

HEALTHCARE

POLICY

Politiques de Santé

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

Volume 10 + Number 4

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by Decision Makers**

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**Regulating Direct-to-Consumer Drug Information: A Case Study of
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POLICY

Politiques de Santé

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Services de santé, gestion et recherche de politique

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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.

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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The Difference a Decade Makes

THE DECADE SINCE *HEALTHCARE POLICY/POLITIQUES DE SANTÉ* WAS FOUNDED HAS seen many waves of change, both within the health sector and beyond. Yet throughout these changes – or perhaps because of them – the need for a credible, curated home for policy-relevant research and evidence-informed discussion and debate has remained constant. Our ability to deliver on this mandate has been helped by our relationship with the Canadian Association of Health Services and Policy Research (CAHSPR), as well as evolving mechanisms for sharing the journal's content. These include an on-going partnership with EvidenceNetwork.ca to make key findings available to the media and full indexing in Medline/PubMed to expand the accessibility and global reach of the content that we publish.

Over the last decade, the journal's most-read articles have reflected a wide range of topical issues in healthcare policy. Examples include publications on benchmarking for continuous quality improvement, logic models for primary healthcare, developing health policy and systems research in Nigeria and global approaches to the evaluation of health services use. Papers on focused topics have also garnered a great deal of interest, including the management of MRI waiting lists, reasons why nurses migrate to the US, and mental health indicators.

There have also been shifting clusters of papers submitted over time. Access to care and waiting times were a focus in the early years of the journal, followed by a large number of papers on primary healthcare. We continue to publish on both topics, but more recently we have seen an increase in submissions related to pharmaceutical policy and comparative health systems. Both are issues of high policy relevance today and are reflected in the contents of this issue of the journal. It features articles addressing a diverse set of questions relevant to pharmaceutical policy, from direct-to-consumer advertising and health technology assessment to reimbursement for drugs for rare diseases and the impact of private insurance on drug use.

One thing that has not changed in the last decade is that producing a high quality journal requires many people to contribute their time and expertise to the cause. Central to the process are *Healthcare Policy/Politiques de Santé's* editors and contributing editors: François Béland, Roger Chafe, Raisa Deber, Mark Dobrow, Bob Evans, Eric Latimer, Steven Lewis, Joel Lexchin and Claude Sicotte, as well as Jean-Louis Denis who edited this year's special issue on Approaches to Accountability. Special thanks are also due to Ania Bogacka, the journal's Managing Editor who tirelessly communicates with us all and coordinates the publication process from start to finish.

In addition, with this issue, it gives me great pleasure to thank the authors from around the globe whose work appears in the journal's pages and the 120 reviewers who have graciously provided their expert advice over the past year (see page 47). *Healthcare Policy/ Politiques de Santé* would not be possible without their contributions. If you are interested in serving as a reviewer for future issues, please register at <http://www.longwoods.com/reviewer-registration/healthcare-policy>. Our online database helps editors to match articles that we send out for comment with reviewers' expertise and interests. By registering, you can help to advance scholarship and evidence-informed debate in health policy, both in the journal's pages and beyond.

JENNIFER ZELMER, PHD

Editor-in-chief

Dix ans d'influences

DEPUIS LA CRÉATION DE *POLITIQUES DE SANTÉ/HEALTHCARE POLICY*, IL Y A DIX ans, on a vu plusieurs successions de changements, tant dans le secteur de la santé qu'ailleurs. À travers ces changements – et peut-être à cause d'eux – il y a toujours eu le besoin d'un lieu crédible et organisé pour nourrir des débats de recherche pertinents et éclairés par les données probantes. Pour exécuter ce mandat nous avons pu compter sur notre partenariat avec l'Association canadienne pour la recherche sur les services et les politiques de santé (l'ACRSPS) ainsi que sur des mécanismes qui permettent de partager le contenu de la revue. Parmi ceux-ci, se trouvent le partenariat avec EvidenceNetwork.ca, qui présente aux médias les résultats clés, ainsi qu'une indexation complète dans Medline/PubMed qui facilite la recherche et accroît l'accessibilité de la planète au contenu que nous publions.

Les articles de la revue les plus consultés au cours des dix dernières années reflètent une vaste gamme de sujets touchant aux politiques de santé. On peut citer, par exemple, des publications sur l'étalonnage visant une amélioration continue de la qualité, des modèles logiques pour les soins de santé primaires, le développement de la recherche sur les politiques et les systèmes de santé au Nigeria ou encore les démarches mondiales pour l'évaluation de l'utilisation des services de santé. Les articles portant sur des sujets plus ciblés ont aussi attiré l'attention, notamment sur la gestion des listes d'attente pour une IRM, sur les raisons pour lesquelles les infirmières migrent vers les États-Unis et sur les indicateurs de la santé mentale.

Il y a aussi eu des changements dans les catégories d'articles présentés au cours du temps. Tout au début de la revue, l'accès aux soins et les temps d'attente formaient un centre d'intérêt prononcé. Puis il y a eu un grand nombre d'articles sur les soins de santé primaires. Nous continuons de publier sur ces deux sujets, mais nous constatons une croissance d'articles liés aux politiques sur les produits pharmaceutiques et aux études comparées sur les systèmes de santé. Il s'agit de deux sujets très pertinents de nos jours et ils sont abordés dans le présent numéro. On y présente en effet des articles qui portent sur un ensemble de questions touchant aux politiques sur les produits pharmaceutiques, que ce soit la publicité directe auprès des consommateurs, l'évaluation des technologies de la santé, le remboursement des médicaments pour maladies rares ou encore l'impact des assurances privées sur l'utilisation des médicaments.

