RWE Workshop

March 31, 2023

CONFIDENTIAL

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Objectives

1. Introduction to RWD
2. Overview of Canadian RWD sources
   - Pros and cons for industry-sponsored studies
3. Process and requirements for accessing data
4. Latest trends
   - Outcomes-based agreements
   - CADTH and INESSS guidance
   - Global (international) applications
5. Case study examples
6. Question & answer period

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Introduction to RWD

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What is Real-World Data?

- **Real-World Data:** "Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources."[1]
- Also typically defined as data arising from outside of controlled clinical trials

"Let's shrink Big Data into Small Data … and hope it magically becomes Great Data."

[1]https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

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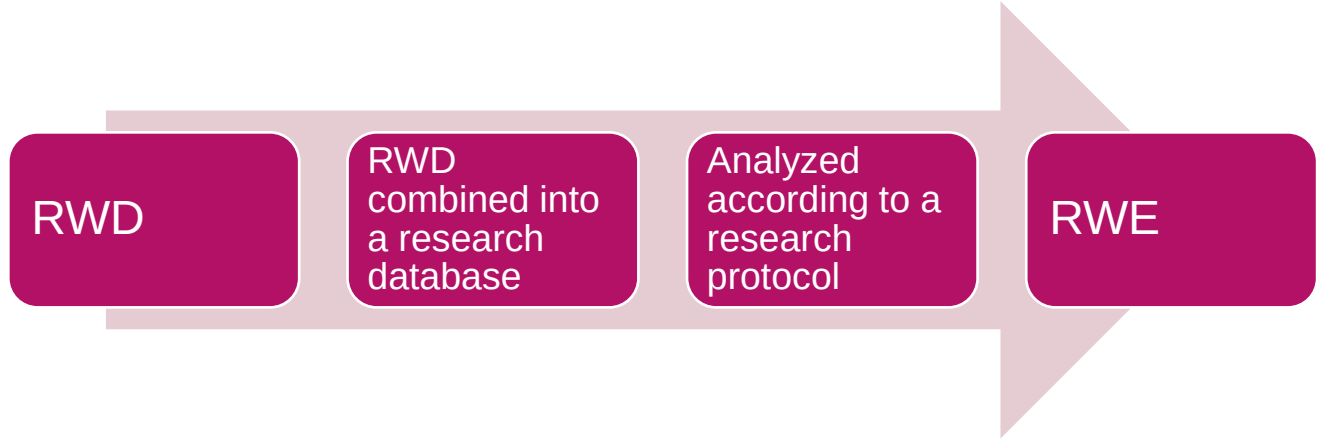
What is Real-World Evidence?

- **Real-World Evidence:** “Real-world evidence is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD.” [1]
- Insights from RWE represent a logical complement to clinical trial/randomized controlled trial (RCT) evidence and offers numerous benefits



[1]https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

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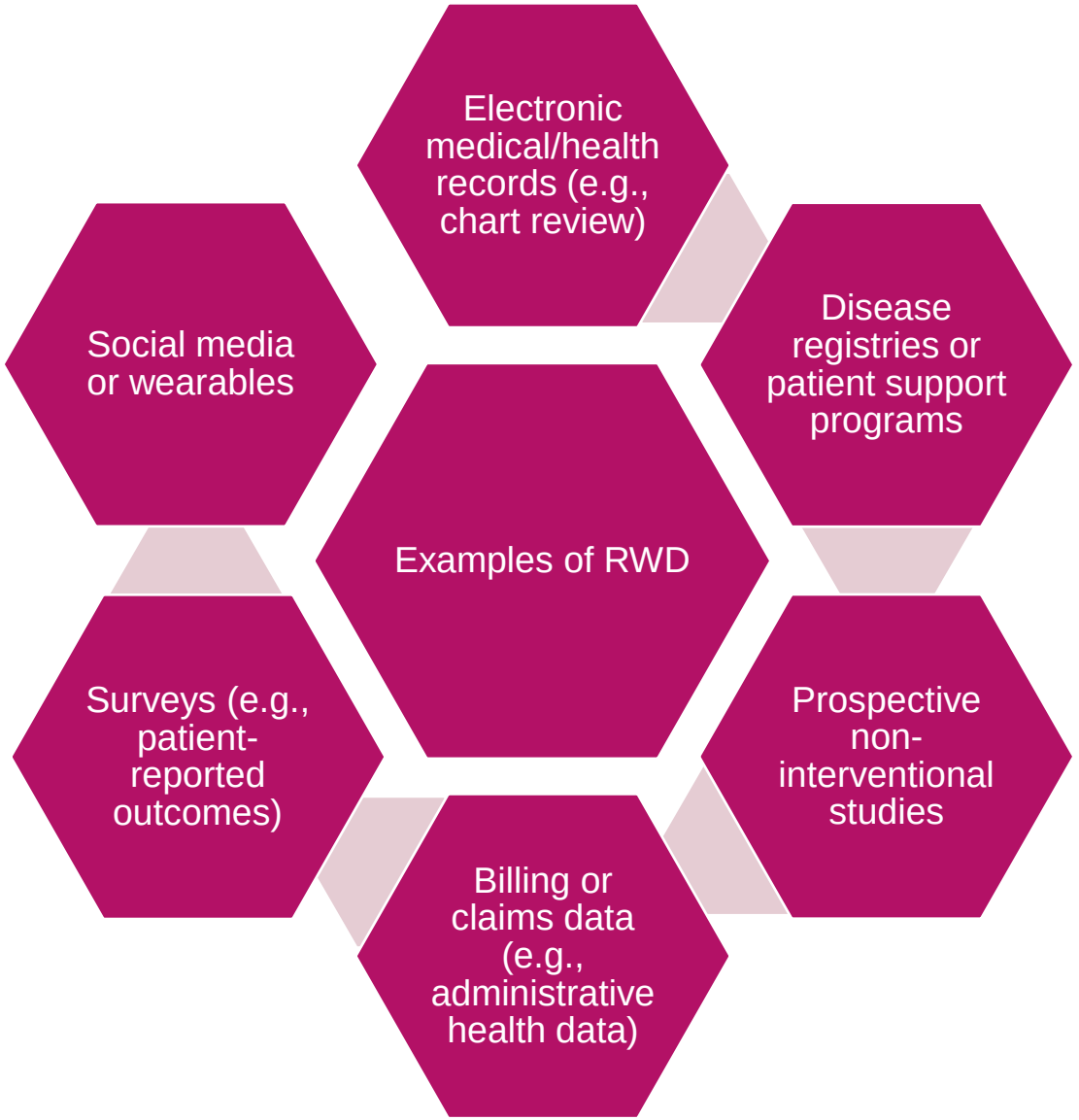
Real-World Data Sources



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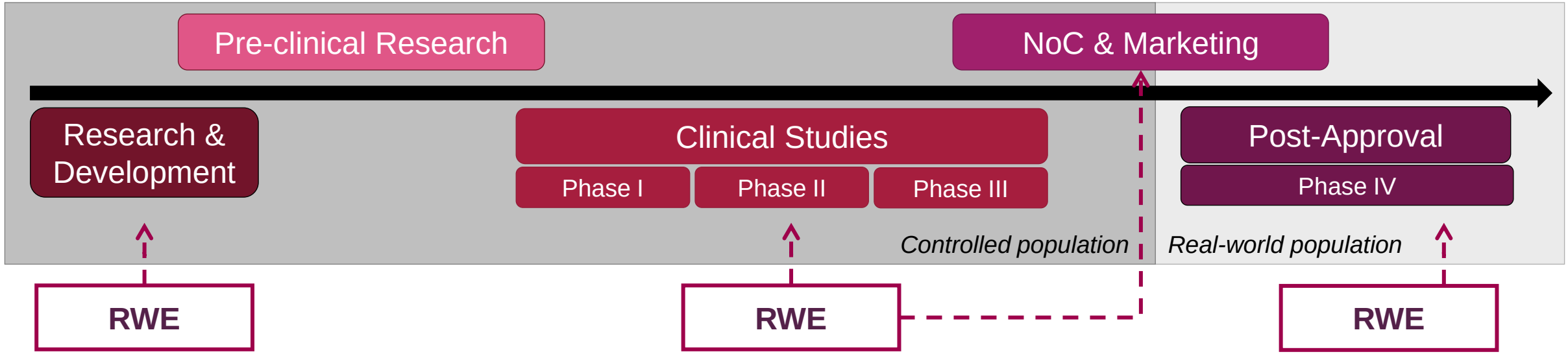
How Does RWE Differ from Clinical Trials?

- While RCTs are still the gold-standard for safety and efficacy, RWE provides evidence reflecting real-life treatment and disease management

RCT	RWE
➢ Controlled population (similar characteristics)	➢ Diverse population (reflective of the real patient population)
➢ Shorter follow-up period	➢ Long-term follow-up period
➢ Limited sample size	➢ Larger sample size
➢ Comparator treatment is limited	➢ All available comparator treatments
➢ Time-consuming data collection	➢ Time-efficient data collection

RWE Research Opportunities

RWE can provide insights for a wide range of research questions:



- Burden of Disease
- Unmet need
- Quality of life
- Treatment landscape

- Inform trial design
- Synthetic control arms
- Cost-effectiveness
- Budget impact
- Value proposition
- Place in therapy
- Inform regulatory approval & HTA strategy

- Real-world safety and effectiveness
- Treatment patterns
- Resource use and costs
- Quality of life
- Patient and physician preferences
- Relative effectiveness

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Regulatory and Reimbursement

- RWE can support decision-making where there is clinical trial uncertainty
  - ✓ Understand generalizability of trial data to Canadian population
  - ✓ Provide data from older patients with comorbidities
  - ✓ Provide data on populations receiving treatment in 2nd or 3rd line
  - ✓ Provide comparison data for standard of care locally (including surrogate arms)
  - ✓ Provide data associating trial end points to end points of interest to decision makers

"Say 'eh.'"

