HEALTHCARE

POLICY

Politiques de Santé

Health Services, Management and Policy Research
Services de santé, gestion et recherche de politique

Volume 10 • Number 2

Data Matters • Discussion and Debate • Research Papers
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Health Services, Management and Policy Research
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VOLUME 10 NUMBER 2 • NOVEMBER 2014

Healthcare Policy/Politiques de Santé seeks to bridge the worlds of research and decision-making by presenting research, analysis and information that speak to both audiences. Accordingly, our manuscript review and editorial processes include researchers and decision-makers.

We publish original scholarly and research papers that support health policy development and decision-making in spheres ranging from governance, organization and service delivery to financing, funding and resource allocation. The journal welcomes submissions from researchers across a broad spectrum of disciplines in health sciences, social sciences, management and the humanities and from interdisciplinary research teams. We encourage submissions from decision-makers or researcher–decision-maker collaborations that address knowledge application and exchange.

While *Healthcare Policy/Politiques de Santé* encourages submissions that are theoretically grounded and methodologically innovative, we emphasize applied research rather than theoretical work and methods development. The journal maintains a distinctly Canadian flavour by focusing on Canadian health services and policy issues. We also publish research and analysis involving international comparisons or set in other jurisdictions that are relevant to the Canadian context.

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Politiques de Santé/Healthcare Policy cherche à rapprocher le monde de la recherche et celui des décideurs en présentant des travaux de recherche, des analyses et des renseignements qui s'adressent aux deux auditoires. Ainsi donc, nos processus rédactionnel et d'examen des manuscrits font intervenir à la fois des chercheurs et des décideurs.

Nous publions des articles savants et des rapports de recherche qui appuient l'élaboration de politiques et le processus décisionnel dans le domaine de la santé et qui abordent des aspects aussi variés que la gouvernance, l'organisation et la prestation des services, le financement et la répartition des ressources. La revue accueille favorablement les articles rédigés par des chercheurs provenant d'un large éventail de disciplines dans les sciences de la santé, les sciences sociales et la gestion, et par des équipes de recherche interdisciplinaires. Nous invitons également les décideurs ou les membres d'équipes formées de chercheurs et de décideurs à nous envoyer des articles qui traitent de l'échange et de l'application des connaissances.

Bien que *Politiques de Santé/Healthcare Policy* encourage l'envoi d'articles ayant un solide fondement théorique et innovateurs sur le plan méthodologique, nous privilégions la recherche appliquée plutôt que les travaux théoriques et l'élaboration de méthodes. La revue veut maintenir une saveur distinctement canadienne en mettant l'accent sur les questions liées aux services et aux politiques de santé au Canada. Nous publions aussi des travaux de recherche et des analyses présentant des comparaisons internationales qui sont pertinentes pour le contexte canadien.

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Telephone: 416-864-9667 Fax: 416-368-4443

ADDRESSES
All mail should go to: Longwoods Publishing Corporation, 260 Adelaide Street East, No. 8, Toronto, Ontario M5A 1N1, Canada.

For deliveries to our studio: 54 Berkeley St., Suite 305, Toronto, Ontario M5A 2W4, Canada.

SUBSCRIPTIONS
Individual subscription rates for one year are [C] $118 for online only and [C] $189 for print + online. Institutional subscription rates are [C] $515 for online only and [C] $678 for print + online. For subscriptions contact Barbara Marshall at telephone 416-864-9667, ext. 100 or by e-mail at bmarshall@longwoods.com.

Subscriptions must be paid in advance. An additional tax (GST/HST) is payable on all Canadian transactions. Rates outside of Canada are in US dollars. Our GST/HST number is R138513668.

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Return undeliverable Canadian addresses to: Circulation Department, Longwoods Publishing Corporation, 260 Adelaide Street East, No. 8, Toronto, Ontario M5A 1N1, Canada.

EDITORIAL
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PUBLISHING
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Healthcare Policy/Politiques de Santé is published four times per year by Longwoods Publishing Corp., 260 Adelaide St. East, No. 8, Toronto, ON M5A 1N1, Canada. Manuscripts are reviewed by the editors and a panel of peers appointed by the editors. Information contained in this publication has been compiled from sources believed to be reliable. While every effort has been made to ensure accuracy and completeness, these are not guaranteed. The views and opinions expressed are those of the individual contributors and do not necessarily represent an official opinion of *Healthcare Policy* or Longwoods Publishing Corporation. Readers are urged to consult their professional advisers prior to acting on the basis of material in this journal.

Healthcare Policy/Politiques de Santé is indexed in the following: PubMed/Medline, CINAHL, CSA (Cambridge), Ulrich's, Embase, IndexCopernicus, Scopus, ProQuest, EBSCO Discovery Service, is archived in PubMed Central, and is a partner of HINARI.

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ISSN No. 1715-6572
eISSN No. 1715-6580

Publications Mail Agreement No. 40069375

EDITORIAL

Learning from Others: International Comparisons of Death and Dying

Death comes to us all, but attitudes towards death vary greatly. Take Dylan Thomas ("Do not go gentle into that good night; rage, rage at the dying of the light") and Elisabeth Kübler-Ross ("Dying is nothing to fear. It can be the most wonderful experience of your life."), as examples.

The Commonwealth Fund's latest International Health Policy Survey (Osborn et al. 2014) also shows that how we prepare for ill health or death varies considerably around the world. In France, for instance, only 12% of seniors living outside of healthcare institutions say that they have had a discussion with family, a close friend or a health professional about the healthcare treatment they want if they become very ill and cannot make decisions for themselves. That compares with 78% in the US, 72% in Germany and 66% in Canada. Across all 11 countries included in the survey, the proportion of seniors who said that they had a written plan describing treatment that they want at the end of life was lower. It ranged from 4-5% in Norway, Sweden and France to 46-58% in Canada, the US and Germany.

Interestingly, there is much less variation in the settings in which older adults die. A 2013 article (Broad et al. 2013) compiled the latest published rates of place of death for 45 populations, including data for 10 of the 11 countries included in the Commonwealth Fund survey. Among these countries, the authors found that deaths in hospital or residential aged care facilities varied from 65% in New Zealand to 80% in Australia.

In both cases, as always, caution is required in interpreting international comparisons. Nevertheless, they do provide a prompt for a number of important policy conversations – comparisons being a type of mirror through which we have the potential to better understand ourselves.

Authors in this issue of *Healthcare Policy/Politiques de Santé* use these and many other techniques to illuminate important policy, research and practice questions facing the health sector today. These range from how to capture patient perspectives on primary and community care outcomes to drivers of spending on prescription medications. Also in this issue are contributions on how best to share and apply new knowledge, including an online repository of documents addressing healthcare renewal and the research–policy divide for oral health inequality.

Whatever your role in health policy, I hope that the thoughtful commentary and new research published in this issue of *Healthcare Policy/Politiques de Santé* offers interesting new insights and perspectives for you.

JENNIFER ZELMER, PHD
Editor-in-chief

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ÉDITORIAL

Apprendre d'autrui : comparaisons internationales sur la mort et le mourir

La mort nous attend tous, mais notre attitude face à elle varie grandement. Prenons seulement comme exemple Dylan Thomas (« N'entre pas apaisé dans cette bonne nuit / Mais rage, rage encor lorsque meurt la lumière »[1]) et Elisabeth Kübler-Ross (« Il ne faut pas avoir peur de la mort. Elle peut être l'expérience la plus merveilleuse de votre vie. »).

La dernière Enquête internationale du Fonds du Commonwealth sur les politiques de santé (Osborn et al. 2014) démontre, pour sa part, que la façon de se préparer à une mauvaise santé ou à la mort varie considérablement d'un pays à l'autre. En France, par exemple, seuls 12 % des aînés qui vivent hors des institutions de soins de santé indiquent qu'ils ont parlé avec leur famille, un proche ou un professionnel de la santé des traitements qu'ils souhaitent recevoir s'ils tombaient très malades et n'étaient plus en mesure de prendre des décisions en leur nom. Aux États-Unis, cette proportion s'élève à 78 %, alors qu'elle est de 72 % en Allemagne et 66 % au Canada. Parmi les 11 pays de l'enquête, le nombre d'aînés qui indiquent avoir mis par écrit la description des traitements auxquels ils consentent pour la fin de vie est moins élevé. Cela va de 4-5 % en Norvège, en Suède et en France à 46-58 % au Canada, aux États-Unis et en Allemagne.

Il est intéressant d'observer qu'il y a moins de variation dans le type d'établissements où les aînés finissent leurs jours. Un article de 2013 (Broad et al. 2013) recense les taux publiés sur les lieux de mort dans 45 sociétés, notamment 10 des 11 pays compris dans l'enquête du Commonwealth. Parmi ces pays, les auteurs notent que les décès à l'hôpital ou dans les établissements pour aînés varient de 65 % en Nouvelle-Zélande à 80 % en Australie.

Dans les deux cas, comme toujours, il faut montrer une certaine prudence au moment d'interpréter et de faire des comparaisons entre pays. Néanmoins, ces chiffres donnent une idée de l'ampleur des débats au sujet des politiques – les comparaisons étant une sorte de miroir qui permet de mieux se comprendre.

Les auteurs qui contribuent à ce numéro de *Politiques de Santé/Healthcare Policy* emploient ces techniques, et bien d'autres, pour éclairer d'importants enjeux auxquels fait actuellement face le secteur de la santé, que ce soit sur les politiques, la recherche ou la pratique. Les thématiques portent sur plusieurs sujets, allant de la façon dont on évalue l'état de santé du point de vue du patient dans les établissements de soins primaires et communautaires jusqu'aux moteurs du gonflement des dépenses pour les médicaments sur ordonnance. Le numéro

présente également des articles sur les meilleures façons de partager et de mettre en pratique les nouvelles connaissances, notamment au sujet d'un répertoire en ligne de documents ayant trait aux politiques du renouvellement des services de santé ou encore au sujet des disparités entre la recherche et les politiques dans les inégalités en santé buccodentaire.

Quel que soit votre rôle dans le milieu des politiques de santé, j'espère que les réflexions et les nouvelles recherches publiées dans le présent numéro vous mettront sur de nouvelles pistes et vous offriront des points de vue intéressants.

JENNIFER ZELMER, PHD
Rédactrice en chef

Note

1. Translation by Lionel-Édouard Martin, http://lionel-edouard-martin.net/2012/07/05/dylan-thomas-nentre-pas-apaise-dans-cette-bonne-nuit-do-not-go-gentle-into-that-good-night/

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"Frankly, My Dear, I Don't Give a Damn"

Franchement, ma chère, je m'en fous

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Abstract

Four years ago, Michelle Holmes, Wendy Chen and collegues reported a significant negative correlation between aspirin use and breast cancer (Holmes et al. 2010). This summer, they noted that no randomized trials have been initiated that test this potentially important association. Why not? Pharmaceutical companies fund most drug research; there is no profit in aspirin. This explanation is incomplete. The deeper issue is a mismatch between the public interest in advancing research, and the interests of the institutions that governments subsidize in different ways for that purpose. In addition to patent protection, governments directly fund public granting agencies and provide the tax relief offered by private charities. Like pharmaceutical companies, these have their own "stakeholders" and objectives. Nobody, it appears, is interested in aspirin.

Résumé

Il y a quatre ans, Michelle Holmes, Wendy Chen ses collègues faisaient état d'une importante corrélation négative entre l'utilisation de l'aspirine et le cancer du sein (Holmes et al. 2010). Cet été, elles notaient qu'aucun essai aléatoire n'avait encore été amorcé pour tester ce lien important. Pourquoi? Ce sont les sociétés pharmaceutiques qui financent la plupart des recherches sur les médicaments; or, il n'y a aucun profit à tirer avec l'aspirine. Cette explication est incomplète. L'enjeu central est un décalage entre l'intérêt public pour la recherche avancée et les intérêts des institutions que les gouvernements subventionnent à cette fin, de diverses façons. En plus de la protection des brevets, les gouvernements financent directement des organismes subventionnaires publics et offrent un allègement fiscal grâce au statut d'organisme de bienfaisance. Tout comme les sociétés pharmaceutiques, ces organismes ont leurs propres « parties prenantes » et leurs propres objectifs. Il semble bien que personne ne s'intéresse à l'aspirine.

Amazing stuff, this willow bark. Its pain-killing properties have been known since ancient times – do the chimpanzees also know about it? More recently, it has been found to be protective against heart attack. For a time, the research evidence pointed towards significant benefits from taking a quarter tablet (80 mg) daily. Later work refined this recommendation to suggest that the benefits are found only among those who have already shown indications of heart disease; the rest of us can pass up the baby aspirin for now. But we are still advised to chew a tablet while calling 911 if we feel a heart attack coming on.

Hangovers, Heart Disease and … Breast Cancer?

But wait! There's more. In an op-ed piece in the *New York Times* this past May, Michelle Holmes and Wendy Chen (2014) state: "We believe that it might be possible to treat breast cancer … with … Aspirin." The authors – physicians and faculty members at the Harvard Medical School – have some basis for their belief: "In 2010, we published an observational study in the *Journal of Clinical Oncology* showing that women with breast cancer who took aspirin at least once a week for various reasons were 50 percent less likely to die of breast cancer." That's big stuff. Holmes and Chen then note that a subsequent British study assembling results from a number of clinical trials of aspirin to prevent heart disease found that aspirin was also associated with a significantly lower risk of breast cancer death.

So far, so good. But observational studies are not randomized controlled trials (RCTs). And there are those who believe, or at least act as if they do, that there is no truth without trial. Everything short of the RCT gold standard is just speculation. So where are the RCTs?

The Root of All Evil … Again

Where indeed? Breast cancer is not exactly a minor threat. In Canada it is (excluding skin cancers), by far, the leading form of cancer among females (Canadian Cancer Society's Advisory Committee on Cancer Statistics 2014). The age-standardized (female) incidence rate in 2014 is estimated to be 99.2 per 100,000 population, more than double the 47.7 rate for lung cancer at #2. (Lung cancer is, however, much more lethal, with an estimated female mortality rate in 2014 of 35.6 per 100,000 population, double the 18.4 for breast cancer.) The prospect of a highly effective treatment, with minimal side effects and costing literally pennies, should have stimulated an immediate mobilization of research to confirm the benefits of aspirin treatment, map out their scope and limitations, figure out the most effective ways of structuring the therapy, and perhaps (but less importantly) determine how and why it works. (If it does work.) That is not happening.

The authors' explanation is a simple one, and surely correct, though incomplete: "Clinical trials are typically conducted on drugs developed by labs seeking huge profits. No one stands to make money off aspirin …" (Holmes and Chen 2014). You cannot patent willow bark, and the German patents on acetylsalicylic acid (ASA) were given up as part of the Treaty of Versailles.

The implication that it is all the fault of Big Pharma drew a rather huffy response in a letter to the NYT from a certain John L. LaMattina of Stonington, Connecticut (helpfully identified by the *Times* as the former president of Pfizer Global Research and Development): "I was dismayed to read Michelle Holmes and Wendy Chen's assertion that part of the blame for not pursuing aspirin as a potential breast cancer treatment rests at the feet of the companies." Their remarks about the role of labs seeking huge profits "perpetuate the belief that pharmaceutical companies alone should be pursuing this line of research" (LaMattina 2014).

Mr. LaMattina does have a point. Holmes and Chen are of course perfectly correct that clinical trials are typically carried out by labs seeking (hoping for) huge profits. And they did not in fact say such research should be carried out by pharmaceutical companies alone – quite the contrary – although, as Willie Sutton famously said of banks, "That's where the money is." Nevertheless, we cannot be too often reminded that pharmaceutical companies are not "eleemosynary outfits." They are strictly for-profit corporations, responsible to their shareholders for maximizing "enterprise value" – a polite term for profit. That rules out spending even small numbers of millions of dollars on research that has little or no prospect of returning a profit. Not our department – let George do it, with someone else's money.

This response does, however, raise a deeper issue. The pharmaceutical industry enjoys the enormous privilege of delegated state authority to suppress normal market competition for its patented products. The industry's corresponding exceptional profits arise from the extraordinary prices, relative to costs of production, that drug patents make possible. These prices, in turn, support not only research, but also – in equal or greater measure – exceptional marketing expenses, including political activities to protect the industry's privileges.

The justification for this extraordinary privilege is the presumed public benefit that flows from the innovative activities of the industry. Patents are intended to induce the industry to "do well by doing good." The public benefits from pharmaceutical innovation are certainly real, although typically greatly overstated by the industry's marketing and public relations. The proportion of true "therapeutic breakthrough" drugs is surprisingly low, compared with the much more extensive activity devoted to "new" products – "me too" molecule manipulation or new combinations of existing drugs, or line extensions, to maintain and extend market position.

While the value of incentives to innovation may be accepted, a number of commentators have pointed out that there could be other ways of inducing innovation that would provide a better balance of public and private benefits. A different mix of policy instruments might result in the industry's doing more good while doing less well (at public expense). One could, for example, scale the degree of market protection to the therapeutic significance of the innovation. Joseph Stiglitz (2012) has suggested a system of prizes, potentially very large for the true therapeutic breakthroughs. Either of these approaches might steer research resources more towards greater benefits for patients rather than for shareholders of Big Pharma. The determination with which the industry has fought off any such proposals for modifying the patent system is testament to how the industry views the current balance of benefits.

But this is a larger topic. The point to note here is that Mr. LaMattina seems perfectly comfortable dismissing the notion that pharmaceutical companies have any social responsibility over and above their duty to their shareholders. If he has any sense that the highly profitable privileges conferred on these companies might carry a corresponding obligation to support research in the public interest, it does not show in his letter.

Now, of course, pharmaceutical firms operate within an extensive web of rules, of legislation and regulation, that impose obligations upon them over and above their duty to their shareholders. This framework is in principle intended to protect public interests that are not necessarily parallel with those of shareholders (or of senior management). But pharmaceutical companies have on numerous occasions not merely ignored the public interest; they have actively flouted it – doing well by doing bad. Pfizer, the company in which Mr. LaMattina was formerly a senior executive, has a long history of criminal convictions for violations of American law in particular, and currently holds the record, $2.3 billion, for fines and penalties arising from these convictions (Harris 2009). But, as in the case of the American banking system, no individuals have been held responsible for what the courts have clearly found to be criminal behaviour. No one goes to jail. So criminal behaviour will persist whenever it is profitable enough to cover any resulting financial penalties and associated legal expenses. Those are just part of the cost of doing business.

La trahison des experts

In this context, it is not hard to understand why there are no company-funded trials of aspirin for breast cancer. Big (and little) Pharma is in the business of producing profits, not knowledge. (Or even of producing drugs. Drugs are simply means to the ultimate end.) That focus is required by the rigorous laws of the market under which companies must operate; if we do not like the result, then we must change those rules. Any attempt to do so, however, will run into fierce opposition from the industry, which rather likes the very favourable regulatory rules they have succeeded in embedding in national laws and international trade agreements.

But what about national governments themselves? There is a view that democratically elected governments, at least, are supposed to act in the general public interest. Sometimes, in fact, they do. Governments channel large amounts of public money directly into medical research, over and above the huge indirect benefits they provide for private pharmaceutical firms. It seems obvious that if aspirin really could significantly improve cancer outcomes, the savings in "blood and treasure" – reductions in mortality, morbidity and health expenditure – could be very large indeed. The case for public funding of a research trial to find out seems compelling, even overwhelming. But it is not happening.

The explanation may lie in the discrepancy between the public interest and the private incentives that drive biomedical researchers and the public agencies over which they exercise substantial influence. Of course, everyone wants to "find a cure for cancer," but Holmes and Chen suggest that there is a strong professional bias towards finding the *right sort* of cure: "[G]eneric drugs, particularly ones as old and familiar as aspirin, just aren't sexy."

> [E]ven as government funding for research is slashed, the government is still willing to test new cancer drugs pushed by pharmaceutical companies, despite very high failure rates for those drugs. Federal grant review panels have no direct financial interest in the studies they approve for funding, but inevitably they are seduced by the more novel treatments – the scientific equivalent of the latest smartphone. (Holmes and Chen 2014)

Nobody is going to win a Nobel Prize for prescribing aspirin; no heroics here.

There is a further important consideration. Successful research teams are not assembled overnight; they can take months or years to develop. Granting agencies know this. You cannot simply turn off the flow of funds to a team one year and turn it back on the next. The people at the core of the team may be gone. So there is a form of co-dependency between research "stakeholders" and the granting agencies that support them. Research teams like to continue doing what they know how to do and are good at. Consistently successful research teams make the agency itself look good and improve its chances in the overall competition for public funds.

This, in turn, may explain the authors' concern for the expense of an aspirin breast cancer trial. Ten million dollars?! That's peanuts – not just for the US, but for just about any public research organization in the high-income world. But if budgets are static or shrinking, those peanuts have to come from someone else's bag, some established research stakeholder, and then things get nasty.

It gets worse still if research funders want to extend their resources by encouraging or even mandating public–private "partnerships," or evaluating research success by whether or not a new patentable product comes to market (hello, universities). The for-profit incentives that inevitably drive private research then determine the allocation of public research dollars as well. But if profit incentives adequately captured the full range of public interests in health research, why would we need public agencies at all?