Il y a une chose qui n'a pas changé au cours des dix dernières années, c'est le temps et l'expertise qu'offrent plusieurs personnes pour produire une revue de grande qualité. Les éditeurs et conseillers de rédaction que voici constituent des éléments centraux du succès de *Politiques de Santé/Healthcare Policy* : François Béland, Roger Chafe, Raisa Deber,

Mark Dobrow, Bob Evans, Eric Latimer, Steven Lewis, Joel Lexchin et Claude Sicotte ainsi que Jean-Louis Denis qui a dirigé cette année le numéro spécial sur les démarches visant l'obligation redditionnelle. Il faut aussi adresser un remerciement spécial à Ania Bogacka, éditrice en chef de la revue, qui communique inlassablement avec nous et coordonne le processus de publication du début à la toute fin.

De plus, dans ce numéro, j'ai le plaisir de remercier les auteurs du monde entier dont les travaux ont paru dans les pages de la revue et les 120 réviseurs qui ont gracieusement offert leurs avis d'experts au cours de l'année écoulée (voir page 47). Il serait impossible de publier *Politiques de Santé/Healthcare Policy* sans leur contribution. Si vous souhaitez devenir réviseur pour les numéros à venir, veuillez vous inscrire à la page <http://www.longwoods.com/reviewer-registration/healthcare-policy>. Notre base de données en ligne permet aux éditeurs de jumeler les articles que nous envoyons pour commentaires à l'expertise et aux intérêts des réviseurs. En vous inscrivant, vous contribuez à faire avancer la recherche sur les politiques de santé et à nourrir les débats éclairés par les données probantes, aussi bien dans ces pages qu'ailleurs.

JENNIFER ZELMER, PHD

Rédactrice en chef

Supporting the Use of Health Technology Assessments by Decision-Makers

Appuyer l'utilisation des évaluations des technologies de la santé chez les décideurs



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Abstract

A perceived gap exists in how well Canadian health technology assessment (HTA) producers are supporting the use of their HTAs by decision-makers. The authors propose that the newly released HTA Database Canadian search interface incorporate structured decision-relevant summaries of HTAs that would be developed by participating Canadian HTA organizations. The registry would serve as a “one-stop shop” by including HTA reports along with their structured summaries in a format that better meets decision-makers’ needs. The Health Technology Analysis Exchange – a Canadian network of publicly funded HTA producers – is well-positioned to undertake this work and would welcome input about both the idea and its execution.

Résumé

On semble percevoir une certaine lacune au Canada dans la façon dont ceux qui préparent les évaluations des technologies de la santé (ETS) appuient l'utilisation de celles-ci chez les décideurs. Les auteurs proposent d'intégrer des résumés structurés pertinents pour la prise de décision dans la nouvelle interface de recherche de la base de données canadienne des ETS, résumés qui seraient préparés par les organismes d'ETS canadiens participants. Ce répertoire servirait de « guichet unique » en comprenant les rapports d'ETS ainsi que des résumés structurés dans un format qui conviendrait davantage aux besoins des décideurs. L'Échange sur les technologies de la santé – un réseau canadien d'organismes d'ETS financés par des sources publiques – serait bien placé pour entreprendre ce travail et souhaite recueillir les commentaires sur cette idée et sa mise en œuvre.

THE USE OF HIGH-QUALITY EVIDENCE FOR POLICY AND MANAGERIAL DECISION-making can be a challenge due to the relationships between knowledge creators (i.e., researchers) and knowledge users (i.e., decision-makers). Challenges exist in part due to the limited communication and a lack of a linkage infrastructure between both parties. Health technology assessment (HTA) producers have developed sophisticated methods for creating knowledge. HTAs, however, may sit unused or, when retrieved, fail to highlight decision-relevant information. A perceived gap exists in how well HTA producers are supporting the use of the HTAs they prepare. We propose that the newly released HTA Database Canadian search interface incorporate structured decision-relevant summaries prepared at the national, provincial/territorial, regional and organizational level in Canada (Pan-Canadian HTA Collaborative 2015). The resulting “one-stop shop” would be a key enabler in efforts to ensure that decision-makers have easy, timely access to research evidence that is provided in a consistent user-friendly format.

In 2014, HTA producers in Alberta, Ontario and Quebec, and the Canadian Agency for Drugs and Technologies in Health (CADTH), partnered with the National Institute for

Health Research’s Centre for Reviews and Dissemination to develop a single repository and search tool for Canadian HTA reports within the HTA database. Each participating HTA organization contributes to the database. The HTA Database Canadian search interface was designed to be a go-to source for Canadian HTA users by incorporating links to full HTA reports and a bilingual and flexible search interface. As more Canadian HTA organizations participate in this initiative, a natural extension to the database would be the addition of decision-relevant summaries for each HTA report (Pan-Canadian HTA Collaborative 2015).

Decision-makers frequently access HTAs prepared outside their context and need to customize this knowledge to inform their particular situations, including the availability of resources, program capacity and competencies of staff. HTA report adaptation is especially relevant in Canada, given our decentralized healthcare system and the variety of organizations involved in decisions about technology. An environmental scan identified several studies of structured decision-relevant summaries that would make it easier for decision-makers in a particular setting to adapt research syntheses from another setting, but so far this has not been operationalized for the field of HTA in Canada (Beynon et al. 2012; Lavis et al. 2009; Lavis et al. 2010; Lavis et al. 2013). Structured decision-relevant summaries of existing HTAs would make it easier to pull key information into their own decision frameworks and then focus on adding the context-specific information that they need to make an informed decision. The user-friendly summaries are as relevant to “generic” CADTH HTAs as they are to a highly contextualized HTA from a hospital or other local organization.