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Regulatory and Reimbursement

- Canadian regulatory & reimbursement agencies accepting RWE (in select situations)
  - ✓ Incidence and Prevalence
  - ✓ Treatment patterns (including adherence)
  - ✓ Comparative effectiveness research
  - ✓ Cost-effectiveness

Health Canada Santé Canada

CADTH Evidence Driven.

Institut national d'excellence en santé et en services sociaux Québec

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Regulatory and Reimbursement

➢ Public payers

- ✓ interest in phase IV trial data to address any trial uncertainty
- ✓ Interest in conditional access to mitigate risks

➢ Private payers

- ✓ interest in employment data (absenteeism/presenteeism)

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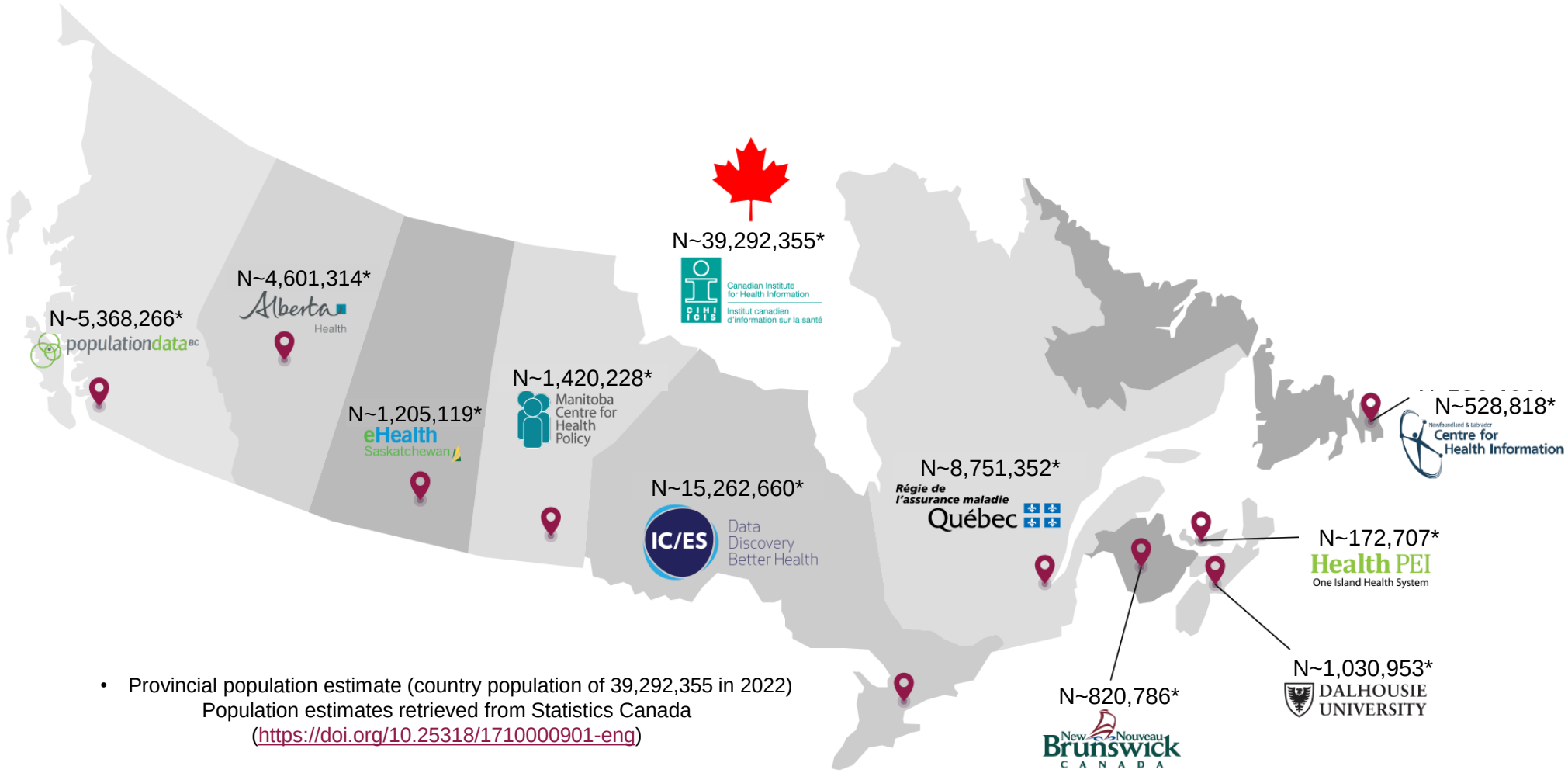
Overview of Canadian RWD Sources

Canadian Administrative Health Data Sources



- Provincial population estimate (country population of 39,292,355 in 2022)
  Population estimates retrieved from Statistics Canada
  (https://doi.org/10.25318/1710000901-eng)

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Canadian Provincial RWD Overview

Province	Health Services			Pharmaceutical		Public Health Insurance	Vital Statistics	Long-term/ Home Care	Provincial Laboratory Data
	Inpatient	Ambulatory Care	Physician Claims	Pharmacy-level	Public plan only				
CIHI (pan-Canadian)	✓	✓	Varies by province		✓	✓	✓	Varies by province	
British Columbia	✓	✓	-	✓	✓	✓	✓	✓	-
Alberta	✓	✓	✓	✓	✓	✓	✓	✓	✓
Saskatchewan	✓	✓	✓	✓	✓	-	✓	✓	-
Manitoba	✓	✓	✓	✓	-	✓	✓	✓	✓
Ontario	✓	✓	✓	-	✓	✓	✓	✓	✓ (80%)
Quebec	✓	✓	✓	-	✓	✓	✓	-	-
New Brunswick	✓	-	✓	Unclear	✓	-	✓	Forthcoming	-
Nova Scotia	✓	✓	✓	-	✓	✓	✓	-	-
Prince Edward Island	✓	✓	✓	✓	-	-	✓	✓	✓
Newfoundland & Labrador	Information not available								

Note: Data availability for research may vary across jurisdiction, data holdings and data custodians/owners.

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Real-World Data in Canada | CIHI

Why CIHI?

- ✓ Comparable, pan-Canadian RWD and information on the health care system
- ✓ Approximately 37 million individuals
- ✓ Data holdings covering pharmaceutical, hospital, community and specialized care
- ✓ Aggregated information on health system costs and health work force
- ✓ Ethics review not required

Canadian Institute for Health Information
Better data. Better decisions. Healthier Canadians.

CIHI Data†

- Hospital data
  - ✓ Acute inpatient *1994*
  - ✓ Day Surgery *1994*
  - ✓ Emergency Department† *2001*
  - ✓ Outpatient clinics *2001*
  - ✓ Rehab inpatient *2000*
  - ✓ Extended stay *2003*
- Community care
  - ✓ Residential care *2003*
  - ✓ Home care *2006*
- Specialized care
  - ✓ Dialysis & transplant *1981*
  - ✓ Joint replacement *2018*
  - ✓ Multiple sclerosis *2012-2016*
- Public Drug claims data *2002-2008*
- Health workforce *1980-2012*
- Public spending *from 1995*
  - ✓ Financial information
    - Public hospitals
    - Health authorities

† *Limited provinces*

Real-World Data in Canada | Statistics Canada

Why Statistics Canada?

- ✓ National statistical office
- ✓ Collect and tabulate information on Canada's economy, society and environment
- ✓ Connect administrative health data to:
  - cause of death
  - quality of life
  - workforce participation
  - productivity

Research Data Centre

- Canadian Community Health Survey (CCHS)
  - ✓ Administrative Health Datasets
  - ✓ Vital Statistics Death Data
  - ✓ Canadian Revenue Agency Tax Returns
- Canadian Vital Statistics Death Data
  - ✓ CCHS
  - ✓ Administrative Health Datasets

Statistics Canada Statistique Canada

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Administrative Data in Ontario

In partnership with the Ontario Ministry of Health (MOH) or the ICES Research Institute

Real-World Data in Canada | Ontario

Why Ontario?

- ✓ Largest province in Canada
- ✓ Electronic medical records available for research for 14 million individuals
- ✓ Health service data available from 1990s
- ✓ Public plan pharmacy claims data
- ✓ Lab services data, covering about 80% of all available lab tests
- ✓ ICES holds linked health administrative data as well as other datasets, including EMRs, and non-health datasets

IC/ES Data Discovery Better Health

Ontario
Ministry of Health
Ministry of Long-Term Care

Administrative Data in Ontario

- Ontario Key Data Sources:

1. **Ontario Health Insurance Plan (OHIP)**
   - Public funding through OHIP covers services such as doctor visits, hospitalizations, ambulance, inpatient medications, and lab services
2. **Ontario Drug Benefit (ODB) claims**
   - Public plan drug claims data for patients 65+ years of age, covers some low income and special drug funding programs, does not include private drug plans or out-of-pocket claims
3. **Lab data**
   - Comprehensive from 2012 onwards; a subset are available for research

- Data access provided through ICES

Ontario Data Linkages

IC/ES Data Discovery Better Health

Ontario
Ministry of Health
Ministry of Long-Term Care

Health Services Data

- Hospitalizations
- Emergency department visits
- Physician claims
- Dialysis and cancer clinic visits
- Inpatient mental health
- Diagnosis and procedure codes
- Length of stay

Lab Data (80% of tests)

- Test name, date *from 1991*
- Test results *from 2007*

Drug Data

- Public plan claims only
  - age 65+
  - Claiming social assistance
  - special drug access programs
- Drug names, Drug identification number (DIN) – level data (e.g. dosage)
- Medication Possession Ratio
- Proportion of Days Covered

Ontario Cancer Registry

- Patient demographics
- Tumour information
  - Site and stage at diagnosis
  - Topography and morphology
- Cancer treatment

Vital Statistics

- Deaths
- Birth registry
- Gender and geographic Information

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Administrative Data in Alberta

In partnership with the Alberta Health (AH) and Alberta Health Services (AHS)

Real-World Data in Canada | Alberta

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Why Alberta?

