Health Policy Research Program
Summary of Research Results

Title:	**Production and Use of Evidence of Drug Effectiveness: Systematic Review, Evaluation and a Guidebook for Decision Makers**
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Summary

Issue

The cost crisis for Provincial Ministries of Health in general, and Drug Benefit Plans in particular, requires more investment in evaluating of drug benefit and risk to determine cost-effectiveness.

Background

Canada is in the early stages of an international drug cost crisis. Public demand for new pharmaceuticals continues to grow, but government resources for drug benefit insurance can no longer keep pace. British Columbia Health Services Minister Colin Hansen has reported that BC's costs are accelerating at 16% per year (currently $110 million/yr). This is a growth of $300,000 per day. Such increases are bringing the pharmaceutical budgets of all provinces under increased scrutiny. Unfortunately, cost concerns distract drug plans from their mission to improve the health of beneficiaries, by optimizing pharmaceutical delivery based on effectiveness, safety and cost implications of drug therapy. Drug plans need researchers to take the initiative and conduct policy-relevant research concerning the effectiveness, safety and cost management of prescription drugs.

Canada is faced with mounting challenges in reforming prescription drug policies to address issues arising from the rapid evolution and increasing use of drug therapy. New prescription medications have produced important health benefits. Chief among these benefits is a reduction in the need for some intensive surgeries and improved management of diseases and symptoms. The Romanow Report reminds readers that benefits of new drug therapies will "only be fully realized if prescription drugs are

integrated into the system in a way that ensures they are appropriately prescribed and utilized and that the costs can be managed". The report also highlights two prescription drug policy issues to be addressed in the near future: (1) improving access to medications and ensuring that Canadians can afford the medications they need; (2) improving the quality, safety and cost-effectiveness of prescription drugs.

Policy Alternatives

- **Produce evidence to inform policy making**
  Pro: Producing evidence will allow decision makers to make more cost-effective decisions that are jurisdictionally relevant.
  Con: (1) Producing evidence requires staff and resources to gather, synthesize and evaluate evidence; (2) decisions may have to be delayed while evidence is gathered.
- **Utilize existing evidence**
  Pro: Utilization data can be readily obtained.
  Con: (1) Existing evidence may not reflect population to be treated; (2) data from across different jurisdictions may be difficult to compare; (3) decisions made in the absence of data can be costly – reliable evidence is needed to improve health care quality and support efficient use of limited resources.

Policy Choice

Production of evidence where definitive evidence of drug effectiveness is NOT available.

Method(s) for Policy Implementation

- **Randomized Controlled Policy Trials (RCPTs)**
  Pro: (1) RCTs are the gold standard among methods to assess drug effectiveness; (2) patient health outcomes as well as costs to the health system can be evaluated.
  Con: (1) Requires expertise in RCT methodology and evaluation; (2) policy decision will be delayed until results are evaluated.
- **N – of –1 Trials**
  Pro: (1) Useful to determine individuals who benefit from therapy when a majority of patients may not derive clinically meaningful benefit; (2) provide a blind test of individual patient response – funding decisions are made on the basis of patient outcome (effectiveness and/or harm).
  Con: (1) Requires drug plan staff time to implement monitor and evaluate trials; (2) individual patient funding approach runs counter to pharmaceutical industry marketing practices.

- **Incentive Trials**
  Pro: An incentive package targeted to physicians can improve cost-effectiveness of prescribing and relieve pressure of a drug benefit plan's "prior authorization" process.
  Con: Requires monitoring of physician prescribing in the intervention and matched control groups to calculate relative savings.

- **Observational or Cross-Jurisdictional Utilization Comparison (Drug Utilization Evaluation)**

  Pro: Utilization data can be readily obtained from many Canadian jurisdictions.
  Con: (1) Evidence of drug effectiveness from pre-market studies comes mainly from studies of middle-aged patients with few co-morbidities and may not reflect post-market use (2) comparing data from different jurisdictions may not be appropriate depending on the questions asked.

Results of Policy Impact Assessment

- **Interviews with Provincial Drug Plans**

In the absence of data drug plan executives: (1) learn from experience in other jurisdictions; (2) institute controls at the patient level e.g. prior authorization; (3) institute controls at the "pharmaceutical company level" to limit drug plan expense; (4) conduct in-house utilization reviews (5) respond to reviews initiated by researchers or disease groups.

Drug plan managers want: (1) clinically meaningful post-marketing safety monitoring by federal authorities; (2) collaborations with credible, independent academic teams; (3) enhanced communication between researchers and decision makers to ensure timely response to program concerns.

- **Results of Patient/Physician/Pharmacist Focus Groups**

Patients believe that governments need better evidence upon which to base decisions. When governments make decisions regarding drug benefits they should consider: (1) evidence from clinical trials; (2) safety profile of the medication; (3) number of people who would benefit; (4) condition for which the drug will be a treatment; (5) cost to the drug plan and (6) alternative available therapy.

Clinicians believe that before decisions are made governments need to consider: (1) drug effectiveness and safety; (2) quality of the evidence available; (3) compliance issues; (4) indication for drug use; (5) cost to the drug plan and (6) experience in other jurisdictions. Clinicians believe RCPTs can be successfully conducted if there is good communication among stakeholders and the trial has been designed by experts in close collaboration with decision makers.

- **Results from a biomedical primary literature search**

A literature search found few relevant published articles in this area of policy research. Articles that have been published show that drug plans can reduce costs by implementing formulary restrictions, prior authorization programs, or limiting coverage based on income or amount of service provided. More health outcome studies of drug policy restrictions are needed.

Implications of Policy

- Researchers should collaborate with drug plans to produce evidence of policy impacts.
- Templates for evidence producing research strategies, relevant to drug plan decision makers, are necessary.
- A guidebook for drug plan executives and drug benefits committees is a first step to providing a framework upon which evidence production strategies can be further integrated into provincial drug policy decision making.

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In addition to the above Summary, the full report can be accessed in the following ways:

- The print version of the full report can be obtained in the language of submission from the Health Canada Library through inter-library loan.
- An electronic version of the report in the language of submission is available upon request from Health Canada by e-mailing rmddinfo@hc-sc.gc.ca.

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