Two types of preparatory work on structured decision-relevant summaries have already been undertaken to pave the way for implementation in the area of HTA. First, Lavis et al. developed structured summary and relevance-assessment prototypes for HTAs using input from 29 Canadian and British managers and policy makers (Lavis et al. 2010). Semi-structured telephone interviews with 19 Canadian study participants indicated support for an HTA summary, and five suggestions emerged from their responses to improve the structured summary prototypes (Table 1).

TABLE 1. Suggestions to improve structured HTA summaries (Lavis et al. 2010)

Summary element	Description
Format	Headings to make it easier for decision-makers to scan for relevant information
Quality assessment	An appraisal of the HTA report using a validated tool
Summary sections	Sections with clear headings and explicit goals
Language	Short plain-language summary with key messages up front
Previous or HTA reports in progress	A listing of previously completed HTA reports or those in progress

HTA = Health technology assessment

Second, the Health Technology Analysis Exchange, a network of HTA producers across the nation established in accordance with Canada’s Health Technology Strategy 1.0 (Health

Technology Assessment Task Group and on behalf of the Federal/Provincial/Territorial Advisory Committee on Information and Emerging Technologies 2004; Canadian Agency for Drugs and Technologies in Health 2014), organized an exploratory workshop in 2013. This workshop focused on decision-relevant criteria for health technologies, with input from HTA producers in Alberta, Ontario and Quebec and from the Evidence and Value: Impact on Decision Making (EVIDEM) Collaboration (EVIDEM Collaboration 2014) and using two reports produced by CADTH as test cases. In relation to the structure of the brief, the participants readily agreed that the recommendation and alternatives should be presented at the top of the brief, as decision-makers typically are interested in the “bottom line.” Additional elements to be included in the brief, for decision-makers who seek additional information, are the context, methods and evidence based on EVIDEM’s top five criteria, and the contextualization of available evidence (Table 2). Several Exchange members also participate in the HTA Database Canadian search interface. As the Exchange encompasses both HTA producers and users across Canada, we propose that its members fill this key gap in the Canadian landscape.

TABLE 2. HTA decision-relevant summary template

Header	Description
Recommendation	<ul style="list-style-type: none"> • Preferred alternative • Reason(s) for recommendations
Alternatives	<ul style="list-style-type: none"> • Pros and cons of each alternative • Feasibility of implementation/operational consideration • Implementation strategies for each alternative • Ethical/legal considerations • Risk assessment and risk mitigation of alternatives
Context	<ul style="list-style-type: none"> • Prevalence • National status (tabular format, by province or health authority) • Stakeholder perspective (medical and patient association and public consultations) <ul style="list-style-type: none"> • What are the issues? • What are the contributions to controversy? • What is known about the intervention(s)? Alternatively, what is not known about the intervention?
Methods	<ul style="list-style-type: none"> • HTA • Stakeholder consultation process
Evidence (based on EVIDEM’s Top 5)	<ul style="list-style-type: none"> • Efficacy/effectiveness • Safety/tolerability • Severity of disease • Impact of healthcare costs (incremental cost-effectiveness) • Quality/uncertainty of evidence
Contextualization of available evidence	Self-explanatory

EVIDEM = Evidence and Value: Impact on Decision Making; HTA = health technology assessment.

The proposal we would like to advance is for a one-stop shop, having all structured decision-relevant summaries from across Canada available in one place, that would address the first of three factors that have been shown to influence the use of evidence in policy making, namely, timing or timeliness (Lavis et al. 2010). Decision-makers need to rapidly

identify what has already been learned about a technology of interest and then adapt this knowledge for their own setting. HTA producers and funders, on the other hand, could use the one-stop shop to identify gaps in the available inventory of HTAs. A subsidiary benefit for HTA producers arises from the primary objective of meeting the needs of policy makers. The registry also would allow decision-makers to access and review HTAs in a timely manner. Previous research has shown that timeliness and discussions between policy makers and researchers can positively impact the use of evidence in decision-making (Moat and Lavis 2011).

Health Systems Evidence is an example of a repository of systematic reviews and economic evaluations on the subjects of governance, financial and delivery arrangements within health systems, as well as the implementation strategies that can support change in health systems (McMaster University 2014). It uses a variety of approaches to ensure comprehensiveness and provides links to structured decision-relevant summaries and full-text reports when freely available (as a way to lead traffic to the sites of the evidence producers) in Canada's both official languages (as well as five others). Similar Canadian and international initiatives that have been developed or are under way include AdHopHTA, Decision Aid Library Inventory and Evidence-Informed Healthcare Renewal Portal, which is a subportal within Health Systems Evidence.

We provisionally propose to develop and test a prototype for structured decision-relevant summaries based on the original prototypes and suggestions for improvement outlined by Lavis et al. and the recommendations from the 2013 workshop, and to use Health Systems Evidence and other complementary databases to garner ideas to expand on the new repository to include decision-relevant summaries. To help frame this initiative, we would apply a framework that encompasses the push, pull and exchange concepts (Gagnon 2009). More specifically, the HTA database and structured summaries would facilitate "user pull" by decision-makers. We also plan to explore the feasibility of building in RSS technology or e-mail alerts that would automatically "push" summaries to decision-makers on topics of interest. Finally, the HTA database can simplify exchange among HTA producers and decision-makers around topics of shared interest. To measure the success for this initiative, we plan to use indicators such as the frequency with which the decision-relevant summaries are accessed, the time spent in the HTA database to view the summaries and the number of subscribers to the RSS feed, as well as through periodic surveys self-reports of user satisfaction with and the perceived usefulness of the HTA database and summaries. The survey also can inquire about the frequency and results of exchanges between HTA producers and decision-makers and about the impact of HTA reports and decision-relevant summaries on decision-making. Comments on any aspects of our proposal can be sent to requests@cadth.ca.