- ✓ Province-wide health authority since 2008
- ✓ Largest fully-integrated health system in Canada
- ✓ Combination of 9 regional health authorities and 3 agencies
- ✓ Electronic medical records available for research for 4.4 million individuals
- ✓ Pharmacy claims data (regardless of payer)
- ✓ Lab services data including cytogenetic and biomarker test results

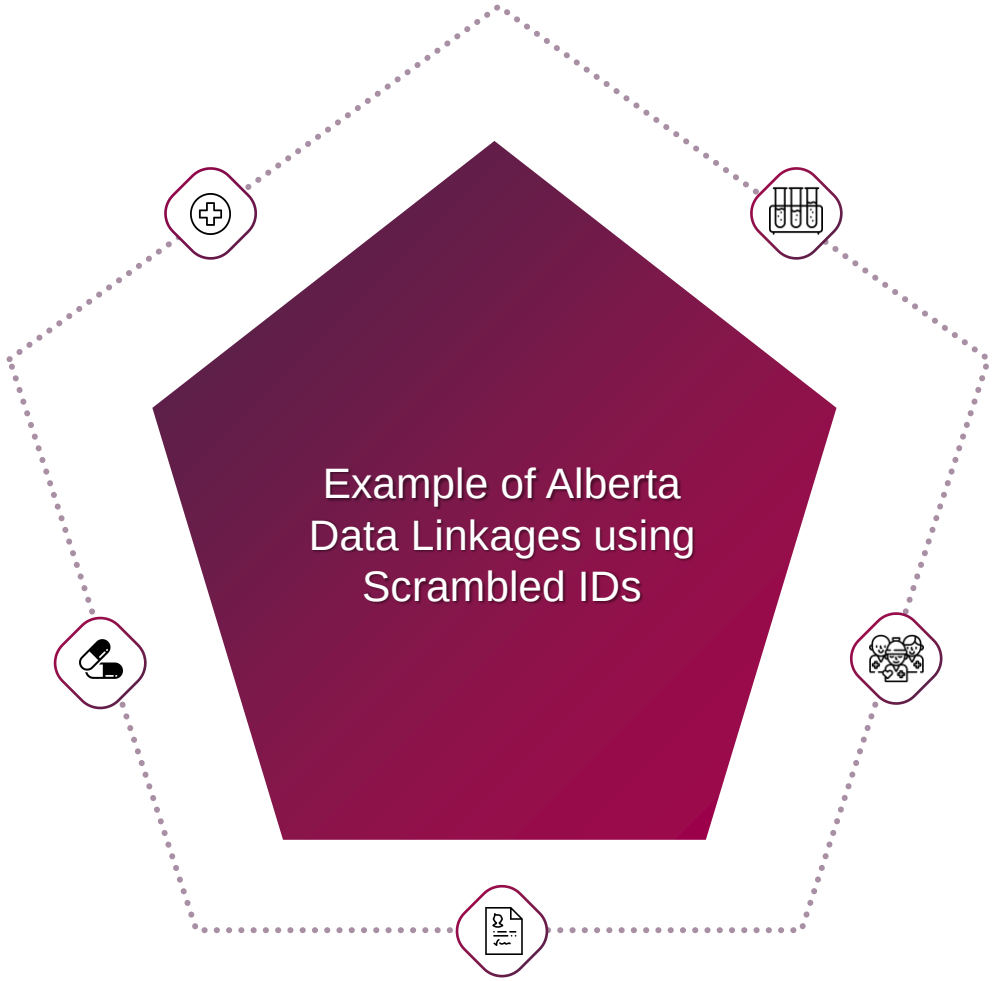
Alberta Health Services

Alberta Health

Alberta Data Linkages

Health Services Data

- ✓ Hospitalizations
- ✓ Ambulatory care visits
- ✓ Physician claims
- ✓ Diagnosis and procedure codes
- ✓ Length of stay



Lab Data

- ✓ Test name, date, results
- ✓ Abnormal diagnosis
- ✓ Reason for test
- ✓ IHC/cytopathology

Drug Data

- ✓ Private and public plan claims
- ✓ Drug names
- ✓ Medication Possession Ratio
- ✓ Proportion of Days Covered
- ✓ Gaps in treatment
- ✓ Treatment switching
- ✓ Concomitant medication use

Alberta Cancer Registry

- ✓ Patient demographics
- ✓ Tumour information
  - ○ Site and stage at diagnosis
  - ○ Topography and morphology
- ✓ Initial cancer treatment

Vital Statistics

- ✓ Births/Deaths
- ✓ Marriage
- ✓ Gender and geographic Information

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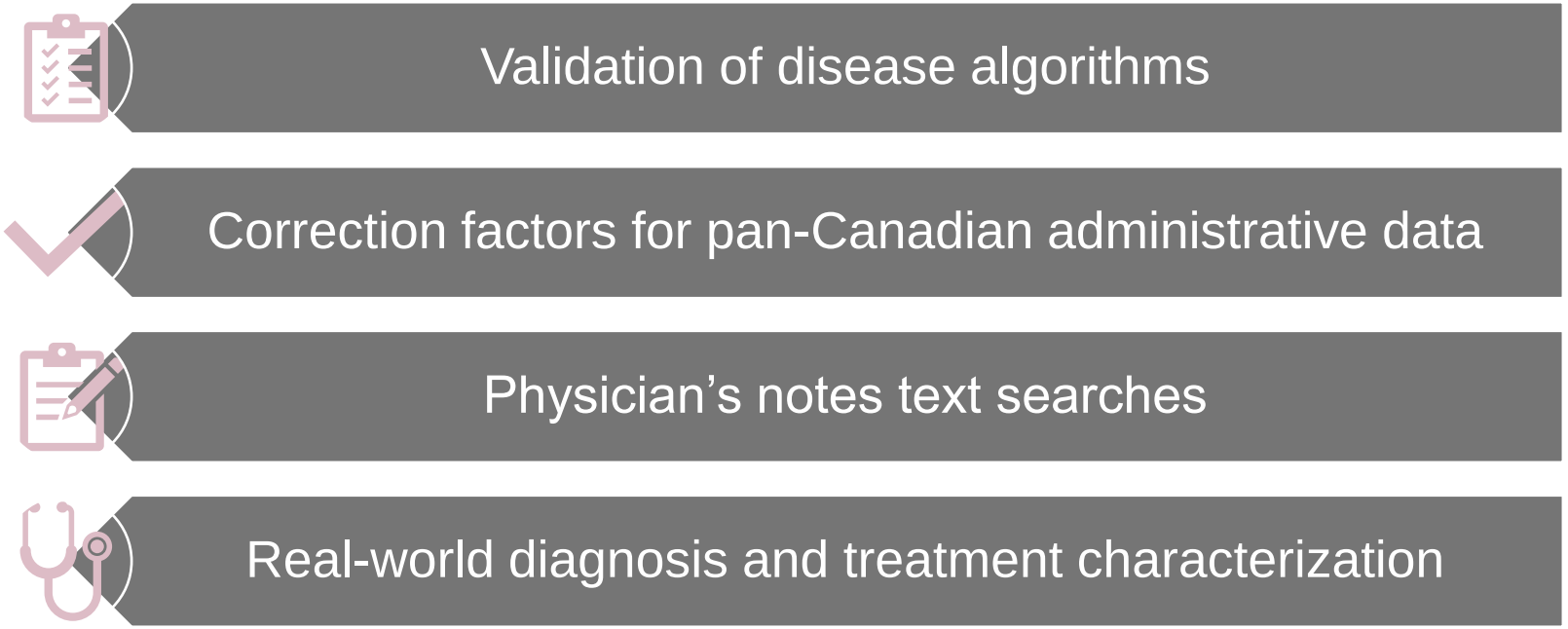
Other Data Sources

Administrative Data is NOT collected for Health Research

Real-World Data in Canada | Electronic Medical Records (EMRs)

Structured & unstructured data available for sub-provincial populations



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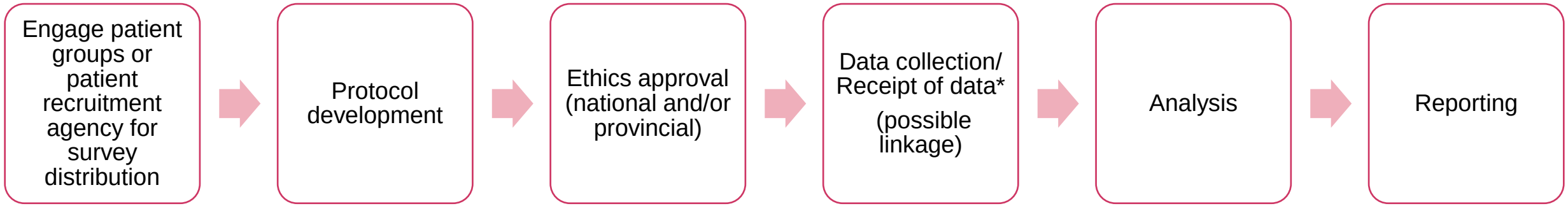
Prospective or Cross-sectional Studies

- Studies on disease/treatment burden can complement results of health system data studies
- Objectives: to understand disease and treatment impact on patients, caregivers, and providers
- Outcomes:
  - Quality of life
  - Disease characteristics
  - Disease and treatment impacts
  - Drivers of treatment patterns
  - Evidence of an unmet medical need
  - Treatment preferences
  - Utility Elicitation
  - Estimate market share of emerging therapies
  - Direct and non-direct costs incurred by patients/families
  - Identify themes to inform survey development



