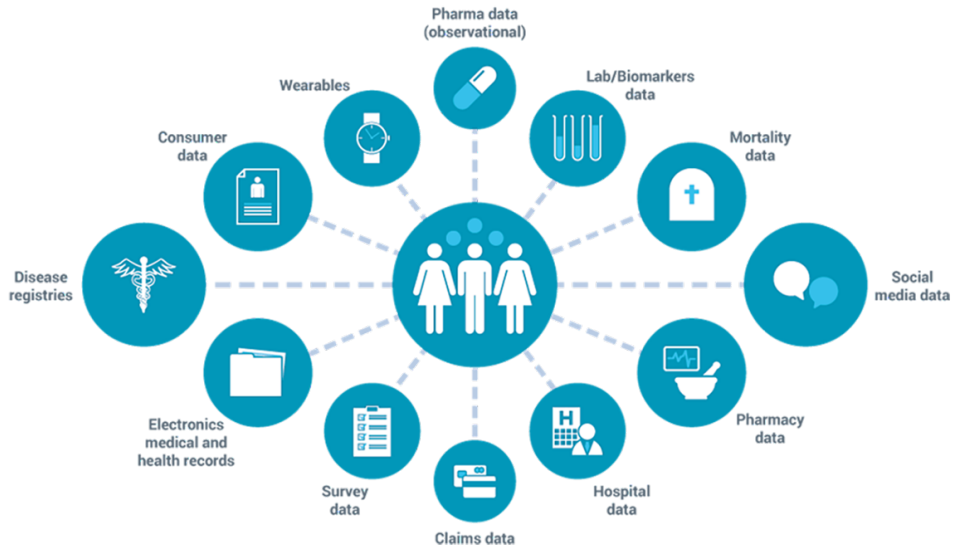
REAL-WORLD DATA (RWD) / REAL-WORLD EVIDENCE (RWE) FOR RARE DISEASE DRUGS



Webinar

September 22, 2021

Canadian Organization for Rare Disorders

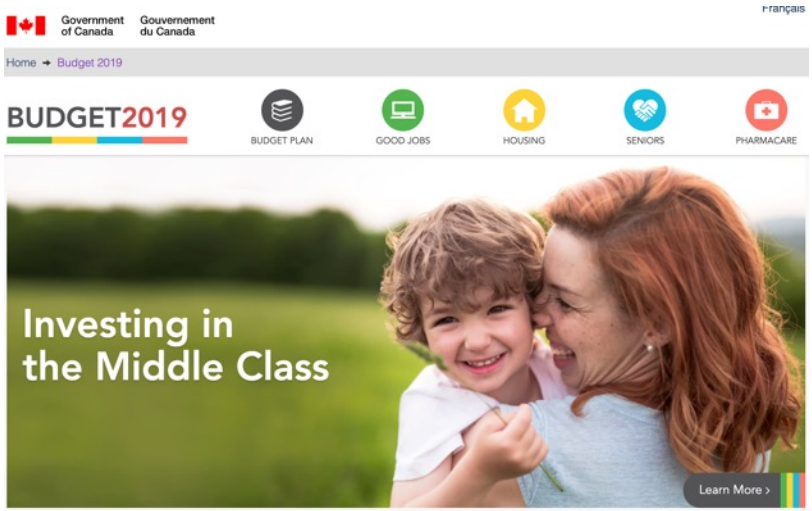
Canadian Rare Disease Drug Landscape

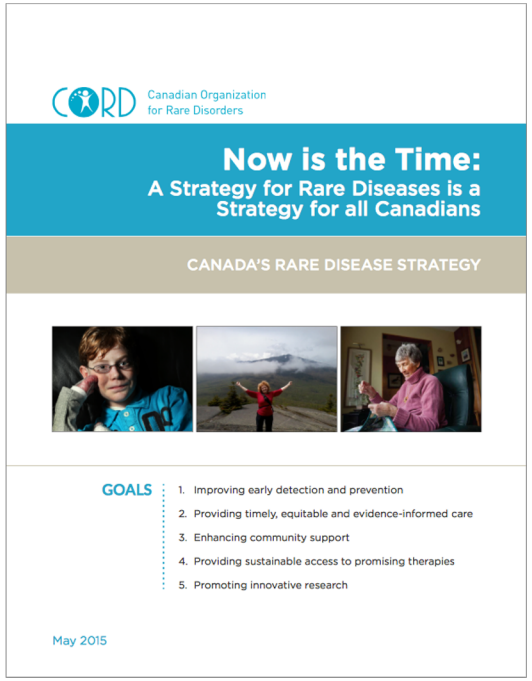
Ready to Go!