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References

- Beynon, P., C. Chapoy, M. Gaarder and E. Masset. 2012. *What Difference Does a Policy Brief Make?* Institute of Development Studies and the International Initiative for Impact Evaluation (3ie), Brighton, United Kingdom. Retrieved September 17, 2013. <http://www.3ieimpact.org/media/filer/2012/08/22/fullreport_what_difference_does_a_policy_brief_make__2pdf_-_adobe_acrobat_pro.pdf>.
- Canadian Agency for Drugs and Technologies in Health. 2014. CADTH. Ottawa, ON: The Agency. Health Technology Analysis Exchange. Retrieved June 10, 2014. <<http://www.cadth.ca/en/advisory-bodies/hta-exchange>>.
- EVIDEM Collaboration. 2014. *EVIDEM™: Evidence and Value: Impact on Decision Making* [Internet]. Montreal: EVIDEM Collaboration. Retrieved December 10, 2014. <<https://www.evidem.org/>>.
- Gagnon, M. 2009. "Section 5.1. Knowledge Dissemination and Exchange of Knowledge." In *Knowledge Translation in Healthcare: Moving from Evidence to Practice*. Ottawa, ON: Canadian Institutes of Health Research. Retrieved February 17, 2015. <<http://www.cihr-irsc.gc.ca/e/41953.html>>. Author's presentation.
- Health Technology Assessment Task Group and on behalf of the Federal/Provincial/Territorial Advisory Committee on Information and Emerging Technologies. 2004. *Health Technology Strategy 1.0: Final Report*. Ottawa, ON: Health Canada. Retrieved April 26, 2013. <http://www.hc-sc.gc.ca/hcs-sss/alt_formats/pacrb-dgacpr/pdf/pubs/ehealth-esante/2004-tech-strateg/2004-tech-strateg-eng.pdf>.
- Lavis, J.N., C. Catallo, G. Permanand, A. Zierler and BRIDGE Study Team. 2013. *Communicating Clearly: Enhancing Information-Packaging Mechanisms to Support Knowledge Brokering in European Health Systems*. Copenhagen: European Observatory on Health Systems and Policies, World Health Organization. (Bridge Series; Policy Summary 7.) Retrieved October 2, 2013. <http://www.euro.who.int/__data/assets/pdf_file/0005/195233/Obs-Policy-Summary-7,-Communicating-clearly.pdf>.
- Lavis, J.N., G. Permanand, A.D. Oxman, S. Lewin and A. Fretheim. 2009. "SUPPORT Tools for Evidence-Informed Health Policymaking (STP) 13: Preparing and Using Policy Briefs to Support Evidence-Informed Policymaking." *Health Research Policy and Systems* 7(Suppl 1): S13. Retrieved October 2, 2013. <<http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3271824>>.
- Lavis, J.N., M.G. Wilson, J.M. Grimshaw, R.B. Haynes, M. Ouimet, P. Raina et al. 2010. "Supporting the Use of Health Technology Assessments in Policy Making about Health Systems." *International Journal of Technology Assessment in Health Care* 26(4): 405–14.
- McMaster University. 2014. *McMaster University*. Hamilton, ON: McMaster Health Forum. Health Systems Evidence. Retrieved June 10, 2014. <<http://www.mcmasterhealthforum.org/policymakers/health-systems-evidence>>.
- Moat, K. and J. Lavis. 2011. *Supporting the Use of Cochrane Reviews in Health Policy and Management Decision-Making: Health Systems Evidence*. London: The Cochrane Collaboration. Retrieved August 5, 2014. <<http://www.thecochranelibrary.com/details/editorial/1018237/Supporting-the-use-of-Cochrane-Reviews-in-health-policy-and-management-decision-.html>>.
- Pan-Canadian HTA Collaborative. 2015. *HTA Database Canadian Search Interface: Background and Key Messages*. Ottawa, ON: Canadian Agency for Drugs and Technologies in Health (CADTH). Retrieved October 19, 2015. <http://www.cadth.ca/media/pdf/pan_canadian/HTA-Pan-Canadian-Key-Messages-en.pdf>.

Regulating Direct-to-Consumer Drug Information: A Case Study of Eli Lilly's Canadian *40over40* Erectile Dysfunction Campaign

Réglementation de l'information destinée directement aux consommateurs : étude de cas de la campagne *40desplusde40* d'Eli Lilly sur le dysfonctionnement érectile



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Abstract

Like most jurisdictions, Canada prohibits direct-to-consumer advertising (DTCA) of prescribed drugs. However, direct-to-consumer information (DTCI) is permitted, allowing companies to inform the public about medical conditions. An analysis of Eli Lilly's *40over40* promotion campaign for erectile dysfunction (ED), which included a quiz on ED, shows that DTCI, like DTCA, can be an effective means of drug familiarization. The pharmaceutical industry is "playing by the rules" currently in effect in Canada. Regulators should thus seriously consider whether existing rules permitting DTCI actually meet stated objectives of protecting the public from marketing campaigns (i.e., DTCA) that may deliver misleading information.