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PSP Data Opportunities

➢ Opportunities for PSPs

❖ Fulfil an unmet need in patient data collection

- ✓ Establish research framework with robust methodology (publish protocol)
- ✓ Engage stakeholders and KOLs for data validation and clinical interpretation
- ✓ New PSP/registries establish research frameworks (e.g. Multiple Myeloma Network)
- ✓ Existing PSP/registries initiate research frameworks and process for patients to opt-in

❖ Collaboration with health system

- ✓ Link to administrative data and examine impact of patient-reported outcomes on utilization
- ✓ Extrapolate provincial-level data to national level by using PSP data
  - ❑ Both rely on health system to link data and patient consent to release data
  - ❑ Limitations in health system resources and capacity – priority projects

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Process and Requirements for Accessing Data

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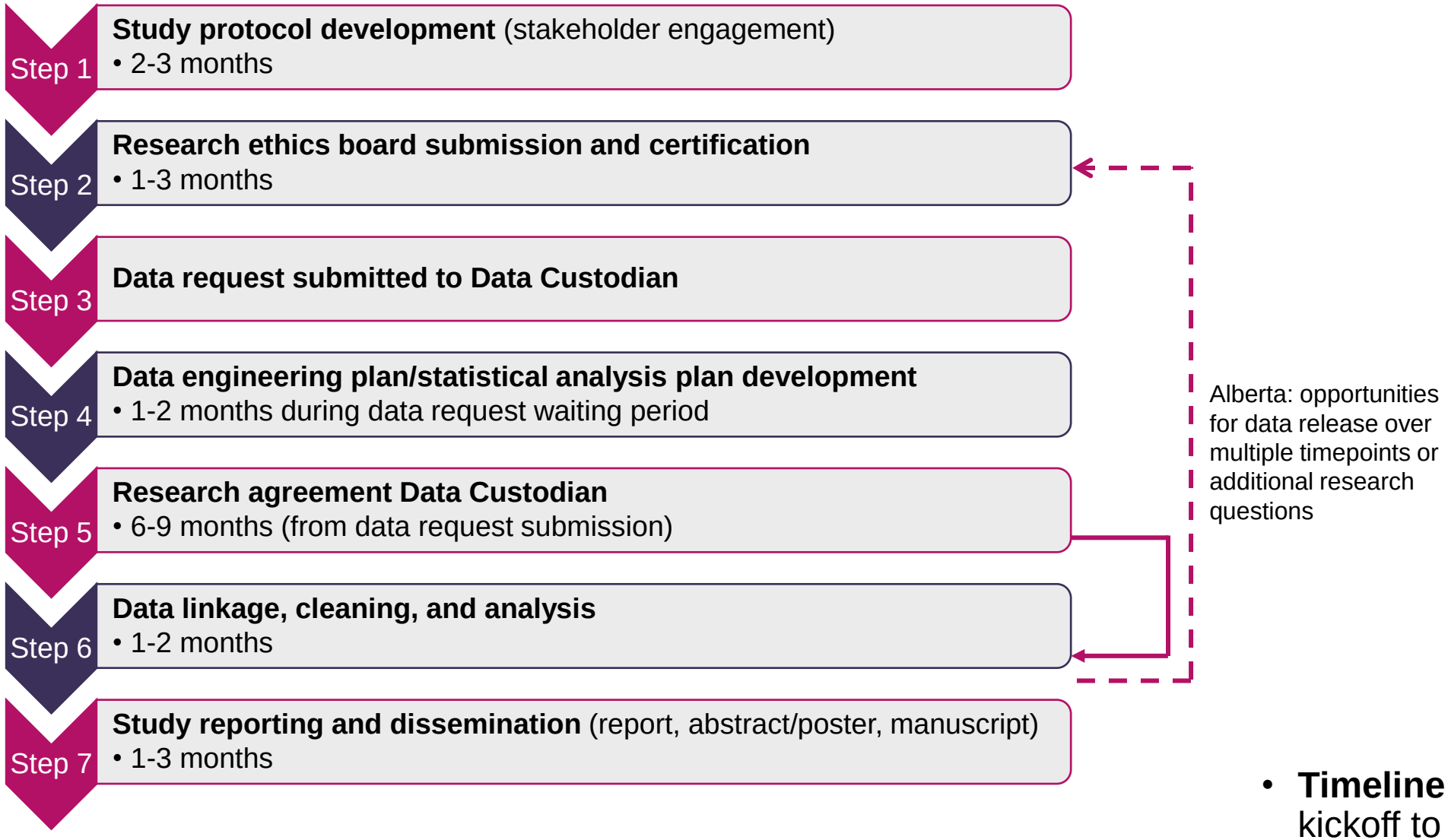
Typical Process/ Timelines



- **Timeline:** 12-18 months from kickoff to reporting

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Latest Trends

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The Case for OBAs

Ideal scenario for payers:

1. High price
2. High level of uncertainty
3. Risk of delayed access (limited evidence to support)

E.g., gene therapies, rare diseases

"high promise, high cost"

Not ideal scenario for payers:

1. Lots of competitors
2. Inferior new product
3. Evidence is well established
4. Outcomes are hard to measure
5. Data to measure is not high-quality

Role for Real World Evidence:

- ✓ Outcomes that are meaningful and measurable
- ✓ Standardized data to pool or compare across jurisdictions
- ✓ Quality of data (validated)
- ✓ Patient-focused
- ✓ Establish and agree surrogate outcomes (short-term)

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What is an outcomes-based agreement?

An OBA is a market access agreement between a 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, as demonstrated by real-world evidence.

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OBAs in Canada: Current State

Why, when, and how?

Patient access to drugs is becoming increasingly challenging in Canada.

With the number of promising therapies with imperfect data increasing, particularly in rare disease and precision oncology, coupled with long reimbursement timelines, timely access for patients to novel therapies has become increasingly challenging.

Why do manufacturers and payers consider using OBAs?

OBAs are a potential solution to support timely market access for patients while mitigating high clinical and/or economic uncertainties associated with a therapy. Unlike some countries, Canada does not have a formalized managed access programs to help manage these types of therapies, so in some cases payers and manufacturers turn to innovative market access agreements, such as outcomes-based agreements.

When should OBAs be used?

OBAs are not appropriate for all drugs. When possible, simple market access agreements are preferrable. OBAs can be used when promising therapies have uncertainties due to limited evidence.

Is there RWD/E available to support OBAs?

The situation for each drug under consideration for an OBA will be unique, and the RWD used to support each OBA will need to be adapted to each unique situation. While national RWD/E solutions are under development, there are interim health outcomes data sources and RWD infrastructure already in place in Canada that could be used to support OBAs. Examples include administrative health data sets, prior-authorization data, and patient support program data.

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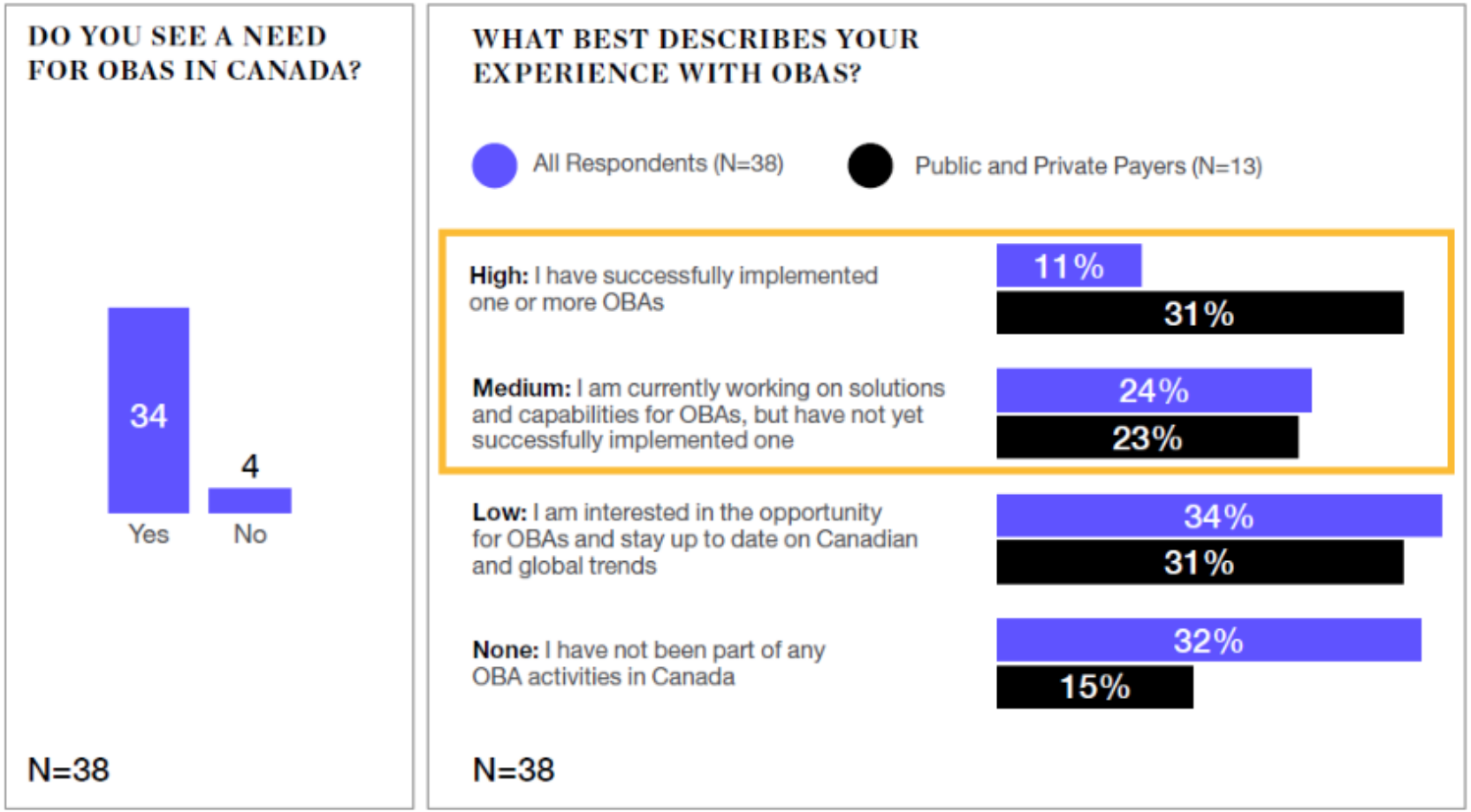
OBAs in Canada: Current State

How many OBAs are there?