Furthermore, although the authors do not mention it, there is an indirect financial interest in the medical community at large. The authors note the exceptional costs of present forms of post-surgical treatment (including drugs) to prevent recurrence, as well as their significant side effects. But it is important to keep in mind that none of the expenditures for cancer treatment are paid out to Martians. Every dollar of cost is income to some individual or organization here on Earth. So if aspirin actually works as suggested, the cost savings will all be income losses to someone, as well as lost opportunities to display professional and technological prowess through more advanced treatments.

In normal markets, cost savings can give an organization a competitive advantage. But despite the fantasies (or even deliberate deceptions) of some economists, healthcare never has been and never will be a normal market.

Cold Charities

There is, however, yet another major source of funding flowing into medical research – private charities. As Holmes and Chen (2014) note, a first RCT of the use of aspirin in cancer treatment is in fact underway in Britain, funded by the non-profit group Cancer Research UK. But this study, of four different cancers, will not be completed until 2025. Many people will die of breast cancer over the next decade. The authors believe that a focused trial of aspirin in breast cancer treatment could be completed in half that time. (Even better, it could be American.)

Significantly, however, a large proportion of the funding that flows through "private" charities is in fact public. Donations to organizations approved by the relevant national taxation authority (such as the Canada Revenue Agency) are offset in part by some form of income tax relief for the donor. The resulting reduction in government revenue (known in public finance as a tax expenditure) means that the state is participating along with the private donor in supporting whichever (state-approved) charity the private donor finds most appealing.

These tax expenditures can be quite substantial. A taxpayer in British Columbia, for example, who donated $1,000 in the 2013 tax year would receive a reduction in income tax of $437 – $290 from the federal government and $147 from the provincial government. In effect, then, the tax credit for charitable donations delegates to individual taxpayers the determination of priorities for a substantial chunk of public spending. A good deal of this donor-directed public money goes to medical charities of various sorts, and cancer societies are among the most prominent.

There are thus parallels among the three possible sources of de facto public funding – patent protection for pharmaceutical firms, public research granting agencies and tax credits for charitable donations. In each case, control over public resources is delegated to more or less arm's-length institutions or individuals, whose objectives presumably (hopefully) overlap with a broader public interest.

The funds transferred appear to be in very different forms. Granting agencies appear explicitly as items in public expenditure budgets. Tax credits represent foregone revenues and do not show in the public accounts, although ministries of finance have pretty accurate estimates of their fiscal impact. The fiscal impacts of patent protection are much more diffuse. They show up, obviously, in the higher prices charged to government programs that pay for drugs. But private employer-paid health insurance coverage is heavily subsidized by governments through tax expenditures, and there is also some tax relief available for individuals with exceptionally large drug bills. So some portion of the higher prices paid by apparently private purchasers comes back indirectly as a charge on the public purse.

The public costs of supporting research through the patent process thus flow through a number of channels, mostly well hidden, but well understood by their corporate beneficiaries.

Come back to the charities themselves. They must depend on individuals and corporations, both for private donations, and for the public money that these draw in their train. That

requires a good deal of imaginative and sophisticated marketing, and that costs money. A (surprisingly?) large share of charitable donations goes to support fundraising and administration. (In this, charities resemble private health insurance, where also a large share of revenues goes to marketing and administrative overhead rather than paying for health services.)

But donors do not want to see their donations diverted into overhead and administrative expenses, essential as these may be in the real world of competition for donor dollars. This may be the explanation for the reluctance (refusal) of the Canadian Cancer Society to disclose the salaries of its top executives. *Frank* magazine, a scurrilous, muck-raking periodical (a dirty job, but someone has to do it – there's a lot of muck out there), recently requested this information from the CCS. While the Society is "fully committed to transparency and accountability" (Canadian Cancer Society 2014), that information was not forthcoming. The US Internal Revenue Service, however, requires the filing of this information as a condition of tax-exempt status. (The CRA, politely Canadian, does not.) So *Frank* gleefully published the salary data from the IRS (Kent 2014).

Are these salaries excessive? I don't know, and I see no reason to reproduce them. (Those with a prurient interest can look the article up.) Senior executives in the public and private spheres do earn a lot of money, and their relative incomes have, as we know, been rising steadily over the past three decades. The Canadian Cancer Society is a large and very successful organization, and its senior management may well be worth their pay.

But the key point is that it is a large and successful organization. The Society's annual report for 2012/13, "Life Is Worth Fighting For," shows total revenues of $223.8 million for the year ended January 31, 2013, of which $204.3 million came through various forms of donation. Direct and indirect fundraising expenditures were $90.4 million; along with $7.4 million for administration, these accounted for 43.7% of total revenues. Could $10 million over five years not be found for a study of a possible major breakthrough in cancer treatment? Come to think of it, there is also an American Cancer Society whose budget must be at least ten times that of the CCS. Might they not be interested? Apparently not.

Why not? The reasons may lie in the same limitations that inhibit the public granting agencies. There may appear to be a lot of money in the pot, but it all has some names penciled in beside it. An established stable of distinguished researchers, pursuing the conventionally accepted, most promising lines of inquiry, become agency "stakeholders." None would be pleased to see even $10 million diverted away from their own, very important, research.

Who Needs an RCT, Anyway?

Whatever the reason, the trials are not happening – except glacially in the United Kingdom. But so what? Holmes and Chen are perhaps barking up the wrong willow tree. They are advocating a trial of aspirin as therapy for second- and third-stage breast cancer. But their own study, and the British heart disease study they refer to, does not address therapy for diagnosed disease. Both studies simply show a significant negative association between cancer mortality and a low level of regular aspirin intake – as little as one a week.

The potential weaknesses of associational studies are well understood and not in dispute. But do you need gold-standard evidence before taking action or giving advice? It depends upon who "you" are. If you are an oncologist treating breast cancer patients and relying on experimentally based protocols, you might well want gold-standard evidence before changing your treatment. After all, the fact that breast cancer mortality is so much lower than disease incidence implies that much of treatment is working. (As does the decline in age-standardized mortality by over 40% during the last 20 years [CCS 2014].) It may not be fun, but it keeps a lot of people on the right side of the grass. If you are a woman in the higher-risk age ranges, however, maybe you should be taking a couple of aspirins (with a glass of milk), say, every Sunday morning?

Of course aspirin has risks, including gastrointestinal bleeding, and people who are already taking other blood thinners should perhaps leave it alone. But older males will recall that when we were all being advised to take a baby (one-quarter strength) aspirin a day – or was it every other day? – as protection against heart disease, the side effects were never a major issue.

The dangers of aspirin have been grossly inflated for good commercial reasons. A long and highly successful advertising campaign (with other forms of brainwashing) by Johnson & Johnson has promoted acetaminophen as a safer alternative to aspirin, and their particular branded version, Tylenol®, as *the* drug of choice.

Today, in my local supermarket, 100 standard Tylenol® tablets sell for $9.13; 100 plain aspirin tablets (generic acetylsalicylic acid, or ASA) can be had for $5.39 – if you can find them. They are down on shelf seven, just off the floor, and occupy about four inches of shelf space. Various versions of Tylenol take up about 10 feet of premium space, on the top two shelves. Tucked in among them, less than a foot of space is allocated to generic acetaminophen, at $6.39 per 100.

Now we know that acetaminophen – even good old Tylenol – also has risks. It can seriously damage the kidneys. Well, all drugs have risks. The marketing machine rolls on.

The observations from associational studies, however, raise a very disturbing possibility. Suppose a well-executed RCT showed that regular, low-level aspirin use was to some degree protective against breast cancer? The pro-Tylenol, anti-ASA campaign must surely have significantly reduced the consumption of aspirin over the decades. Has this, then, contributed materially to the incidence of breast cancer?

All the more reason for doing the trial, and doing it soon. While we are waiting for Godot, however, why not advise (hello, CCS) those of the female persuasion to take a couple of aspirins? You are unlikely to do harm, and you might save a few lives.

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The Drivers of Overspending on Prescription Drugs in Quebec

Les moteurs du gonflement des dépenses pour les médicaments sur ordonnance au Québec

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Abstract
According to data from the most recent edition of the *Canadian Rx Atlas*, Quebec was the province with the highest total spending per capita on prescription drugs. The difference between Quebec and the rest of Canada was 35%, which translates into $1.5 billion dollars of extra spending. This analysis explores the economic cost drivers of the higher level of pharmaceutical spending in Quebec. While much of the additional spending was driven by a higher volume of drugs being prescribed overall, the factors contributing to higher spending differed greatly within particular therapeutic categories. The results and their implications are discussed in the context of pharmaceutical policy environment.

Résumé
Selon les données présentées dans la dernière édition du *Canadian Rx Atlas*, le Québec est la province où l'on observe le total des dépenses par personne le plus élevé pour les médicaments sur ordonnance. La différence entre le Québec et le reste du Canada est de 35 %, ce qui équivaut à 1,5 milliards de dollars de dépenses supplémentaires. Cette analyse explore les

inducteurs économiques de coût du plus haut niveau de dépense en produits pharmaceutiques au Québec. Alors que la plupart des dépenses supplémentaires sont induites par un plus grand volume de médicaments prescrits, les facteurs qui contribuent au gonflement des dépenses diffèrent grandement selon les catégories pharmaceutiques. Les résultats et leurs répercussions sont discutés dans le contexte propre aux politiques sur les produits pharmaceutiques.

The recently published *Canadian Rx Atlas* documented vast provincial variations in prescription drug spending (Morgan et al. 2013b). Retail spending per capita on prescription drugs was the highest in Quebec, at 35% above the rest of Canada. As prescription drugs make up one of the largest components of healthcare spending, important questions arise as to why so much more is spent on them in Quebec.

Because average levels of prescription drug use and cost increase as people age, provincial differences in population age might explain some of the difference in prescription drug spending. However, Quebec's population is only slightly older than the Canadian average (per cent of individuals 65 years or older is 16.2 in Quebec and 14.4 in the rest of Canada); the difference in population age alone would only translate into a difference in per capita spending of about 5%, leaving 30% still to be explained (Statistics Canada 2012).

To put that 30% difference in perspective, Quebecers spent $187 per capita more on prescription drugs than other Canadians in 2012/2013. This translates into a price tag of $1.5 billion dollars of additional money spent than otherwise would be the case if per capita spending in Quebec were equal to that for the rest of Canada. The $1.5 billion dollar figure is considerable, given that Quebec's total annual prescription drug retail spending was $6.6 billion.

Another plausible explanation for the higher level of spending might be the health status of the population. Yet Quebec does not stand out either as the sickest or the healthiest population in Canada – generally, their disease rates and health behaviours are comparable to or better than residents of other provinces (INESSS 2013).

If age and health status are unlikely candidates, what drives the extra spending in Quebec? This analysis explores the economic cost drivers of prescription drug spending in the historical context of pharmaceutical policy developments.

Data and Analysis

The data used for this study are derived from data obtained under license from IMS Brogan (IMS Health Canada Inc.). We obtained retail sales volumes for every province from the IMS CompuScript database. We obtained population estimates from Statistics Canada. Sales information from IMS pertains to total retail sales, including all markups and pharmacists' fees. Our analysis does not include drugs sold over the counter, diagnostic agents (e.g. glucose test strips) and devices.

For analysis of provincial variation, we computed the impact of six age-standardized pharmaceutical cost drivers that fall into three broad categories: volume effects, therapeutic choice effects and price effects.

Volume effects relate to the absolute amount of drug therapy received by a population. They are a function of the prescription volume – per capita volume of prescriptions received – and prescription size, the average amount of drug per prescription.

Therapeutic choice effects relate to the average selection of general and specific types of drug per course of treatment. They include choices from both the broad drug classes and from specific drug types within a particular drug class.

Price effects reflect both the use of generics and price paid. Generic use is the average savings generated from using a generic drug as opposed to the brand name alternative. This is influenced by the availability of generics, the relative price of generics versus brands and the extent to which generics are selected when available. The price paid reflects the average price paid per unit of a given brand or generic drug product.

The impacts of cost drivers were computed as per the methods described in the *Canadian Rx Atlas*, which presented impacts in terms of percentage differences in per capita spending between each province and the rest of Canada. Here, we compute the total value of these differences for Quebec by multiplying differences in per capita spending by Quebec's total population.

Results

Table 1 breaks down the extra $1.5 billion dollars spent on prescription drugs in Quebec by cost driver, for all drugs and for select therapeutic categories. Overall, much of the additional spending in Quebec was driven by a higher volume of drugs purchased. Higher per capita volumes of prescription drugs purchased were enough to increase spending in Quebec by $1.2 billion relative to what it would have been at averages observed in the rest of Canada.

Averaged across all drug classes, the types of drugs chosen for Quebecers were slightly less costly than the choices made for other Canadians. Although this held overall, there were a number of therapeutic categories where Quebecers were prescribed more expensive treatment options. In those cases, they were prescribed a more expensive drug within a particular sub-class as opposed to a drug from a different sub-class. For example, it may not be common knowledge that esomeprazole – a proton pump inhibitor used to treat gastrointestinal disorders – costs three times as much as another proton pump inhibitor, pantoprazole, even though both are similar in terms of their therapeutic efficacy (INESSS 2013).

TABLE 1. Drivers of the difference in total spending on prescription drugs between Quebec and the rest of Canada, 2012/2013

Therapeutic category	% difference in spending per capita in QC vs. the rest of Canada*	Total extra spending in QC as a result, $ millions	Driver contrition to difference in spending, $ millions (% total)		
			Higher volume prescribed	More expensive drugs chosen	Higher prices paid
All drugs	30	1,508.8	1,200.3 (80)	-79.3 (-5)	387.8 (26)
Top seven categories where QC spends more					
Antihypertensives	34	180.5	19.4 (11)	17.4 (10)	143.7 (80)
Cholesterol-lowering drugs	46	165.0	76.9 (47)	38.6 (23)	49.5 (30)
Acid-reducing drugs	50	134.4	9.0 (7)	90.6 (67)	34.9 (26)
Gabapentin and pregabalin	118	87.0	29.6 (34)	56.1 (64)	1.3 (1)
Antipsychotics	60	83.3	78.1 (94)	1.1 (1)	4.1 (5)
Drugs for ADHD	106	72.1	65.3 (91)	6.8 (9)	0.01 (0)
Drugs for respiratory conditions	22	67.5	48.6 (72)	34.6 (51)	-15.8 (-23)
Categories where QC spends less					
Insulins	-24	(32.9)**	-17.4 (53)	-7.6 (23)	-8.0 (24)
Opioids	-37	(68.8)**	-88.0 (-128)	4.1 (6)	15.1 (22)

* Age-standardized; ** Savings.

ADHD = attention deficit hyperactivity disorder; QC = Quebec.

Source: Morgan et al. 2013b.

Quebecers paid higher average prices than would have been paid in the rest of Canada. While this was partially a result of higher unit prices for specific brand or generic drugs, an important driver of this is the difference in average length of prescriptions in Quebec versus the rest of Canada: across all drug classes, prescriptions in Quebec were for approximately half as many days of therapy as were prescriptions in the rest of Canada (Table 2).

In addition to higher unit costs, higher average prices in Quebec also reflect lower generic substitution rates relative to the rest of Canada. Overall and in most therapeutic categories, the share of total spending that went to generics as opposed to brand name drugs was higher in the rest of Canada. The most extreme example is antiplatelet drugs, with Quebec spending only 32% on generics in this category compared with 65% spending on generics for the rest of Canada.

TABLE 2. Average length of prescriptions across provinces in Canada, all drugs and top three categories where Quebec spends more, 2012/2013

Province	Estimated average days of treatment per prescription (days)			
	All drugs	Antihypertensives	Cholesterol-lowering drugs	Acid-reducing drugs
British Columbia	44	79	63	48
Alberta	46	79	73	53
Saskatchewan	36	46	39	37
Manitoba	39	59	56	41
Ontario	41	65	61	44
Quebec	***20***	***26***	***29***	***24***
New Brunswick	47	79	75	56
Nova Scotia	51	74	75	60
Prince Edward Island	48	84	58	47
Newfoundland	50	76	70	54

Source: *Canadian Rx Atlas*, 3rd edition

The magnitude of higher spending in Quebec and factors contributing to it were not the same across drug classes. As can be seen in Table 1, Quebecers spent considerably more than the rest of Canada on classes of commonly prescribed drugs. For example, Quebecers spent significantly more on medications for cardiovascular disease, which are the largest therapeutic categories in terms of prescription rates and average spending per capita in Canada. For antihypertensives, Quebecers spent more in large part because they paid higher unit prices and therefore had the highest cost per day of treatment compared to all other provinces. For cholesterol-lowering drugs, higher spending reflected more drugs being prescribed, more expensive drugs being chosen and higher prices being paid.

Differences in average spending per capita on some less commonly prescribed classes of medicines were even greater. For example, for drugs used for attention deficit hyperactivity disorder (ADHD) and neuropathic pain (gabapentin and pregabalin), spending per capita in Quebec was more than double the level in the rest of Canada.

Discussion

Prescription drugs spending per capita is far higher in Quebec than in the rest of Canada, and this is largely because Quebecers purchase more prescriptions and pay higher prices for the medicines they use. These differences may have as much to do with policy and practice variations than with health needs of the population. For example, the shorter prescription length in Quebec – typically, one month in length as opposed to the customary three months elsewhere

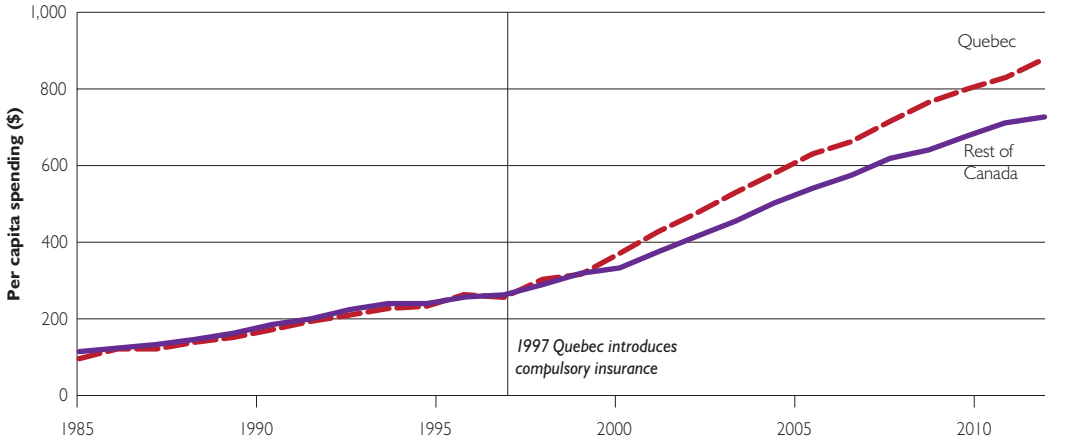
in Canada – adversely affects population spending. As unit prices include pharmacy fees, for a three-month supply of a particular drug, Quebecers pay a pharmacist fee three times, compared to once for other Canadians.

Similarly, the lower use of generics in Quebec can be partly explained by the unique-in-Canada "15-year rule." To incentivize the pharmaceutical industry to do business in Quebec, this legislation was introduced in 1994 and allows brand name drugs to qualify for reimbursement for 15 years after inclusion on the provincial formulary, even if their patent expires and the generic version becomes available on the market. This in turn provides two to five additional years of patent protection (Gagnon 2011; Paris and Docteur 2006). However, in an attempt to control prescription drug costs, the government recently abolished the 15-year rule as part of the 2013/2014 budget (Marceau 2012).

Finally, the higher overall use of prescription drugs in Quebec versus the rest of Canada may, in part, be explained by the introduction of Quebec's compulsory drug insurance program in 1997, the first and still the only one of its kind in Canada. It requires all residents of Quebec to have drug insurance coverage, either privately through group insurance or employee benefit plans, or, if they are not eligible for a private plan, through Quebec's public health insurance agency, RAMQ. As Figure 1 illustrates, the effect of this legislation appears observable in longer-term trends in total prescription spending in Quebec and the rest of Canada (CIHI 2013).

The finding of higher spending on drugs for ADHD is troubling: either Quebec is experiencing a significantly higher incidence of ADHD than the rest of Canada, or ADHD is being overdiagnosed, or it is being overtreated. Any of these scenarios is worrisome and warrants further investigation.

FIGURE 1. Total per capita spending on prescription drugs, Quebec and the rest of Canada, 1985–2012



Source: CIHI Drug Expenditure in Canada Report, 1985–2012

We also found that Quebecers are spending less than the rest of Canada on opioids, almost exclusively driven by a lower volume of drugs prescribed. This is significant for two reasons. First, Canada is the country with the second-highest level of opioid use in the world, and opioid-related issues of addiction, overdose and abuse are an important public health concern (Fischer and Argento 2012). Second, the key driver behind the lower spending in Quebec is volume, and particularly, the number of prescriptions given out is significantly lower than the rest of Canada. It is possible that other provinces may learn something from Quebec's clinical practice on prescription opioids.