Résumé

Comme dans la plupart des pays, le Canada interdit la publicité directe auprès des consommateurs (PDAC) pour les médicaments sur ordonnance. Toutefois, on y autorise l'information destinée directement aux consommateurs (IDDC), permettant ainsi aux compagnies d'informer la population sur certains états de santé. Une analyse de la campagne de promotion *40desplusde40* d'Eli Lilly sur le dysfonctionnement érectile (DE) – laquelle comprenait un questionnaire sur le DE – démontre que l'IDDC, comme la PDAC, peut constituer un moyen efficace de familiarisation à un médicament. L'industrie pharmaceutique « se plie aux règles du jeu » actuellement en vigueur au Canada. Mais les organismes de réglementation devraient sérieusement s'assurer que les règles qui permettent l'IDDC atteignent réellement les objectifs en place visant à protéger la population des campagnes de marketing (à savoir la PDAC), lesquelles peuvent donner une information trompeuse.

FOR DECADES, ACCESS TO AND USE OF PRESCRIPTION DRUGS HAS BEEN CONTROLLED by health professionals, and almost exclusively by physicians. It is widely recognized that, due to their potency and potential harms, many drugs should be available only by prescription, i.e., their use authorized by and made available to patients under the supervision of a physician (Donohue 2006). Most developed countries have implemented legislation to control how drugs are developed and marketed to the public (Carter 1999; Rosenthal et al. 2002), because prescription drugs should not be marketed like other commodities.

In the Canadian context, as in most jurisdictions (the US and New Zealand being notable exceptions), direct-to-consumer advertising (DTCA) of prescription drugs is prohibited because of important concerns about patients' misunderstanding of a drug's benefits/risks, thereby contributing to potential misuse, specifically increased demand by patients (Findlay 2001) and pressure on physicians (Lurie 2009) to prescribe marketed drugs (usually brand-name drugs), and thus increasing pressure on the budgets of health insurers (public or private) (Mintzes et al. 2003). The Canadian Food and Drugs Act prohibits the use of promotional activities and advertising that includes "false, misleading or deceptive" information, and it also

restricts drug promoters from “mak[ing] any representation other than with respect to the brand name, proper name, common name, price and quantity of the drug” (Food and Drugs Act 2013). Unfortunately, as Lexchin and Mintzes (2014) demonstrate, there are important weaknesses in the enforcement of Canadian DTCA regulation, most notably with regard to the promotion of “off-label” uses, financial inducements to use a product, fear-generating advertisements and advertising of products with serious safety concerns.

However, Canadian regulation has undergone a series of reforms that have allowed the pharmaceutical industry to employ other strategies to promote their products (Gardner et al. 2003; Lexchin 2013). In the wake of these reforms, Health Canada mandated two independent organizations – Advertising Standards Canada (ASC) and the Pharmaceutical Advertising Advisory Board (PAAB) – to oversee the application of Food and Drugs Act provisions regarding drug promotion. Termed “direct-to-consumer information” (DTCI) by ASC, informational campaigns – which may include brochures and websites, help-seeking announcements (e.g., TV spots) and social media – are permitted in Canada when the putative aim is to raise awareness about a particular medical condition and available treatments, but this information cannot mention a specific product or manufacturer (ASC 2011). This distinction between non-permitted “advertisement” and permissible “information” is based on provisions in the Health Canada policy, *The Distinction Between Advertising and Other Activities*, which “recognizes the importance to the pharmaceutical industry and to the general public of being able to disseminate and access non-promotional information regarding drugs for human use” (Health Canada 2005). It should be noted that the mandate of ASC is limited to materials submitted on a voluntary basis by pharmaceutical companies, and it can only provide non-binding recommendations. Further, even if ASC issues guidelines, it does not have the authority to adjudicate complaints, which remains the responsibility of Health Canada.

When assessing the impact of particular marketing strategies on peoples’ perspectives and knowledge about available treatment options, attention to purpose and business context (e.g., competitor drugs or treatments on the market) is also important when regulators try to distinguish between advertising and the more ambiguous concept of promotion. Both advertising and promotion are ways to increase customer attention towards and sales of a product (Canadian Marketing Association 2013), and these may be used to present or reinforce a product’s image (e.g., as the gold-standard treatment) and/or a corporate brand (Leiss et al. 2013). However, there is a fine line between the two concepts. Rather than being treated as advertising, i.e., “Any paid form of non-personal communication about an organization, product, service, or idea by an identified sponsor” (Alexander 1965: 9), we suggest that DTCI – with its multifaceted design – lies more in the realm of promotion, i.e., “The coordination of all seller-initiated efforts to set up channels of information and persuasion to sell goods and services or to promote an idea” (Belch and Belch 2008).

Considering that, in terms of regulation, the ASC is responsible for framing Health Canada provisions on drug-related communication activities, it is pertinent to use their distinction between DTCI and DTCA to better understand how DTCI works in practice

under current Canadian regulation, and then to evaluate whether this distinction is valid (i.e., whether DTCI is in fact free from the problems associated with DTCA). To facilitate this analysis, we examine Eli Lilly's *40over40* DTCI campaign about the problem of erectile dysfunction (ED). We conclude that this campaign – and DTCI in general – can be a very effective and subtle means of building public familiarization with a particular product (e.g., Cialis), raising most if not all of the same concerns that led governments to restrict DTCA.

