e.g., limited follow-up time, no follow-up once the PSP closes) and perceived bias with industry-funded PSP, which may require additional collaborations with independent experts/payers.

**Adequate planning and time** are required to ensure appropriate consents/governance and address the challenges identified above (e.g., additional data collection through data linkages).

This case study showed that it was **feasible to generate robust high-quality RWE using PSP data**. RWE using PSP data offers **unique opportunities to enhance decision making and improve patient outcomes**. However, RWE leveraging the PSP should be planned early; ensure transparency; employ strategies for methodological rigor, improved data collection, and robust analyses; and outline all known limitations.