In summary, there are a number of factors that contribute to Quebec's higher spending on prescription drugs, and the importance of each differs substantially depending on the drug class. However, in one way or another, higher volumes of drugs prescribed, more expensive treatment options chosen by prescribers, higher unit prices and lower use of available generics all contribute to the additional $1.5 billion annual price tag. It remains to be seen if the elimination of the 15-year rule will have a significant impact on Quebec's prescription drug spending.

In all likelihood, further policy options will be required, with the objectives of lowering unit drug prices and encouraging physicians to prescribe only what is necessary and opt for more cost-effective options. But this will be challenging: with a mixed public/private payer system, the government has a limited arsenal of effective cost-control mechanisms at its disposal (Morgan et al. 2013a; Gagnon 2014). Structural inefficiencies created by the mixed funding model – including a dramatic weakening of purchasing power and negotiating power for product listing agreements – are difficult to overcome in the current environment.

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Supporting Evidence-Informed Health Policy Making: The Development and Contents of an Online Repository of Policy-Relevant Documents Addressing Healthcare Renewal in Canada

Appuyer l'élaboration de politiques de santé fondées sur les données probantes : développement et contenu d'un répertoire en ligne de documents ayant trait aux politiques du renouvellement des services de santé au Canada

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Abstract

Objectives: (1) To develop an online repository of policy-relevant documents, other than and complementary to those from the peer-reviewed scientific literature, addressing healthcare renewal in Canada; and (2) to describe the distribution of document contents.
Methods: An iterative scoping review approach was undertaken. Documents were identified through website hand-searches and referrals from 19 Canadian health organizations. Descriptive frequencies were calculated, such as for document type.
Findings: In July 2014, 1,034 documents were in the Evidence-Informed Healthcare Renewal Portal. The top three types of documents were situation analyses (n = 390, 38%), health and health system data (n = 191, 18%) and jurisdictional reviews (n = 115, 11%). The top three national priority areas addressed were health human resources (n = 778, 75%), quality as a performance indicator (n = 502, 49%) and information technology (n = 385, 37%).
Conclusion: The process of developing a systematic method for identifying these documents has yielded a new resource to support evidence-informed health policy making and has identified a large volume of policy-relevant documents addressing healthcare renewal priority areas in Canada.

Résumé

Objectifs : (1) Développer un répertoire en ligne de documents ayant trait aux politiques, autre que et complémentaire à ceux issus de la littérature scientifique évaluée par les pairs, dans le contexte du renouvellement des services de santé au Canada; et (2) décrire la distribution du contenu des documents.
Méthode : Une revue itérative de délimitation de l'étendue a été entreprise. Les documents ont été identifiés au moyen de recherches manuelles sur le site Web et en fonction des références provenant de 19 organisations de santé au Canada. Les fréquences descriptives ont été calculées, notamment pour le type de document.
Résultats : En juillet 2014, 1 034 documents étaient présents sur le Portail du renouvellement des soins de santé fondé sur des données probantes. Les trois principaux types de documents étaient les analyses de situation (n = 390, 38 %), les données sur la santé et le système de santé (n = 191, 18 %) et les examens juridictionnels (n = 115, 11 %). Les trois principales priorités nationales traitées étaient les ressources humaines en santé (n = 778, 75 %), la qualité comme indicateur de performance (n = 502, 49 %) et les technologies de l'information (n = 385, 37 %).
Conclusion : Le processus pour le développement d'une méthode systématique afin d'identifier ces documents a mené à une nouvelle ressource pour appuyer l'élaboration de politiques fondées sur les données probantes et a permis d'identifier un grand volume de documents qui traitent des secteurs prioritaires pour le renouvellement des services de santé au Canada.

Health policy makers need quick and easy access to many different types of evidence that can help them to make well-informed decisions about health systems. Researchers, policy makers and other stakeholders have undertaken many initiatives to facilitate the use of research evidence in health policy making, such as preparing evidence briefs and convening stakeholder dialogues (Lavis et al. 2006). Online one-stop-shops have also been created that identify, add value to and make readily available different types of evidence (e.g., Health Evidence[1] for systematic reviews of the effects of public health programs and services and Health Systems Evidence [HSE][2] for many types of research evidence – including systematic reviews – about governance, financial and delivery arrangements, as well as implementation strategies, in health systems). The majority of these online repositories focus on research evidence. There is a need for an online repository that identifies, adds value to and makes readily available other types of evidence, such as colloquial evidence (Lomas et al. 2005). This type of evidence can support a better understanding of contextual factors related to the health system.

Policy-relevant documents can be considered a type of colloquial evidence, and are typically not published in peer-reviewed journals; Lomas and colleagues (2005) explain that the use of colloquial evidence prevails among decision-makers. This type of evidence is expansive and includes the expertise, views and realities of experts and professional opinion, political judgment, values, habits and traditions, input from lobbyists and pressure groups and the particular pragmatics and contingencies of the situation, such as resources. Although policy-relevant documents are primarily of the colloquial evidence type, these documents may also fit under the context-free or context-sensitive types of scientific evidence, as defined by Lomas et al. (2005). Context-sensitive scientific evidence is adapted to the circumstances of the local context and often addresses attitudes, implementation, organizational capacity, forecasting, economics/finance and ethics (Lomas et al. 2005). The most narrow type is context-free scientific evidence and is defined as methodologically explicit, systematic and replicable and is most closely aligned with the evidence-based medicine approach. Ultimately, a combination of these three types of evidence, considered alongside the many other influences on the policy process (possibly through deliberative processes), can best support evidence-informed health policy making.

Evidence-Informed Healthcare Renewal Initiative

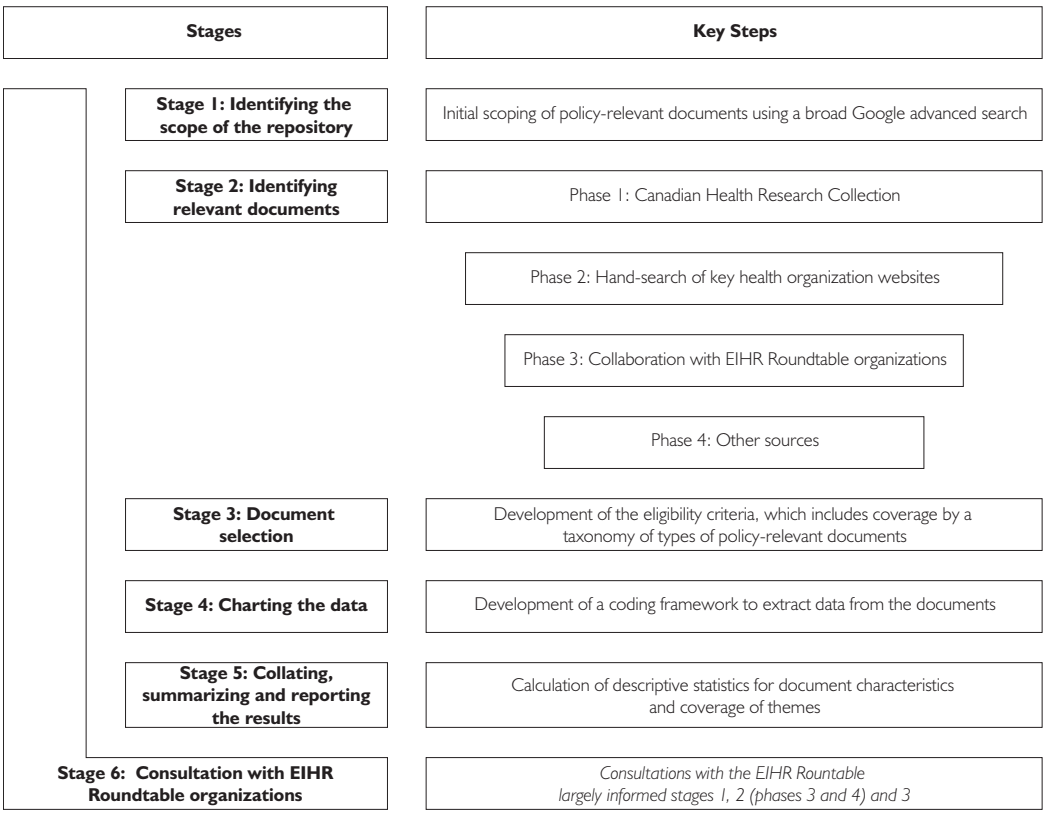
In Canada, the Evidence-Informed Healthcare Renewal (EIHR) initiative was created to support the producers and users of evidence to work collaboratively to "translate evidence for uptake into policy and practice to strengthen Canada's healthcare systems" (CIHR 2012a). In October 2011, the EIHR initiative convened the first EIHR Roundtable with participants from 41 federal, provincial and territorial governments and a broad range of national and provincial stakeholders and analysis organizations. One of the objectives of the EIHR Roundtable is to work together to ensure the collective outputs (i.e., policy-relevant documents) of each of the partner organization's work are available and accessible to the

public and health system leaders through a public knowledge platform (CIHR 2012b). To operationalize this objective, the EIHR Roundtable chose to collaborate with the HSE team at McMaster University (HSE 2012) to create the EIHR Portal, which is "a continuously updated repository of policy-relevant documents that address 'healthcare renewal' in Canada" (CIHR 2012c). The development and contents of the EIHR Portal are the focus of this paper. More detailed information about this process is available through the master's thesis on which this paper is based (Kowalewski 2012).

Methods

An iterative approach similar to Arksey and O'Malley's (2005) scoping review framework was used in developing the methods for creating the EIHR Portal (Figure 1). The Arksey and O'Malley (2005) scoping review framework suggests five stages: identifying the research objective; identifying relevant studies, or in this case documents; selecting documents; charting the data; and collating, summarizing and reporting the results. Note that the fifth stage of the scoping review approach was modified to collate, summarize and report on the general focus of the documents identified and not the specific content of the documents themselves. An optional sixth stage involves consultation with stakeholders to ensure the identification of all relevant material.

FIGURE 1. Overview of the modified scoping review approach to develop the EIHR Portal



Identifying the objective

As previously described, one of the objectives of the EIHR Roundtable was to create an online repository of policy-relevant documents addressing healthcare renewal in Canada (but not published in peer-reviewed journals). To help define the scope of this repository, a broad Google advanced search was conducted to gain a sense of the different types of policy-relevant documents related to Canadian health systems. A scan of the results revealed that many different types of documents are available on the Internet from a number of different sources. However, the breadth of the search produced a large number of results that included many documents that were not relevant to the scope of this project. This initial scoping revealed that a more targeted search strategy was necessary to feasibly and efficiently identify and retrieve these types of documents.

The EIHR Roundtable provided input for the scope of the repository, which was then used to develop and refine the objective and eligibility criteria (see *"Selecting documents"* further). Nineteen EIHR Roundtable member organizations agreed to contribute documents to assist with populating the online repository. Consultations with the EIHR Roundtable helped to ensure that the contents of the EIHR Portal are of broad interest and usefulness to different policy maker and stakeholder groups in Canada. A descriptive analysis of the general contents of the documents helped to identify any gaps in the EIHR Portal content to ensure comprehensiveness as it continues to grow.

Identifying relevant documents

The identification of relevant documents occurred in four iterative and gradually more targeted search phases (Figure 1). The first and second phases of this stage were conducted before consultations with the EIHR Roundtable began and helped to gain a sense of the available pool of policy-relevant documents. The first phase targeted the Canadian Health Research Collection electronic database, which is a collection of publications from Canadian research institutes, government agencies and university centres working in the area of health and medical research. The second phase was a hand-search of key national health organization websites. The third and fourth phases were conducted in collaboration with the EIHR Roundtable and were critical to the development of the online repository. The third phase involved identifying documents produced by EIHR Roundtable organizations. The final phase involved identifying and signing up for listservs, as well as establishing a mechanism to support internal referrals from the research team.

All searches were focused on documents produced since January 2003 (and documents from before that time were included only if they were perceived as landmark by EIHR Roundtable members, such as the *Canada Health Act*, although we return to exceptions below). The start date of 2003 was chosen for two main reasons. First, this was the year of the First Ministers' Accord on Health Care Renewal, signifying a major political shift in focus and

support for healthcare renewal in Canada. Second, the (then) time span of 10 years enabled the comparison of document content across a significant length of time but also ensured that the documents are still relevant in the current political and system context.

Selecting documents

The inclusion/exclusion criteria were devised iteratively throughout the four phases of the search strategy. The EIHR Roundtable was consulted extensively during the development of the criteria and specifically, during the development of the taxonomy of document types (Table 1). There were two categories of criteria: process-related and content-related. The main initial process-related inclusion criterion was that the document was referred by an EIHR Roundtable organization or was referred by the research team and approved by an EIHR Roundtable organization. The first content-related inclusion criterion was that the document had to address healthcare renewal, which was defined as renewing, reforming or strengthening governance, financial and delivery arrangements within health systems. The second content-related criterion was that the document had to be one or more of a list of 24 (later 25) document types (see Table 1 at www.longwoods.com/content/24034).

The taxonomy of document types was created using a sample of 50 documents from a broad range of sources that were all referred by the EIHR Roundtable. Two individuals from the research team worked through several different versions of the taxonomy before deciding on the current version. Consultations with the EIHR Roundtable informed this iterative process. The following types of documents/sources were excluded: e-newsletters, one-stop shops, podcasts and videos, peer-reviewed journal articles, derivative products of relevant healthcare renewal documents, annual reports that only describe basic activities and outputs or that only present audited financial statements and opinion pieces showing only one individual's opinion on issues related to healthcare renewal (although one-stop shops, podcasts and videos are captured through an "additional resources" document on the EIHR Portal).

Two independent reviewers assessed for eligibility all documents identified in Search Phases 3 and 4. The same two reviewers also assessed the eligibility of those documents identified in Search Phase 2 and by the research team in Search Phase 4 that were sent to EIHR Roundtable organizations for approval to include in the EIHR Portal (any documents not identified by an EIHR Roundtable member had to be approved by the EIHR Roundtable). Both reviewers read the full documents and then met to reconcile any discrepancies in assessments of eligibility.

Charting the data

Two independent reviewers extracted information from the documents assessed as eligible for the EIHR Portal using a coding taxonomy for health-system topics developed for HSE (Lavis et al. 2014, Manuscript under review). The documents were also coded for national health system priority areas, as identified in the 2003 First Ministers' Accord on Health Care Renewal (Health Canada 2006). Documents can be coded for more than one topic and prior-

ity area. Finally, the two independent reviewers extracted descriptive characteristics, such as document type, jurisdictional focus and general citation information (note that documents that did not specify a jurisdictional focus were coded as Canada).

Collating, summarizing and reporting the results

The data were summarized to present a "descriptive epidemiology" of policy-relevant documents addressing healthcare renewal in Canada that are contained in the EIHR Portal. The focus of the analysis was to calculate descriptive statistics for both the general characteristics of included documents and their coverage of national priority areas.

Maintaining the EIHR Portal

While not formally part of a traditional scoping review approach, the McMaster Health Forum has continued to develop and execute a strategy to keep the EIHR Portal up to date. Every month, EIHR Roundtable member organizations are sent a reminder e-mail to submit their list of referrals for the EIHR Portal (please refer to eihrportal.org for a list of all current participating healthcare organizations). On average, the EIHR Roundtable members refer 50 documents per month; however, the number of referrals varies greatly on a month-to-month basis. EIHR Roundtable organization referrals are augmented by internal referrals from the research team that are then vetted by one or more EIHR Roundtable members. The sources of these internal referrals are listservs from and ongoing website hand-searches of a broad range of governments, government agencies, institutional associations, professional associations and colleges, patient- and disease-based groups and research centres. The websites are hand-searched monthly, quarterly or yearly depending on their past yield (with prolific sites searched more frequently). All documents continue to be assessed and coded by two independent reviewers.

Results

A total of 1,034 documents were included in the EIHR Portal as of July 2014. Three quarters of documents were published between 2009 and 2014 (n = 780, 75%). Sixty-six per cent (n = 687) of documents focus on Canada as a whole. More than one in ten province-territory-focused documents address healthcare renewal in Ontario (n = 118, 11%). The top three types of documents are situation analyses (n = 390, 38%), health and health system data (n = 191, 18%) and jurisdictional reviews (n = 115, 11%). There is no representation of government/third-party accords (see Table 2 at www.longwoods.com/content/24034).

Document themes by 2003 First Ministers' Accord on Health Care Renewal national priority areas

Many of the documents address a number of national priority areas as identified in the 2003 First Ministers' Accord on Health Care Renewal (Table 3). The top three national priority areas addressed are health human resources (n = 778, 75%), quality as a performance

indicator (n = 502, 49%) and information technology (n = 385, 37%). The least commonly addressed national priority areas are technology assessment (n = 25, 2%), prescription drug coverage (n = 128, 12%) and diagnostic/medical equipment (n = 157, 15%). Although more attention is being paid to these issues, as evidenced by the increasing number of documents addressing these topics across the three time points (Table 3), they are still not addressed as frequently as the other national priority areas.

TABLE 3. Number of documents by 2003 Health Accord national priority areas

National priority areas*	Number of documents (%)			
	Pre-2003 (n = 30)	2003–2008 (n = 257)	2009–2014 (n = 780)	Total (n = 1,034)**
National priority funding areas				
Information technology	16 (53)	112 (44)	269 (34)	385 (37)
Primary healthcare	13 (43)	83 (32)	260 (33)	344 (33)
Home care	10 (33)	83 (32)	169 (22)	255 (25)
Electronic health record	11 (37)	49 (19)	156 (20)	210 (20)
Diagnostic/medical equipment	9 (30)	56 (22)	98 (13)	157 (15)
Prescription drug coverage	7 (23)	30 (12)	93 (12)	128 (12)
Other priority areas				
Health human resources	26 (87)	213 (83)	561 (72)	778 (75)
Innovation and research	16 (53)	83 (32)	192 (25)	282 (27)
Patient safety	6 (20)	68 (26)	201 (26)	266 (26)
Healthy Canadians/determinants of health	16 (53)	39 (15)	214 (27)	264 (26)
Aboriginal health	17 (57)	54 (21)	128 (16)	194 (19)
Technology assessment	4 (13)	4 (2)	19 (2)	25 (2)
Performance indicators				
Quality	19 (63)	145 (56)	347 (44)	502 (49)
Timely access/waiting lists	12 (40)	100 (39)	270 (35)	372 (36)
Sustainability	15 (50)	62 (24)	262 (34)	355 (34)
Health status and wellness	16 (53)	87 (34)	213 (27)	307 (30)

* Documents could be coded as multiple national priority areas.

** Total includes documents without years of publication, which were not included in the year range calculations; therefore, the total number does not always equal the sum of the year range totals.

Discussion

The EIHR Portal provides a one-stop shop for policy-relevant documents that can support health policy makers who make decisions about healthcare renewal in Canada. It is

continuously updated based on protocols refined during its development. The EIHR Portal contains 1,034 policy-relevant documents that address healthcare renewal in Canada and that can be categorized into 24 document types; the largest category of which is situation analysis (n = 390, 38%). The documents address national priority areas identified by federal, provincial and territorial governments, such as health human resources (n = 778, 75%) and information technology (n = 385, 37%). The lack of focus on certain national priority areas, such as technology assessment (n = 25, 2%), seen from a system level (as opposed to in terms of particular programs, services and drugs) can spur discussion about current gaps in Canadian healthcare renewal.

Strengths and limitations

This study has four key strengths and two limitations. The first strength is that the results of the initiative present the first effort to systematically identify, gather and describe policy-relevant documents that address healthcare renewal in Canada and that have not been published in the peer-reviewed literature. The novel taxonomy of document types can contribute to future systematic analyses of this unique body of evidence. Second, the study involved key stakeholders, which was pivotal to its success. This approach is commonly called linkage and exchange in the Canadian context (Lomas 2000). Arksey and O'Malley (2005) explain that including the perspectives of those with knowledge of, and a vested interest in, the area of investigation, in this case Canadian healthcare renewal, is invaluable to the research process. Third, the integration of the EIHR Portal into HSE permits users who identify policy-relevant documents to examine related systematic reviews and other types of research evidence (e.g., economic evaluations and health reform descriptions) and the prompting of users who identify research evidence to examine related policy-relevant documents. Fourth, the EIHR Portal was designed to be continuously updated, which will help to ensure its continued relevance to Canadian policy makers and other stakeholders. The first limitation of this study is that the quality of the documents included in the EIHR Portal was not appraised, because no systematic quality-appraising tool exists for most document types. The second limitation of this study is that, given the nature of the process used to identify documents, there is a possibility that this process may have not captured all documents. However, a comprehensive search of all databases containing grey literature (as would be typical of systematic reviews) would be highly challenging for identifying this unique body of evidence.

Implications for health policy making in Canada

There are a number of implications for health policy making that arise out of the development of a one-stop shop for policy-relevant documents that address healthcare renewal in Canada. The one-stop shop expands the breadth of documents available for policy makers to easily access and use to inform their health system decisions. The EIHR Portal, which provides policy makers with colloquial evidence primarily, in combination with the research evidence available in HSE, facilitates the development of well-informed health policies that can be

based on a truly comprehensive understanding of the best available global evidence and of the local health system context.

