PSP Real-World Readiness Framework Whitepaper

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Impact where it matters.

Introduction

Patient Support Programs (PSPs) within Canada are privately operated services designed to assist patients, caregivers, and providers with various therapy-related needs. The primary objectives of PSPs are to facilitate patient access to specialty medications, provide reimbursement tools and resources, and offer ongoing support services to help patients adhere to their prescribed treatment regimens. These programs play a pivotal role in integrating financial support, education, and navigation assistance within the Canadian healthcare system (e.g., instances where PSPs deliver therapies via infusions).

As part of the standard of care, various patient level data points are collected as part of the PSP to support the program goal. The value of PSP programs can be further enhanced by expanding the utility of the data collected, ensuring consistency across data variables, and optimizing its application for decision-making. Led by the Canadian Personalized Healthcare Innovation Network (CPHIN), this initiative seeks to elevate PSP data quality into a robust source of Real-World Evidence (RWE).

This project aims to develop a practical Real-World Readiness Framework for use by healthcare system stakeholders to assess the data reliability and relevance of PSP data. By doing so it may contribute to the design of more effective PSP programs and increase the likelihood that the collected data will be sufficiently high quality for informed decision making.

The framework consists of three main components:

(1) Definition and identification of use cases in which PSP data can support decision-making by various stakeholders.

(2) Identification of data variables that can inform these use cases.

(3) Establishment of "fit for purpose" data criteria, outlining methods for data collection, reliability, and relevance checks for different use cases.

PSP Real World Readiness Framework

The PSP Real World Readiness Framework (Figure 1) details a step-by-step process designed to help manufacturers and vendors to proactively design programs that facilitate future real world data (RWD) analyses. The set of recommendations established by the framework can also be leveraged in analyzing the ability to leverage current PSP data for decision making.

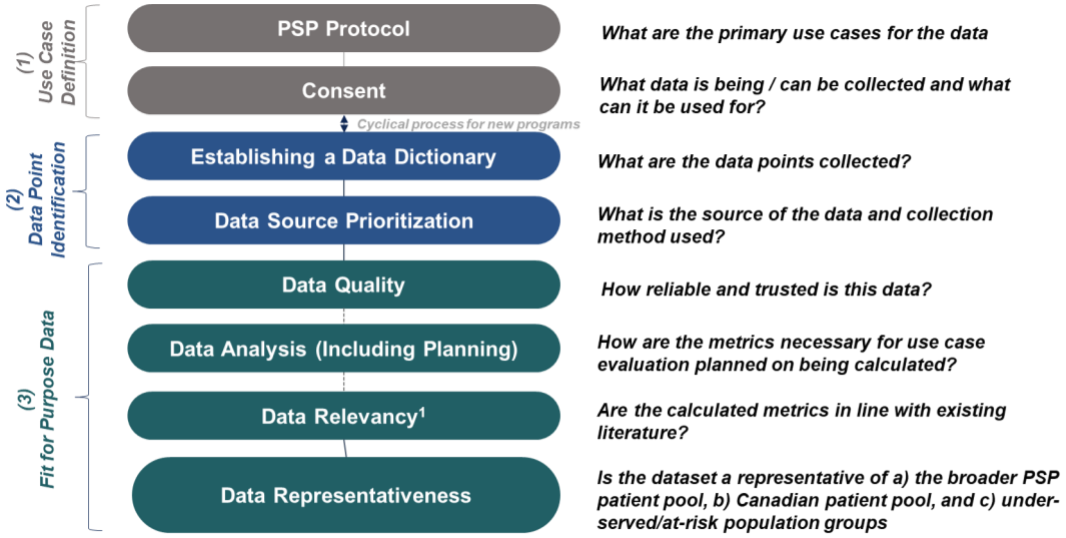

Figure 1. PSP Real World Readiness Framework step-by-step process detailing use case identification, data point identification and assessing fit-for-purpose data.

1. Use Case Definition

Initially, the intended use of the PSP collected data must be clearly articulated. Table 1 lists five, prioritized use cases based on their potential to improve patient access and enhance the patient care/experience.

The intent of these use cases is to delineate the various opportunities where data collected as part of Patient Support Programs (PSPs) can be leveraged by stakeholders to inform decision-making processes. Note that each use case requires a different level of breadth of data quality to support decision-making. Maintaining rigorous standards of data quality and relevance is essential to generating insights that can meaningfully inform decision-making and, ultimately, contribute to improved patient outcomes.

Table 1. PSP use case category definition detailing the five prioritized use cases.