- Picking Up from Pre-Election
- Health Canada's Report on "What We Heard" during the Rare Disease Drug Strategy Consultations.
- Patented Medicines Prices Review Board: Delay in "restrictive pricing" guidelines and Federal Court of Appeals: Reason for hope in new drug launches in Canada?
- CADTH & INESSS: What (when) is role in NEW Rare Drug Strategy?
- pCPA: Slow-walking pricing agreements; why no one is holding them accountable for delayed access?

Context and opportunity

- Liberal election 2021 = recommitment to Rare Disease Drug Strategy
- 2019 federal budget includes a commitment to invest in a national strategy for rare disease treatments: **up to $1 billion** over two years starting in 2022-23, with **up to $500** million per year afterwards
- 2020 fall economic statement and Speech from the Throne reaffirmed this commitment
- National support for better access to rare disease medicines has been building steadily since 2015 and CORD's launch of **Canada's Rare Disease Strategy**





Panelists

- Tara Cowling, Medlior
- Laurie Lambert, CADTH
- Craig Campbell, London Health Sciences
- Sandra Anderson, Innomar Strategies
- Brad Alyward, Canadian Organization for Rare Disorders
- Durhane Wong-Rieger, Canadian Organization for Rare Disorders

Moderator

- Bill Dempster, 3Sixty Public Affairs

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Agenda

- Introductions
- What are RWD/RWE
- What are situations where RWE is making a difference?
  - Use of RWE in HTA
  - Monitoring and evaluating patient benefits and safety outcomes to confirm and expand clinical trial data
  - Using RWE from Patient Support Programs to support reimbursement decisions
  - Payer perspectives on value and challenge of RWE
- How RWE can optimize rare drug access
  - Case 1: 1st in class new therapy for previously untreated condition
  - Case 2: Significant improvement over existing therapy
  - Case 3: Therapy targeted to genomic mutation (subtype)
  - Case 4: Gene therapy
- Next steps

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Rare Disease Strategy - Five-point Action Plan

1. Improving early detection and prevention,
2. Providing timely, equitable and evidence-informed care,
3. Enhancing community support,
4. Providing sustainable access to promising therapies and
5. Promoting innovative research

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12-Steps to a National Rare Disease Framework

- Patient empowerment: Empower patient organizations and patient advocates as active full partners
- Creation of a Canadian Rare Drug Agency: independent, transparent, publicly accountable agency with responsibility for all aspects of the review of drugs for rare diseases, in coordination with Health Canada
- Create R&D incentives: Invest in Research and Development to support therapeutic product accessibility, monitoring, and evaluation. Build capacity for drug discovery, technological innovation, manufacturing and production, and (global) distribution.
- Speed up access to treatment: Ensure timely availability of new treatments by establishing a competitive and viable environment, including supportive mechanisms for clinical trials, early access programs, clinical site development, patient registries, and patient support programs

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12-Steps to a National Rare Disease Framework

- Address regulatory barriers: Ensure PMPRB guidelines do not exceed a "reasonable" threshold of fairness compared to comparable countries; roll back 2019 PMPRB regulatory changes by removing use of economic factors
- Improve regulatory approvals process: Ensure Health Canada continues to update its regulatory process to encourage clinical trial and new drug submissions for rare disease drugs
- Ensure pathways for special cases: For urgent need, timely access provided through Special Access Program (SAP) prior to Health Canada approval; create pathway through Early Access Programs.
- Need for multiple funding options: Multiple separate pathways based on population size, disease severity, unmet need, evidence uncertainty, potential therapeutic value, budget impact, annual unit price, and industry