An analysis of the general contents of the documents in the EIHR Portal also has implications for health policy making in Canada. Based on the descriptive epidemiology of policy-relevant documents, it is evident that certain Canadian health system national priority areas are receiving more attention than others. Policy makers should consider whether all national priority areas are receiving the necessary attention.

Implications for research

The EIHR Portal described in this study is the first of its kind. In time, the EIHR Portal could include documents that address healthcare renewal or other macro-level health system topics in other countries; the potential for inter-country collaboration is great. The process of developing a systematic method for identifying policy-relevant documents and retrieving useful information from these documents can be reproduced by anyone interested in using this type of evidence to inform their health policy making. Of course, the methods described here can only serve as a guide because the process depends on the context and resources available to create one-stop shops like the EIHR Portal. Future research should devise a method to appraise the quality of these documents, so that this could be reported in the EIHR Portal and policy makers would not have to rely on their own critical appraisal skills.

Conclusion

There is growing recognition that a range of evidence is required for evidence-informed health policy making that addresses not only questions of effectiveness but also describes contextual factors, such as the ideas, interests and institutions that shape health policies. Ultimately, only through concerted efforts that facilitate wholly informed policies can we effectively strengthen the health system and improve the health of Canadians.

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Notes

1. See www.healthevidence.org.
2. See www.healthsystemsevidence.org.

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RESEARCH PAPER

Choosing Your Partner for the PROM: A Review of Evidence on Patient-Reported Outcome Measures for Use in Primary and Community Care

Choisir son partenaire pour le PROM : examen des données sur l'évaluation de l'état de santé du point de vue du patient dans les établissements de soins primaires et communautaires

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Abstract

Patient-reported outcome measures (PROMs) are assessments of health status from the patient's perspective. The systematic and routine collection and use of PROMs in healthcare settings adds value in several ways, including quality improvement and service evaluation. We address the issue of instrument selection for use in primary and/or community settings. Specifically, from the large number of available PROMs, which instrument delivers the highest level of performance and validity? For selected generic PROMs, we reviewed literature on psychometric properties and other instrument features (e.g., health domains captured). Briefly we summarize key strengths of the three PROMs that received the most favourable psychometric and overall evaluation. The Short-Form 36 has a number of strengths, chiefly, its strong psychometric properties such as responsiveness. The PROMIS/Global Health Scale scored highly on most criteria and warrants serious consideration, especially as it is free to use. The EQ-5D scored satisfactorily on many criteria and, beneficially, it has a low response burden.

Résumé

Les PROM (*patient-reported outcome measures*) sont des méthodes d'évaluation de l'état de santé du point de vue du patient. Le recours systématique et routinier aux PROM dans les établissements de santé apporte une valeur ajoutée de diverses façons, notamment en ce qui a trait à l'amélioration de la qualité et à l'évaluation des services. Nous nous penchons sur la question du choix d'instrument à utiliser dans les établissements de soins primaires et/ou de soins communautaires. Plus précisément, quel instrument parmi le vaste nombre de PROM disponibles obtient le meilleur rendement et offre la plus grande validité? Nous avons évalué la littérature sur les propriétés psychométriques et autres caractéristiques (par exemple, les domaines de santé saisis) de certains PROM génériques. Nous avons fait un bref sommaire des forces clés des trois PROM qui ont obtenu la meilleure évaluation psychométrique et générale. Le formulaire *Short-Form 36* présente plusieurs points forts, notamment ses solides propriétés psychométriques telles que la réactivité. Le *PROMIS/Global Health Scale* a obtenu un score élevé pour la plupart des critères et mérite une attention particulière, particulièrement en raison de sa gratuité. L'*EQ-5D* a obtenu un score satisfaisant pour plusieurs critères et présente un faible fardeau de réponse, ce qui est un avantage.

Introduction

Patient-reported outcome measures (PROMs) are assessments of health status or health-related quality of life from the patient's perspective, a viewpoint not captured by clinical outcomes (Dawson et al. 2010). The process typically used to collect such data is for the patient-reported outcome (PROM) questionnaire to be completed by the patient at specific times in his/her clinical trajectory (e.g., at regular intervals following surgery or at defined follow-up points after diagnosis of a chronic condition).

The systematic and routine collection and use of PROMs in healthcare settings can add value in several ways, including direct patient management, quality improvement and service evaluation, technology assessment and research and the assessment of practitioner performance. In a previous paper, "Let's All Go to the PROM" (McGrail et al. 2011), we set out the arguments for routine collection of PROMs data. In this paper we address the issue of instrument selection – there are many PROMs to choose from but which ones deliver the highest level of performance and validity?

The scope of the project reported here was to consider PROMs for use in primary and/or community care settings, as opposed to an elective surgery context which has received more attention in the research literature. We worked closely with stakeholders at a provincial Ministry of Health whose primary interest was in the use of PROMs to support evaluation of integrated primary and community care initiatives. This work thus provides information and a summary of evidence to inform PROM selection in a primary/community care context.

For a number of widely used PROM instruments, this paper reports: (1) the psychometric properties of the instruments; (2) health domains captured; (3) implementation considerations (respondent burden, administration methods, translations available, readability and cost); and (4) availability of population norms and utility scoring algorithms. These areas were identified as important to instrument selection through consultation with a range of knowledge users (health researchers, practitioners, administrators and government).

Selection of PROMs as a Focus for This Study

This rapid review was undertaken for the BC Ministry of Health. As such, we were given six months for completion of the review. The initial weeks of the project were spent clarifying with the Ministry their specific goals and questions for the project so that our research team could provide the Ministry with an appropriate selection of instruments. Due to time constraints, a systematic review was not feasible; therefore, a rapid review was undertaken. The methods used made every effort to ensure reproducibility and rigour, and to avoid bias. Below we describe our approach to the selection of the shortlisted instruments.

According to the Mapi Research Trust's Patient-Reported Outcome and Quality of Life Instruments Database ("PROQOLID. Patient-Reported Outcome and Quality of Life Instruments Database. [internet] 2013 [cited 2012 December]; available from: http://www.proqolid.org/,"), there are now close to 800 PROM instruments, with more being developed all the time. Therefore, the first stage of this research was to select a shortlist of PROM instruments, and hence define a manageable task.

Given that our study focused on applications in primary and community care, the most relevant PROMs are comprised of generic measures that provide the opportunity to compare outcomes across many clinical conditions. Specifically, a measure applicable to a primary and community care setting was defined as one that is broad enough in content to capture important differences in health status resulting from a wide array of health conditions typically seen in primary/community care. As such, we focused on generic measures of health status that

cover domains used to assess overall health status. The benefit of a generic measure, with its ability to make comparisons across diverse clinical conditions, involves a tradeoff: by excluding condition-specific instruments, some sensitivity and responsiveness is almost inevitably sacrificed in subsets of the clinical population. On the other hand, one of the advantages of a generic PROM is that it provides information about a respondent's overall health status or health-related quality of life, which often includes multiple aspects or domains (i.e. physical, emotional, mental, social and general health).

The process for uncovering PROM measures involved re-examining existing reviews and performing structured searches of instrument databases and consulting measurement experts. We acquired a PROQOLID membership for the project. However, we did not rely exclusively on PROQOLID to identify relevant instruments. We used PROQOLID as a tool to screen all indexed generic instruments. Of note, several instruments that were not indexed in PROQOLID but were identified through expert and stakeholder consultations were also included. Internet and database searches were conducted to verify the completeness of our long- and short-lists of instruments. PROQOLID provided a comprehensive and up-to-date listing of 115 generic PROMs. From this, we developed a short-list of 25 potential generic PROMs applicable for use in a primary care setting. To be considered in this rapid review, the generic PROMs needed to include the following characteristics:

- designed for use among adults;
- able to be self-administered;
- capable of generating a summary score to assess overall health status;
- applicability to a primary care and community care setting; and
- widespread in their current use (evaluated via a formal citation search).

Project experts also suggested including the measures How's Your Health, PROMIS and RAND-36, none of which was included in PROQOLID. The total number of eligible instruments was, therefore, 28. The "impact" of each instrument was considered by reviewing the number of cited references for the original papers (over the past six years) in the Web of Science, to ensure that the selection of instruments was targeted to those in widespread and current use (see Appendix 1 for the citation search details). Our final short-list of candidates included 8 PROM instruments:

- Assessment of Quality of Life (AQoL-8D)
- EuroQol EQ-5D-3L
- Health Utilities Index (HUI3)
- Nottingham Health Profile (NHP)
- PROMIS-Global Health Scale (GHS)
- Quality of Well-Being Scale (QWB)
- Short-Form 36 (SF-36)
- World Health Organization Quality of Life Instrument (WHOQoL-BREF)

For many of these instruments, there exist numerous versions. For example, there are three versions of the Health Utilities Index (HUI1, HUI2 and HUI3), and the SF "family" of instruments includes the SF-36 (versions 1 and 2), the SF-12 (versions 1 and 2) and the SF-8. For each instrument we made a pragmatic decision to focus primarily on the latest, most commonly used version that was most applicable to primary/community care settings. However, some of the review work, especially where studies researched multiple instruments, reports evidence from multiple-instrument versions.

Our review and evaluation efforts had a two-pronged focus: (1) ascertaining the psychometric properties of the instruments; and (2) identifying data and information regarding important instrument properties, such as domain coverage, implementation considerations and the availability of utility scores and population norms.

Methods

Psychometric review

We synthesized evidence from existing reviews of the psychometric properties of our eight candidate instruments (Khangura et al. 2012).

Our searches were developed in MEDLINE (OvidSP) and Embase (OvidSP) using keywords because most instruments were not indexed as subject terms in the MeSH or Emtree thesauri. We used two search filters. The first, a filter specifically for measurement properties of health instruments reported by Terwee and colleagues (2009) and recommended by the Consensus-Based Standards for the Selection of Health Measurement Instruments (COSMIN) group (Mokkink et al. 2010), was used in PubMed and then adapted for MEDLINE and Embase (OvidSP). The second filter, developed by the Scottish Intercollegiate Guidelines Network (Scottish Intercollegiate Guidelines Network; available from: http://www.sign.ac.uk/methodology/filters.html), is also used for systematic reviews and the publication-type "reviews." Our search comprised three components: terms for the eight PROMs that were then combined with the measurement filter and then the review filter.

We also performed grey literature searches. The majority of the instruments have websites that were also examined for evidence relating to psychometric properties. Further details on our search strategy are given in Appendices 2 and 3, and are available in the main project report (Bryan et al. 2013).

Two researchers (J.B. and J.C.D.) independently evaluated the titles and abstracts. Articles were selected for further review that assessed an instrument's psychometric performance in a general population applicable to primary and community care (i.e., not specific clinical populations) and articles that focused on at least one of the psychometric properties of the instrument as suggested by the COSMIN guidelines. Specifically, the extracted information included the following: reliability (internal consistency and test–retest reliability), validity (content, construct, cross-cultural and criterion validity), responsiveness, generalizability

and comparability with other candidate PROM instruments. Further, only manuscripts that focused on at least one of the eight selected candidate PROMs were included. Discrepancies between the two reviewers were discussed and resolved by a third reviewer (S.B.), yielding 21 articles selected for full-text review. An additional article on the PROMIS Global Health Scale was added to provide coverage for all candidate instruments, bringing the total number of articles to 22 (Anderson et al. 1993, 1996; Bouchet et al. 2000; Brazier et al. 1999; Butterworth and Crosier 2004; Coons et al. 2000; Doward et al. 2004; Ford et al. 2000; Furlong et al. 2001; Gandek et al. 2004; Hawthorne and Richardson 2001; Hays et al. 2009; Haywood et al. 2005; Horsman et al. 2003; Kopec and Willison 2003; McHorney and Tarlov 1995a; Revicki and Kaplan 1993; Sintonen 2001; Skevington et al. 2004; Ware 2000; Wiklund 1990).

The data extraction process was guided by the COSMIN criteria and a corresponding data extraction template was created covering reliability (internal consistency and test–retest reliability), validity (content, construct, cross-cultural and criterion) and responsiveness. Reviewers (J.B. and J.C.D.) provided a score by strictly adhering to guidelines established by COSMIN for each of the data extraction items listed above (Mokkink et al. 2010). Scores ranged from very strong positive evidence (+++) to very strong negative evidence (−−−). Conflicting evidence was also noted (+/−), as was an absence of evidence in that particular category (?). Further details on the scoring approach are given in Appendix 4. We also evaluated, but did not score, additional information not included in the COSMIN guidelines, including generalizability and comparability with other candidate PROM instruments, which we considered important for decision-making.

Two reviewers (J.B. and J.C.D.) also extracted all text from these articles pertaining to each instrument's psychometric properties. This information was entered into a central database and subsequently scored independently by the two reviewers (J.B. and J.C.D.), in line with the COSMIN guidelines (Consensus-Based Standards for the Selection of Health Measurement Instruments [COSMIN] 2013; Mokkink et al. 2010).

Review of other performance attributes

The second focus of this rapid review was on other performance attributes of the selected candidate instruments. The instruments were reviewed in terms of their domain coverage through detailed inspection of the questions asked. The domain framework of the PROMIS instrument, given its comprehensive and inclusive nature, was used as a reference point (www.nihpromis.org/Documents/PROMIS_Full_Framework.pdf).

In terms of implementation considerations for policy makers and patients, we also sought information on respondent burden, readability, cost of using the instrument and the availability of official translations. Data sources for this information were primarily materials published by the instrument's developers and third-party websites focused on evaluating PROM tools. We independently assessed readability using the Flesch–Kincaid grade-level

test, which assesses an individual's education grade level equivalent (Microsoft 2013).

Finally, information relating to selected decision-making criteria was also obtained. These criteria included: norm reference sets (to allow comparison of sample data to the general population), and utility/preference scoring algorithms (to allow the calculation of quality-adjusted life years and so facilitate cost-utility analyses). Information on these criteria was obtained for each instrument through examination of the appropriate website or scoring guides.

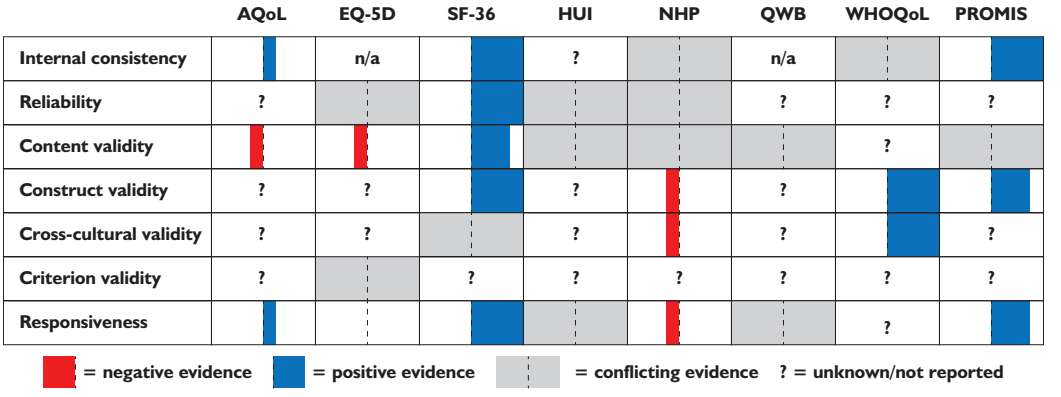
Results

Psychometric review

The details of the 22 articles providing information about the psychometric properties of the candidate PROM instruments (Anderson et al. 1993, 1996; Bouchet et al. 2000; Brazier et al. 1999; Butterworth and Crosier 2004; Coons et al. 2000; Doward et al. 2004; Ford et al. 2000; Furlong et al. 2001; Gandek et al. 2004; Hawthorne and Richardson 2001; Hays et al. 2009; Haywood et al. 2005; Horsman et al. 2003; Kopec and Willison 2003; McHorney and Tarlov 1995a; Revicki and Kaplan 1993; Sintonen 2001; Skevington et al. 2004; Ware 2000; Wiklund 1990) are reported in Table 1 (see Table 1. at www.longwoods.com/content/24035). Ten of the articles also contained direct comparisons between two or more candidate instruments. All of the articles provided a good rationale and key objectives for the review, and most also provided a summary of their results in relation to the key objectives. Each of the 10 psychometric categories we considered was evaluated for most of our candidate instruments. However, none of the reviews explicitly discussed measurement error, an important psychometric category related to reliability.

A brief summary of results from our rapid review of the candidate instruments' psychometric properties is presented in Figure 1 and Table 2, and an overview of the main sources for evidence on each criterion is given by Table 1.

FIGURE 1. Overview of results from psychometric review



Note: The width of the bars indicates the volume of available evidence demonstrating the observations (i.e., narrow bars are indicative of fewer studies and wide bars are indicative of a larger number of studies)

The SF-36 had a very large evidence base and performed as well as, or better than, the other instruments in most of the psychometric domains we considered. It was noted to be reliable (Gandek et al. 2004; Haywood et al. 2005), to be comprehensive in its coverage of various aspects of health (Haywood et al. 2005; Ware 2000) and for having strong content validity, which indicates that it taps into the domains it proposes to examine (Butterworth and Crosier 2004; Gandek et al. 2004; Hays et al. 2009; Skevington et al. 2004; Ware 2000). Its strong score in the responsiveness category, indicating the ability to detect change when it was known to have occurred or differences between groups known to vary in health status, suggested that it would be better suited for evaluation studies than some of the other instruments (Anderson et al. 1996; Bouchet et al. 2000; Hays et al. 2009; Kopec and Willison 2003; McHorney and Tarlov 1995; Ware 2000). PROMIS-GHS also performed well in most categories, demonstrating good internal consistency, construct validity and responsiveness. However, the evidence base is smaller, in part due to its more recent development (Hays et al. 2009). While the WHOQoL-BREF did not perform as well as the SF-36 in some domains, and had a smaller evidence base, it did have stronger evidence for cross-cultural validity than any of the other instruments (Skevington et al. 2004).

Several limitations were noted for the remaining instruments. Both the HUI3 and QWB-SA were reported to be lacking in their coverage of mental health (Brazier et al. 1999; Coons et al. 2000). While the NHP does perform well in populations with major burdens of disease, and may be more responsive than the SF-36 in those groups, it may be less relevant for general population samples with lower burdens of disease (Haywood et al. 2005; Wiklund 1990). The response format of the QWB-SA is problematic for detecting changes in severity of illness, and may overweight minor health conditions such as wearing eyeglasses (Brazier et al. 1999). Finally, we found the evidence base for the AQoL-8D to be smaller than some of the other instruments we considered, despite it being in use for over 10 years, which has also been noted by other authors (Hawthorne and Richardson 2001). The limited psychometric information uncovered relating to AQoL-8D may have been due in part to the rapid review methodology.

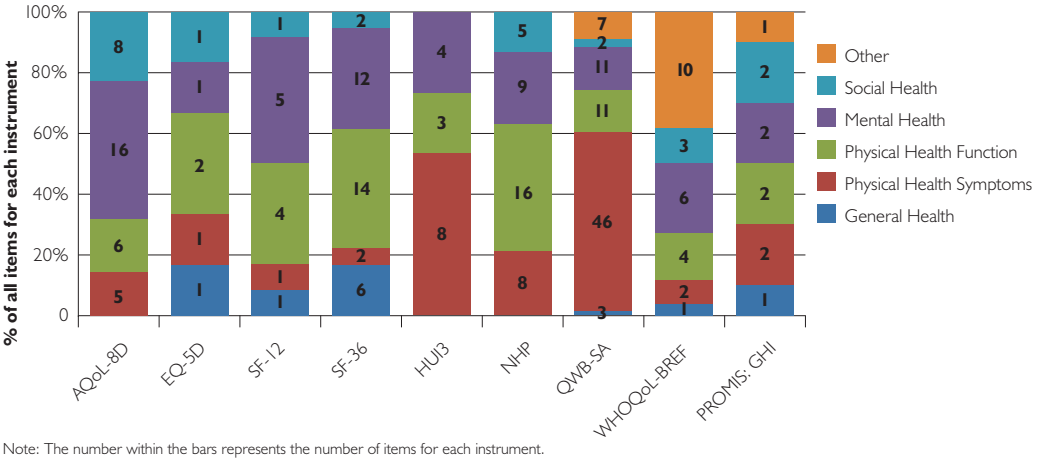
In several cases, the evidence reported in the papers reviewed may have provided useful information that was not part of the COSMIN scoring framework. An example of this is cross-cultural validity. The WHOQoL-BREF, which prioritized cross-cultural validity during its initial development, performed best in this category. This instrument has been systematically evaluated in many different contexts, and only a few meaningful differences have been noted (Skevington et al. 2004). Both the EQ-5D and HUI are used in different cultural and linguistic contexts. The SF-36 is also widely used in many contexts, and some evidence of validation across different groups (using differential item functioning analysis) has been reported for both the SF-36 and SF-12 (Ford et al. 2001; Gandek et al. 2004). The NHP had positive evidence in terms of how it was developed and its widespread use (Anderson et al. 1996;

Coons et al. 2000), but one review noted potential differences between how the instrument functions in French versus English (Anderson et al. 1996). There was no information on the cross-cultural validity of the QWB-SA, PROMIS and the AQoL.

