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Conditionally Funded Field Evaluations: PATHs Coverage with Evidence Development Program for Ontario

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Introduction

Faced with escalating health-care costs, in 2003 the Ontario Ministry of Health and Long-Term Care (MOHLTC) decided to embark on a new evidence-based platform for decision-making around medical devices, procedures, and programs. This new venture was predicated on the belief that assessment of technologies using a more systematic and rigorous process could improve efficiencies in the health-care system, potentially control rising health-care costs, and ultimately improve the overall health of Ontarians. In the case where the evidentiary base for a new technology is strong and fairly conclusive, making recommendations about reimbursement, implementation, or uptake of the technology is relatively straightforward. However, what if the evidentiary base is of poor quality, conflicting, not based on "real world" effectiveness studies or there are concerns about implementation and uptake of the technology for a particular jurisdiction? For example, economic evaluation evidence may exist, but because of known differences in unit costs, practice patterns, or patient preferences across jurisdictions, this might affect the transferability of economic evaluation data across jurisdictions. There may even be concerns about the generalizability of clinical evidence from other jurisdictions for local decision-making needs. For example, differences in patient characteristics like demographics or rates of compliance with therapies, or provider characteristics such as level of expertise or training, or health-care system characteristics like payment incentives or available infrastructure, can all affect whether, and to what extent, a technology works in a particular jurisdiction. In these cases, assessing the technology using local context-specific data collection may be necessary.

The Programs for Assessment of Technology in Health (PATH) Research Institute is the longest established group undertaking conditionally funded field evaluations (CFFEs) of health-care technologies in Ontario. CFFEs are safety, efficacy, effectiveness, or cost-effectiveness studies conducted in the "real world" (i.e., more pragmatic) and where funding for the technology or use of the technology is conditional on sites or professionals participating in data collection for evaluation purposes. There are other groups in Ontario conducting CFFEs and each research group not only addresses slightly different levels of decision uncertainty, but each group also approaches and conducts CFFEs in slightly different ways. The CFFE process used by PATH, illustrative examples of completed CFFEs and their impact on policy and reimbursement in the province are discussed. Finally challenges for government and researchers are highlighted with some conclusions for moving forward.

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Ontario's Evidence-Based Health Technology Assessment (HTA) Process

An overview of Ontario's evidence-based HTA process and the role of CFFEs are provided in Figure 1. The process begins when a health-care organization, health-care facility or health-care provider requests that the MOHLTC consider purchasing or reimbursing a new technology in the province. The funding requests for surgical or diagnostic procedures, devices or products, or new programs or services are submitted to a division of the MOHLTC called the Medical Advisory Secretariat (MAS), which conducts an initial scan of the technology and prioritizes using a standardized scoring algorithm. This initial scan and scoring is then presented to the Ontario Health Technology Advisory Committee (OHTAC), which meets once a month to review evidence around technologies and makes recommendations to the Deputy Minister of Health. OHTAC was formed in 2003 to create an evidence-based single point of entry for the uptake and diffusion of health technologies in the province and consists of clinical epidemiologists, clinicians, health economists, health policy analysts, health services researchers, bioethicists, senior hospital administrators, and representatives from the Ontario Hospital Association, the Ontario Medical Association, Medical Device Manufacturing Association, and community-based health-care programs. Based on the initial scan and prioritization, OHTAC may reject the request for review, request more information or may decide that MAS proceed to conduct a Health Technology Policy Analysis (HTPA) around the technology. An HTPA is completed internally by MAS within 16 weeks, where the technology's safety, efficacy, effectiveness, and cost-effectiveness are reviewed. Guided by a rating of the technology using the Grades of Recommendation, Assessment, Development and Evaluation (GRADE), the evidence around the technology is deliberated by OHTAC at which point OHTAC may make a policy recommendation regarding the uptake and diffusion of the technology. OHTAC may also conclude that there is not enough information to make an evidence-based decision, and recommend that a CFFE be undertaken.

