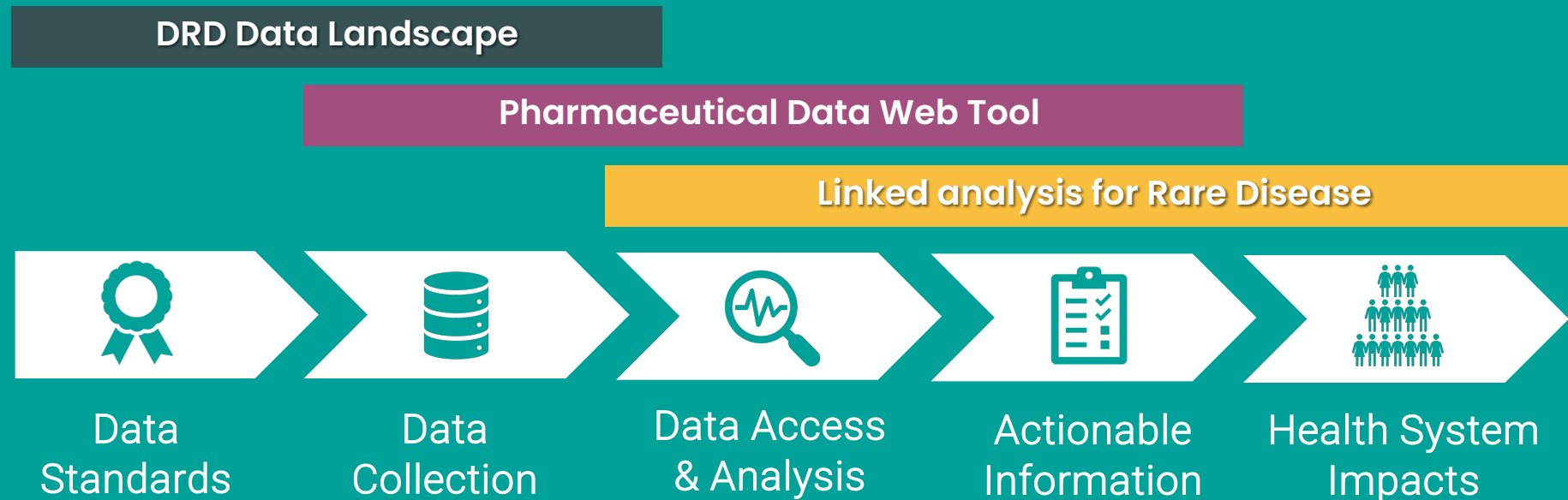


Support for the Strategy for Drugs for Rare Diseases



Drugs for Rare Diseases Data Landscape

As the number of drugs for rare disease (DRD) has increased, there is an even greater need for a strong pan-Canadian pharmaceutical data foundation to support the management of post-market drug utilization, effectiveness, and safety of drugs for rare diseases.

Existing Challenges

- **Lack of Standardization** – Canada has data assets that are not standardized and centralized for optimal health care management and decision-making
- **Small Volumes** – Given the diseases are rare, combined data is needed to effectively evaluate drug treatments
- **Inaccessible Data** – Data on drugs for rare diseases are collected in disparate systems across the country, making it difficult to provide consistent, effective and sustainable coverage while balancing spending on DRDs

CIHI's Vision for DRD

Build on the value of CIHI's data infrastructure and demonstrate how our data can be leveraged as real-world evidence for a sustainable pan-Canadian rare-disease strategy

- ✓ Identify and document what health administrative data on drugs for rare disease is available
- ✓ Identify where advancements in data collection can be made more quickly for drugs for rare diseases
- ✓ Leverage data to support decision making