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12-Steps to a National Rare Disease Framework

- Leverage Managed Access Programs (MAPs): For drugs receiving an NOC-C where there is uncertainty about the evidence at the time of approval but where the unmet needs and benefits outweigh the risks.
- Facilitate concurrent Health Canada and HTA reviews: Joint Health Canada and HTA application (where appropriate)
- Support real-world evidence generation: Be responsible for real-world monitoring, data collection, evaluating benefits, risks, and uncertainty
- Enhance centres of clinical expertise: Partner in developing Networked Centres of Expertise for specific rare diseases related to management of a therapy.

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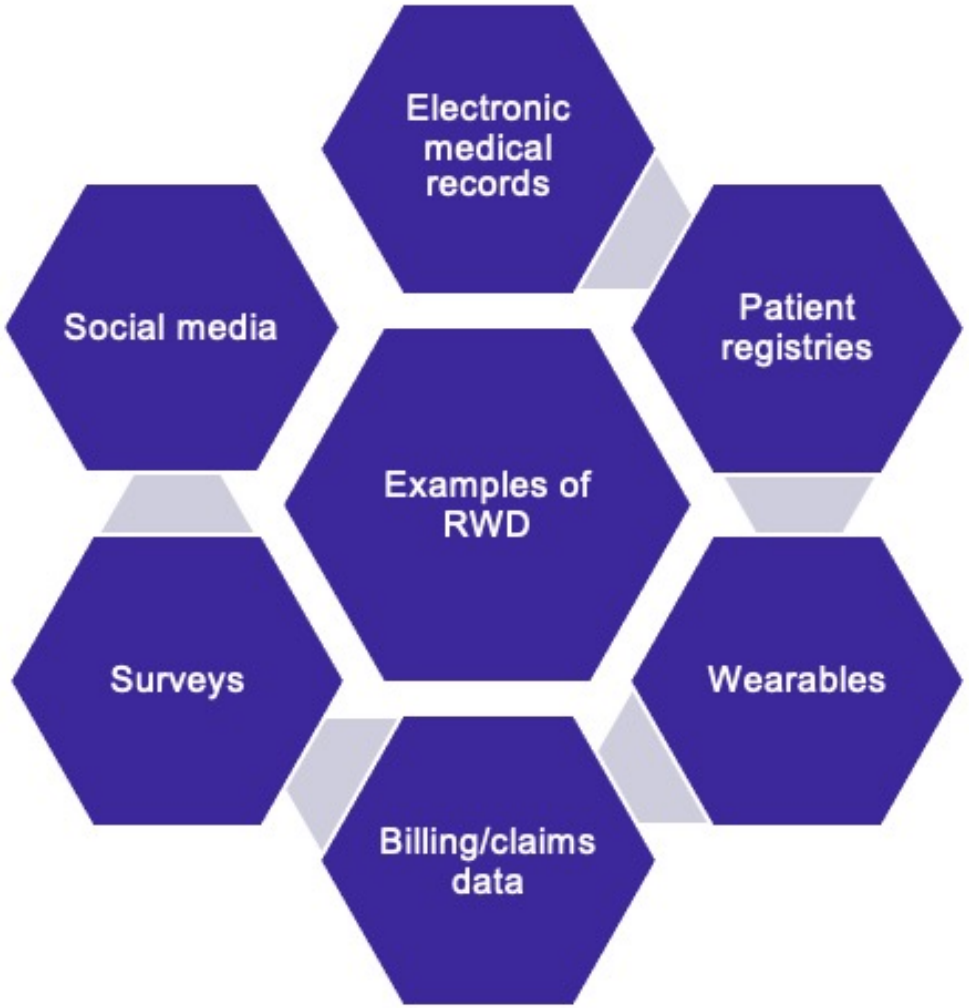
What is RWD?