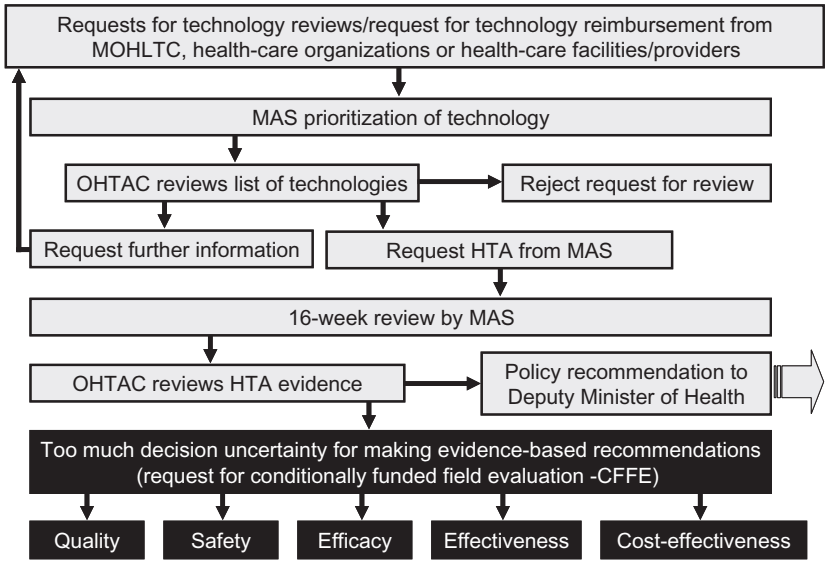

Figure 1 Overview of Ontario's evidence-based HTA process and the role of conditional funded field evaluations. CFFE: Conditionally Funded Field Evaluation, HTA: Health Technology Assessment; MAS: Medical Advisory Secretariat; MOHLTC: Ministry of Health and Long-Term Care; OHTAC: Ontario Health Technology Advisory Committee [1].

Role of CFFEs in Ontario's Evidence-Based Process

As shown in Table 1, OHTAC's decision uncertainty around a technology may be based on a lack of conclusive evidence on quality, safety, efficacy, effectiveness, or cost-effectiveness. These categories of uncertainty are akin to what are referred to as the "hurdles for reimbursement" decision-making for drugs. For example, there may be concerns that the technology may potentially harm patients or health-care providers and, in this case, a CFFE may be conducted to assess safety or develop guidelines or standards of practice for use of the technology. Similarly, there may be concerns about whether the technology could work even under ideal experimental trial conditions and, in this case, a CFFE may be recommended to assess the efficacy of the technology (e.g., an explanatory randomized controlled trial (RCT)). There may also be concerns about whether the technology will work in "real world" practice and it may be recommended that a pragmatic RCT or observational study be undertaken. And finally, there may be concerns over value for money of the technology and it may be recommended that a cost-effectiveness analysis be undertaken where resource use, practice patterns, unit costs, and patient preference information are collected to help reduce uncertainty.

Once a CFFE is commissioned, a study team is put together based on key opinion leaders in the province and a protocol is developed to collect the evidence needed to reduce decision uncertainty. The CFFEs not only vary in the uncertainty being addressed, but also vary considerably in terms of study design, outcomes measured, study duration, sample size, and site participation. To date, CFFEs have ranged in duration from about 1 to 4 years from study initiation to completion.

Examples of PATH's Completed CFFEs

The PATH Research Institute has been in existence since 2003. During the pilot phase of this program, PATH initiated three CFFEs per year and is now initiating four new CFFE per year. Most of the CFFEs conducted by PATH have either been efficacy, effectiveness, or cost-effectiveness evaluations; however, PATH has also been actively involved in developing general disease policy models, which can be used to assess the cost-effectiveness of a number of alternative treatment alternatives at the same time using a common disease modeling platform. For illustrative purposes, examples of two completed CFFEs are presented below, along with their subsequent impact on policy in the province.

Table 1 Attributes of technologies, questions asked, uncertainty faced by decision makers, and type of conditionally funded field evaluations used to reduce decision uncertainty [2]

Attribute of technology	Questions HTAs typically address	Typical uncertainty in decision-making	Types of CFFEs used to address uncertainty
Quality	Is the technology consistent and of high quality?	Lack of quality evidence or inconsistency in quality of the technology	Technology quality assurance assessments
Safety	Does the technology harm patients or health-care professionals?	Safety concerns in general or in context-specific application of the technology	Safety assessments, development of guidelines or standards of practice
Efficacy	Can the technology work under ideal experimental trial conditions?	Poor quality evidence, lack of evidence, or conflicting evidence of efficacy	Explanatory RCTs
Effectiveness	Does the technology work in "real world" practice?	Concerns over generalizability of efficacy evidence or transferability of clinical evidence from another jurisdiction	Pragmatic RCTs, observational studies (e.g., cohort, registries)
Value for money	Is the technology cost-effective Compared with alternative ways of treating patients?	Concerns over transferability of economic and patient preference evidence from another jurisdiction	Effectiveness studies including collection of resource use, practice pattern, unit cost and patient preference information

CFFE, conditionally funded field evaluation; HTA, health technology assessment; RCT, randomized controlled trial.