Source: Canadian Outcomes-Based Agreements Experience and Perceptions: Survey Results, RWE & OBA Working Group, October 2021. https://bit.ly/3JhICRr

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RWD for OBAs: Current State

Is there RWD available to support OBAs?

Are there RWD sources ready to use, as-is, for OBAs? No.

Are there RWD sources with the potential to use for OBAs? Yes.

RWD solutions for OBAs will not be one-size-fits-all.

Administrative health data sets

Prior-authorization data

Patient support program data

The situation for each drug under consideration for an OBA will be unique, and the RWD used to support each OBA will need to be adapted to each unique situation. While national RWD/E solutions are under development, there are interim health outcomes data sources and RWD infrastructure already in place in Canada that could be used to support OBAs.

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Case Study Examples

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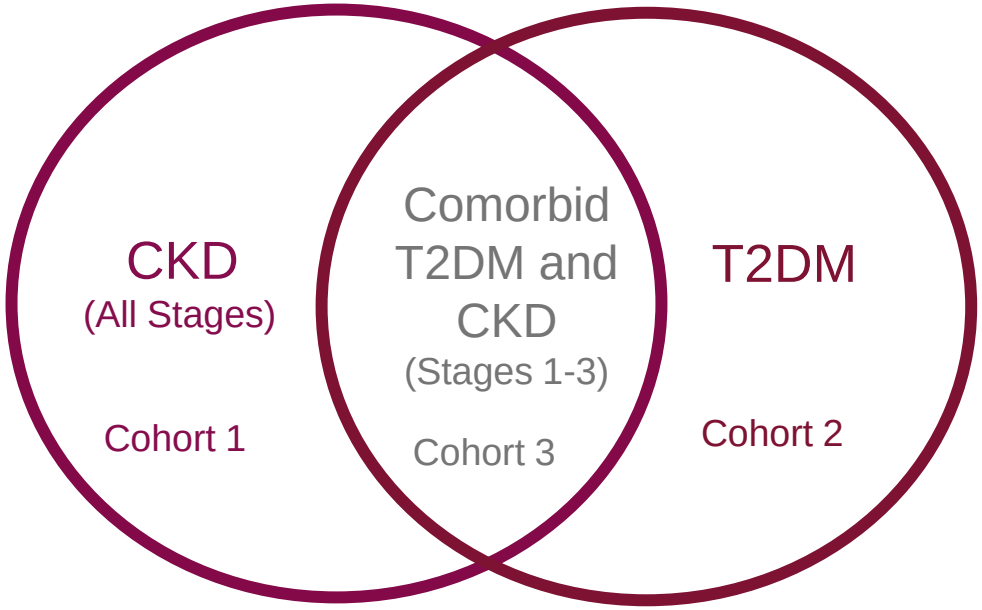
Current Medlior Project | Burden of Chronic Kidney Disease (CKD) and Type 2 Diabetes Mellitus (T2DM)

Outcomes (among 3 study cohorts):

- Epidemiologic characteristics: annual and period incidence and prevalence
- Demographic and clinical characteristics: demographics, complications/comorbidities, treatment patterns, CKD progression
- HRU: ambulatory care visits, physician claims, hospitalizations, prescription medication dispenses



What makes this study unique?

- **Early-stage** CKD is often present before patients are captured using ICD diagnosis codes
  - This study utilized **laboratory data** to define the study cohort, in addition to diagnosis codes to identify early-stage CKD
  - Our finding showcase the extensive province-wide laboratory available for research
- A comprehensive list of complications was examined in each population (cardiovascular, diabetic, renal, other outcomes), which has not been previously conducted in the literature

Protocol published on clinicaltrials.gov and EU PAS Register

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Current Medlior Project | Hematological Oncology*

Objectives:

1. Evaluate treatment patterns and overall survival
2. Estimate market share for novel therapy (to inform budget impact model)
3. Estimate HCRU and costs

Cancer Care Alberta | CANCER RESEARCH & ANALYTICS

What makes this study unique?

- This is a **rare** disease without specific ICD-9 codes to identify the cohort.
  - **Alberta Cancer Registry** will be used to identify the patient cohort based on morphology and topography codes.
- Project began with an **advisory board** comprised of physicians and Canadian payers
  - Trial endpoints do not include overall survival, which is what payers have expressed being a key area of uncertainty
  - Ad Board gave recommendations for where Canadian RWE would best fill current gaps in evidence or areas of uncertainty from the payer perspective

*Specific disease area cannot be specified as this a current study and the protocol has not be published at this time.

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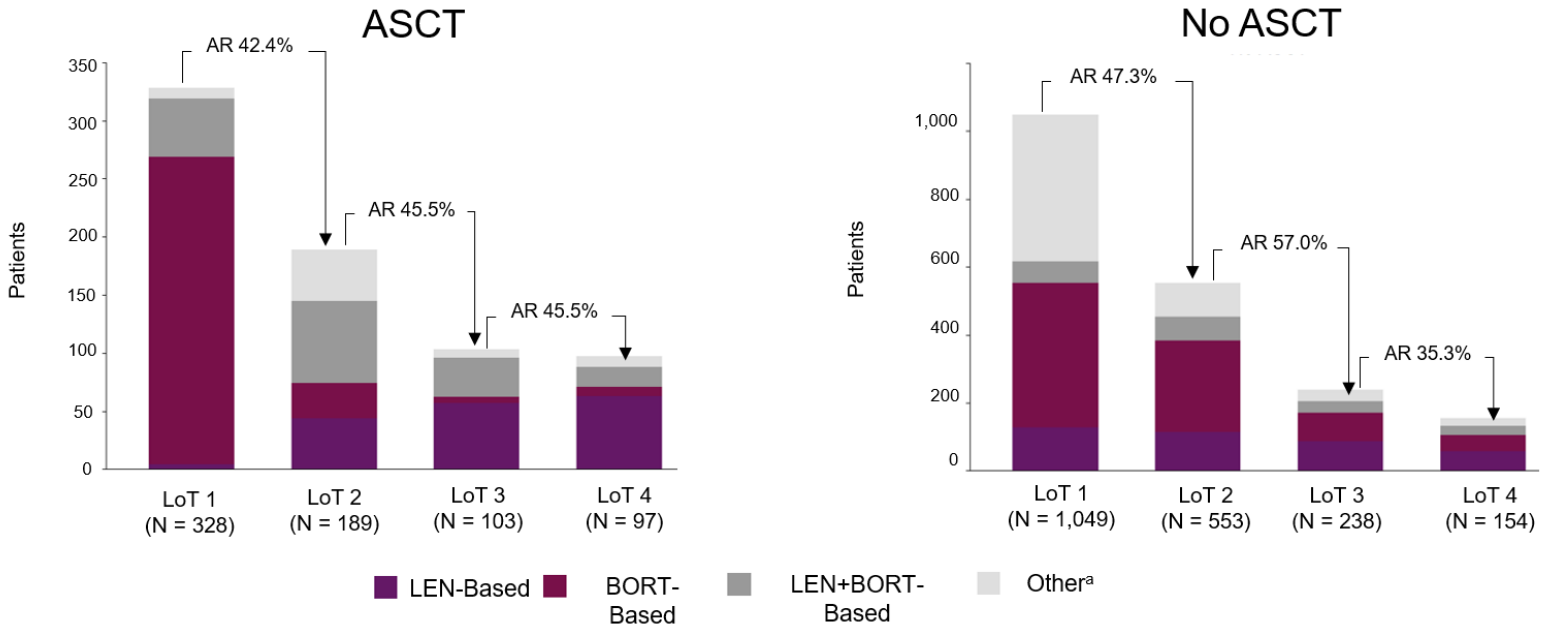
Previous Medlior Project | Multiple Myeloma

Objective: To describe the real-world multiple myeloma (MM) population in Alberta from 2011-2016

Outcomes:

- Treatment patterns: lines of therapy, treatment regimens, autologous stem cell transplant, duration of therapy
- Healthcare utilization: ambulatory visits, physician claims, hospitalizations and costs

Treatment regimens and attrition rates for each line of therapy



Characteristic	Overall N = 1377
Follow-up Time, years (Mean ± SD)	2.3±1.6
Age, years (Mean ± SD)	68.9±12.2
ISS Stage (n=816) n (%)	
I	278 (20.2)
II	213 (15.5)
III	325 (23.6)
ISS-FISH (n=487) n (%)	
Low	300 (21.8)
Intermediate	128 (9.3)
High	59 (4.3)

NPL for unstructured lab data

Abbreviations: AR, attrition rate; ASCT, autologous stem cell transplant; BORT, bortezomib; LEN, lenalidomide; LoT, line of therapy
[a]Other medications predominantly consisted of dexamethasone and prednisone.