Use Case Category	Description	Example Questions
External Organization Focused Use Cases		
1. **Reimbursement** *Helping payers, both public and private, understand the real-world impact of products*	Demonstrate the real-world impact of products using PSP data through the standardized reporting of key metrics. Real world metrics include patient outcome measures, adherence, compliance, patient reported outcomes (PRO) (e.g., quality of life) and total cost of care	• What is the compliance to therapy m6, m12, m18, m24? • What is the persistency to therapy m6, m12, m18, m24? • What dosing is used long-term in the real world? • …
2. Patient Experience & Outcomes *Enabling identification of gaps in care and assess of the value of adjunct services*	Report on the patient experience to quantify the clinical and societal impact of the services offered within PSPs by analyzing key metrics to identify gaps in care, inform value-based healthcare and impact of adjunct services (e.g., mental health support)	• What is the use and retention of PSP services by patients? • How does the utilization of PSP services correlate to patient persistency, compliance to therapy? • …
3. Regulatory *Enabling regulatory decision makers to inform or reinforce decisions, allowing them to support product approval*	Inform drug effectiveness studies (e.g., post-market drug effectiveness studies) to support regulatory applications including label updates, expansion (e.g., dose optimization)	• What is the distribution of dosing patterns for patients on-therapy? • …
Internal Manufacturer Focused Use Cases		
4. Medical Education *Enabling organizations to evaluate the success of medical education programs and its impact on patient outcomes*	**Internal:** Demonstrate the impact of medical education programs on patient outcomes, QoL and other metrics **External:** Leverage insights from internally focused success of medical education programs to identify the trends of HCPs to be targeted by programs and understand patient discontinuation rate (e.g., impact of dosing)	• What is the impact of medical education programs on patient self-management and adherence? • …
5. Process Improvement *Allowing organizations to identify PSP process improvement opportunities*	Using the framework (project outcome) to identify gaps in data quality / data collection methodology that makes it difficult to leverage the PSP data for primary use cases	• What are areas where there are data quality gaps in current methodologies? • …

2. Data Variable Identification

Once use cases are selected, it is essential to identify the relevant data variables needed to address the key questions. The selected data variables must be fit-for-purpose for the intended analysis. Data points required to address most use case questions would likely fall within one of the following categories:

- Patient Characteristics
- Physician Characteristics
- Treatment Characteristics
- Patient Interactions
- Patient Reported Outcomes (if applicable)
- Adverse Reaction Documentation (if applicable)

The comprehensive list of data points can be found within Appendix A.

3. Establishing Fit for Purpose Data

A. Fit for Purpose Definition

Once the use case corresponding data variables have been defined, retrospective or prospective PSP data could be considered fit for purpose. The fit-for-use assessment has been delineated into two key components: evaluating data relevance and data reliability (Figure 2). Data relevance is specific to the therapeutic area under investigation and is determined by assessing whether (1) the collected data can be utilized to accurately quantify the metric of interest and (2) the calculated metric value aligns with existing literature. Evaluating data reliability should also involve examination of the documentation and processes in place to ascertain the high quality (completeness, accuracy, transparency, and provenance).

A crucial element of data reliability is clarity on data accrual, which is considered central to ensuring the trustworthiness of RWD. The RWD should be collected in a document that pre-specifies the data variables, provides clear definitions (i.e., a data dictionary to establish a common definitional framework), standardizes reporting and delineates the relevant time windows for data element collection (i.e. a common temporal framework) [1] [2].