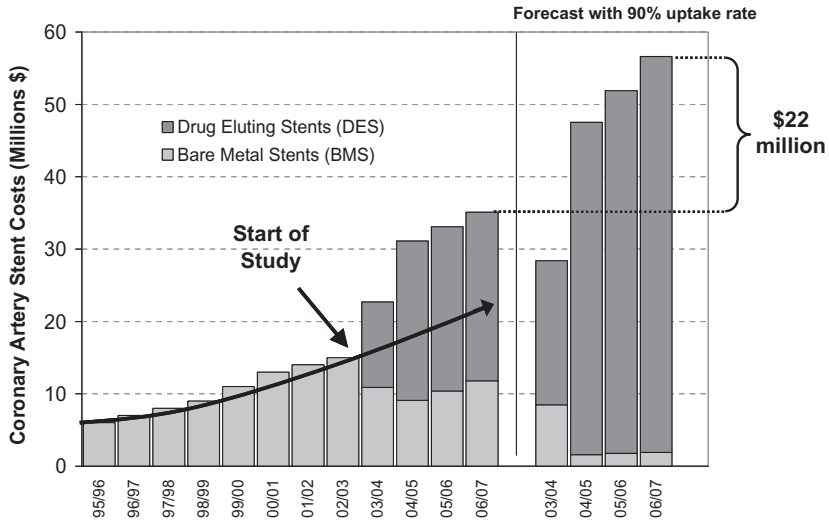

Figure 2 Illustrative example of monetary impact of CFFE research in Ontario: Actual and projected expenditures ($Cdn) on bare metal and drug eluting stents in Ontario, by year [2].

Drug-Eluting Stents vs. Bare Metal Stents for Coronary Artery Disease

Early trials of drug-eluting stents (DES) suggested that this was a very effective new technology. These data led to considerable pressure from health professionals and professional associations on government to comprehensively replace bare metal stents with DES. It was suggested that an immediate substitution of the technologies in the range of 60% of cases, followed by a gradual phase-in over a few years to achieve 100% usage of the new technology, would be appropriate. However, based on reports in the literature, OHTAC had concerns about the generalizability of the clinical trial results to patients in the "real world." To address these concerns, PATH, in combination with clinical opinion leaders in the province and key stakeholders conducted a study where DESs were conditionally funded in a large study of almost 20,000 patients to determine the relative effectiveness of DES in different patient populations. The study was designed based on input from key clinical experts, from the Ontario Cardiac Care Network and from consultation with both suppliers of DESs in the province at the time of the study. It was found that the effectiveness of DES varied by patient characteristics and based on these study results, OHTAC recommended that DES should be offered to high-risk patients only (i.e., patients with diabetes and long or narrow lesions).

The policy impact of OHTAC's recommendation was immediate and funding was built into subsequent year budgets. Expenditures on bare metal and DES in the province prior to, and during, the CFFE are shown in Figure 2. Expenditure forecasts based on stent volumes in the province in the absence of DES funding are shown as the trend time line, while actual expenditures, including the CFFE, are shown as cumulative bar charts. As a result of OHTAC's recommendations based upon the findings from the CFFE, funding for stents in the province was $38 million in 2006–2007. This level of funding contrasts sharply to what the province would have been spending on stents without controlled diffusion of the technology. Based on uptake rates of 90% (i.e., similar to what happened in the United States with no diffusion control), Ontario spent $22 million less on stents for the 2006–2007 fiscal year alone (as shown in the second panel in Fig. 2). Compared with the cost of conducting the study at approximately $0.5 million, the benefits in terms of cost-savings for 2006–2007 alone were substantial, and this does not even account for potential cost-savings moving forward into future fiscal years.

Endovascular Repair (EVAR) of Abdominal Aortic Aneurisms (AAA)

EVAR was first introduced in 1991 and since then, its use as a treatment for AAA has become widespread. At the time OHTAC considered funding for EVAR in the province, no large RCTs had been conducted comparing EVAR with open surgical repair (OSR) and there were concerns about which patients could benefit most from EVAR (i.e., patients at low or high risk for surgical complications) and what was the long term safety of EVAR. As a result, PATH undertook a CFFE to address these decision uncertainties. It was found that EVAR was slightly cost saving and resulted in improved patient outcomes compared with OSR. Based on these CFFE findings, OHTAC recommended that there should be increased access to EVAR for patients at high risk for surgical complications, including operative mortality. As with DESs, the policy implications of the study happened quickly: EVAR was changed from an uninsured to an insured service; a physician billing code was introduced; hospitals restructured their operations to accommodate EVAR; and hospitals began receiving program-specific funding on top of their global budgets. The overall result of the CFFE for Ontario was a modest saving in health-care spending for the estimated 635 high risk AAA cases per year, while at the same time improving patient overall outcomes and quality of life.

