OPERATIONALIZING AN OUTCOMES-BASED MARKET ACCESS AGREEMENT USING REAL-WORLD DATA FROM THE CANADIAN NEUROMUSCULAR DISEASE REGISTRY

20SENSE PT37

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BACKGROUND

The number of promising novel therapies with clinical uncertainty or immature data is growing, especially in rare disease and precision oncology. This trend, combined with long reimbursement timelines, has resulted in Canadians having to wait an average of 1.5 years or more from Health Canada regulatory approval to initial listing on a public plan before gaining access to a new drug, if they obtain access at all.

Outcomes-based agreements (OBAs) have the potential to enable timely patient access to such therapies, while mitigating risk for payers. An OBA is an 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. OBAs can be used to address uncertainties that create access barriers; they are not a replacement for clinical trials, and they are not appropriate for all drugs or for all reimbursement scenarios.

Barriers to implementing OBAs in Canada include the time and resources associated with real-world data (RWD) collection. Leveraging existing data sources and infrastructure, including patient registries, could help resolve these barriers.

The **Canadian Neuromuscular Disease Registry (CNDR)** is a Canada-wide registry of people diagnosed with neuromuscular diseases including spinal muscular atrophy, Duchenne muscular dystrophy, and amyotrophic lateral sclerosis (ALS). The CNDR was launched in 2011 and is run by the University of Calgary.

Spinal muscular atrophy (SMA) is a genetic disease affecting the central nervous system, peripheral nervous system, and voluntary muscle movement. The CNDR dataset for SMA first launched in 2012 and was expanded in 2019. There are currently 3 disease-modifying therapies (DMTs) for SMA available in Canada, however there are provincial reimbursement gaps, notably for adult patients. OBAs, as well as other managed access mechanisms such as managed entry agreements, have been used in international jurisdictions to enable timely access to SMA therapies – but not in Canada.

RESEARCH QUESTION

Could an OBA be operationalized using RWD from the CNDR?

METHODS

Using the Focus Group Discussion method, individuals were selected with expertise in the areas of the CNDR, SMA patients, health technology assessment and OBAs.

The group convened 17 times from February to September 2024. Each meeting focused on a specific topic, including the CNDR, SMA and its health outcomes, data collection processes, current available SMA therapies, international OBAs, payer processes, and others. External sources including literature reviews and interviews with subject matter experts were conducted as input to the group discussions. An iterative process was used to summarize the data collected, develop study results, and review results with the group to achieve consensus. The results were then reviewed with 14 external stakeholders including physicians, public and private payers, manufacturers, and health technology assessors.

RESULTS

1. Health Outcomes

Eight health outcomes (HO) for SMA were identified and evaluated for data readiness, data interpretation, and data timeframe.

- Data readiness: Accessible for an OBA, complete and accurate.
- Data interpretation: Health outcome is clear and simple.
- Data timeframe: Can be collected in a reasonable timeframe.

One HO was deemed suitable for an OBA (motor scales), and four additional HOs were deemed suitable in combination with others (survival; ventilation; feeding tube; duration of therapy). See Table 1.

Health outcome suitability for an OBA using RWD from the CNDR

Note: For all health outcomes listed, patient data is only available in the CNDR if the patient has registered in the CNDR.

HEALTH OUTCOME	SUITABLE FOR AN OBA?
1 Motor Function Scale (ability appropriate – sitter, walker)"	Yes
2 Overall Survival (OS)	In combination with other health outcome(s)
3 Permanent Ventilation	In combination with other health outcome(s)
4 Feeding tube	In combination with other health outcome(s)
5 Duration of Therapy	In combination with other health outcome(s)
6 PROMs (PEDS QL, SMAFRS, SMAIS)	Future potential
7 Hospitalizations	No
8 Scoliosis surgery	No

2. Process Gaps and Solutions

Three key process gaps and their solutions were identified in order to use the CNDR for an OBA, including patient participation in the CNDR is voluntary (only 30% of SMA patients are currently registered); patient consent does not allow individual level data to be shared outside of the CNDR; reporting timelines require 3 months for data quality control processes. See Table 2.