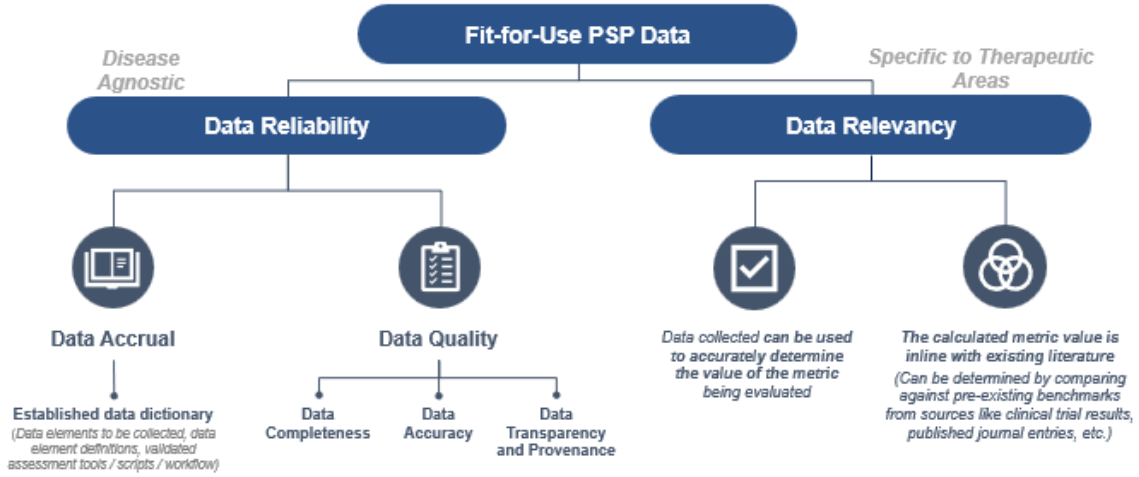

Figure 2. Schematic for assessing the fit for use of PSP data – a process consisting of two key steps, determining data reliability and data relevancy [1] [2].

B. Data Collection

When collecting data as part of the PSP, an established and detailed data dictionary will allow for a high level of data transparency and provenance. The data dictionary should outline definitions for individual data points as well as how they are sourced, collected and stored. Ensuring consistency in the level of detail captured for each data point and keeping the data dictionary up to date will help achieve high levels of data transparency. An illustrative sample of a data dictionary which includes documentation of fit-for-use data can be seen within Figure 3 below.

Data Field	Date of Data Refresh	Data Type	Predefined Value Sets	Definition / Comments	Data Collection Source(s)	Method of Data Collection	Data Completeness Checks Performed	Data Accuracy Checks Performed
Enrollment Start Date	**Date of data refresh:** YYYY-MM-DD	Numeric Date	YYYY-MM-DD	-	• **P1: Medical Records:** Healthcare Providers Collecting Patient History and Medical Care Data for PSP Administration	**M5:** Raw data manually collected and stored within digital system	-	-
Patient Coverage Type	**Date of data refresh:** YYYY-MM-DD	Pre-Defined Selection	• Compassionate • Bridging • Commercial • …	-	• **P2: Standardized Health Adjacent Records:** PSP Administration Data	**M5:** Raw data manually collected and stored within digital system	-	-
Reason for Withdrawal	**Date of data refresh:** YYYY-MM-DD	Free Text	N/A	-	• **P2: Standardized Health Adjacent Records:** PSP Administration Data	**M5:** Raw data manually collected and stored within digital system	-	-

Figure 3. Illustrative example of a data dictionary which depicts the desired level of detailed documentation.

C. Data Input Methods

There are many ways that RWD can be captured as part of a PSP with varying levels of quality associated with each of the data input methods.

Data collection sources are categorized into three tiers. The prioritization order decreases from P1 to P3 as the potential level of quality and accuracy decreases (Figure 4).

- **P1 Medical records –** Medical records are considered the highest priority of data input method. These records originate from routine medical care under the supervision of healthcare professionals [3]

- **P2 Standardized health adjacent records -** Standardized health adjacent records are the second priority of data input methods and include data which is originating from the supervision of a trained professional [3]

- **P3 Other sources -** Other sources are the lowest tier of data input method, which includes data of a wide range of sources without the supervision of any trained professional [3]

The data input method associated with a variable may influence use case results and subsequent acceptance by different stakeholders.

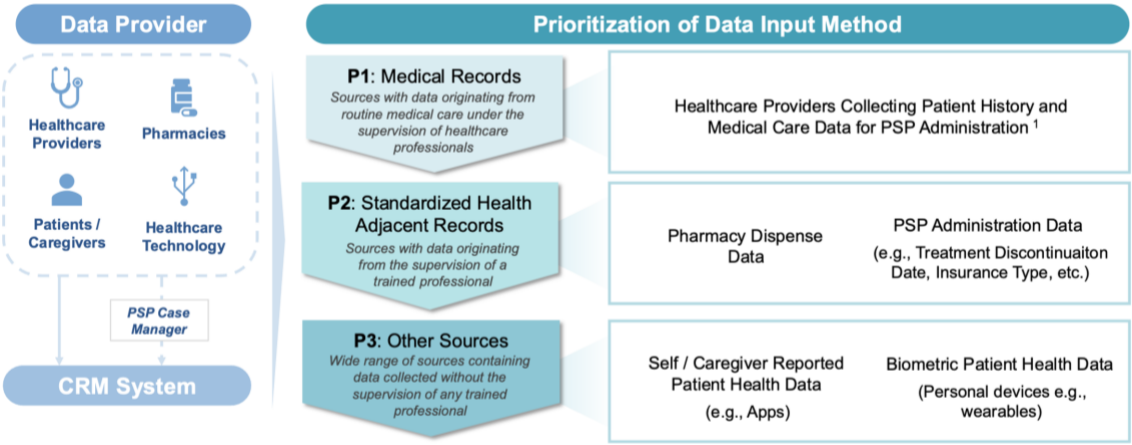

Figure 4. Health record prioritization hierarchy established to determine the quality of PSP data [3]

D. Data Collection Methods

Methods of data collection and storage can be categorized into two broader groups (Figure 5).