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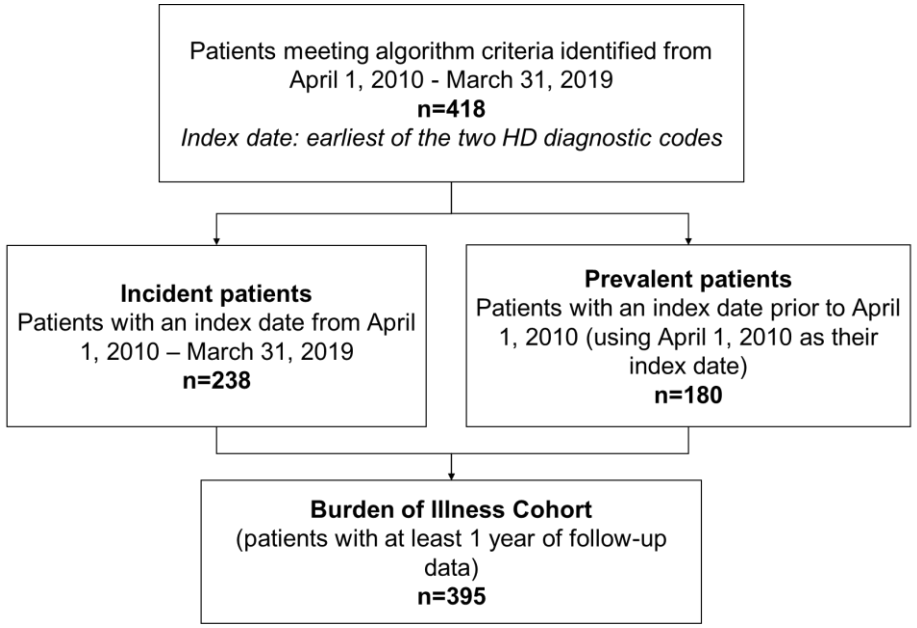
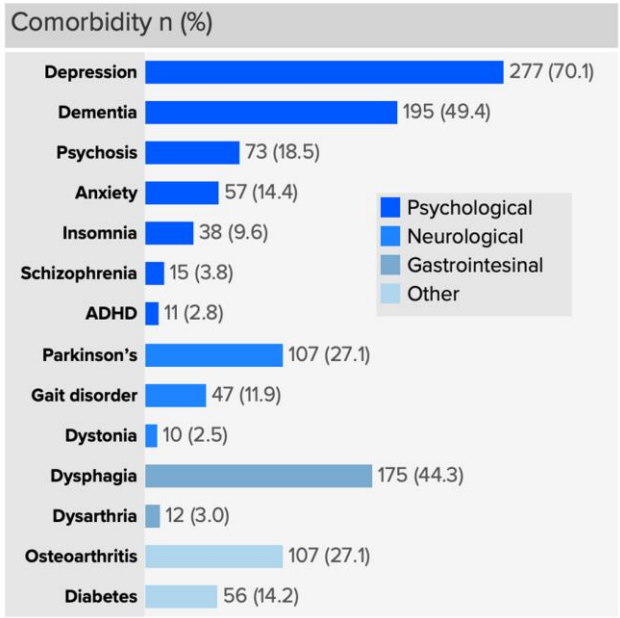
Previous Medlior Project | Burden of HD

Objective: To characterize the burden of disease of HD (epidemiology and healthcare resource utilization/costs) in a Canadian setting with a publicly-funded healthcare system, using administrative health data in Alberta.

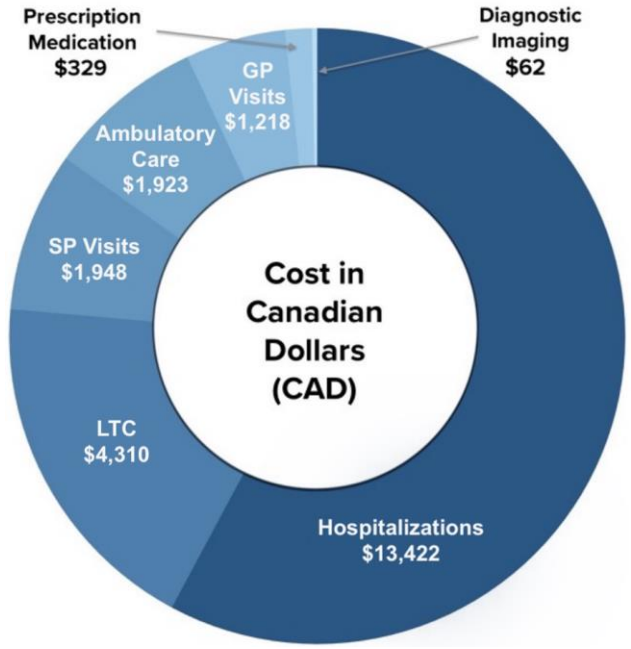


HD Study Population

Presence of comorbidities of interest in the HD BOI cohort (n=395)



NOTE: Comorbidities were extracted using diagnostic codes from the DAD, NACRS, and Practitioner Claims datasets in the two years prior to index date and during the entire follow-up period.



Mean all-cause costs among patients with HD per person-year of follow-up

Key Findings:

- The annual prevalence increased slightly over time, and the 5-year average annual incidence was 0.83 per 100,000 person years, while the 5-year period prevalence was 12.15 per 100,000 PY.
- The sizable number of physician visits and high cost of hospitalizations illustrates the need for continued research and development to advance the care available to patients diagnosed with HD in Canada.

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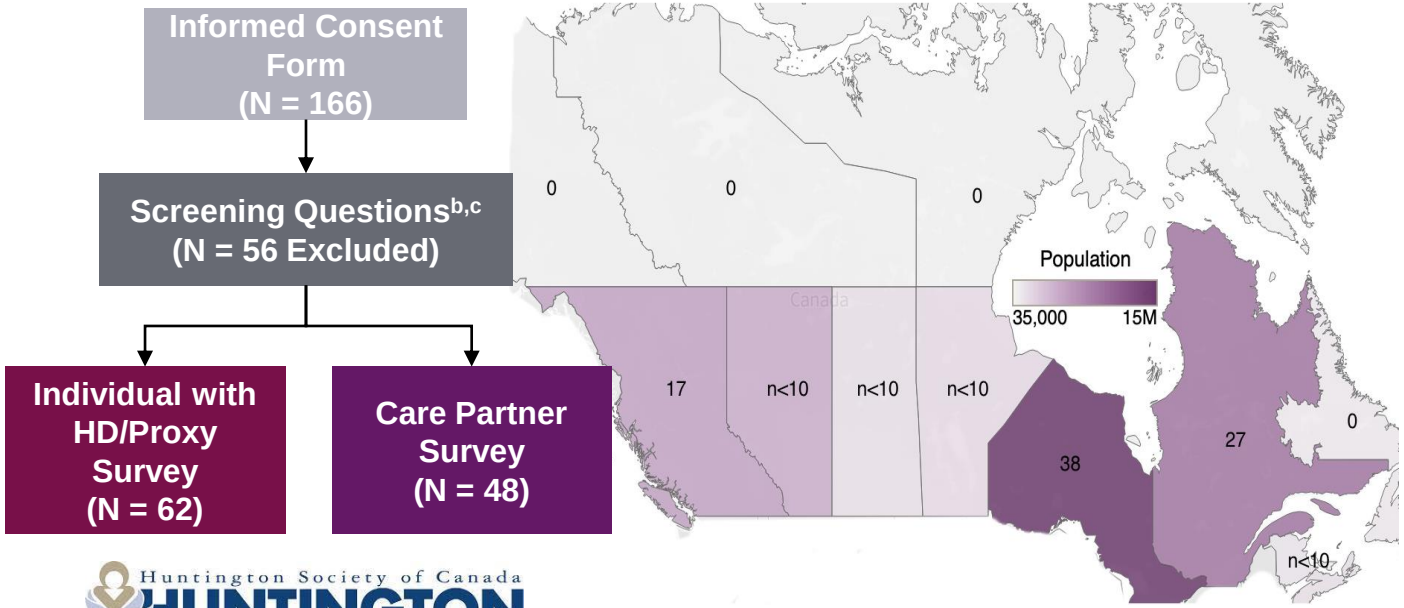
Previous Medlior Project | HRQoL of HD

Objective: To evaluate the impact of Huntington's disease (HD) on health-related quality of life (HRQoL) for individuals with HD and care partners in Canada.

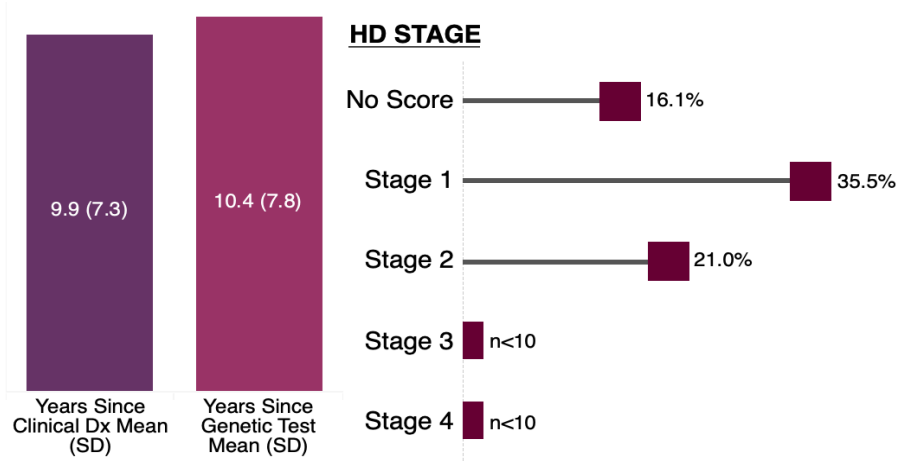
Methods: An online survey was distributed by Canadian patient advocacy groups from September – November 2020 to evaluate demographic and clinical characteristics, HRQoL, and care partner burden.