Definition

"Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources."[1]

"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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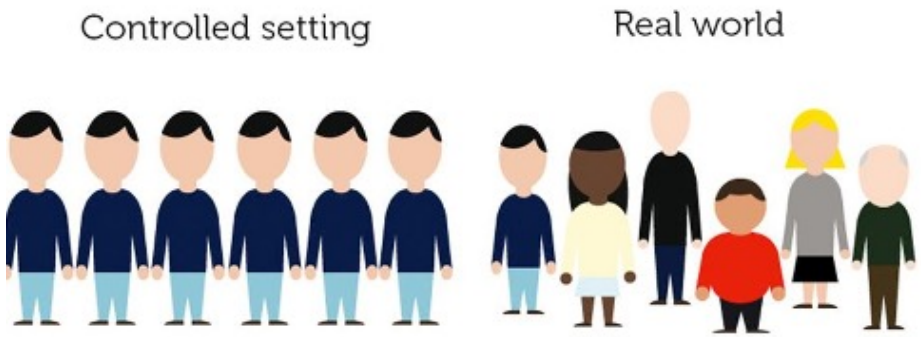
What is RWE?

Definition

"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]



RWD

↓

RWD combined into a research database

↓

Analyzed according to a research protocol

↓

RWE

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

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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

Canadian Health System Data

Canadian health system data includes:

- Health insurance plan registration
- Vital statistics
- Health service records
- Pharmaceutical claims
- Laboratory service tests
- Diagnostic imaging
- Cancer Registry

*Provincial population estimates (country population of 37,971,020 in 2020)
Population estimates retrieved from Statistics Canada

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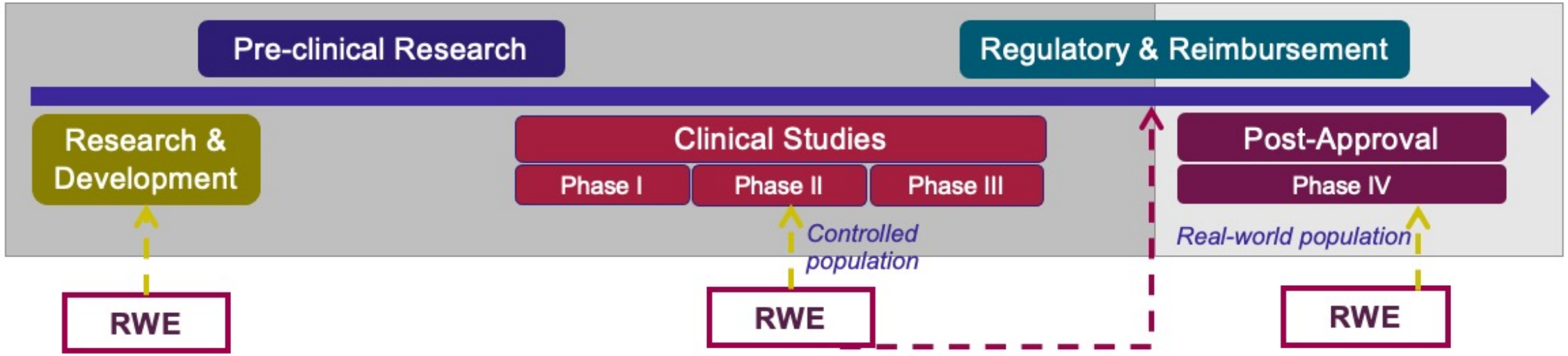
RWE & Decision-Making

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



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

- **Regulatory Approval & HTA strategy**
- Relative efficacy
- Cost-effectiveness
- Budget impact
- Value proposition
- Place in therapy
- HTA/scientific advice

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

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Regulatory and Reimbursement

"Say 'eh.'"

- RWE can support decision-making where there is clinical trial uncertainty
  - ✓ Provide 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)
  - ➢ Public payers - interest in phase IV trial data to address any trial uncertainty
  - ➢ Private payers - interest in employment data (absenteeism/presenteeism)