- Information collected directly into digital system – *Although the preferred method of data collection would be direct capture into a digital system, most PSP RWD is currently manually entered from different sources over time.*

- Manually collected information entered into a digital system

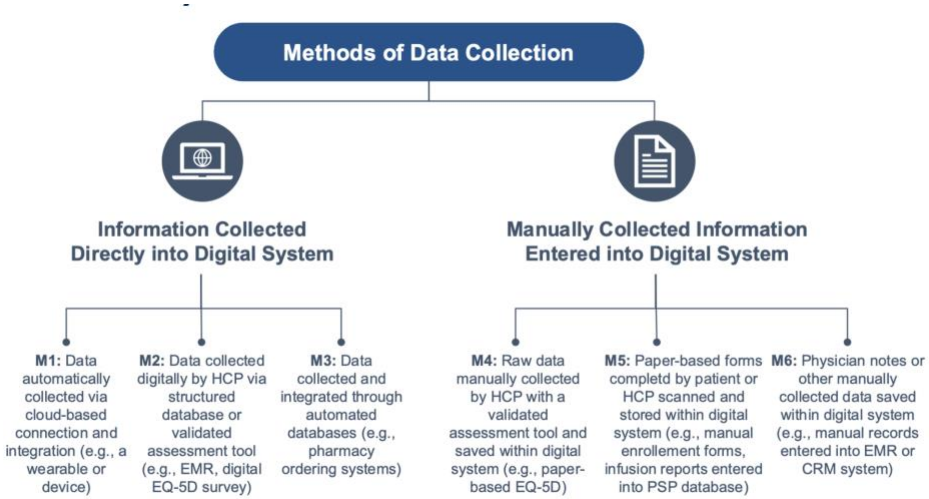

Figure 5. Prioritized methods of data collection to determine the quality of PSP data

Note: Validated assessment tools are questionnaires or methods of collecting information with established guidelines which have been leveraged for capturing patient reported outcomes in clinical studies.

E. Consent

PSPs must design specific consent language and consent processes to enable the secondary use of data for various use cases. If proper consent is not obtained upfront, then re-consenting with patients must occur before analysis can take place. Clear data governance processes should be established early to facilitate the secondary use of data.

F. Data Quality

Data quality within this framework is categorized into three main areas (Figure 6) [1] [4] [5]:

1) **Data completeness -** The level of data completeness should be evaluated at both a micro and macro level. At the micro level, an example is to consider the percent of missing data fields of each PSP record. At the macro level, an example is to consider the generalizability of the patients enrolled in the PSP to the total patient population. Understanding the completeness of the dataset is important when interpreting use case results and will enable identification of potential limitations and biases [4].

2) **Data accuracy -** Accuracy is determined by understanding the method of data collection used to capture the information as well as quality measures in place to validate the data points (e.g., data quality management framework in place, method by which refreshed data is captured, how historical data is stored, etc.) [4].

3) **Data transparency –** Documentation specifying the origin of a piece of data, outlining where it has moved from to where it is presently (if applicable), data refresh descriptions and efforts to address / mitigate source of bias are all critical for high quality data [4]