Review of other performance attributes

In terms of domain coverage, Figure 2 provides a summary of our data. While the number of questions in each domain varied across instruments, all of our candidate instruments included questions that assessed physical and mental health. Questions regarding social health were absent from the HUI3, and questions on general health were absent from the HUI3, AQoL-8D and NHP.

FIGURE 2. Domain coverage for selected PROMs



Note: The number within the bars represents the number of items for each instrument.

All of our candidate instruments are available in the two official languages of Canada (English and French). The EQ-5D and SF-36 are available in more languages than the other instruments, including Mandarin Chinese, Punjabi, Korean and Tagalog, which are frequently spoken at home in British Columbia.

The mode of PROM administration may also affect the decision-making process. Our initial inclusion criteria required that all of our candidate PROMs could be self-administered, and all are able to be administered on paper. Alternative administration modes, such as by telephone and online, have been validated for some of the instruments, particularly for SF-36, EQ-5D and PROMIS/GHS.

Other key considerations are an instrument's readability and the amount of time required to complete it, presented in Table 3. Readability scores reflect the estimated grade level required to understand the instrument. The EQ-5D required the highest reading level, grade 11, due to its long questions and use of multi-syllabic words. All other instruments are within the recommended ranges for working with adult populations. All of our candidate instruments are relatively short, taking between 2 and 15 minutes to complete.

TABLE 2. Summary of strengths and weaknesses of selected PROMs

Instrument	Strengths	Weaknesses
AQoL	Discriminates between groups with clinical variations in health.	Smaller evidence base.
EQ-5D	Discriminates between groups with clinical variations in health.	Not as comprehensive. Not sensitive to small changes, limited responsiveness in healthy populations.
SF-36	Top instrument in most psychometric categories. Widely used, multiple cultural contexts and many versions available.	
HUI	Can distinguish between groups with clinical variations in health, and widespread use in a variety of cultural contexts.	Lacking in mental health. Less reliability. Less responsive in populations of fairly good health.
NHP	More responsive than SF-36 in populations with poor health. Widespread use in a variety of cultures.	Not ideal for use in general population, or outside of populations with major health issues.
QWB	Good for capturing change in primarily healthy populations.	Lacking on mental health, may overweight minor conditions.
WHOQoL	Very strong cross-cultural validity. Correlated with groups with clinical variations in health.	Smaller evidence base.
PROMIS GHS	Good internal consistency, responsiveness and correlation with other instruments.	Smaller evidence base.

TABLE 3. Respondent burden and readability

Instrument	Number of items	Word count	Time for completion (min)	Flesch–kincaide grade level
AQoL-8D	35	1,188	5	5.3
EQ-5D	6	239	"few minutes"	10.6
SF-36®	36	692	10	5.9
HUI3®	15	1,173	8–10	7.4
NHP	38	353	5–15	2
QWB-SA	80	1,934	15	5.6
WHOQoL-BREF	26	607	5	6.7
PROMIS/GHS	10	217	2	7.6

A final practical consideration is the financial costs associated with using the instrument. While many of the candidate instruments are free to use (AQoL-8D, NHP, WHOQoL-BREF and the PROMIS/GHS), others require payment to the developers when used outside of a research context (EQ-5D, SF-36, HUI3 and QWB-SA). The SF-36 requires licensing fees.[1]

Depending on the context under which the PROM will be used, the availability of population norms for comparison to a general population, and utility scores for use in cost-effectiveness evaluation, may be important decision-making criteria. Population norms are available for all of the instruments, but only the EQ-5D, SF-36 and SF-12 and HUI3 have norms from Canadian samples. Utility scores are also available for most instruments, with the exception of the NHP and WHOQoL-BREF, but value weights from Canadian samples are only currently available for the EQ-5D and HUI3.

Discussion

Summary and key findings

Our research team collaborated closely with colleagues working in primary care and those in health policy positions at regional and provincial levels. Further, we consulted widely with academic partners with knowledge and experience of PROM use in primary and community care. The culmination of the project was an end-of-project knowledge translation workshop, hosted by the BC Ministry of Health. In sharing our findings with health sector evaluators, researchers and administrators, we asked for views on the top instruments for use in a primary/community care setting. A "dotmocracy" process was used whereby all workshop participants were able to express their overall instrument preference using dot stickers. The highest-ranking instruments were SF-36, EQ-5D and PROMIS/GHS.

Based on the review of evidence presented in this paper, and when thinking specifically about PROMs in the context of primary and community care, the SF-36 has a number of strengths, chiefly its strong psychometric properties (particularly its responsiveness) and widespread usage facilitating comparisons, as well as the availability of population norms and utility scores. The EQ-5D scored satisfactorily on many of the criteria we considered, and has a lower response burden than the SF-36. However, for evaluation research and quality improvement purposes, there is a concern relating to responsiveness and hence its ability to detect change. It may be better suited than the SF-36 where brevity outweighs the importance of responsiveness. The PROMIS/GHS also scored highly and should be given serious consideration. Despite its smaller evidence base, its relatively strong psychometric properties, absence of licensing fees, increasing utilization and integration into a broader information system are all strong arguments in its favour (Cella et al. 2010).

The selection of a PROM instrument is a complex task that will inevitably involve trade-offs. The time for completion of the instrument is, not surprisingly, correlated with the number of items included in the instrument, which also influences its ability to detect changes when they occur (see psychometric results above). For example, the EQ-5D takes less time to complete than the SF-36, but is also less responsive to detecting change. Decision-makers must, therefore, consider the relative importance of being able to detect changes, which is crucial for use in evaluation and quality improvement, with the burden placed on respondents.

Despite general acknowledgement that the responsiveness of the SF-36 was a strong point compared to other instruments, there was concern that the instrument took too long to complete. Practical considerations pertaining both to how it would be administered, and by whom, featured prominently in the discussion. Some workshop attendees expressed a preference for the EQ-5D, despite its lower responsiveness, because it would be quicker to administer and therefore a lesser burden to patients. Cost was another important consideration and was considered a particular strength of the PROMIS/GHS instrument.

Limitations

We acknowledge the following limitations of this rapid review. A systematic review may have produced a greater breadth and depth of evidence relating to generic PROMs applicable to a primary care setting. Specifically, a search of the primary literature may have uncovered other PROMs not identified in our search. Further, we may have missed some papers providing more depth compared with the review papers selected that addressed the psychometric properties of the eight instruments. A quality assessment of the selected papers was not undertaken and thus the conclusions need to be interpreted with caution. We made a pragmatic decision to focus on versions of instruments that were the latest or most commonly used within a primary/community care setting. Ideally, our review would have included all available versions to first establish which is most scientifically robust within a primary care setting to allow all instruments equal opportunity to emerge into practice. Although we report that there were no psychometric weaknesses for the SF-36, we highlight the following feasibility considerations. The SF-36 has a longer completion time, which may predispose this instrument to reduced response rates and greater cumulative missing data, but we did not review such evidence in this project. Finally, the quality of the available evidence detailing population norms was not evaluated in this rapid review due to time constraints. As such it is possible that for some of the instruments, there may be issues relating to the external validity of these published norms. The final limitation pertains to the observed data extraction discrepancies between J.C.D. and J.B. These discrepancies were largely due to the heterogeneity of the included studies. As such, a third reviewer (S.B.) was added to resolve all differences.

Conclusions

The results provide a summary of the characteristics of and evidence base for some of the most commonly used generic PROM instruments. Identifying the instrument best suited for an evaluation in healthcare, or for routine data collection, depends on the context (i.e., population of interest and research question) in which it would be used. By providing information pertaining to psychometric and practical considerations, we hope to encourage the wider utilization of PROMs in Canadian healthcare, and make the process of selecting a suitable PROM instrument easier.

Note

1. Some alternative instruments that share many similarities with the SF-36 (RAND-36, RAND-12, Veteran's RAND-36 and Veteran's RAND-12) are available without cost.

Acknowledgements

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Janice M. Murphy, PhD, Research Consultant, Balfour, BC; and Rick Sawatzky, PhD, Associate Professor, School of Nursing, Trinity Western University, Scientist, Centre for Health Evaluation and Outcome Sciences, Providence Healthcare, Langley, BC.

This study was supported by a grant from the Canadian Institutes of Health Research. The authors would also like to thank their other research team colleagues, Kacey Dalzell and Martin Dawes, and colleagues at the BC Ministry of Health and the Michael Smith Foundation for Health Research.

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RESEARCH PAPER

The Primary-Specialty Care Interface in Chronic Diseases: Patient and Practice Characteristics Associated with Co-Management

Interrelation entre services de première ligne et soins spécialisés dans les cas de maladie chronique : caractéristiques des patients et des cliniques associées à la cogestion

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Abstract

Objective: Specialist physicians may act either as consultants or co-managers for patients with chronic diseases along with their primary healthcare (PHC) physician. We assessed factors associated with specialist involvement.

Methods: We used questionnaire and administrative data to measure co-management and patient and PHC practice characteristics in 702 primary care patients with common chronic diseases. Analysis included multilevel logistic regressions.

Results: In all, 27% of the participants were co-managed. Persons with more severe chronic diseases and lower health-related quality of life were more likely to be co-managed. Persons who were older, had a lower socioeconomic status, resided in rural regions and who were followed in a PHC practice with an advanced practice nurse were less likely to be co-managed.
Discussion: Co-management of patients with chronic diseases by a specialist is associated with higher clinical needs but demonstrates social inequalities. PHC practices more adapted to chronic care may help optimize specialist resources utilization.

Résumé

Objectif : Les médecins spécialistes peuvent agir comme consultants ou cogestionnaires pour les personnes atteintes de maladies chroniques, et ce, de pair avec le médecin de première ligne. Nous avons évalué les facteurs associés à l'implication du spécialiste.
Méthode : Nous avons employé des données administratives et des données recueillies par questionnaire pour mesurer le degré de cogestion ainsi que les caractéristiques des patients et des cliniques de première ligne auprès de 702 patients atteints de maladies chroniques fréquentes. L'analyse comprenait une régression logistique multiniveau.
Résultats : En tout, 27 % des participants étaient cogérés. Les personnes atteintes de maladies chroniques plus graves et dont la qualité de vie est plus affectée par la maladie ont plus de probabilités d'être cogérées. Les personnes plus âgées, qui ont un statut socioéconomique moins élevé, qui résident en milieu rural et qui sont suivies par une clinique de première ligne dotée d'une infirmière clinicienne ont moins de probabilités d'être cogérés.
Discussion : La cogestion par un spécialiste de patients atteints de maladies chroniques est associée à de plus grands besoins cliniques mais fait état d'inégalités sociales. Les cliniques de première ligne mieux adaptées pour les maladies chroniques pourraient aider à optimiser l'utilisation des ressources des spécialistes.

Introduction

Ambulatory management of chronic diseases relies mainly on the primary healthcare (PHC) setting; however, involvement of medical specialist physicians may improve process and outcomes of care for patients with chronic diseases that are within their field of expertise (O'Malley and O'Malley 2007; Smetana et al. 2007; Stange and Ferrer 2009). Specialist involvement can be of two types – co-manager or consultant – depending on whether or not the specialist provides ongoing management in addition to the primary care physician (PCP) (Forrest 2009). Specialists are involved as co-managers when they act as regular care providers for the patients, sharing responsibilities with the PCP for long-term follow-up for the patient who was referred (Forrest 2009). Specialists are consultants when their role is limited to providing diagnostic/management advice to PCPs (cognitive consultation) or performing

diagnostic/curative technical interventions (procedural consultation), without providing ongoing management to the patient for the health problem (Forrest 2009). For example, in the case of consultation, a PCP could refer a patient with knee osteoarthritis to the orthopaedist for an injection. Once the intervention would be completed, the patient would return to the primary care provider for follow-up and would only return to see an orthopaedist when further advice or intervention would be sought. In the case of co-management, the patient would return regularly to the orthopaedist for monitoring of his arthritis and adjustment of the treatment plan. The patient would still be attended by the PCP for management of other health needs. The consultant usually requires fewer specialist visits than the co-manager; thus, acting as a consultant can free up the specialist's time to act as consultant/co-manager for more patients (Forrest 2009; Jiwa et al. 2008; Starfield 2010).

As specialist resources are limited (Fye 2004; Hanly 2004; Shipton et al. 2003; Stewart 2008), it is essential that patients receive the appropriate specialist involvement. Evaluation of need, enabling and predisposing factors (Andersen 1995) associated with type of specialist involvement would consequently be useful for clinicians, researchers and policy makers. Studies on referral and utilization patterns of specialists' services have observed that beyond clinical needs, there were social inequities and that provider and practice characteristics further predisposed and enabled these patterns (Feldman et al. 2007, 2009; Forrest et al. 2006). However, no study has addressed patient, physician or practice characteristics associated with type of specialist involvement in populations with chronic diseases. The aim of the present study was therefore to determine the clinical, socio-demographic, PCP and PHC practice characteristics associated with involvement of a medical specialist physician as a co-manager for adults with chronic diseases managed in the primary care setting.

Methods

Design, recruitment and data collection

The present study consisted of cross-sectional secondary analyses using data from a multilevel design cohort study conducted between 2006 and 2008 to assess the impact of PHC practice models on the process of care and health outcomes of patients with chronic diseases (Feldman et al. 2012; Lemieux et al. 2011). A sample of 90 PHC practices in Montréal and Montérégie regions of the province of Québec (Canada) that managed patients with chronic diseases were contacted to participate in the study. Thirty-three PHC practices referred 1,031 patients with diabetes, arthritis, congestive heart failure (CHF) or chronic obstructive pulmonary disease (COPD), of whom 776 provided written informed consent and entered the cohort. Patients were interviewed at baseline and subsequently at 6, 12 and 18 months using standardized questionnaires regarding socio-demographic and clinical characteristics, utilization and quality of care and health and quality of life outcomes. We also linked patient data from the provincial physician reimbursement administrative database, including information over the period of one year prior to entry into the study until one year after entry. As in other Canadian provinces, the entire population of Quebec is covered by provincial health insur-

ance, and physicians bill the province for ambulatory services rendered to patients. In Canada, patients usually require a doctor's referral to consult a specialist. The study was approved by the research ethics committees of the relevant institutions.

Co-management by a specialist

Specialist involvement was first determined using the physician reimbursement database and was defined as having at least one outpatient encounter with a relevant specialist in the 12 months prior to or after entry in the study (total period = two years). A two-year period was preferred to a one-year period because patients with co-management may not necessarily be followed yearly by the specialist. Relevant specialists considered for each diagnosis were cardiologist for CHF, respirologist for COPD, endocrinologist for diabetes and rheumatologist or orthopaedist for arthritis. Patients with specialist utilization were further classified according to the type of involvement using the following question at baseline: Which clinic mainly follows you for your (diagnosis)? (a) Your primary care clinic, where your general practitioner is; or (b) your specialized clinic, where your specialist doctor is or specialist doctors are. Those answering (b) were classified as being co-managed by a specialist. For those who answered (a), we used a second question asked at the 18-month follow-up to further determine the type of involvement: In addition to being followed by a general practitioner for your (diagnosis), are you also followed by a specialist doctor? If yes, for how many years have you been followed by the specialist? Participants who reported being followed by a specialist for at least two years were also classified as being co-managed at entry into the study. All remaining participants were not considered as being co-managed – any contact with a specialist was considered to be on a consultant basis.

Independent variables

Patients' clinical and socio-demographic characteristics (diagnosis, severity of disease, co-morbidity, age, gender, region [urban = Montréal, rural = Montérégie]), highest level of education and perceived income] were measured at baseline using the survey and classified as potential need, predisposing or enabling factors for specialist services utilization (Andersen 1995; Feldman et al. 2007, 2009). Co-morbidity level was measured by the number of reported conditions from a list of 17 common chronic diseases (see Appendix available online at: www.longwoods.com/content/24036). Disease-specific health-related quality of life (HRQoL) questionnaires were used as a proxy for disease severity. The following tools were used: Health Assessment Questionnaire for arthritis (Bruce and Fries 2003; Maska et al. 2011), the Minnesota Living with Heart Failure (Garin et al. 2009; Sneed et al. 2001), the Chronic Respiratory Questionnaire (Lacasse et al. 1996; Schünemann et al. 2005) and the Audit of Diabetes-Dependant Quality of Life (Bradley et al. 1999). Scores were standardized on a common scale with a mean of 50 and a standard deviation (SD) of 10, with lower scores representing more severe cases. PCP's working experience was determined based on graduation year of the physician, which was available from administrative data of the Quebec College of Physicians (licensing board). Finally, at baseline, we determined the type of practice

arrangement (community health centre [CHC]/hospital-based family medicine unit, family medicine group [FMG], other physician group practice and solo practice) and the following organizational components: amount of diagnostic/therapeutic procedures available on-site, remuneration mode of physicians (fee-for-services vs. fixed salary/mixed) and presence of nurse with an innovative role (see Appendices A–D for description of all these practice variables). These practice data were determined based on the results from a survey of PHC practices completed one year prior to patient enrolment (Pineault et al. 2009).

Statistical analyses

Because of the nested sampling design and the possible clustering of patient/physician characteristics inside practices, hierarchical two-level logistic regression models with random intercept were computed to assess bivariate and multivariate associations between independent variables and co-management by a specialist. For multivariate analyses, patient's diagnosis, severity of disease, co-morbidity, age, gender, education, perceived income and PCP's experience were entered at the individual level, followed at the practice level by region and practice arrangement. To isolate the role of organizational components from practice arrangement and avoid model over-fitting, we calculated a separate model that included mode of remuneration, role of nurse and level of procedures without practice arrangement. Presence of collinearity between variables was assessed using Pearson and Spearman correlations, as well as contingency tables (see Appendices E and F for results). The likelihood ratio test was performed to assess model fit. The Wald test was used to assess if unexplained variance at patient level (τ_0^2) differed from zero, and the extended R^2 formula of McKelvey and Zavoina was used to determine the explained proportion of total variation of co-management (Snijders and Bosker 1999). We used HLM 6.03 software (Scientific Software International, Lincolnwood) with the Laplace estimation method and an alpha level fixed at 0.05 for all analyses.

Sensitivity analyses

To assess robustness of our results, we repeated the modelling strategy with a definition of co-management based solely on specialist utilization pattern using the provincial physician reimbursement administrative database. There is no operational definition of specialist co-management that has been proposed in the literature for administrative data. We chose to emphasize regularity of encounter with the specialist rather than PCP/specialist ratio or number of encounters to capture what we considered the most important aspect of co-management: being followed over the course of the disease. Therefore, patients who visited the relevant specialist at least once in each of the two consecutive years of available data were considered to be co-managed. Moreover, to further assess how our findings were specific to co-management, we also modelled factors associated with specialist involvement as a consultant in comparison to having no specialist utilization (patients with co-management were thus excluded from the analysis). Due to limitations in statistical power resulting from low rates

of specialist involvement as a consultant, this later analysis included only patient characteristics and was therefore performed using a standard logistic regression model.

Results

At baseline, 702 patients had complete data and were included in the present analysis. There were no significant differences between participants and non-participants according to diagnosis, gender and age (results not shown). Characteristics of participants are summarized in Table 1. The majority of participants were at least 65 years old (54.2%, minimum = 22, maximum = 97), had at least one co-morbidity (74.8%, minimum = 0, maximum = 13), had a PCP with at least 15 years of experience (90.0%) and were managed in a PHC group practice (91.5%).

TABLE 1. Characteristics of participants ($N = 702$)

Level	Characteristics	Proportion of participants* (%)
Patient	Diabetes as main diagnosis	34.6
	Congestive heart failure as main diagnosis	19.4
	Chronic arthritis as main diagnosis	26.8
	COPD as main diagnosis	19.3
	Co-morbidity, median (IQR) number	3.0 (1–4)
	HRQoL, mean (SD) score	50.1 (10.0)
	Age, mean (SD) years	67.1 (11.7)
	Female gender	54.8
	No high school diploma	46.7
	High school diploma	29.1
	Diploma > high school	24.2
	Income perceived less or much lesser	22.5
Physician practice	Experience of PCP, mean (SD) years	27.2 (8.5)
	Urban region	59.0
	Solo practice	8.5
	Physician group practice	34.9
	Family medicine group (FMG)	21.7
	Community health centre (CHC)/hospital-based family medicine unit	35.0
	Fee-for-services remuneration of PCP	65.1
	Nurse with innovative roles	51.5
	High level of diagnostic/therapeutic procedures	42.3

* Values are in % unless otherwise indicated.

COPD = chronic obstructive pulmonary disease, IQR = interquartile range, SD = standard deviation, HRQoL = health-related quality of life, PCP = primary care physician.

