CORD – Fall Conference

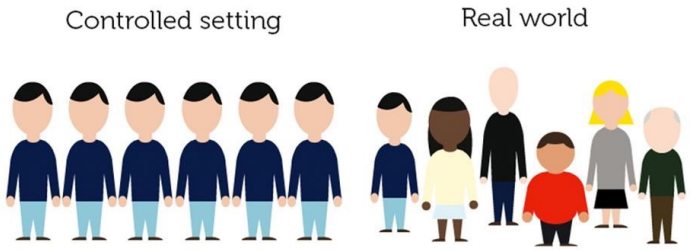
Calgary, AB

November 2023

MEDLIOR™
HEALTH OUTCOMES RESEARCH

How Does RWE Differ from Clinical Trials?

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/resource consuming data collection	➢ Time/resource efficient data collection



RWE for Rare Disease
Fit for Purpose

	Health System Data	Registry data
Benefits	• Robust source of provincial-level data ✓ Larger population, routinely captured • Real-world population-level data ✓ Demographics, comorbidities, tx patterns, overall survival, cause of death • Strong utilization data ✓ Hospitalizations, procedures, emergency visits, outpatient visits, lab tests, etc • Longitudinal data (+10 years) ✓ Long-term safety and effectiveness • Potential for AI/ machine learning	• National level data ✓ Valuable for smaller/ rare populations • Rich source of self-reported data ✓ Quality of life, caregiver burden, barriers to treatment, income status • Potential to import novel data ✓ Wearables, health/fitness apps, patient journals for symptom tracking • Ability to collect longitudinal data ✓ Change in self-reported data over time • Potential for AI/ machine learning

RWE for Rare Disease
Fit for Purpose

	Health System Data	Registry data
Drawbacks	• Limited primary care data • Limited structured data for clinical outcomes • No data on inpatient medications • Limited patient-reported outcomes data • Some provinces limited to public plan drug data • Time lag of >6 months for data release • Access is variable across provinces • Lack of standardization to pool data nationally	• Concerns with data quality/ bias • Patient consent needed for secondary use • Resources to analyze and report data

RWE for Access

Unmet need for national registry data/ platform

✓ Patient-centric data collection

- Collect measures that matter to patients to demonstrate value of therapy
- Research framework with robust methodology for analysis/reporting

✓ Data linkages

- Link to administrative data to examine impact of patient-reported outcomes on utilization and costs
- Extrapolate provincial-level admin data to national level using registry data

✓ Long term surveillance

- Longer-term data collection is feasible for monitoring outcomes

✓ Automated reporting

- Agreement on reporting needs to inform and improve access to therapies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