Challenges

Although very successful in changing behavior and influencing policy and funding changes in the province, there have been challenges faced when trying to implement the CFFE program in Ontario and a number of lessons have been learned. These challenges relate to funding, timing, and to the conduct of the studies themselves.

Funding

The biggest challenge for government is finding the resources to fund the infrastructure associated with an evidence-based decision-making platform and process, which can also include

the option of funding for the conduct of CFEEs of technologies with uncertain risks, benefits and costs. For example, the field evaluation program in Ontario is modestly funded at about $250,000 per field study for core support for PATH and an additional $250,000 per evaluation for the field work itself (e.g., research nurses, primary data collection). Although $500,000 per evaluation is a very reasonable cost for conducting primary data collection studies, the overall cost of such a program can become large if there are a number of CFFEs undertaken each year. However, given the political will, there are a number of potential funding sources. For example, although it is well recognized that it is difficult to remove ineffective or obsolete technologies from a health-care system, there is potentially a large source of funds that can be generated through the adoption of an evidence-based process that includes removal of these technologies. Another potential source of funding for field evaluations is from the cost-savings generated by controlled diffusion of a new technology. As demonstrated for DESs, this strategy generated millions of dollars of cost avoidance that could prudently be re-invested back in the CFFE evidence-generating process rather than simply being absorbed by the health-care system. Another potential source of funding is from reduced out-of-province procedures. For example, instead of paying for procedures that are conducted in the United States because they are determined to be "medically necessary" but not available in Ontario, these procedures could be made available in Ontario as part of a CFFE. Finally, financing through cost-sharing in the form of unrestricted grants, or provision of the technology or the supplies associated with the use of technology, on an experimental basis by technology manufacturers could be encouraged. As part of the PATH's CFFE process, industry stakeholders are consulted for advice around possible study designs and use of the technology during the evaluation (e.g., training and level of experience). Possible financial or "in kind" support from industry stakeholders is facilitated if they are consulted early, and throughout, the CFFE process.

Timing

One of the obstacles facing government is timing needed for the conduct of CFFE. Well designed and conducted CFFEs are similar to any clinical trial and often take considerable time before results become available. With a typical three- to five-year political cycle, there is often tension between research and political needs as senior management of government typically try to enforce a quick turnaround of research studies and this is not always conducive to conducting the high quality evaluations that are required to inform health policy. The process benefits hugely from the presence of champions within senior management of government who understand the importance of research and the need for a long-term perspective when conducting the high quality research studies that are needed in order to be accepted by the various stakeholders of the technology.

Research Conduct

The biggest concern from a research perspective is the compromising of scientific rigor because of time pressures and restrictions. By nature, CFFE is a lengthy complex process. It requires the creation of working groups made up of key stakeholders and opinion leaders who are involved in designing the study questions and methods from the beginning of the process. Then there is protocol development, sample size and site determination, case report form development, contracts with sites and investigators and dealing with multiple research ethics board (REB) submissions. For example in one study, almost 100 REBs were involved. It is worth noting that some studies require large sample sizes, some require long follow-up periods and some require a range of outcomes in order to achieve important and valid data, with study initiation often subject to contractual and legal delays.

PATH's due diligence systems require that all studies go through a peer review process, as well as a number of other quality assurance activities that PATH has insisted upon. For example, consistent training of health-care professionals with respect to the technologies has been essential to ensure that the technology is being used safely. At times this may be done with the support of the manufacturer where alternative training programs are not available. In addition, a number of procedures around the actual conduct and reporting of the study results are in place to substantiate the validity of our results in the event that the assessment of the technology is against the perception and desires of key stakeholders associated with the technology. Above all else, the CFFEs have to be relevant, rigorous, conclusive and defensible.

Closing Remarks

Although we have only a limited number of completed CFFEs to date, all of the studies have been very successful, as government, researchers and health-care professionals have all worked in harmony toward a common goal. Furthermore, it is noteworthy that local data collection appears to carry significant weight within the medical community, who accept that "real world" patient cohorts in a specific setting may be significantly different from clinical trial patient cohorts. Very encouragingly, despite a continuous background of tension around funding of field evaluations and time to achieve results, government has been very receptive to policy recommendations arising from CFFEs. Of even more policy relevance is the fact that the Ontario government has responded by changing policy and increased funding for all technologies where OHTAC has provided a positive recommendation based on findings from a CFFE.

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