Table 2: Process gaps and solutions identified to enable the use of the Canadian Neuromuscular Disease Registry for an OBA

GAP	SOLUTION
1 Patient participation in the CNDR is voluntary, ~30% of SMA patients are currently enrolled.	Mandatory participation in the CNDR for patients partaking in the OBA. The OBA would replace the current payer prior authorization / special access program.
2 Patient consent for data sharing - current CNDR consent does not allow sharing of individual level data outside of the CNDR.	Current consent is being revised to share aggregated data externally, which would need to be assessed if sufficient for an OBA. If not, a seperate consent would need to be developed for patients partaking in the OBA.
3 Reporting - 3 months required to run a report in order to perform data quality checks, and for clinic sites to correct data.	No change. Interim and final reports would have to adhere to this constraint and be agreed to upfront as part of the OBA.

3. Operational process for an OBA using the CNDR

An operational process using the CNDR as the data source for an OBA was developed by using the current state process (see Figure 1) and the solutions to the gaps (Table 2). See Figure 2.

Figure 1: Current state process for patient drug access and CNDR

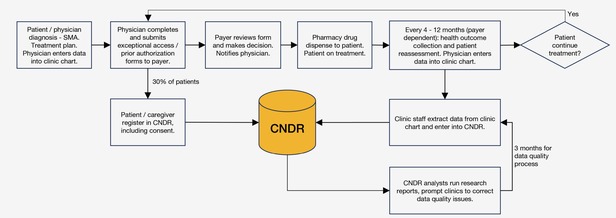
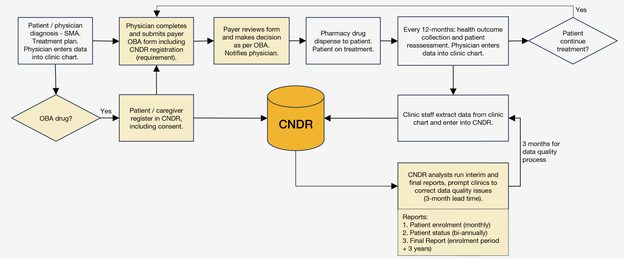


Figure 2: Future state process for patient drug access and CNDR with OBA



CONCLUSIONS

Using the CNDR to operationalize an OBA for SMA appears to be feasible, with the caveat that the solutions for the 3 identified gaps are deemed sufficient and can be implemented. Additional feedback from stakeholders is required to determine if further refinement to the proposed process is required, particularly from Canadian public payers and the pan-Canadian Pharmaceutical Alliance.

DISCUSSION

In addition to the findings that the CNDR could be used for data collection for an OBA, it was also identified that payers are currently evaluating patient renewals for the 3 SMA therapies as part of their **exceptional access / prior authorization processes**. In these processes, each patient's health outcomes are being provided to payers, typically annually, to allow the payer to evaluate the eligibility for the continuation of treatment (see Figure 1). Using these existing payer processes could be another method of operationalizing an OBA in Canada.

It must be noted that **patient-report outcome measures (PROMs)** are highly important health outcomes for SMA patients, due to the high heterogeneity in progression of the disease for each patient. Physicians confirmed the use and importance of physician-administered PROMs in addition to other health outcomes to assess patients.

A key learning from the project is that **a significant amount of preparatory work is required to enable an OBA to support a first-in-class drug.** The findings from this research show that in current state the CNDR can support RWD for an OBA, however, in the case of SMA, physicians noted that this may have been challenging when the new therapies were first available. At that time, new processes were needed to introduce the drugs into practice, including – for example – defining a standard of care (i.e., PROMs, motor scales), building infrastructure to measure patient health outcomes (i.e., occupational therapists for motor scales), and assessing the patient registry for data requirements and gaps.

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