Pharmaceuticals Data and Information Roadmap

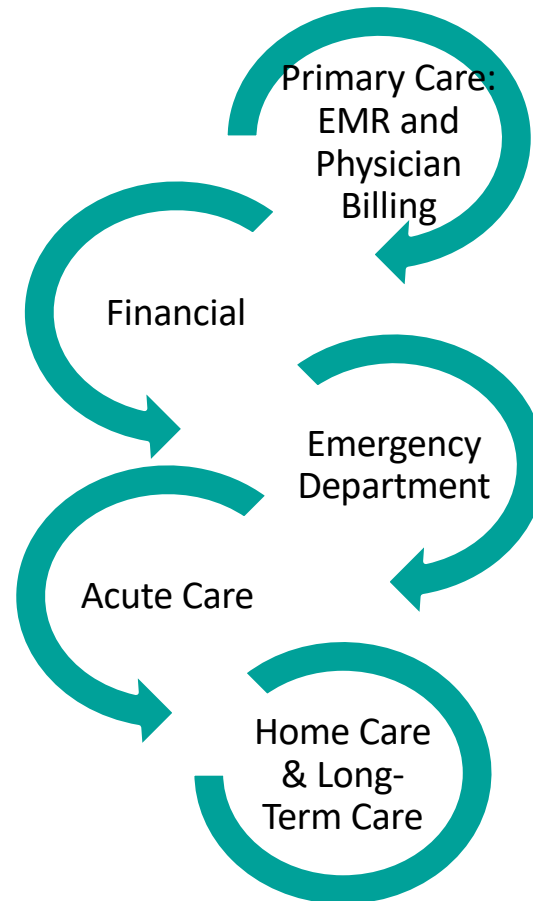
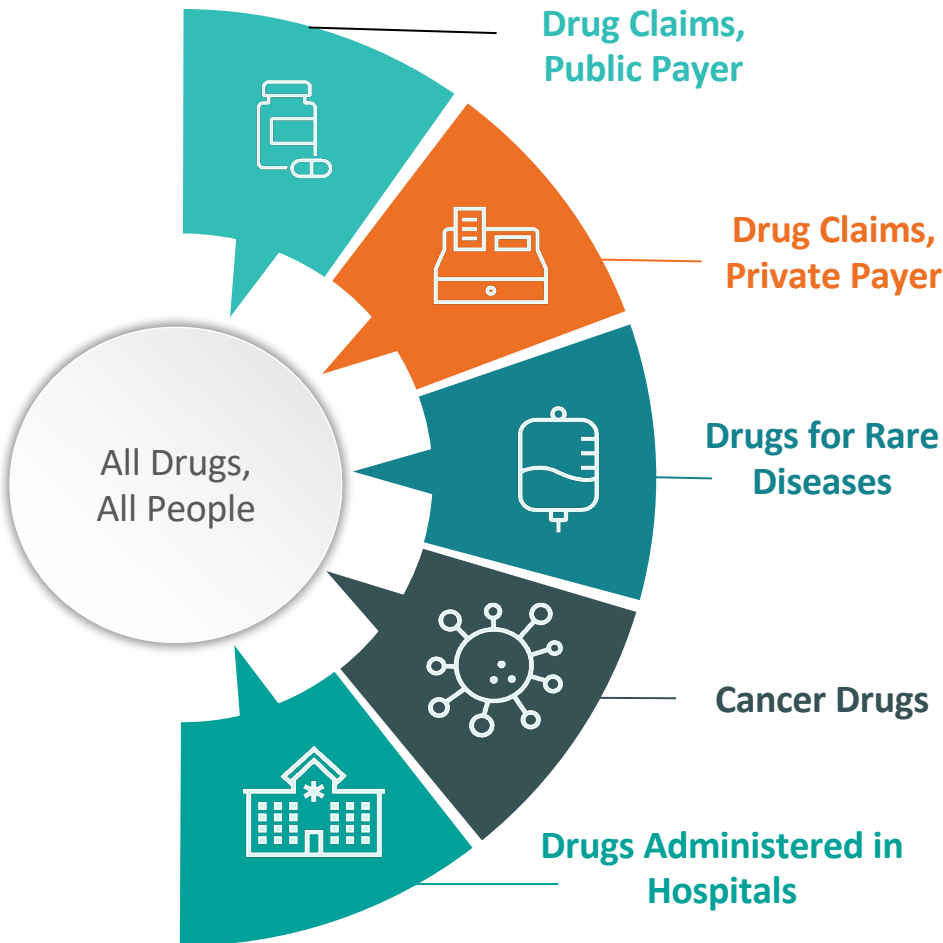
Develop an “All Drugs, All People”
Data Landscape



Enhance Linkage to
Existing Health Admin Data
at CIHI



Optimize DRD Management in
Canada



Appropriateness,
effectiveness, &
outcomes



Cost and
budget
management



Payment
models



Equitable access

Our Pharmaceuticals Roadmap in Action – DRD

CIHI's work on drugs for rare diseases (DRDs) aligns with each of the goals in our Pharmaceuticals Roadmap and is an example of the value of the pan-Canadian Prescription Drug Data and Analytics Initiative.