- Patient HRQoL was assessed using the SF-36v1
- Care partner HRQoL was assessed using the Caregiver Strain Index (CSI) and the Huntington's Disease Quality of Life Battery for Carers (HDQoL-C)

Survey Respondents and Geographic Distribution[a]



Characteristics of Individuals with HD



Huntington Society of Canada
HUNTINGTON
Société Huntington du Canada

Société Huntington du Québec
HUNTINGTON
Huntington Society of Quebec

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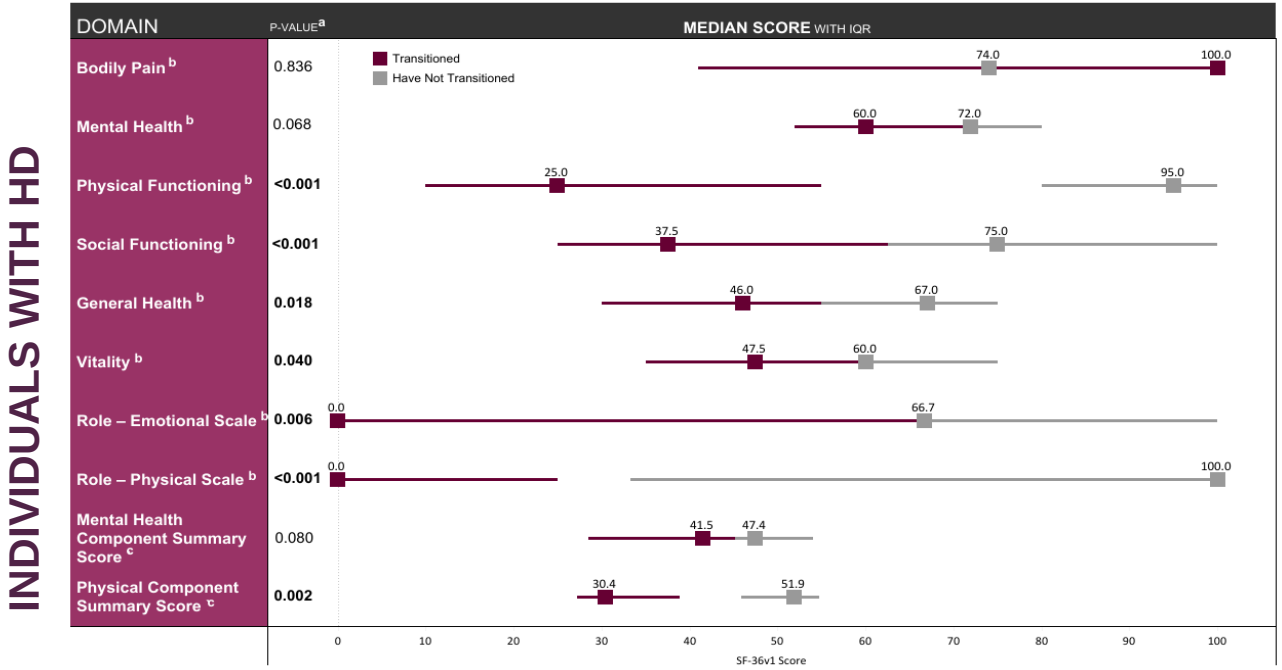
Previous Medlior Project | HRQoL of HD

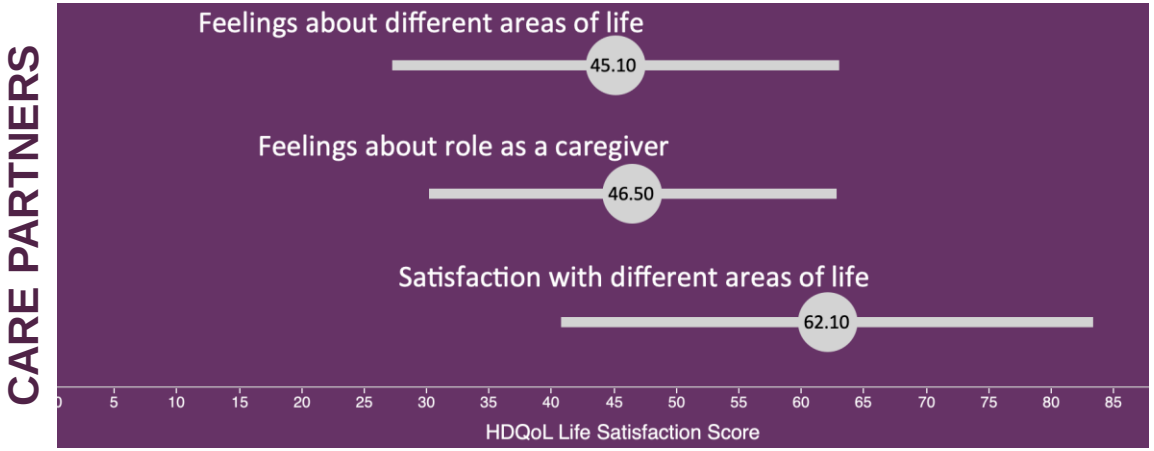
Key Results:

- HRQoL decreased significantly across domains for respondents who had motor progression compared to those who had not.
- Over half of care partners indicated a high stress/burden.

SF-36v1 Domain and Summary Scores for Individuals with HD Stratified by Motor Transition Status



Care Partner HDQoL-C Summary Scores[a]



Conclusion: This study quantified the substantial burden that HD places on the HRQoL of both individuals with HD and caregivers in Canada, addressing a critical knowledge gap.

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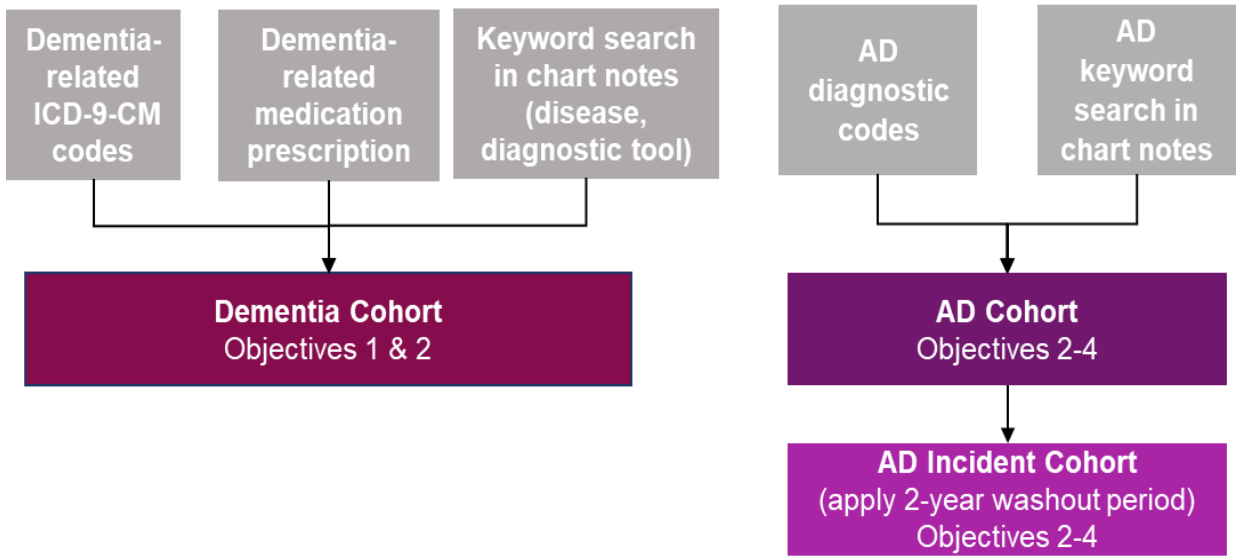
HEALTH OUTCOMES RESEARCH

Current Medlior Project | Early AD

- **Purpose:** to understand the clinical practice patterns and variations in the diagnosis of Alzheimer's disease (AD) in primary care using electronic medical record (EMR) data from Ontario
- **Objectives:**
  1. Identify the different types of diagnostic and screening tools used in primary care clinical practice to diagnose dementia patients.
  2. Describe the demographic and clinical characteristics of patients with dementia and AD seen in primary care clinical practice in Ontario.
  3. Describe the range of cut-off scores for different levels of disease severity among diagnostic and screening tools currently used in primary care clinical practice in patients with AD.
  4. Describe the treatment patterns in patients diagnosed with AD in primary care clinical practice.
- **Methods:**
  - **Data source:** EMR data and de-identified chart data from the Queen's Family Medicine Restricted Data EnviroNment (RDEN) database from August 2011 to August 2021 (Canadian Primary Care Sentinel Surveillance Network network)
  - **Cohorts:** Patients with dementia and AD will be identified using a combination of ICD-9-CM codes and a keyword search in chart notes using natural language processing (NLP)



- **Outcomes:**
  - **Diagnostic and screening tools**: frequency and average rate of use for tools including neuropsychological tools, imaging techniques, and laboratory tests
  - **Cut-off scores used for disease severity:** range of cut-off scores
  - **Demographic and clinical characteristics**: age, sex, comorbidities
  - **Treatment patterns**: medication type, class, duration, dosage, time to first medication