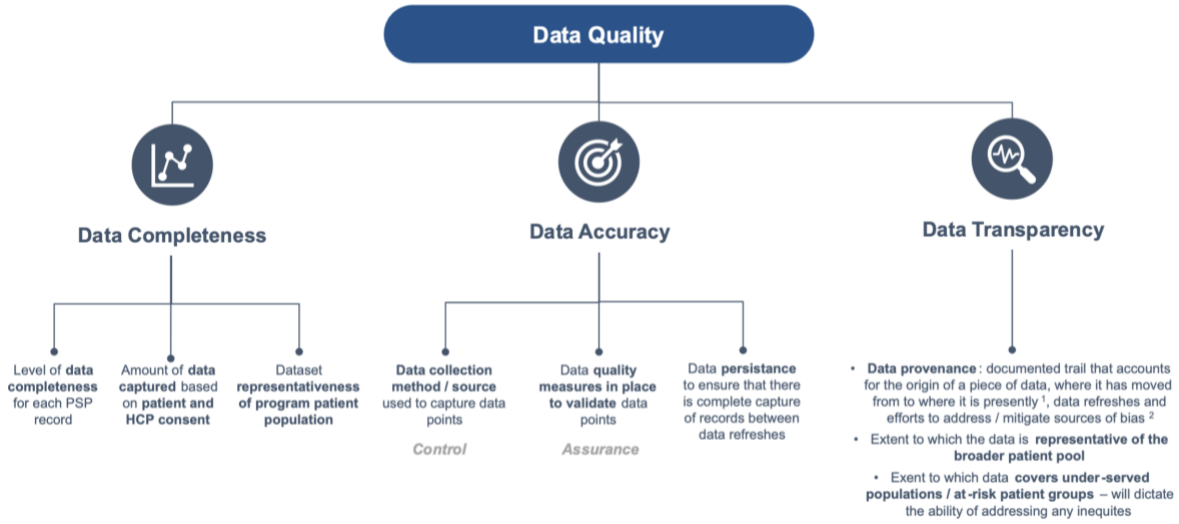

Figure 6. PSP Real World Readiness breakdown of the components of data quality [4] [6].

Next Steps

As next steps, the involved organizations will be analyzing data quality in existing PSPs. The intent of the analysis is to understand the current state of Canadian PSP data, identify common issues and provide recommendations for general improvements that might elevate the data quality and ultimately the utility of PSP data to be included in decision making.

Please contact us to understand how the CPHIN PSP Real World Readiness framework outlined can be leveraged to improve your organization’s PSP data collection.

Appendix A - Data Points

Data Type	No.	Data Point
Patient Characteristics	1	Patient Age Group (0-18, 18-29, 30-39, 40-49, 50-59, 60-69, 70+)
	2	Patient Gender
	3	Patient Sex
	4	Province
	5	Patient Diagnosis History
	6	Patient Previous Medical Treatment History
	7	Patient Concurrent Medical Treatment (if applicable)
	8	Patient Vaccination History (where applicable)
	9	Program Enrollment Date
	10	Program Re-Enrollment Date(s) (if applicable)
	11	On-Drug Start Date
	12	On Drug Start Type (patient support program, clinical trial or other)
	13	Coverage Type (compassionate, bridging, commercial)
	14	Coverage Type Start Date and End Date (if applicable)
	15	Historical Coverage Type (compassionate, bridging, commercial)
	16	Historical Coverage Type Start Date and End Date (if applicable)
	17	Disruption or Withdrawal Date (if applicable)
	18	Patient Drug Discontinuation Date (if applicable)
	19	Reason for Discontinuation (if applicable)
	20	Mortality Date (if applicable)
	21	Primary Payer Name
	22	Primary Payer Type (public, private, federal, cash, etc.)
	23	Historical Primary Payer Name(s)
	24	Historical Primary Payer Type(s) (public, private, federal, cash, etc.)
	25	Secondary Payer Name (if applicable)
	26	Secondary Payer Type (if applicable)
	27	Historical Secondary Payer Name(s)
	28	Historical Secondary Payer Type(s) (if applicable)
	29	Reimbursement Status by Payer (approved, denied, pending)

Physician Characteristics	30	HCP Name
	31	HCP Specialty
	32	Primary Hospital Affiliation
	33	Province / Territory of Primary Hospital Affiliation
Treatment Characteristics (Each Product Administration)	34	Product Administered
	35	Product Indication
	36	Dose Strength
	37	Dose Frequency
	38	Dose Quantity
	39	Mode of Administration
	40	Site of Injection, Infusion (if applicable)
	41	Infusion date (derived from post-injection / infusion reports, scheduling date within CRM)
	42	Patient Infusion Appointment History
	43	Dispense date (derived from pharmacy dispense data)
	44	Dispensed Product Quantity (derived from pharmacy dispense data)
	45	Pharmacy Name
	46	Pharmacy Location
Patient Reported Outcomes (if applicable)	47	Quality of life surveys (e.g., EQ5D, Work Productivity and Activity Impairment, Healthy Days Measure, Other validated QoL surveys, etc.)
	48	Caregiver Reported Information
	49	Biometric Data Reported from Wearable Device
Patient Interactions	50	Patient Call (e.g., adherence call) Type
	51	Patient Call Date
	52	Description or Categorization of Service (e.g., diagnostic test) Provided to Patient or Care Giver
	53	Result of Service Provided to Patient or Caregiver (e.g., test result)
	54	Date of Services Provided to Patient or Care Giver
Adverse Reaction (if applicable)	55	Type of Adverse Reaction (e.g., regular, severe, hospitalization required, etc.)
	56	Date of Adverse Reaction
	57	Additional Information on Adverse Reaction

Sources

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