Overall, 314 participants (42.5%) had at least one encounter with a specialist in the two-year study period (median visits = 3, 1st quartile = 1 visit, 3rd quartile = 4 visits and maximum = 39 visits over two years). Of these 314 participants, 193 (61.6%) were classified based on their survey answers as co-managed by the specialist (i.e., 27.4% of the entire sample of 702). Co-managed patients had a mean number of 5.1 visits with the relevant specialist (SD = 4.6; 95% CI = 4.4–5.7), with 74.6% having more than two visits over the two-year period. The proportion of co-management varied between the 33 practices from 0% to 66.7% (median = 24.3%, interquartile range = 25.1%).

Table 2 (shown at www.longwoods.com/content/24036) presents proportions, crude odds ratios (ORs) and adjusted ORs of being co-managed for each of the factors. Depending on the diagnosis, 10% (diabetes) to almost half (CHF) of the patients were co-managed. Probability of being co-managed doubled or tripled depending on the type of practice arrangement (from 15% in FMGs to 40% in solo practices). Unadjusted analyses indicated that probability of being co-managed was significantly lower for those with less co-morbidity, lower education, lower perceived income, living in a rural region, having diabetes, less severe state of disease or being followed in an FMG practice. Multivariate analyses further indicated that probability of being co-managed by a specialist was lower for those who were older or were being managed in a PHC practice with a nurse assuming an advanced role, but was not associated with co-morbidity.

Assessment of model fit indicated that the proportion of total variation that remained unexplained at the practice level once individual-level variables were entered in the model was 6.0% ($\tau 0^2 = 0.26$, $p = 0.001$). It then decreased to 2.2% ($\tau 0^2 = 0.09$, $p = 0.019$) after introducing area of residency. Only 1.2% ($\tau 0^2 = 0.05$, $p = 0.035$) or 0.6% ($\tau 0^2 = 0.027$, $p = 0.090$) were remaining when practice arrangement or organizational components were respectively added. The explained proportion of the total variation of the logit of being co-managed by a specialist was 13.8% for clinical factors only, 21.1% with addition of socio-demographic ones and 22.3% after including practice arrangement.

Sensitivity analyses

Overall, 191 (27.2%) patients visited the specialist in each of the two consecutive years and were consequently classified as co-managed based solely on their utilization pattern. Similar results (not shown) were obtained using this definition of co-management, with the exception that patients managed in CHCs/hospital-based family medicine units demonstrated a lower probability of being co-managed compared with those managed in conventional group practices (adjusted OR = 0.57, 95% CI = 0.34–0.98). In fact, both definitions agreed on type of specialist involvement for two out of three patients (kappa statistic = 0.68; $p < 0.001$), and 76.1% of the patients identified as co-managed based on the survey were also classified as co-managed in these sensitivity analyses. When we computed a multivariate model of specialist involvement as a consultant (vs. no encounter with a specialist) as a function of patient characteristics, only diagnosis and area of residency were associated with specialist involvement as a consultant.

Discussion

We found that among those who had contact with a specialist, a majority had the specialist involved as a co-manager and that, overall, a quarter of adults with chronic diseases managed in PHC settings were co-managed by a physician specialist. The probability of being co-managed was associated with patient morbidity (index diagnosis and its severity), but not co-morbidity. Socio-demographic/economic characteristics of patients also accounted for a relatively important portion of the variation in being co-managed. Finally, we found that the prevalence of co-management varied between PHC practices, but that region was the main source of this variation rather than organizational practice characteristics.

Involvement of the specialist as a co-manager may be needed for health problems falling outside PCP management competencies such as severe or uncommon conditions (Starfield 2010). In contrast, for less severe and/or more common health problems, PCPs may have sufficient expertise to adequately manage the patient with the involvement of the specialist solely as a consultant to reduce clinical uncertainty or provide access to technical interventions/tests outside the PCP's scope of practice (Forrest 2009; Starfield et al. 2003). Our results are in accordance with these recommendations. Persons with CHF (a more severe condition) were most likely to be co-managed by a specialist (more than a third of patients), whereas persons with diabetes (a common chronic disease which may require less complex interventions and may be less symptomatic than the other ones) were the least likely to be co-managed (only a tenth of patients) (Deshpande et al. 2008; Halpin and Miravitlles 2006; Mosterd and Hoes 2007). Furthermore, persons who had more severe disease (lower HRQoL) were more likely to be co-managed. Co-morbidities, which add to the difficulty of patient management (Bayliss et al. 2008), would have been expected to increase probability of being co-managed. However, we found no association between co-morbidity and co-management after controlling for other covariates. Previous studies found that time to specialist consultation after onset of the chronic disease decreased with increasing burden of comorbidity (Feldman et al. 2007, 2009). Interestingly, they used the Charlson Co-morbidity Index, which also captures the severity of co-morbidities by weighting them according to their mortality (Charlson et al. 1987). There is currently no gold standard for measuring co-morbidity (Alibhai et al. 2008; Valderas et al. 2009), and for practical reasons, we created our own index limited to a co-morbidity count. Perhaps severity rather than quantity of co-morbidities may be the actual driver of the decision-making process to involve specialists in chronic disease care. Further research is thus needed to clarify the true impact of co-morbidity on specialist involvement in ambulatory patients with chronic diseases and help determine the best co-morbidity index to use in this research area.

We found that lower education and perceived income, as well as older age, were associated with decreased probability of being co-managed. This is consistent with findings in specialist services utilization literature (Chan and Austin 2003; Feldman et al. 2007, 2009). The present findings may reflect differences in preferences and/or barriers to specialist care (Chan and Austin 2003). However, they add to the literature indicating that even under universal health

insurance for physician services, access to specialist care contradictorily remains prone to social inequalities among patients with different socioeconomic status (Chan and Austin 2003; Feldman et al. 2007, 2009). Our results also suggest that the probability of co-management is almost halved for patients living in rural regions compared with those in urban ones. It is possible that physicians in rural regions prioritize specialist involvement as consultants in a context of limited availability of specialized resources. Indeed, the ratio of specialists per person is four times higher in the Montréal urban area than in the Montérégie rural region (Gouvernement du Québec 2004), and it is well-documented that referral, access and utilization of specialist services decrease with lower availability of resources (Boyle et al. 2006; Chan and Austin 2003; Feldman et al. 2009; Forrest et al. 2006; Jaakkimainen et al. 2003). As these socio-demographic discrepancies accounted for a large amount of variation in being co-managed, further studies are needed to determine whether they reflect underuse versus overuse to promote health equity.

Experience of the PCP could affect the decision to refer to a specialist (Feldman et al. 2007, 2009; Forrest et al. 2006). However, physician experience (PCP or specialist) may not affect the type of involvement desired (Swarztrauber and Vickrey 2004) and our results are in accordance with this. Further research on attitudes and beliefs of physicians is needed to better understand the decision-making process leading to co-management.

Consistent with the literature suggesting that PHC practice characteristics account for only 5%–10% of specialist services referral and utilization (Forrest et al. 2006; Sullivan et al. 2005), we found that PHC practice characteristics may account for a limited amount of total variation of specialist co-management. Our results suggest that practice models with more attributes valued for chronic care (e.g., teamwork, increased accessibility, networking activities with community), such as FMGs and CHCs/hospital-based family medicine units, may be associated with less patient co-management, contrarily to those with lesser ones, such as solo or conventional group practices (Adams et al. 2007; Dennis et al. 2008; Levesque et al. 2012; Stange et al. 2010). However, having a nurse with an innovative role (e.g., practice nurse) sharing clinical activities with physicians may contribute significantly to explaining these findings. Indeed, we found that patients who were managed in practices that had a nurse in an innovative role (e.g., FMGs and CHCs/hospital-based family medicine units) were less likely to be co-managed than those in solo and conventional group practices (who typically do not have nurses in these positions). In accordance with the literature, we did not find an association between physician remuneration mode and co-management (Swarztrauber and Vickrey 2004).

Limitations

Our study did not assess the quality of collaboration with the specialist, health outcomes or appropriateness of co-management. Practices that participated in the study likely have PCPs with favourable attitudes towards research and management of chronic diseases. Thus, differences observed between practices participating in our study may be less than true dif-

ferences in the entire population of PHC practices. Our context is one in which all residents are insured for medical care and self-referral is limited. Thus, there may be limits in terms of generalization to healthcare systems with different accessibility to specialist services. Finally, this study addressed only physician specialist involvement. Therefore, results may not apply to paramedical specialists' involvement such as dieticians for diabetes or physiotherapists for arthritis patients.

Conclusions

About one-fourth of patients with chronic diseases managed in the primary care setting are also co-managed by a medical specialist physician. Severity of the index condition is strongly associated with being co-managed by a specialist. Younger persons, those living in urban regions and those with higher level of education and financial means were further more likely to be co-managed. Organizational practice characteristics may account only for a limited amount of variation in distribution of co-management among patients, and interdisciplinary group practice involving a nurse with an innovative role may decrease co-management by a specialist. Outcome research studies are obviously needed to determine whether type of specialist involvement (consultant vs. co-manager) relates to different outcomes for persons with chronic diseases beyond the difference in frequency of contacts with the specialist.

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RESEARCH PAPER

Understanding the Research–Policy Divide for Oral Health Inequality

Comprendre le rôle des disparités entre recherche et politiques dans les inégalités en santé buccodentaire

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Abstract

Background: No studies exist of the congruence of research in oral health to policy. This study aimed to examine the broad congruence of oral health research to policy, and implications for developing oral health research that is more policy relevant, particularly for the wider challenge of addressing unequal oral health outcomes, rather than specific policy translation issues.

Methods: Bayesian-based software was used in a multi-layered method to compare the conceptual content of 127,193 oral health research abstracts published between 2000–2012 with eight current oral health policy documents from Organisation for Economic Co-operation and Development countries.
Findings: Fifty-five concepts defined the research abstracts, of which only eight were policy-relevant, and six of which were minor research concepts.
Conclusions: The degree of disconnection between clinical concepts and healthcare system and workforce development concepts was striking. This study shows that, far from being "lost in translation," oral health research and policy are so different as to raise doubts about the extent to which research is policy-relevant and policy is research-based. The notion of policy relevance encompasses the lack of willingness of policy makers to embrace research, and the need for researchers to develop research that is, and is seen to be, policy-relevant.

Résumé

Contexte : Il n'y a pas d'étude sur la congruence entre la recherche et les politiques en matière de santé buccodentaire. Cette étude vise à examiner la congruence générale entre la recherche et les politiques en matière de santé buccodentaire ainsi que ses répercussions pour le développement de recherches plus pertinentes pour les politiques, particulièrement face aux grands défis d'inégalités en matière de santé buccodentaire, plutôt qu'en fonction d'enjeux touchant au transfert des politiques.
Méthode : Nous avons employé un logiciel de type bayésien pour effectuer une comparaison multiniveau entre, d'une part, le contenu conceptuel de 127 193 résumés de recherche en santé buccodentaire publiés entre 2000 et 2012 et, d'autre part, huit documents actuels de politiques en matière de santé buccodentaire provenant de pays membres de l'Organisation de coopération et de développement économiques.
Résultats : Cinquante-cinq concepts définissaient les résumés de recherche, dont seulement huit étaient pertinents pour les politiques et six présentaient des concepts de recherche mineurs.
Conclusions : Le degré de disparité est frappant entre, d'une part, les concepts cliniques et, d'autre part, les concepts touchant au système de santé et au développement de la main-d'œuvre. Cette étude montre qu'au-delà de se perdre dans les processus du transfert, les politiques et la recherche en santé buccodentaire sont tellement différentes qu'on en vient à douter de la pertinence politique des recherches et du bien fondé scientifique des politiques. La notion de pertinence politique comprend le manque de volonté de la part des responsables de politiques d'adhérer à la recherche tout comme le besoin pour les chercheurs de développer des projets qui sont (et semblent) pertinents du point de vue des politiques.

Policy studies are defined as scholarly, peer-reviewed analyses of the nature of the processes by which policy is formed and such studies form a small but growing body of work within the oral health literature. As of May 24, 2013, there were 1,213 items in PubMed with the terms "oral health" OR "dental" OR "dentist" OR "periodontal" AND "policy" – terms that are likely to capture broad oral health research, as well as explicit policy content. A total of 762 of these items had been published since 2000. A far smaller set focussed on the translation of research into practice (Rogers 2012): improving the translation of data from clinical trials (Barnett and Pihlstrom 2004), for clinical guidelines (Clarkson 2004; Clarkson et al. 2010) and for specific challenges such as women's oral health (Daley et al. 2013).

Currently, no studies exist of the congruence of oral health research to policy, nor of the implications of this congruence for developing more policy-relevant oral health research. Between 2000 and 2012, at least 127,193 unique papers with abstracts were published in oral health. This body of literature has not been systematically analyzed for its content relevance to oral health policy priorities. A focus on the content of this literature is particularly relevant to the study of evidence translation. While a quality hierarchy influenced by the "blue chip" standard of randomized controlled trials dominates health sciences research, health policy research suggests that the congruence of research content to policy may be more important than research methodology in policy take-up of research (Bell 2010; Dobbins et al. 2007a, 2007b; Mays et al. 2005; Sorian and Baugh 2002).

If the content of research can be determined through research databases, indicative evidence of oral health policy priorities exists in the form of oral health policy statements produced by governments. In a previous study (Bell and Seidel 2012), we explored how disadvantaged groups are represented in oral health policy statements from Organisation for Economic Co-operation and Development (OECD) countries. We found that concepts for "vulnerable or disadvantaged groups are usually, but not always, the least frequent concepts" in these documents. The current study describes the conceptual content of the entire corpus of oral health research abstracts from 2000 to 2012 and compares this content to that of oral health policy documents. The study aims to build an understanding of the nature of the research–policy divide so that research can better serve policy efforts to address oral health inequity.

Method

This study used Leximancer semantic analysis software developed at the University of Queensland, Australia (https://www.leximancer.com/). It is useful for researchers who need to explore large text-based data sets. Leximancer is a Bayesian-based application of computational linguistics in which "machine learning" methods are applied to the analysis and synthesis of language. We compared two different samples. The first sample was 127,193 oral health abstracts published from January 1, 2000 to December 31, 2012, treated as indicative of all

oral health research. The second sample comprised government oral health policy documents, published between 2004 and 2012, from eight OECD countries. A quantitative content analysis was performed using the Leximancer software to describe the changing content of oral health research, by year, from 2000 to 2012. This was compared with the results of the same procedure for analyzing the content of the eight policy documents.

Research questions

The research questions were "How well-matched is the content of research to oral health policy?" and "What are the implications of this for developing oral health research that is more policy relevant, particularly for the challenge of addressing unequal oral health outcomes?"

Oral health literature sample

The oral health abstracts were obtained using search terms in the Scopus database as follows: "oral health" OR "dental" OR "dentist" OR "periodontal." The terms were developed in discussion with oral health practitioners to avoid the systematic exclusion of a large corpus of oral health literature. The sample of abstracts was designed to be sufficiently large and the findings sufficiently broad so as not to be substantively different with the addition of more specific words. One difficulty in having too large a sample size is that as the number of found concepts increases, the concept map becomes too detailed, and the manual checks become impractical, yet often the substantive broad findings do not change. The addition of the word "caries," for example, increases the sample size but only by 4%, and separate analysis of that relatively small corpus does not change the findings obtained from the broad sample that includes 15,928 abstracts containing the word "caries" and the chosen search terms.

The available evidence suggests that the Scopus database offers 20% more coverage than Web of Science; PubMed being better for biomedical sources, and Google Scholar being less accurate (Falagas et al. 2008). Scopus is therefore the largest abstract and citation database of peer-reviewed literature in the world, including for all countries in this study. Yet the study is not a review of databases but rather an analysis of one highly authoritative database. The research database search period began on January 1, 2000; the earliest date for the policy sample search was 2004 to allow for a reasonable lag time for the translation of research into policy. The results of both searches were exported as CSV files in manually set batches of 2,000 abstracts (a download limitation of Scopus). The downloaded abstracts were subsequently added to a SQLite database to generate CSV files with abstracts for a full year. This procedure involved manual checks of data consistency and removal of duplicate abstracts. Table 1 provides the number of unduplicated abstracts, published by year, for the sample of 127,193 abstracts.

TABLE 1. Number of oral health research abstracts, by year, from 2000 to 2012

Year	Number of research abstracts
2000	6,073
2001	5,624
2002	6,566
2003	8,417
2004	9,504
2005	10,084
2006	10,153
2007	11,125
2008	12,057
2009	11,714
2010	12,127
2011	11,570
2012	12,179
Total	**127,193**

Oral health policy sample

The oral health policy documents published between 2004 and 2012 came from Wales (WG 2012; a draft report), the US (CDC 2011), Northern Ireland (DHSSPS 2007), New Zealand (MOH 2006), Canada (FPTDDWG 2005), England (DH 2005), Scotland (SE 2005) and Australia (NACOH 2004). These oral health policy documents, like the abstracts, were treated as indicative evidence, i.e., of oral health policy understandings, not necessarily what has been implemented. Our previous study of the representation of disadvantaged groups in oral health policy (Bell and Seidel 2012) used criteria from other international comparative studies to define a policy statement as follows:

- "current statements in English (not in languages other than English unless an English version of the statement is also available);
- statements by government agencies (not health professional associations or other organisations);
- explicit policy guidelines and planning statements, such as oral health plans, strategies and vision documents (not oral health implementation or activities reports or indicators for oral health system performance or policy recommendations in oral health research reports, etc.);
- national (not international, multi-country, state or provincial) policy statements; and
- 'stand-alone' oral health policy documents (not general health policy documents or health policy documents with a single oral health section)."

These criteria resulted in a total of eight documents yielded by searches of the websites of agencies in 34 OECD countries and follow-up queries for publicly available documents. Non-sovereign countries, i.e., of the United Kingdom, were included as were draft documents (WG 2012) if they met the sample criteria. Snowballing techniques scrutinizing the applied and scholarly literature also were applied to confirm the sample set. Accordingly, the study was not a study of the multi-faceted politics that influence dental care systems and their development, and the roles of professional and consumer groups in policy advocacy. Rather, it was a study of the translation of scholarly oral health research into oral health policy statements as they relate to health inequalities.

Analytic procedure

OVERVIEW

The analytic procedure had two stages: (1) mapping concepts in the research abstracts and then the policy documents, and (2) manually comparing these two sets of concepts. These analyses were performed using the Leximancer software (version 4). In Leximancer, the unit of analysis is a "text block" about the length of a paragraph. The software is able to learn from a corpus of uploaded texts in an iterative fashion. The analysis creates a network of Venn diagrams as a spatial representation of interconnected concepts — a concept map.

The technical aspects of the Bayesian-based Leximancer software have been explored in validity studies (Smith and Humphreys 2006) and in hundreds of applications (Baker et al. 2011; Cretchley et al. 2010; Hepworth and Paxton 2007; Hewett et al. 2009; Kuyini et al. 2011; Kyle et al. 2008; Pakenham et al. 2012; Travaglia et al. 2009). The algorithm-based nature of Leximancer draws on the discipline of computational linguistics. Leximancer has been defined as an automated approach to transforming co-occurrence information about words into semantic patterns. The algorithms used in Leximancer involve machine learning as summarized in the technical validity study.

A unified body of text is examined to select a ranked list of important lexical terms on the basis of word frequency and co-occurrence usage. These terms then seed a bootstrapping thesaurus builder, which learns a set of classifiers from the text by iteratively extending the seed word definitions. The resulting weighted term classifiers are then referred to as concepts. Next, the text is classified using these concepts at a high resolution, which is normally every three sentences. This produces a concept index for the text and a concept co-occurrence matrix. By calculating the relative co-occurrence frequencies of the concepts, an asymmetric co-occurrence matrix is obtained. This matrix is used to produce a two-dimensional concept map via a novel emergent clustering algorithm. The connectedness of each concept in this semantic network is used to generate a third hierarchical dimension, which displays the more general parent concepts at the higher levels (Smith and Humphreys 2006).

The usefulness of the software lies not simply in its concept maps and supporting quantitative data that allow scoping of a large body of qualitative data. Leximancer also facilitates manual checks of large qualitative data sets. It provides multiple data viewing windows that allow the analyst to scrutinize the text on which the data are based, in the context of the original uploaded text. In the current study, this allowed sequential manual checking procedures to extend the machine-driven findings.

STAGE 1. MACHINE-AUTOMATED MAPPING OF THE CONTENT OF ABSTRACTS AND POLICY DOCUMENTS

In the first stage of this study, the automated Leximancer procedures were used to ensure that the two different samples – the research abstracts and the policy documents – were subjected to the same research procedure for their analysis. The research abstracts were uploaded in Leximancer to produce a concept map, and, in a separate procedure, the policy documents were uploaded in Leximancer to produce a second concept map. Concepts were therefore selected by the software with only one kind of intervention by the analyst. Meaningless concepts such as structural features of abstracts ("aims" or "conclusions") were removed as mapping concepts as were names of countries, i.e., this content was not excised but rather subsumed by Leximancer under other mapping concepts. This intervention was designed to ensure that the data output produced by Leximancer represented the conceptual content of the two different corpuses. This analysis produced concept maps showing not only the semantic placement of one concept relative to all other concepts, but also the pathways or connections across multiple concepts.