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

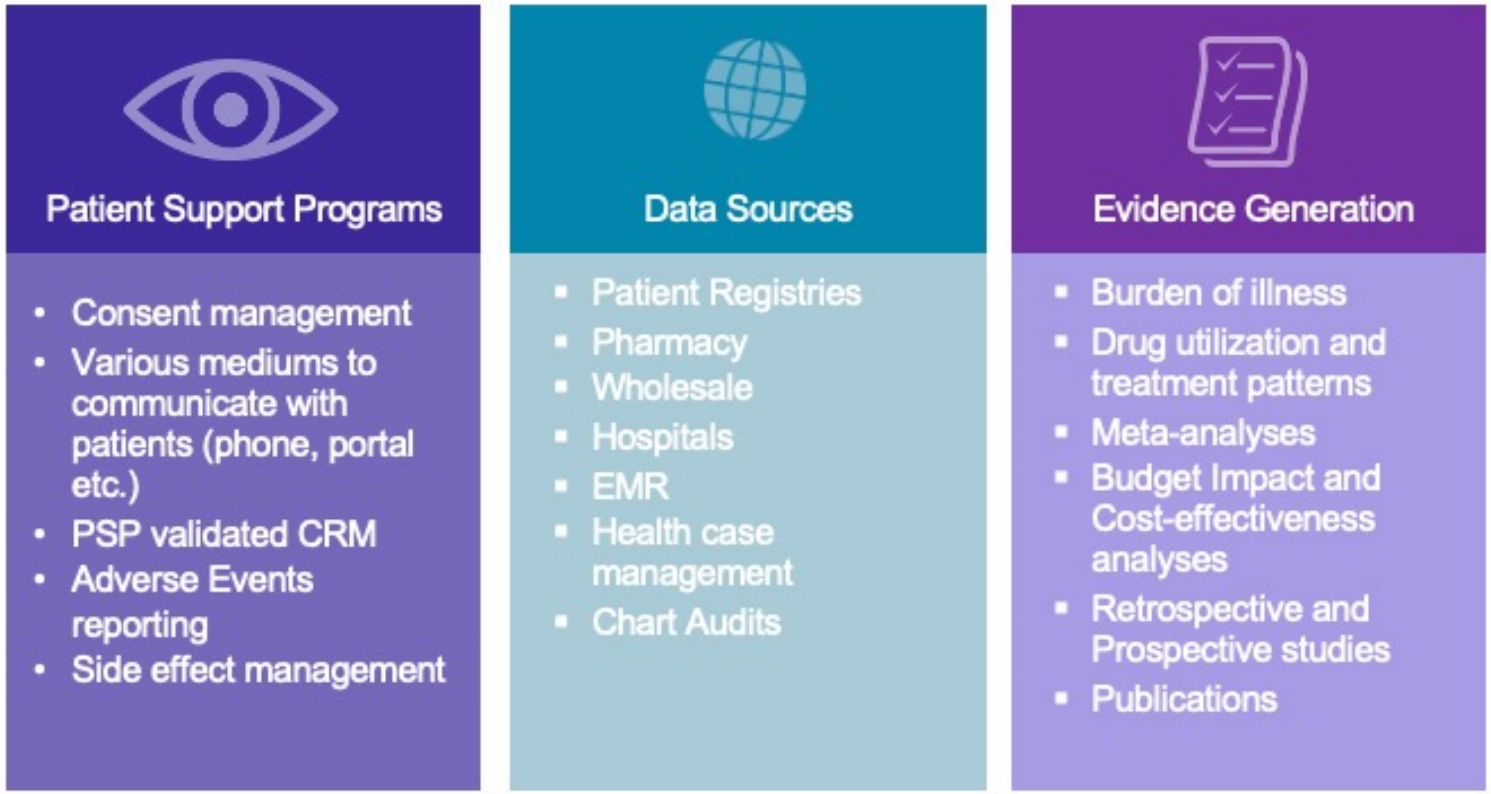
AmerisourceBergen
Innomar Strategies

Real World Evidence and Patient Support Programs

Sandra Anderson,
SVP, Commercialization and Strategy

September 22nd , 2021

RWE through the patient journey

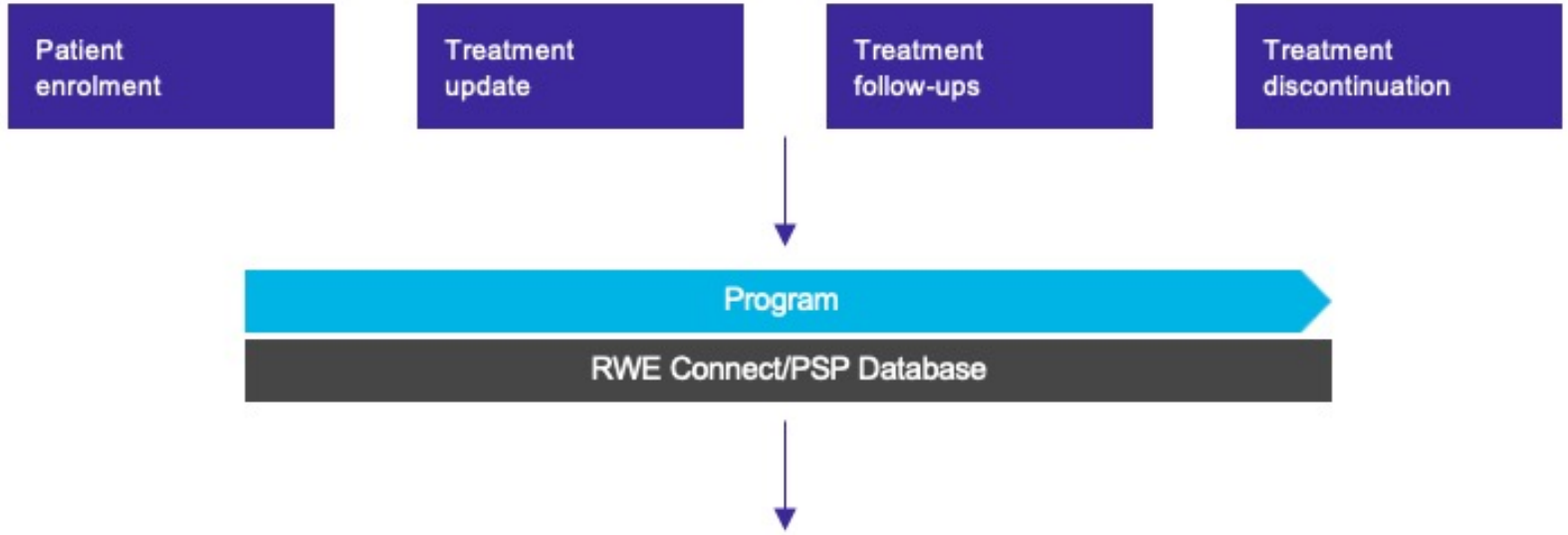


Leverage insights across the therapeutic lifecycle

Data that is payer driven for PLAs, OBAs, Renewal Criteria

Bergen

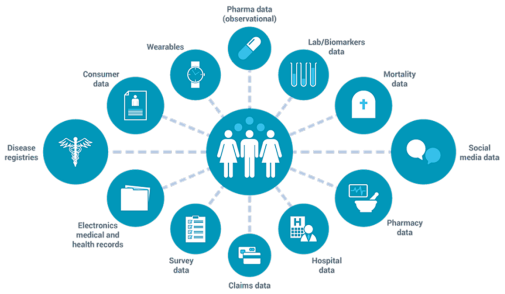
Real world data collection through PSP



Data collection

Baseline information	Patient characteristics including age, weight (dose related), and treatment dose Baseline characteristics e.g., age at diagnosis, disease severity
Treatment patterns	• Duration of therapy • Reasons for discontinuation • Medication adherence
Healthcare resource utilization	Healthcare utilization/burden e.g., physician visits, ER visits, hospitalizations, costs to system
Productivity and Societal outcomes	• Work/school productivity • Disability • Caregiver burden questionnaires

Health outcomes (Secondary Measures)	Baseline and clinical response measures ▪ Laboratory reports QoL measures
Other Market Research and KOL Opportunities	PSP Satisfaction Surveys Abstract and Publications Conference Presentations



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Today's slides available on CORD's SlideShare page

(https://www.slideshare.net/raredisorders)

Today's webinar will be posted to CORD's YouTube channel

https://www.youtube.com/user/CORDRareDisorders)

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