Pharmaceuticals Roadmap Goals

DRD Activities in Progress



Re-develop National Prescription Drug Utilization Information System (NPDUIS) system and standards to accommodate new data feeds



- Prescription Drug Data Landscape Report identifies DRD data across Canada for potential inclusion in NPDUIS



Acquire additional data to achieve a pan-Canadian “All Drugs, All People” pharmaceutical data holding



- Acquire additional DRD data to expand what is already captured in NPDUIS



Update and expand access to pharmaceuticals data



- Develop a Pharmaceutical Data Web Tool to make information on drug plans and formularies covering DRDs across Canada more accessible



Enhance pharmaceutical data analysis



Conduct linked analyses:

- Baseline analysis using CIHI's existing linked health administrative data
- Feasibility assessment of the linking registry data to CIHI administrative data

Projects Underway: Foundational Data & Tools

Foundational work to establish CIHI's role in supporting the National Strategy, explore data availability and make existing information more accessible



CIHI's DRD Projects Underway

1

DRD Webpage

CIHI webpage to speak to CIHI's support of the Strategy and highlight project outputs. *Target July 2024.*

2

DRD Analysis Selection Criteria

Criteria to support the identification and prioritization of rare diseases and DRDs for analysis in alignment with PT priority information needs. *Complete.*

3

Data Availability and Suitability

Summary of findings on CIHI data availability and suitability to support analysis on specific DRDs and rare diseases. *Target Summer 2024.*

4

pan-Canadian Prescription Drug Data Landscape Report

Report summarizing pharmaceutical drug data availability across Canada, with a focus on drugs for rare diseases. *Target March 2024.*

5

Pharmaceutical Data Web Tool

Interactive tool on drug plan information and formularies to support evaluation of consistency in coverage for DRDs across Canada. *Target March 2024.*

Data Landscape for Drugs for Rare Diseases – Description

- Data on drugs for rare diseases (DRDs) are collected in disparate systems across the country.
- The pan-Canadian Prescription Drug Data Landscape Report provides a picture of existing DRD data:
 - Documenting what health administrative data on DRDs is available.
 - Identifying where advancements in data collection can be made for DRDs.
 - Identifying where standardization in data collection may be needed.
- Supports forward planning for collecting and using data and evidence for DRDs to unlock the potential of this information for decision-makers and patients.



Benefit

- More comprehensive, accessible data to support RWE generation that can inform formulary listing decisions to improve access to drugs for patients.

Pharmaceutical Data Web Tool – Description

- An interactive tool that supports querying of public drug plan coverage and formularies (drugs that are covered in a province), as well as some high-level contextual information about what is in NPDUIS.
- Allows comparisons of PT formularies, including public coverage of drugs for rare diseases across Canada.
- Improves access to information on public drug plans and formularies with the goal of enabling more consistent access to DRD for people across Canada.



Benefit

- Accessible information on drug plans and formularies provides contextual information needed to understand differences in drug utilization and achieve more consistent access to DRD across jurisdictions.

Projects Underway: Rare Disease Focused Analysis

Examples of how CIHI data can be leveraged for real-world evidence in the rare diseases



CIHI's DRD Projects Underway

1

NPDUIS DRD Analysis

Analysis to identify and document DRDs available in NPDUIS. *Target Fall 2024.*

2

Baseline Analysis: Cystic Fibrosis

Analysis on hospital and emergency department use for patients on Trikafta. *Target Fall 2024.*

3

Baseline Analysis: Huntington's Disease

Portrait of care analysis for Huntington's Disease patients ahead of disease-specific drug treatment. Focus on clinic, long-term care and drug data. *Target Fall 2024.*

4

Registry Linkage Feasibility

Feasibility assessment to explore if and how registry data can be linked to CIHI administrative databases for real-world evidence. *Target Fall 2024.*

Linked Analysis for Rare Disease – Description

- Focused analyses linking multiple data sources on specific rare diseases and/or DRDs, prioritized to align with provincial stakeholder needs to achieve greater insights on:
 - the natural history of rare disease,
 - effectiveness of various treatment options,
 - factors that influence a patient’s care journey, and
 - a range of individual and system-level outcomes.

- Two streams:
 - Baseline – Analysis using CIHI’s existing linked health administrative data
 - Registry Linkage Feasibility – Explore feasibility and value of linking registry data to CIHI administrative data



Benefit

- Leverages real-world evidence to answer system questions about the use, safety, and effectiveness of DRDs to inform funding decisions with a goal to improve patient access.



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