STAGE 2. MANUAL CHECKS AND COMPARISON OF CONCEPTS

In the second stage of the study, manual checks aimed to further document substantive conceptual differences and similarities between the research abstracts and the policy documents, particularly as they related to health inequity. These checks were undertaken in two steps as follows.

STEP 2.1

A set of categories to support the manual comparisons was developed by scrutinizing all concept words – the tag word used to describe a concept by Leximancer, as identified in Stage 1. Four possible categories for the concept words were decided: (1) matching concept words (in whole or part of case) that had the same apparent meaning, (2) matching different concept words that had the same or very similar meaning, (3) matching concept words (in whole or part of case) that had a different substantive meaning and (4) identifying concept words that did not match and were most unlikely to have the same or similar meaning. All concepts from both the Stage 1 analysis of the abstracts and the policy documents were placed in these four categories.

STEP 2.2

The qualitative data relevant to the first three categories of concept words were scanned in both the abstracts and the policy documents using Leximancer's data viewing windows. A specific data viewing window that extracted the sentence in which the concept appeared expedited the scan. The larger context of the sentence was extracted only when the meaning of the concept word in the sentence was not obvious. This exercise focused only on obvious, not subtle, differences in meaning; for instance, the difference between the word "system" used to refer to biological systems in the research abstracts and healthcare systems in the policy documents. It also focussed on identifying whether the concept sentences belonged in the category at least two-thirds (>66%) of the time.

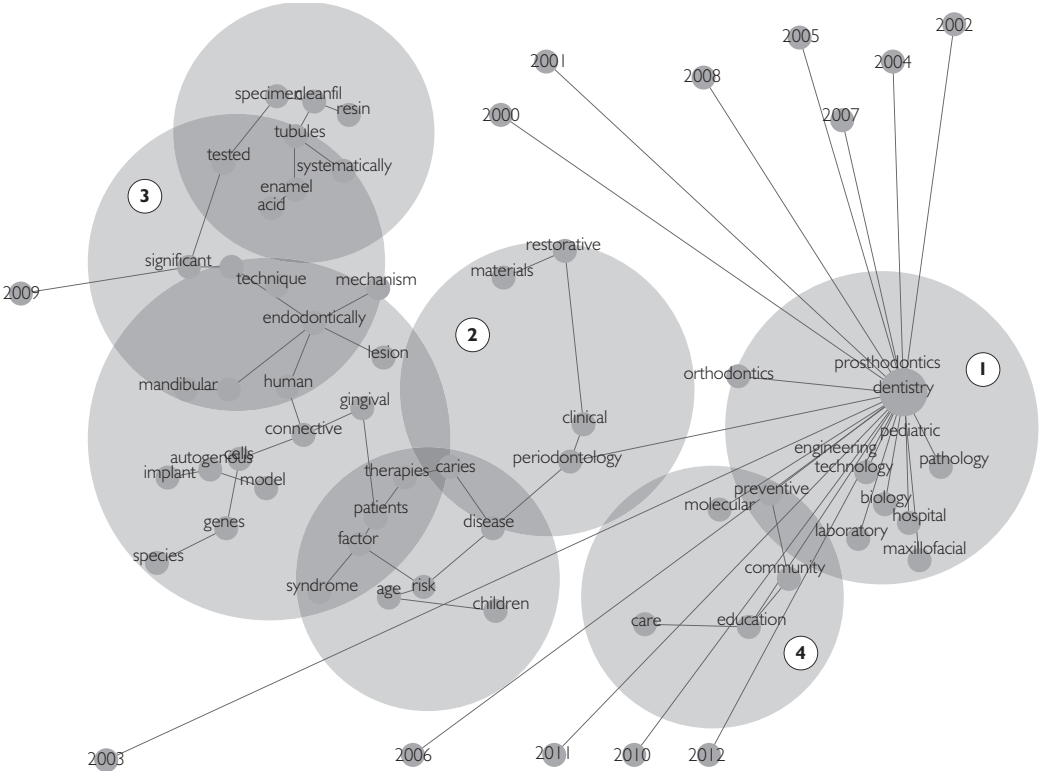
Results and Discussion

Machine-automated content mapping

Two concept maps with supporting data were produced: one map with 55 concepts for the research abstracts and another map with 33 concepts for the policy documents. In the 127,193 research abstracts, 1,604,212 instances of the mapped concepts were found by Leximancer in 1,197,367 distinct text blocks. In the policy documents, 14,612 instances of the mapped concepts were found in 3,482 distinct text blocks. The use of two different sets of data analyses, one for each data set, means that differences in the size of the data sets are not an issue.

Figure 1 illustrates the map of the 55 concepts identified in the research abstracts using the search terms "oral health, dental, dentist" and "periodontal." As is evident in Sphere #1, abstracts from all years except 2009 related directly to dentistry with immediate linkages to periodontology, clinical and restorative work (Sphere #2), and continuing pathways to less frequently occurring concepts. Abstracts from 2009 focused on the concepts of significant techniques in orthodontics (Sphere #3). When the relationships between concepts are close, the software application presents these concepts as overlapping, e.g., "significantly" and "canals" in Sphere #3. For clarity, all 55 concepts are listed individually in Table 2 (see Table 2 at www.longwoods.com/content/24037). The placement of concepts in the concept map is determined by their overall co-occurrence – for an individual concept and for its relationships with all other concepts. The lines within and between the circles show the relationships among multiple concepts. The size of the dots within each circle reflects the extent to which a concept co-occurs with all the other concepts; the larger the dot, the greater the co-occurrence.

FIGURE 1. The 55 identified concepts in oral health research abstracts, by year, and their inter-relationships



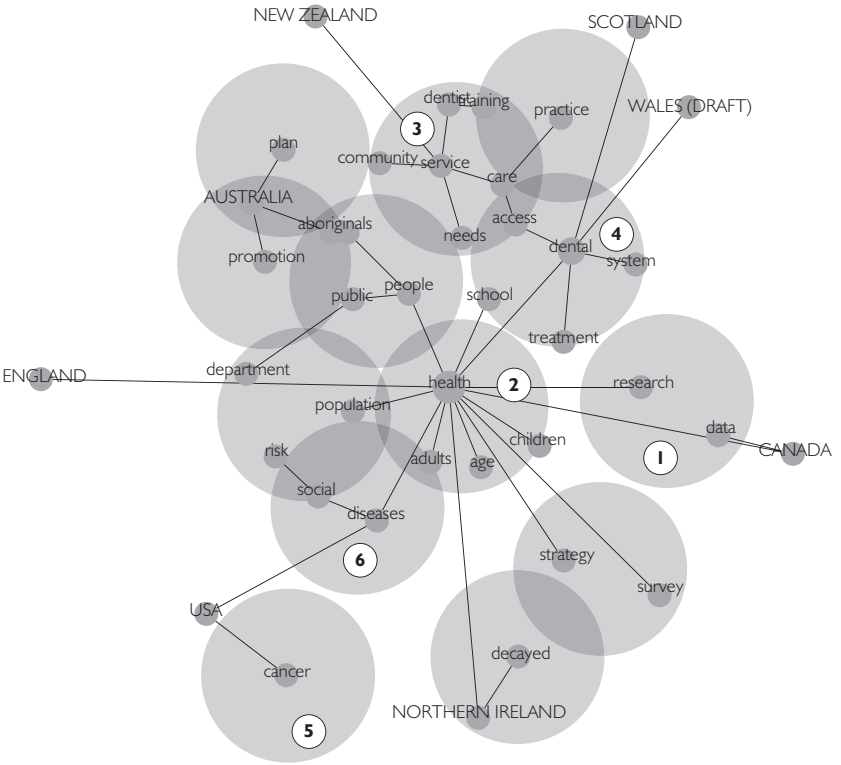
Note: The software application presents close relationships as overlapping concepts. Spheres 1 and 2 = the most frequently occurring concepts and their linkages; Sphere 3 = concepts from 2009 abstracts showing overlapping relationships; and Sphere 4 = less frequently occurring concepts with fewer linkages. All concepts are listed individually in Table 2.

In Figure 1, clinical concepts, predominantly laboratory-based and clinical evidence concepts, dominate and are well-connected. Sphere #1 indicates the most frequently occurring concepts and a high degree of co-occurrence. In contrast, healthcare system and workforce development concepts, such as "care," "education" and "community," seen in Sphere #4, are less frequent and poorly connected, as suggested by the lack of lines connecting them to one another or to other concepts. This indicates that, even when healthcare system and workforce development concepts appear in research-based literature, these concepts are not part of well-developed theoretical constructs that allow connections to be developed between them and clinical research. The concept map in Figure 1 suggests a visually striking degree of disconnection between clinical concepts and healthcare system and workforce development concepts. Yet, the abstracts for the most recent years (2010–2012) are closest to the healthcare system and workforce development concepts, suggesting oral health research has included more of this kind of content in recent years.

Figure 2 maps the 33 concepts identified in the oral health policy documents from the eight OECD countries, and their inter-relationships. These 33 concepts also are listed individ-

ually in Table 2. The lines from each country to a particular sphere, e.g., from Canada to "data" in Sphere #1 and to "health" in Sphere #2, identify the primary concepts in the oral health policy documents of each country. The overlap between planning, promotion and aboriginal issues in oral health policy for Australia reflects a focus on indigenous issues that then links to people and health. At the time of data collection, the oral health policy for Wales was in draft form and this is noted in the Figure.

FIGURE 2. The 33 identified concepts in oral health policy documents, by country, and their inter-relationships



Note: The software application presents close relationships as overlapping concepts. Spheres 1, 5 and 6 illustrate the limited links between disease and research in policy documents. Sphere 2 shows the focus on health for people of all ages in policy documents. Sphere 3 illustrates the most frequently occurring concepts in policy documents. Sphere 4 illustrates the least frequent concepts. All concepts are listed individually in Table 2.

When compared with the concept map of oral health research abstracts in Figure 1, the map in Figure 2 suggests how much more the concept of health, not clinical dentistry concepts, is integral to policy. The more common concepts in the spheres in Figure 2 are concepts about services, care, needs, access, community and training – not clinical concepts. Where the concept of dentist occurs (Sphere #3 in Figure 2), it is proximate to the concept of training. Similarly, where the concept of dental occurs (Sphere #4), it is typically connected with the system concept.

When comparing Figures 1 and 2, it appears as if oral health research preoccupations are the opposite of policy preoccupations. Figure 1 demonstrates that clinical and disease concepts

are the best connected and most frequent concepts (as the supporting data suggest), with the opposite being true of the healthcare system and workforce development concepts. In striking contrast, mapping the policy documents in Figure 2, specific disease concepts such as cancer (Sphere #5) and research and data concepts (Sphere #1) tend to be less common (as the supporting data also suggest) and less well-connected to the workforce and healthcare system concepts. Disease, as a general concept, is in fact proximate to the social concept in the policy documents, as shown in Sphere #6 (Figure 2). The data in Figures 1 and 2 raise the question of whether similar concepts in the two figures mean different things or whether apparently different concepts mean the same thing. In other words, is the content of oral health research even more different, or more similar, than these figures suggest? Manual checks comparing the substantive meaning of the different concepts allowed exploration of the answers to this question.

Manual checks and comparison of concepts

Table 2 lists the 55 concepts identified from the 127,193 oral health research abstracts and the 33 concepts identified from the eight oral health policy documents, along with their frequency and likelihood of occurrence, as documented by the Leximancer analyses. In a manual check of the data, it was not necessary to read the entire corpus of oral health research abstracts, as few of the concepts in the research abstracts actually matched or appeared related to the policy concepts. In the research abstracts, the 10 concepts highlighted in Table 2 were scanned. These 10 concepts represented a total of 488,525 sentences. In the policy documents on the right side of Table 2 also, 10 concepts were scanned. These 10 concepts represented a total of 5,372 sentences.

The data in Table 2 demonstrate that only eight of the 55 research concepts appear policy-relevant in the sense that they mean the same as concepts used in policy language. These shared concepts are dentistry/dental, patients/people, care, disease/diseases, age, children, risk and community. However, only two of these concepts (dentistry, patients) are in the top 12 most common research concepts, whereas seven of these concepts (dental, care, people, promotion, diseases, children and community) are in the top 12 policy concepts. Only six of the eight shared concepts involve using the same concept word to mean the same thing. The concept of community is one of the least important research concepts with a likelihood of occurring 3% of the time in any one of the distinct text blocks in the corpus of abstracts. However, the concept of community is four times more likely to appear in the policy documents with an occurrence likelihood of 12%. Most concepts relevant to healthcare system and workforce development in the policy concepts in this analysis have no equivalent concept in the research concepts.

Conclusions and Implications

This study has suggested a striking degree of disconnection between oral health clinical

research concepts and healthcare system and workforce development concepts. Its findings indicate that, far from being "lost in translation," oral health research and policy are so different as to raise doubts about the extent to which research is policy-relevant or policy is research-based. That is, most of the research does not appear to be policy-relevant, and most of the time, policy does not appear to be drawing on research. Measuring the research–policy divide helps to better understand the difficulty of reforming oral healthcare. It suggests not only why, but also where, research could better address the healthcare system priorities of policy makers.

In this study, the key directions for oral health research suggested by leading policy concepts lie in developing a health-based concept of oral health, informed by services and workforce development research that would allow better design of oral healthcare delivery. In so doing, this study indicates that the nature of the research–policy divide for addressing oral health inequality is not simply about evidence translation. It is also about the nature and sufficiency of particular kinds of evidence in oral health. Research cultures are sometimes arguably more focused on generalizable measures of significance to do with disease and risk factors rather than local healthcare systems and communities. Therefore, it is possible that differences between research and policy would have most likely been even larger if provincial or state policy documents were the focus of the study. The present research reflects the fact that the specialty area of dental public health, which is more likely to focus on policy-relevant concepts such as access, systems delivery and community perspectives, is a small and sometimes marginalized area within dentistry. Therefore, the study is potentially useful to those who wish to call for a greater emphasis on dental public health, including professional and consumer associations acting as policy advocates.

The debate about the policy relevance of research is ultimately about values. No claim is made here that there should be a perfect alignment of research and oral health policy. Rather, the study raises the question of whether the divide should be as large as the indicative evidence here suggests. It is hoped that this study will contribute to deeper discussion in oral health about the extent to which research is serving the development of sound policy – one way in which research can contribute to solutions for disadvantaged groups. If the results of the knowledge production system are not empirically measured – to demonstrate the extent to which policy and research are aligned – it is difficult for those who want to question such knowledge production systems to have any basis for doing so.

The notion of "policy persuasive research" may help frame these findings. It would appear from the results of this study that high-quality research does not always influence policy. While professional bodies may advocate for evidence-based research to be used in policy, this study suggests that little of this research actually becomes policy. The broader policy literature suggests many explanations for the research–policy disconnect in health – what is lacking is empirical measurement of the divide, particularly in oral health. It is known that the research–policy divide in health has a complex causality (Nutley et al. 2007): policy making is aligned

to political, financial and strategic imperatives (Corrigan and Watson 2003; Lavis et al. 2001); evidence does not always capture different stakeholder needs (Lavis et al. 2002); and many "real-world" local contextual limitations unaccounted-for by researchers apply to policy making (Innvaer et al. 2002). Analysis of the literature explores debates about how quantitatively defined disciplines such as oral health too often fail to capture the complex contexts of policy, while qualitative research is perceived as lacking the defensible rigour required for the adversarial and warring interests found in policy contexts (Bell 2010; Bell and Seidel 2012).

Whatever the agreed-on cause, or however the values define the debate, this study has suggested that research in oral health and the development of oral health policies are two conversations happening in parallel. It provides some support for those wanting to make the two conversations coalesce and result in changes to oral health policy. Some public oral health debates have managed to achieve this single conversation, such as the water fluoridation debate. Oral health researchers might use results of the current study and the fluoridation example to develop strategies to make their research more policy-persuasive.

Finally, this study has suggested that machine- and algorithm-based approaches can help measure language phenomena where anecdote and opinion about research translation have not worked so well to create a basis for change. There are around 1,000 published papers applying text analytics, computational linguistics or natural language processing in the health sciences, but none of these is a study of the research–policy divide. The corpus of policy research is vast, and deals extensively with the research–policy divide; however, to date, it has not used algorithm, machine-based language approaches such as Leximancer to help measure that divide, even indicatively. *The Lancet* has published an influential suite of papers on research waste arguing that 85% of medical research investment is wasted (Chalmers and Glasziou 2009); however, these papers focused on research translation into practice, not policy (Chalmers et al. 2014). Results of the current study hopefully will contribute to informed debate about what kinds of methods can help evaluate the value to the community of its investment in research, particularly for the most vulnerable and disadvantaged, and especially in fields such as oral health where the problem of research translation has been so little treated.

Acknowledgements

The authors wish to honour the extraordinary life and work of Dr. Erica Bell and her drive in having this paper published. Erica published over 100 academic research papers and five books during her 10 years at the University of Tasmania, as well as publishing two historical novels. Erica is remembered by her family, friends and colleagues as a warrior of creative thought through her positive attitude, her all-encompassing love of life and her outstanding achievements.

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RAPPORT DE RECHERCHE

Trois types de stratégies des fabricants pour la fidélisation aux médicaments de marque

Three Types of Brand Name Loyalty Strategies Set Up by Drug Manufacturers

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Résumé

La restructuration de l'industrie pharmaceutique a mené au développement de trois nouveaux types de stratégies commerciales pour la fidélisation de différentes cohortes de patients à des médicaments : la fidélisation par le rabais, par l'accompagnement et par la compassion. La fidélisation par le rabais vise à maintenir les traitements au produit de marque et décourager la substitution au produit générique. La fidélisation par l'accompagnement est basée sur une offre des services de suivi et d'accompagnement à domicile et par téléphone afin d'encourager les patients à adopter un traitement puis d'en améliorer l'observance. Enfin, l'industrie offre des programmes de compassion où les patients peuvent recevoir des traitements avant même que le médicament ne soit généralement disponible ou remboursé par son assureur. Dès que le médicament (le plus souvent très dispendieux) est inscrit à la liste des médicaments remboursés, le manufacturier met fin au programme de compassion et bénéficie d'une importante cohorte de patients déjà sous traitement.

L'impact de ces programmes sur les politiques publiques et les droits des patients soulève de nombreuses préoccupations, au nombre desquelles figurent au premier plan l'accès direct du fabricant au patient et ses données de santé et la pression à la hausse sur les coûts de l'assurance-médicaments.

Abstract

The recent restructuring of the pharmaceutical industry has led to three new types of promotional strategies to build patient loyalty to brand name drugs: loyalty through rebates, patient support, and compassion programs. Loyalty through rebates seeks to keep patients on a brand name drug and prevent their switch to the generic equivalent. Loyalty through patient support provides aftersales services to help and support patients (by phone or home visits) in order to improve adherence to their treatments. Finally, compassion programs offer patients access to drugs still awaiting regulatory approval or reimbursement by insurers. When and if the approval process is successful, the manufacturer puts an end to the compassion program and benefits from a significant cohort of patients already taking a very expensive drug for which reimbursement is assured.

The impact of these programs on public policies and patients' rights raises numerous concerns, among which the direct access to patients and their health information by drug manufacturers and upward pressure on costs for drug insurance plans.

Introduction

Contexte général

L'industrie pharmaceutique de médicaments novateurs[1] est en profonde restructuration et ses pratiques de promotion du médicament en mutation. La convergence assez unique de fin de brevet de plusieurs médicaments vedettes dits « blockbusters » (générant un revenu supérieur à 1 milliard $ par année) sur une courte période de temps[2], affecte une très grande part des revenus de l'industrie. De plus, contrairement aux décennies précédentes, l'industrie pharmaceutique ne dispose pas suffisamment de nouveaux médicaments novateurs en réserve pour prendre le relais. Un rapport d'enquête de la Commission européenne identifiait dès 2008 ces deux phénomènes comme étant à la source d'une restructuration importante de l'industrie pharmaceutique et comme cause de la mise en place par les fabricants de nouvelles stratégies, notamment pour prolonger la durée de vie de leurs médicaments vedettes arrivés en fin de course (European Commission 2008). Réagissant à son environnement, l'industrie pharmaceutique remodèle ses stratégies industrielles, mais aussi de commercialisation des médicaments (Abecassis et Coutinet 2008 et 2009).

Même si la valeur commerciale d'un brevet ne prend pas fin dès l'expiration du brevet (Hudson 2000), les fabricants s'ajustaient à la réduction importante de leurs ventes, quoique

graduelle, en présence de la concurrence des génériques (Avorn 2011). En effet, l'introduction à un prix inférieur des médicaments génériques bio-équivalents permet la substitution graduelle du médicament, se traduisant par des économies significatives pour les patients et leurs assureurs. L'industrie aurait maintenant décidé de modifier sa stratégie face à son nouvel environnement.