DTCI in Action: Eli Lilly's *40over40* Campaign

In 2010, Eli Lilly launched *40over40*, a Canadian DTCI campaign for ED to promote its drug Cialis. The campaign complied with Canadian drug marketing legislation and was certified by the ASC. A *help-seeking* television advertisement presented the medical condition and the burdens of living with ED and referred viewers to the *40over40.ca* website for more information about treatment options. Among an array of information about ED and possible treatments, one of the main features of the website was a quiz that men could take to evaluate if they were among the 40% of Canadian men over 40 years old supposedly with ED (i.e., a good marketing claim that is not adequately referenced on the *40over40* website).

The quiz is a shorter version of the *International Index of Erectile Function (IIEF) Questionnaire*, which was “designed to provide sensitive and specific outcome assessments in clinical trials of ED [with the goal to] develop a self-administered questionnaire that would be suitable for use by clinicians and researchers” (Rosen et al. 1997: 823). The *40over40* campaign used a modified version of the IIEF, a self-assessment quiz for patients (Cappelleri and Rosen 2005). After five general and non-contextualized questions (each scored out of five), if a man's score is lower than 22 out of 25 points, he is identified as being in need of treatment for ED. Interestingly, if he answers “No sexual activity” to the second question “When you had erections with sexual stimulation, how often were your erections hard enough for penetration?” or selects “Did not attempt intercourse” to any of the three next questions, he loses all the points related to that question, thereby placing him in a category considered as abnormal and thus requiring treatment. No matter the context of or the reasons for a lack of sexual function or activity, the website and quiz reports that this is likely due to a problem of ED that can and should be treated. Further, whether or not the score reaches the “abnormal” threshold, the same general statement is presented to the viewer:

If your score is 21 or lower, you may want to speak with your doctor. Only your doctor can confirm if you have ED, so talk to him or her about these results. If you do have ED, remember that you're not alone. There's no need to worry or feel embarrassed. ED is a very common condition affecting about 40% of men over 40 years of age. Luckily, there are many available treatments to consider, and up to 95% of ED cases can be treated. Learn about your options and then make an appointment to discuss them with your doctor (Eli Lilly Canada Inc. 2014).

The quiz's form and presentation give the impression that it is a standard clinical evaluation, but without any empirical justification to support its claims or the need for respondents to seek medical advice. Overall, because of logical shortcuts (e.g., that no sexual activity necessarily implies ED) and lack of references, the scientific validity and trustworthiness of the tool is questionable. But the intent is clear: to convince men that, no matter their situation (i.e., their score on the quiz), they should still talk to their physician about ED and seek treatment.

The Business Context of Drug Familiarization

The business context of a drug information campaign can be an important factor in familiarizing the public with a drug, an element that current legislation is unable to take into account. To continue with the *40over40* example, Cialis has dominated Canadian public media in recent years, with a noted increase in its media presence (e.g., TV, websites or social media) compared with a significant decrease for Viagra. In part, this can be explained by the fact that:

1. Viagra is a slightly older drug and so is marketed less than more recently commercialized drugs (Wienke 2005).
2. There is a general absence of advertising from the other competitors, Bayer and GlaxoSmithKline, mostly because of recent market saturation and lack of features to differentiate their drugs from the market leaders, Cialis and Viagra (Dawar 2013).
3. Pfizer (makers of Viagra) lost its Canadian patent two years before its legal expiration in 2014, following a Supreme Court of Canada decision that voided the patent because of a lack of disclosure in the original patent application of the actual compound treating ED (Teva Canada Ltd. v. Pfizer Canada Inc. 2012). This likely dampened Pfizer's interest in marketing Viagra.

As a result, Cialis is the only product being actively promoted for ED in Canada, which may subtly orient people to think of this drug as the gold-standard treatment, rather than considering alternative drugs or non-medical interventions. So even if the stated (and government-approved) purpose of the *40over40* campaign is help-seeking, questions should be raised by the ASC as to whether this campaign is not *too effective* at achieving this goal, i.e., that it may be very effective at familiarizing the population with a particular and one-sided view of a condition, in terms of severity, incidence and treatment options. For example, there is evidence that drugs are not the sole effective treatment of ED, and that improvement in erectile function is possible through a risk reduction approach (Martin et al. 2014) and behaviour modification, such as choosing more healthy lifestyles, addressing the co-morbidities of aging, stress reduction or seeking counselling or psychotherapy. But this evidence is not presented in the information campaign, and so, viewers are left with a limited array of possible treatment options, of which drugs are favoured (Bélisle Pipon and Williams-Jones 2015).

The Impact of Familiarization

Even with the limited amount of information permitted in DTCI campaigns in Canada, they can, thus, still be a very effective and subtle means of familiarizing the public with a prescription drug, a disease or a company. Further, the public may be unable to evaluate the veracity of advertised claims, especially when the apparent purpose of the marketing campaign is to inform. Familiarization is particularly effective in an era where patients are increasingly seen – and may see themselves – as consumers of health services (Featherstone 2010), and where pharmaceutical drugs are seen as commodities (Cohen et al. 2001). Because they are familiarized with a specific drug, consumers of medical information may develop misconceptions with regards to the nature of their medical condition (e.g., incidence, severity) and the benefits and (lack of) risks of drugs (or other products) promoted to treat the condition (Bélisle Pilon and Williams-Jones 2015). Such misconceptions may be especially problematic in the case of ED, with drugs such as Cialis and Viagra having a highly symbolic role in the collective imagination.