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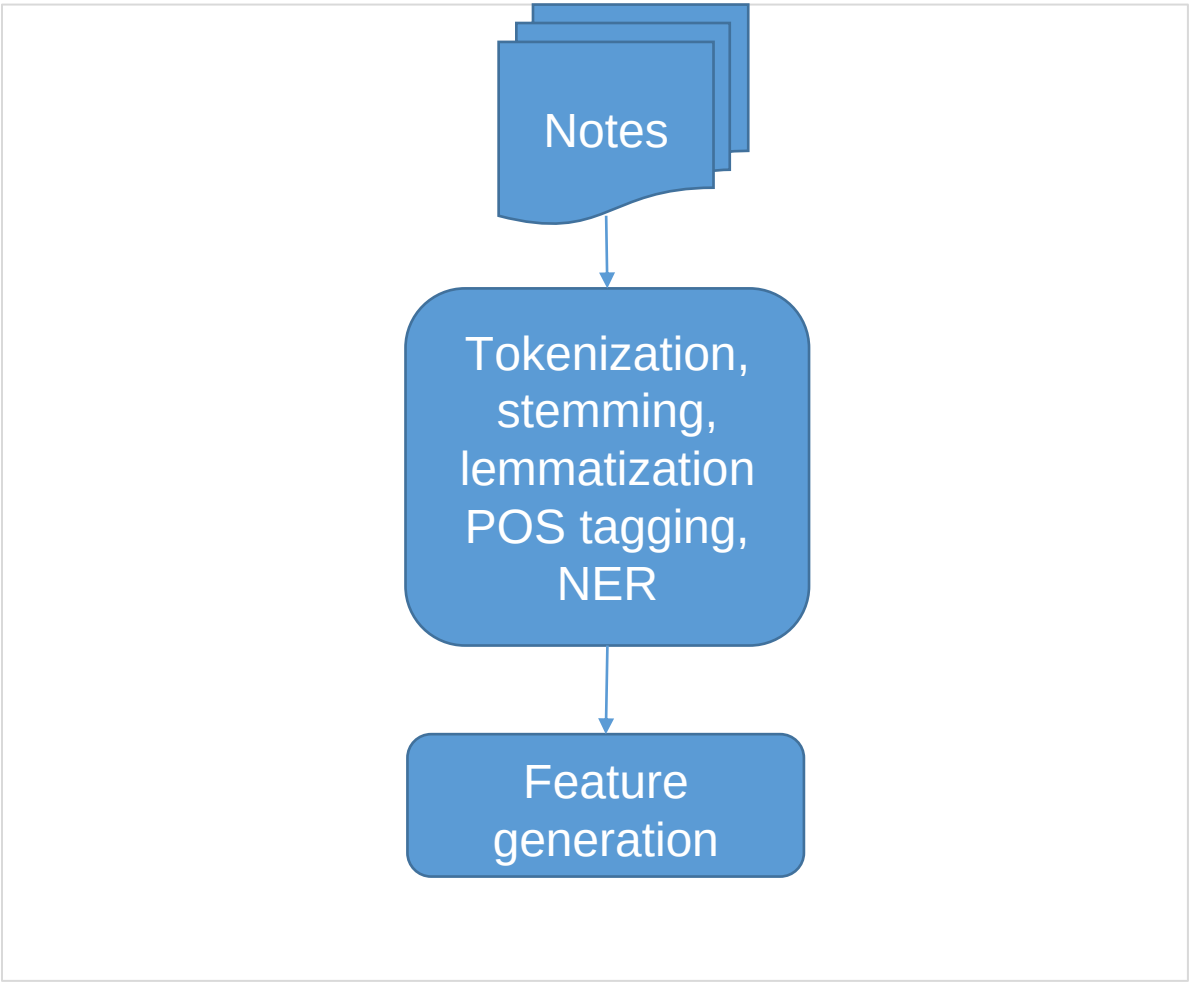
Current Medlior Project | Early AD

Medlior is partnering with the Bigdata Analytics and Management (BAM) laboratory at Queen's University directed by Dr. Farhana Zulkernine to conduct data analysis and NLP

- **NLP process:**
  - **Data preparation**: data will be extracted and anonymized
  - **NLP pipeline**: tokenization, transforming words to basic form, identify parts of speech for semantic and syntactic analysis, process negative expressions, and extract relevant medical terms
  - **Cleaning, filtering and transforming data:** remove empty chart notes and duplicates
  - **Extraction and organization of information**: data organized into databases to facilitate analysis
  - **Validation**: manual and automated processes



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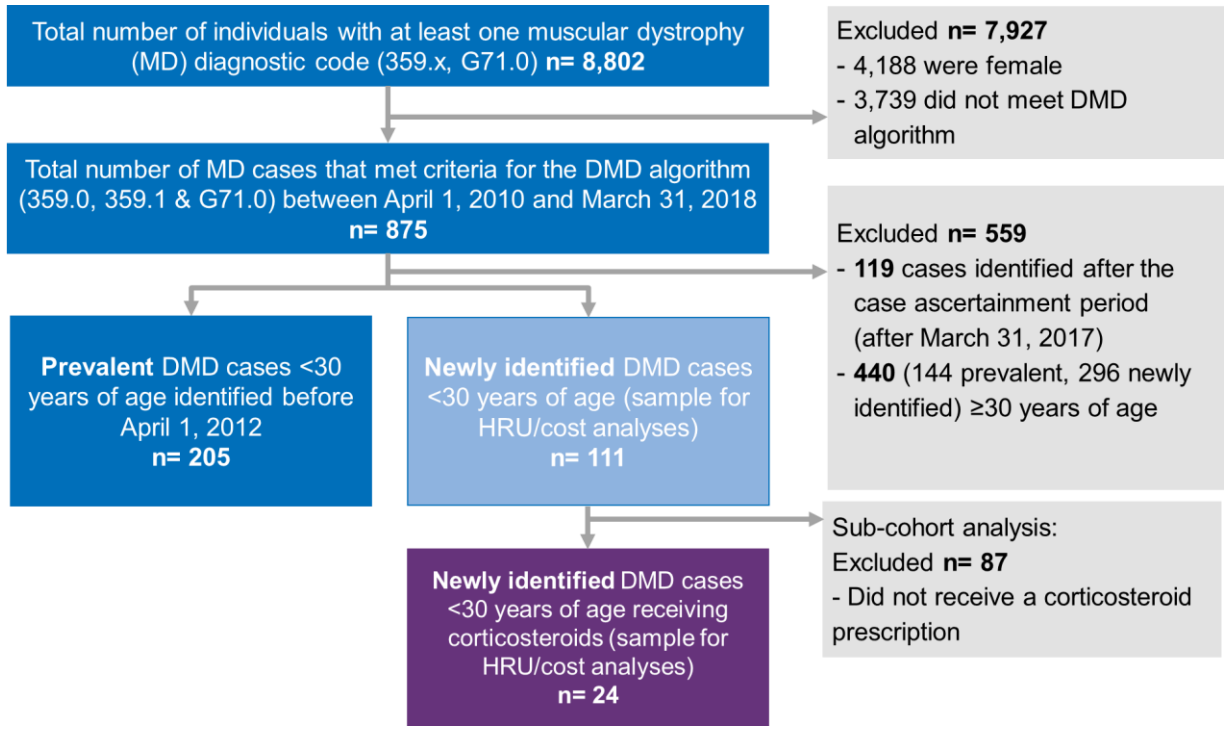
Previous Medlior Project | DMD

Objective: To describe burden of illness in the first year post-DMD diagnosis – epidemiology, healthcare resource utilization and direct medical costs

Study Design:

- Retrospective cohort study of incident patients
- Individuals <30 years of age with DMD were identified utilizing muscular dystrophy diagnostic codes from a published algorithm4 by linking several Alberta administrative datasets.



- This was the first study to investigate the epidemiology and direct costs of DMD in Alberta using population-based administrative healthcare data.
- As ICD codes alone cannot identify DMD cases, this study applied a published algorithm, as well as age and drug utilization, to identify a relevant study cohort of newly diagnosed patients for the cost analysis.
- Patients with DMD had multiple interactions with the healthcare system in the year following diagnosis, resulting in substantial direct medical costs.

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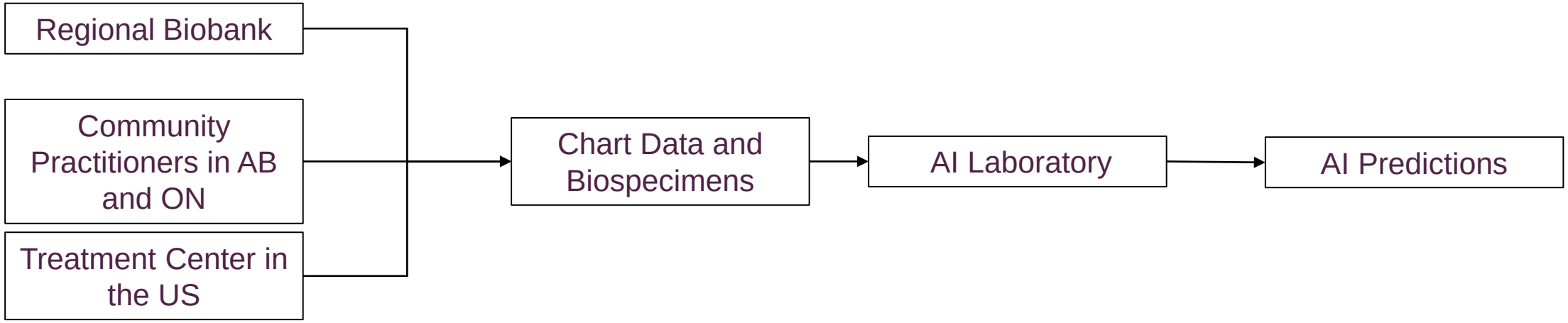
Current Project | Validation of Prediction Tool

Objective: To assess the value of a digital model for predicting the transformation of potentially malignant lesions to squamous cell cancer



Outcomes:

- The diagnostic prediction tool demonstrated better specificity and sensitivity of cancer progression than clinical diagnosis only, among patients with moderate or severe dysplasia
- In patients with mild, moderate and severe dysplasia, elevated risk scores using this prediction tool was associated with an increased risk in disease progression.

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Question & Answer Period

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Thank you for your time!