Objet de l'article

L'Ordre des pharmaciens du Québec nous a mandatés pour préparer et animer une journée de discussion tenue à Montréal le 27 novembre 2013 avec les principaux intervenants québécois du domaine de la santé et des médicaments, portant sur les stratégies de fidélisation de l'industrie pharmaceutique. Afin d'embrasser un cadre plus large que celui du Québec et mieux cerner les ressorts internationaux et systémiques du phénomène, nous avons fait une revue de la littérature sur les programmes de fidélisation au Canada, aux États-Unis et en France. Au-delà des publications académiques, nous avons incorporé à notre analyse divers documents gouvernementaux, corporatifs et promotionnels du Canada, publiés sur le web ou remis par l'Ordre. Nous présentons ici les premiers résultats de cette analyse sous la forme d'une typologie des principaux mécanismes de fidélisation mis en œuvre par l'industrie pharmaceutique. Le but est de distinguer trois types de stratégies qui, chacune à leur façon, visent à fidéliser des cohortes de patients à la prise de certains médicaments. Nous présentons ces trois stratégies, en identifiant le contexte et le type de produit visé, les mécanismes de fonctionnement de la stratégie et les principales critiques formulées.

Des stratégies commerciales de fidélisation

Les médicaments viennent au deuxième rang des coûts de santé au Canada et au Québec, après les hôpitaux et avant les médecins. Le secteur du médicament est le seul de ces trois principaux segments dont le marché est mondialisé, directement soumis à des forces déployées à l'échelle internationale (OCDE 2008; Schoonveld 2011). Ainsi, les mesures de contrôle des coûts des médicaments mis en place par les pouvoirs publics sont particulièrement vulnérables aux stratégies commerciales de la grande industrie pharmaceutique.

Les fabricants de médicaments ont toujours usé de stratégies pour maximiser la valeur de leurs brevets (Pearce 2006), mais l'industrie a quelque peu modifié ses pratiques au cours des dernières années. En plus des stratégies promotionnelles traditionnelles comme les visites chez le médecin, la remise d'échantillons, ou les rencontres promotionnelles (Gagnon et Lexchin 2008), les fabricants déploient de nouvelles stratégies en offrant aux patients un rabais, un service, une assistance, ou encore un médicament à titre gracieux non encore disponible sur le marché.

L'observation des stratégies implantées à l'échelle internationale (Canada, États-Unis et France) nous permet de constater que les stratégies présentent des points communs tout en s'adaptant aux différents contextes règlementaires et assurantiels. La stratégie de fidélisation s'ajuste au statut du médicament sur le territoire de distribution et aux conditions de sa prise en charge par l'assureur.

Pour maintenir ou hausser leurs ventes ou encore percer le marché des médicaments, les fabricants usent de stratégies commerciales que nous distinguons en trois types. Une première stratégie vise à maintenir les patients sur des médicaments d'origine dont le brevet est échu, et ce malgré l'entrée de génériques sur le marché : il s'agit de la fidélisation par le rabais. Un deuxième type oriente les patients et les professionnels de la santé vers un traitement particulier en concurrence avec d'autres produits également sous protection de brevet, en offrant des services d'appoint ou de gestion de soins : il s'agit de la fidélisation par l'accompagnement du patient. Enfin, un troisième type se met en place pour des médicaments qui ne sont pas encore reconnus par les autorités publiques pour fins de remboursement et dont le prix peut parfois atteindre des sommes colossales. Les fabricants proposent alors des programmes de compassion qui permettent au patient d'obtenir le médicament à titre gracieux ou à prix fortement réduit. Il s'agit de la fidélisation par la compassion.

Nous allons reprendre chacune de ces stratégies afin d'en présenter les principales caractéristiques, sachant que davantage de recherche sera nécessaire pour mieux identifier les contours. On observe que la documentation est plus importante pour la stratégie par le rabais, et ce, surtout aux États-Unis. Les deux autres types de stratégies sont encore peu documentés.

La fidélisation par le rabais : les médicaments en fin de course

Le contexte et le produit visé

La stratégie de la fidélisation par le rabais est une conséquence directe de la fin du brevet de nombreux médicaments vedettes. Les fabricants tentent de convaincre les patients de poursuivre la prise du produit de marque au lieu de transférer leur médicament au générique. Les médicaments visés par ces programmes présentent deux caractéristiques : ils sont largement prescrits par les médecins et leur brevet est échu (ou le sera sous peu).

Sans surprise, c'est dans le plus grand marché mondial du médicament que les fabricants ont d'abord mis en place les programmes de fidélisation par le rabais. La stratégie vise essentiellement à convaincre le patient à prendre le produit de marque en le remboursant de sorte que l'original ne lui coûte pas plus cher que la copie.

La forme des programmes : l'expérience américaine et canadienne

La fidélisation par le rabais s'insère toujours dans le marché des assurés. Il est important de distinguer la fidélisation traitée ici des formes caritatives d'assistance offertes par l'industrie pharmaceutique aux États-Unis pour les segments de population qui ne disposent d'aucune assurance médicaments (Chauncey et al. 2006; Chisholm et DiPiro 2002; Felder Palmer et al. 2011).

Les programmes américains de fidélisation par le rabais, comme les programmes canadiens, s'adressent aux personnes qui détiennent une assurance-médicaments. Ils se présentent sous la forme de cartes de co-paiement ou de coupons, appliqués pour réduire le coût direct d'acquisition du médicament de marque. L'adhésion au programme réduit le coût de

co-assurance du patient au montant équivalent pour le médicament générique. La stratégie vise toujours à inciter le patient (et le professionnel de la santé) à ne pas y substituer le produit générique (Choudhry et al. 2009).

Une grande part du coût supplémentaire induit par la prise du médicament de marque est toutefois transférée à l'assureur, par le jeu conjugué de la réglementation et des contrats d'assurance. Prenons l'exemple fictif du patient dont l'assurance rembourse 80 % du coût du médicament. Il est placé devant l'option offerte par un programme de fidélisation par le rabais, avec un médicament d'origine de 100 $ pendant que le générique se vend 30 $. Sans programme, le patient qui opte pour le générique paie 6 $ de co-assurance, tandis qu'il doit débourser 20 $ pour obtenir le produit de marque. Le programme de rabais intervient en lui offrant de payer la différence de co-assurance entre les deux produits, c'est-à-dire 14 $ (20 $ moins 6 $), afin de lui procurer le médicament de marque. Le mécanisme se répercute par contre sur l'assureur qui doit assumer la plus grande part de l'accroissement de coût, soit 80 % du produit de marque au lieu du générique. Ceci représente pour l'assureur une augmentation de coût de 56 $ (80 $ moins 24 $). La firme qui met en place un tel programme n'assume que 14 $, pendant qu'elle répercute un coût additionnel de 56 $ à l'assureur.

La distribution des cartes de co-paiement au Canada peut prendre trois voies: le médecin, le pharmacien, ou directement sur un portail web. Introduits d'abord sous la forme de programmes mis de l'avant par chacun des fabricants, les programmes font maintenant l'objet de regroupements importants sous le parapluie d'entreprises qui en assurent la gestion. Ces regroupements auprès d'une entreprise tierce permettent de faciliter la gestion et d'accélérer la diffusion des programmes. Le regroupement vise aussi à réduire les appréhensions face à la communication directe au fabricant de renseignements personnels sensibles sur la santé des patients. Il sert aussi à aplanir la complexification du processus de remboursement en uniformisant les procédures.

Deux grands regroupements sont disponibles au Canada : la carte InnoviCares, gérée par l'entreprise STI de la Nouvelle-Écosse qui regroupe sous une même carte près de 100 produits différents, et le groupe RxHELP, géré par l'entreprise Cameron Stewart LifeScience de Mississauga en Ontario qui offre au Québec plus de 34 produits.

Critiques

Les programmes de fidélisation par le rabais se sont d'abord fait connaître sur le marché des statines en raison des chiffres records de vente que tentaient de sauvegarder les fabricants (Avorn 2011). Une étude publiée en 2012 souligne que les fabricants touchés (Pfizer pour le Lipitor, Merck pour le Zocor et AstraZeneca pour le Crestor) rivalisent d'ingéniosité pour tenter de prolonger au maximum la durée de vie de leur médicament en fin de cycle (Jackevicius et al. 2012). Les auteurs concluent que les énormes économies anticipées pour le système de santé américain avec l'arrivée des produits génériques sont susceptibles d'être sérieusement compromises, à moins d'une réaction vigoureuse des parties prenantes.

Les répercussions énormes sur les budgets de santé ont été relevées et les programmes ont été vigoureusement dénoncés par des universitaires comme contraires à l'intérêt public puisque, même en réduisant le coût direct d'acquisition du patient, ils augmentent de manière injustifiée les coûts de santé du système, sans bénéfice sanitaire (Grande 2012; Ross et Kesselheim 2013).

Les auteurs ont aussi souligné que la promotion de la fidélisation aux médicaments d'origine était contraire aux principes de santé publique en dévalorisant aux yeux du public et des professionnels de la santé la bioéquivalence des médicaments génériques (Grande 2012).

La stratégie impose à terme la hausse des primes d'assurance afin d'absorber les hausses de coûts. Pendant que le fabricant fait cadeau d'un rabais au patient, il refile le plus gros de la note à l'assureur et à terme, à l'ensemble des assurés. Le caractère insidieux des conséquences économiques de la fidélisation par le rabais fait partie de sa raison d'être. Dès qu'il devient impossible au fabricant de transférer les coûts sur l'assureur, en raison de l'adoption de mesures de protection (lorsque l'assureur peut le faire), il réduit ou met abruptement fin à son programme de fidélisation[3].

La stratégie exige la collaboration étroite des professionnels de la santé (le médecin prescripteur ou le pharmacien délivreur), ce qui soulève d'importantes questions déontologiques quant à la participation professionnelle à un programme commercial de l'industrie. La transmission par le pharmacien des données nécessaires au paiement partiel de la prescription par l'industrie pharmaceutique pose également d'importantes questions quant à la protection des renseignements personnels des patients et au respect du secret professionnel.

La fidélisation par l'accompagnement : les traitements complexes

Le contexte et le produit visé

Plusieurs options peuvent parfois se présenter pour traiter une même condition médicale. Le choix de traitement se fait alors entre médicaments différents, souvent tous sous protection de brevet, mais dont le principe actif ou la méthode d'application se distingue. Des programmes sont alors mis en place pour promouvoir un produit ou un traitement par rapport à ses concurrents.

La forme des programmes : l'expérience française

Pour mettre en valeur son produit, le fabricant peut élaborer une stratégie servant à guider et diriger le patient (ainsi que le professionnel de la santé) vers son produit. La stratégie propose d'accompagner le patient dans son traitement, en le renseignant sur sa maladie et ses effets, en lui offrant une formation à l'usage du médicament, un service d'écoute et des conseils de santé. En somme, le programme propose au patient un suivi à caractère médical ou infirmier. L'observance au traitement est l'un des principaux bénéfices mis de l'avant par certains programmes. La firme reconfigure ainsi son statut de fabricant et vendeur de médicaments pour adopter celui de la vente d'un « bénéfice thérapeutique dans le cadre de ces bonnes pratiques » (Abecassis et Coutinet 2009, p. 149).

C'est la France qui a le plus contribué à l'analyse de ce type de stratégie. Comme le régime légal d'assurance santé français ne prête pas facilement flanc aux stratégies de fidélisation par le rabais, on y voit plutôt le développement de programmes d'accompagnement à divers traitements. Les autorités françaises ont vite réagi et ont commandé une enquête de l'Inspection générale des affaires sociales (IGAS 2007).

Les pratiques sont apparues en France en 2003 et se sont accélérées à compter de 2006. Une grande partie des programmes d'accompagnement visent des produits injectables avec formation à l'auto-injection. Les programmes peuvent aussi aller plus loin et devenir de véritables programmes d'accompagnement en cours de traitement (*disease management*). Les pathologies les plus souvent visées répertoriés par l'IGAS sont : la sclérose en plaques, l'hypertension artérielle, le diabète, l'ostéoporose, la polyarthrite rhumatoïde, l'infection au VIH, le surpoids et l'asthme. Les fabricants expliquent leur stratégie par la complexité de la pathologie et son traitement, les interventions à domicile devenues incontournables à la suite du virage ambulatoire et l'amélioration de l'observance du patient.

La forme des programmes : l'expérience canadienne

On observe au Canada l'émergence de stratégies d'accompagnement par des entreprises qui se présentent comme des « navigateurs de soins »[4] et qui accompagnent les patients et les professionnels de la santé en cours de protocole de soins faisant appel à des médicaments ou des produits particuliers. Certains distributeurs ou grossistes, actifs depuis longtemps dans la chaîne du médicament, développent de nouvelles lignes d'affaires en offrant une panoplie de services intégrés destinés au patient, au médecin et sa clinique, à l'établissement de santé ou aux pharmacies. Des distributeurs de médicaments s'introduisent dans cette nouvelle ligne d'affaires, notamment la firme McKesson et le distributeur des pharmacies de la bannière Pharmaprix (Shoppers Drug Mart, par le biais de sa filiale Shoppers Drug Mart Specialty Health Network Inc.).

Critiques

L'IGAS s'est montrée très sévère dans ses conclusions en soulignant cinq grandes classes de risques soulevés par les stratégies d'accompagnement des grands laboratoires pharmaceutiques. On craint d'abord que soient mal protégés les droits des patients (sur-médication; protection des renseignements personnels). L'IGAS note que les programmes peuvent court-circuiter le réseau socio-sanitaire et entraîner une confusion des rôles dans l'esprit du patient quant au professionnel responsable du suivi médical. Les programmes contournent la prohibition de la publicité auprès du public et les fabricants se placent ainsi en situation de conflits d'intérêts. Au nom de la défense des intérêts sanitaires du patient, ils poursuivent leur intérêt commercial dans la vente d'un produit. Enfin, le recrutement de clientèles et le suivi à des fins commerciales est contraire, selon l'IGAS, aux exigences des règles professionnelles. Le rapport émet cinq grandes recommandations, dont la principale demande que soit consacré le principe de la prohibition de tout contact direct ou indirect entre un fabricant de médicament et un patient.

On connaît peu de choses de l'accompagnement par des « navigateurs de soins » au Canada. Il soulève toutefois d'emblée d'importantes préoccupations en raison du lien direct entretenu entre la firme qui vend le médicament prescrit et le patient. L'émergence de réseaux parallèles de distribution des fabricants, par le biais d'ententes d'exclusivité avec certaines pharmacies ou distributeurs, soulève en outre des questions d'accès au médicament et de liberté de choix du patient de son pharmacien.

La fidélisation par la compassion : les nouveaux médicaments non approuvés

Le contexte et le produit visé

La troisième catégorie de mécanismes de fidélisation vise les nouveaux médicaments qui n'ont pas encore fait l'objet d'une pleine reconnaissance par les autorités publiques, que ce soit pour leur mise en marché ou aux fins de remboursement par les assureurs. Nous retenons le terme de la « compassion » que les firmes utilisent au Québec pour caractériser ces programmes qui offrent au patient d'obtenir un médicament autrement non disponible.

Les programmes de compassion s'inscrivent dans un contexte où les autorités publiques sont plus prudentes avant d'accepter l'inscription de nouveaux médicaments aux coûts importants, particulièrement pour les médicaments biologiques et oncologiques, pour lesquels les firmes tentent de convaincre les autorités réglementaires que la valeur thérapeutique du produit en justifie le coût. Ces programmes côtoient de nouvelles formes de partage de risque conclues par contrat entre les fabricants et les autorités sanitaires (de Pouvourville 2012). Nous limitons ici la discussion aux programmes du Québec.

La forme des programmes au Québec

Le médicament peut être en attente d'une reconnaissance par les autorités d'homologation de Santé Canada (programme d'accès spécial – PAS, ou programme du Patient d'exception au Québec), ou être en attente d'évaluation par l'Institut national d'excellence en santé et en services sociaux (INESSS) ou de son approbation par le ministre pour une inscription à la Liste des médicaments. Les programmes de compassion sont aussi parfois offerts pour un médicament dont la reconnaissance est partielle (Liste des médicaments d'exception) ou susceptible d'être rétrogradé de la Liste ordinaire à la Liste du médicament d'exception, ou au mécanisme du Patient d'exception.

Ces programmes sont implantés pendant la période préalable au remboursement du médicament par les autorités publiques ou les assureurs privés. La plupart des programmes fonctionnent sous la dénomination de « Programme d'assistance au processus de remboursement du médicament X® ». Un document du CHUM indique que l'aide financière offerte au patient cesse dès que le médicament est inscrit à la liste des médicaments remboursés (CHUM 2013).

Les programmes touchent surtout les traitements en oncologie, en établissement ou en ambulatoire, ou encore le traitement de maladies rares (souvent génétiques). La promotion est davantage dirigée vers les médecins chez qui on recherche la prescription au médicament.

Certains programmes de compassion exigent une coordination des soins hors réseau, autant pour la livraison du médicament que pour son administration. En effet, le médicament qui n'est pas sur la liste des médicaments remboursés est en principe non disponible dans l'Établissement de santé. Certains auteurs avaient soulevé ce problème de l'accès aux médicaments non remboursés par le régime de l'Ontario, lorsque les patients étaient prêts à se les payer (Flood et Hardcastle 2007). Cette situation n'est pas étrangère au développement par certaines entreprises d'un réseau national de centres de perfusion et d'injection pour certains médicaments au statut de remboursement précaire. Par exemple, on trouve au Québec douze cliniques qui procèdent à la perfusion du Perjeta, pour lequel aucune reconnaissance au remboursement n'est encore acquise. Ces cliniques parallèles peuvent aussi servir à l'injection hors indication de médicaments inscrits à la Liste des médicaments d'exception. Certains programmes conjuguent compassion et accompagnement pour financer une partie du coût d'acquisition et offrir le service de perfusion au patient.

Critiques

Le fabricant offre une aide financière aux médicaments qui ne jouissent pas encore de la pleine reconnaissance afin d'en ouvrir l'accès au patient. Ce mécanisme prépare en même temps le terrain à la reconnaissance en mobilisant professionnels et patients et facilite la pénétration du marché. Comment refuser un médicament expérimental offert gracieusement? La fin du statut précaire d'un médicament et sa pleine inscription à la Liste des médicaments remboursés sonnent le glas du programme de compassion. Ainsi, dès qu'un médicament est approuvé pour fins de remboursement, la RAMQ doit prendre le relais et payer le plein prix pour l'ensemble de la cohorte des patients déjà sous médication.

Le programme exige une gestion administrative importante. Les fabricants offrent une aide au patient qui doit transmettre ses données d'assurance afin d'engager les démarches auprès de l'assureur. Comme l'oncologie représente une part importante du terrain d'application des programmes de compassion, les fabricants proposent maintenant aux hôpitaux de rémunérer les personnes requises pour faire les démarches administratives nécessaires et faciliter l'inscription des patients au programme.

Conclusion

Nous avons distingué trois types de stratégies commerciales de fidélisation des firmes pharmaceutiques dans un contexte de restructuration de l'industrie. Les efforts de promotion des deux premières stratégies, soit la fidélisation par le rabais et la fidélisation par l'accompagnement, sont surtout dirigés vers le patient appelé à prendre une part plus active quant au choix ou à l'administration de son médicament. Parmi les principales réserves ou critiques de ces programmes, figure justement le contournement de la prohibition de la publicité directe au patient ici souvent mise en œuvre par le web. Ces stratégies illustrent bien la diversification des voies de promotion de l'industrie où le médecin n'est plus le seul destinataire principal des efforts de diffusion au Canada et en Europe. Les professionnels ne sont toutefois pas en

reste, puisque l'adhésion du patient exige la participation étroite des professionnels de la santé. En outre, l'impact économique de la fidélisation par le rabais démontre que le rabais offert au patient cache une augmentation importante des coûts pour l'ensemble des assurés. La troisième catégorie se distingue de façon importante des deux premières en raison du caractère nouveau du médicament. Les programmes sont surtout dirigés vers les médecins spécialisés afin de favoriser le recrutement de patients.

Les stratégies commerciales de l'industrie pharmaceutique semblent avoir pris de court les politiques publiques du Canada et du Québec sur plusieurs fronts. Certains hésitent à questionner ces programmes puisque, d'une certaine façon, ils offrent un service au patient en réduisant ses dépenses personnelles, en lui offrant des services complémentaires ou encore en lui donnant accès à un médicament autrement inaccessible. La première étape d'une réaction éclairée des pouvoirs publics passe par une meilleure compréhension des logiques et des mécanismes qui animent les trois stratégies commerciales ici présentées. Cette étude se veut une contribution en ce sens.

Remerciements

Nous remercions l'Ordre les pharmaciens du Québec qui a financé en partie la recherche et Johanne Préval, professionnelle de recherche à l'ENAP, qui a participé à la revue de littérature. Nous remercions également les évaluateurs anonymes et les éditeurs de la Revue pour leurs commentaires judicieux et utiles.

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Notes

1. C'est-à-dire pouvant bénéficier de la protection du brevet.
2. On parle en anglais de « patent cliff ».
3. Par exemple, en novembre 2013, le Site de RXHelp annonçait que Pfizer se réservait le droit de ne plus rembourser au complet la différence de la co-assurance du patient entre le médicament de marque et le médicament générique.
4. Voir notamment la section « Services spécialisés aux patients » du site web de McKesson: https://www.mckesson.ca/fr/mckesson-specialty (lu le 10 janvier 2014).

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