Interestingly, Canada is not the only jurisdiction that struggles with regulating DTCI-like activities; for instance, the Netherlands has similar dispositions allowing disease awareness campaigns that are framed by a self-regulated agency. A study from Leonardo Alves et al. (2014) demonstrated that there is low compliance with self-regulation guidelines in the Netherlands. Even if their study was focused on the print media, the authors raised questions about the growing interest for online drug information and the importance of evaluating “the content and quality of disease awareness websites” to determine the effect on consumer behaviour (Leonardo Alves et al. 2014: 8).

In the same vein, while assessing a low-testosterone, unbranded online campaign in the US, (Schwartz and Woloshin 2013) identified three familiarization strategies that can also be found in the *40over40* campaign: lowering the bar for diagnosis (e.g., by using an exaggerated abnormal threshold in the ED self-assessed quiz), overemphasis of the risks to push patients to consult their doctor (e.g., by exaggerating the potential consequences of ED) and orienting interpretations of the evidence about drug benefits and harms (e.g., by emphasizing drug functioning rather than other potential options to address the causes of the disorder, such as stress reduction). These strategies have a significant familiarizing effect, because they are integral to “well-coordinated campaigns [that] are more subtle than drug-specific campaigns, and they blur the line between public health or professional education and marketing” (Schwartz and Woloshin 2013: 1461).

Conclusion

It is important to note that pharmaceutical companies engaged in DTCI in Canada, such as Eli Lilly in its *40over40* marketing campaign for ED, are playing by the rules set by Health Canada. Thus, if the goal of health regulators is to mitigate the potential undue influence created by drug advertisement (i.e., current restrictions on DTCA) (Bélisle Pilon 2013), then regulators should acknowledge that DTCI raises similar ethical problems as DTCA. They

should protect the public from activities that have a familiarizing component that undermines the ability of people (i.e., patients and health professionals) to make free and informed decisions about how to best manage and find treatments for particular medical conditions. More specifically, regulators should consider treating DTCI as an indirect but powerful form of advertising that can familiarize people with certain drugs, and so apply similar restrictions to DTCI as for DTCA. Rather than DTCI being treated as a self-regulated activity, and thus being subject to only voluntary evaluation by ASC, information campaigns should be assessed under the current and more strict (if still limited) regulation for DTCA. In addition, the business context should be considered when a campaign is assessed, so that a non-promotional campaign does not end up promoting one drug as the gold standard. In so doing, regulators could eliminate important ambiguities surrounding the notion of DTCI as being relatively neutral “information provision” and close important loopholes in current regulations.

Recognizing that DTCI is most often drug promotion – in the sense of the World Health Organization (1988: 5) definition, where “promotion’ refers to all informational and persuasive activities by manufacturers and distributors, the effect of which is to induce the prescription, supply, purchase and/or use of medicinal drugs” – and thus an indirect form of advertising means that it should, more generally, be regulated alongside DTCA activities. This would, we suggest, help to make the “rules of the game” for drug marketing clearer and more robust, and also better protect the public and help professionals from initiatives designed to subtly influence their behaviour.

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References

- Advertising Standards Canada (ASC). 2011. *DTCI Guide*. Toronto, ON: Advertising Standards Canada. <<http://www.adstandards.com/en/clearance/ConsumerDrugs/DTCIGuideEN.pdf>>.
- Alexander, R. 1965. *Marketing Definitions: A Glossary of Marketing Terms*. Chicago, IL: American Marketing Association.
- Belch, G. and M. Belch. 2008. *Advertising and Promotion: An Integrated Marketing Communications Perspective* (8th ed.). New York, NY: McGraw-Hill/Irwin.
- Bélisle Pipon, J.-C. and B. Williams-Jones. 2015. “Drug Familiarization and Therapeutic Misconception via Direct-to-Consumer Information.” *Journal of Bioethical Inquiry*. doi 10.1007/s11673-015-9634-8.
- Bélisle Pipon, J.-C. (2013). *De la binarité au modèle AI3R : la bioéthique écosystémique comme modèle d'analyse normative de l'industrie biopharmaceutique*. (Master Thesis). Université de Montréal, Montréal, Québec.
- Canadian Marketing Association (CMA). 2013. *Code of Ethics and Standards of Practice*. Toronto, ON: Canadian Marketing Association.
- Cappelleri, J.C. and R.C. Rosen. 2005. “The Sexual Health Inventory for Men (SHIM): A 5-year Review of Research and Clinical Experience.” *International Journal of Impotence Research* 17(4): 307–19. DOI:10.1038/sj.ijir.3901327.
- Carter, P.I. 1999. “Federal Regulation of Pharmaceuticals in the United States and Canada.” *Loyola of Los Angeles International and Comparative Law Journal* 21: 215.

