Health Policy Research Program
Final Report Executive Summary

Title:	**Evaluation of an Integrated Model and Iterative Loop for Assessment of Drug Effectiveness in the "Real World"**
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Executive Summary

In April 2001, Health Canada, through the Health Policy Research Program, provided funding to identify policy options and methodologies leading to assessment of drug effectiveness in Canada. Drug *effectiveness* is a measure of the extent to which a specific drug--when deployed in everyday clinical settings rather than in highly controlled research contexts--does what it is intended to do for a specified population. In today's context, the necessity for ascertaining the effectiveness and safety of a new drug is important for giving physicians and patients an idea of how drugs compare in their benefit and risk.

Regardless, the original imperative for the research still stands:

- How could information on drug effectiveness be used in establishing and revising formulary listings? As effectiveness information improves, what implications could this have for pharmaceutical policy development? For example: limiting access and second line approval for certain prescription drugs; and developing a common F/P/T drug review process.
- What are the strengths and weaknesses of existing analytical methods and sources of data for measuring drug effectiveness in the post-approval setting i.e., not in pre-Notice of Compliance clinical trials. How can these be improved to yield useful evidence for policy making? What changes to federal and provincial regulatory processes would be required?
- How is information on drug effectiveness currently communicated to policy makers, prescribers and consumers? How can this communication be improved to ensure evidence-based decision-making?
- Who/what types of organizations should be engaged to carry out effectiveness analyses?

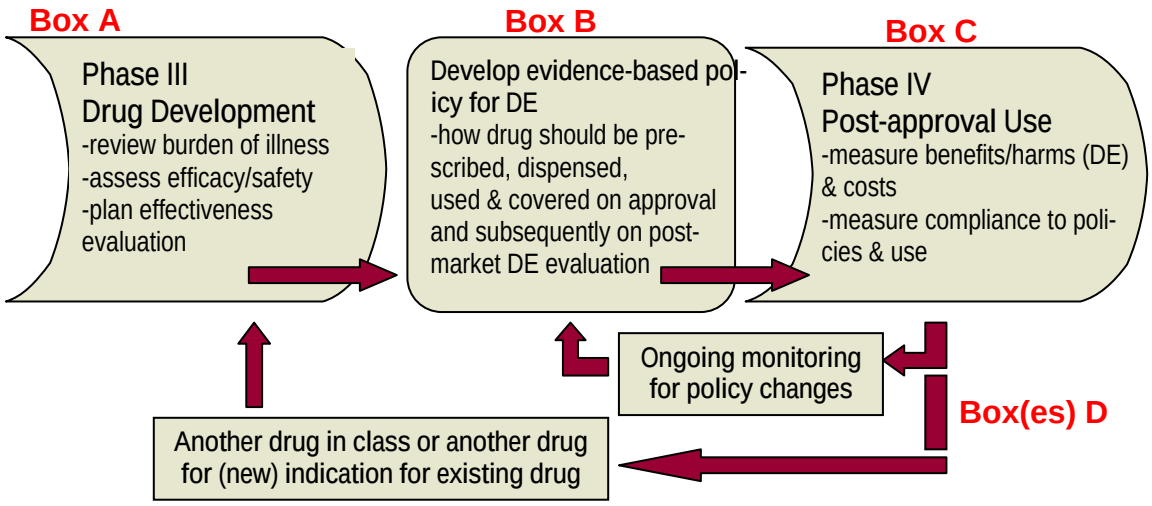
Four universities drew on their combined capacity to undertake a project which would develop and test strategies for assessing drug effectiveness. The project assessed a systematic model for evaluating whether a drug or classes of new drugs are effective. In turn, the project determined

what kind of resources that are required to undertake the task of determining whether drugs really 'do' work in the "real world" using ways that are systematic and nation-wide.

Health services researchers who concentrate on the use of pharmaceuticals in Canadians and hail from British Columbia, Manitoba, Quebec and Nova Scotia used a variety of methods to answer key questions regarding the assessment of drug effectiveness in the "real world". We concentrated, specifically, on two classes of drugs: anti-inflammatories (used for arthritis) and drugs used for the treatment and prevention of osteoporosis. This national team of researchers:

(1) Systematically put together the evidence to answer: How information on drug effectiveness could be used in establishing and revising formulary listings?
(2) Analyzed computerized administrative claims databases to answer: What are the strengths and weaknesses of existing analytical methods and sources of data for measuring drug effectiveness?
(3) Evaluated the process of having 78 community-based pharmacists follow-up with persons taking new drugs to establish whether the drug has worked or caused side effects and
(4) Administered a series of surveys to see what it would take for policy-makers to use drug effectiveness assessments to make drug insurance coverage decisions.

The following was our visual guide in placing the research on drug therapy effectiveness in the 'real world'.



Sub-study	*Box*	*Policy Implications*
A. Case Studies	A	The 1st step in the integrative/iterative model establishes a plan (through case study) for analyzing effectiveness; this would be undertaken during a drug's pre-approval period. The case studies inform on how to measure benefits and harms (post-approval) using population-based analyses (sub-study B) & field studies (sub-study C).

Sub-study	*Box*	*Policy Implications*
B. Population-bases Analyses	C	The 3rd step tested in the model helps to inform policy makers on how closely consensus guidelines on how the drug should be used are followed by the prescriber and the patient; within the limits of this data there is a characterization of the populations who actually use the drugs in the "real" world.
C. Field Study	C	The 3rd step tested in the model also helps to inform about the post-approval *persistence in use of the drug*, *adherence to dosing instructions*, *tolerability of the drug*, and the characterization (beyond that available using administrative databases) of the populations who actually use the drugs in the "real" world.
D. Uptake & Adoption Surveys	B/D	The 2nd & 4th steps tested help to inform us as to how use of a systematic means of evaluating drug therapy effectiveness (the integrative/iterative loop model) would be accepted in the development of policy and implementation of optimal pharmaceutical use strategies.

Our Findings:

- One needs a strong case for monitoring therapeutic effectiveness and safety including an evidence-based synthesis process. An evaluation plan can be drafted in a month based on established criteria; ideally, a broadly-based case for undertaking the evaluation should be made

- A system for ***pharmacist-based surveillance of drug effectiveness and safety*** in a community pharmacy setting can be undertaken.

- Provinces have variable access to administrative data to undertake therapeutic effectiveness and safety studies based on quantifiable endpoints and comorbidity status. It is often difficult to undertake timely and person-level analysis that is comparative across different provinces

- Decision-makers need access to comparative effectiveness information and to persons with better skill sets to be able to interpret the information on effective and safe therapeutic outcomes.

Our recommendation

- Establish a central coordinating office for facilitating ***synthesis*** on comparative effectiveness of pharmaceuticals and ***research*** into therapeutic effectiveness and safety on an ongoing basis.
    - Ensure its independence from pharmaceutical manufacturers and the agency which approves the drugs

The views expressed herein do not necessarily represent the views of Health Canada

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

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

This research has been conducted with a financial contribution from Health Canada's Health Policy Research Program. For permission to reproduce all or part of the research report, please contact the Principal Investigator directly at the following address: Richard.crilly@sjhc.london.on.ca.

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