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- Cohen, D., M. McCubbin, J. Collin and G. Pérodeau. 2001. "Medications as Social Phenomena." *Health* 5(4): 441–69. DOI:10.1177/136345930100500403.
- Dawar, N. 2013. "When Marketing Is Strategy." *Harvard Business Review*. <<http://hbr.org/2013/12/when-marketing-is-strategy/ar/1>>.
- Donohue, J. 2006. "A History of Drug Advertising: The Evolving Roles of Consumers and Consumer Protection." *Milbank Quarterly* 84(4): 659–99. DOI:10.1111/j.1468-0009.2006.00464.x.
- Eli Lilly Canada Inc. 2014. "Symptoms – Take This Self-Assessment Quiz to Find Out if You May Have ED." 40over40. <<http://40over40.ca/symptoms.php#b>>.
- Featherstone, M. 2010. "Body, Image and Affect in Consumer Culture." *Body and Society* 16(1): 193–221. DOI:10.1177/1357034X09354357.
- Findlay, S.D. 2001. "Direct-to-Consumer Promotion of Prescription Drugs." *Pharmacoeconomics* 19(2): 109–19. DOI:10.2165/00019053-200119020-00001.
- Food and Drugs Act, L.R.C. 2013. 1985, ch. F-27. <<http://lois-laws.justice.gc.ca/eng/acts/F-27/>>.
- Gardner, D.M., B. Mintzes and A. Ostry. 2003. "Direct-to-Consumer Prescription Drug Advertising in Canada: Permission by Default?" *Canadian Medical Association Journal* 169(5): 425–27. <<http://www.cmaj.ca/content/169/5/425>>.
- Health Canada. 2005. The Distinction between Advertising and Other Activities. Ottawa, ON: Government of Canada. <http://www.hc-sc.gc.ca/dhp-mps/advertpublicit/pol/actv_promo_vs_info-eng.php>.
- Leiss, W., S. Kline, S. Jhally and J. Botterill. 2013. *Social Communication in Advertising: Consumption in the Mediated Marketplace* (3rd ed.). Routledge. New York, NY.
- Leonardo Alves, T., A.F. Martins de Freitas, M.E.C. van Eijk and A.K. Mantel-Teeuwisse. 2014. "Compliance of Disease Awareness Campaigns in Printed Dutch Media with National and International Regulatory Guidelines." *PLoS ONE* 9(9): e106599. DOI:10.1371/journal.pone.0106599.
- Lexchin, J. 2013. "Health Canada and the Pharmaceutical Industry: A Preliminary Analysis of the Historical Relationship." *Healthcare Policy* 9(2): 22–29. DOI:10.12927/hcpol.2013.23621.
- Lexchin, J. and B. Mintzes. 2014. "A Compromise Too Far: A Review of Canadian Cases of Direct-to-Consumer Advertising Regulation." *The International Journal of Risk and Safety in Medicine* 26(4): 213–25. doi:10.3233/JRS-140635.
- Lurie, P. 2009. "DTC Advertising Harms Patients and Should Be Tightly Regulated." *The Journal of Law, Medicine & Ethics* 37(3): 444–50. DOI:10.1111/j.1748-720X.2009.00405.x.
- Martin, S.A., E. Atlantis, L. Lange, A.W. Taylor, P. O'Loughlin, G.A. Wittert and Florey Adelaide Male Ageing Study (FAMAS). 2014. "Predictors of Sexual Dysfunction Incidence and Remission in Men." *The Journal of Sexual Medicine* 11(5): 1136–47. DOI:10.1111/jsm.12483.
- Mintzes, B., M.L. Barer, R.L. Kravitz, K. Bassett, J. Lexchin, A. Kazanjian et al. 2003. "How Does Direct-to-Consumer Advertising (DTCA) Affect Prescribing? A Survey in Primary Care Environments With and Without Legal DTCA." *Canadian Medical Association Journal* 169(5): 405–12. <<http://www.cmaj.ca/content/169/5/405>>.
- Rosen, R.C., A. Riley, G. Wagner, I.H. Osterloh, J. Kirkpatrick and A. Mishra. 1997. "The International Index of Erectile Function (IIEF): A Multidimensional Scale for Assessment of Erectile Dysfunction." *Urology* 49(6): 822–30. DOI:10.1016/S0090-4295(97)00238-0.
- Rosenthal, M.B., E.R. Berndt, J.M. Donohue, R.G. Frank and A.M. Epstein. 2002. "Promotion of Prescription Drugs to Consumers." *New England Journal of Medicine* 346(7): 498–505. DOI:10.1056/NEJMsa012075.
- Schwartz, L.M. and S. Woloshin. 2013. "Low "T" as in "Template": How to Sell Disease." *JAMA Internal Medicine* 173(15): 1460–62. <<http://archinte.jamanetwork.com/article.aspx?articleID=1726956>>.
- Teva Canada Ltd. v. Pfizer Canada Inc., 2012. 2012 SCC 60 625–659. <<http://scc-csc.lexum.com/scc-csc/scc-csc/en/item/12679/index.do>>.
- Wienke, C. 2005. "Male Sexuality, Medicalization, and the Marketing of Cialis and Levitra." *Sexuality and Culture* 9(4): 29–57. DOI:10.1007/s12119-005-1001-1.
- World Health Organization (WHO). 1988. *Ethical Criteria for Medicinal Drug Promotion* (No. 88/7708-WHO/Glor43WO). Geneva: WHO. <<http://apps.who.int/iris/handle/10665/38125>>.

A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada? Insights from Across the Ponds

Démarche nationale quant aux décisions de remboursement des médicaments pour maladies rares au Canada? Pistes provenant d'outremer



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Abstract

Introduction: Regardless of the type of health system or payer, coverage decisions on drugs for rare diseases (DRDs) are challenging. While these drugs typically represent the only active treatment option for a progressive and/or life-threatening condition, evidence of clinical benefit is often limited because of small patient populations and the costs are high. Thus, decisions come with considerable uncertainty and risk. In Canada, interest in developing a pan-Canadian decision-making approach informed by international experiences exists.

Objective: To develop an inventory of existing policies and processes for making coverage decisions on DRDs around the world.

Methods: A systematic review of published and unpublished documents describing current policies and processes in the top 20 gross domestic product countries was conducted. Bibliographic databases, the Internet and government/health technology assessment organization websites in each country were searched. Two researchers independently extracted information and tabulated it to facilitate qualitative comparative analyses. Policy experts from each country were contacted and asked to review the information collected for accuracy and completeness.

Results: Almost all countries have multiple mechanisms through which coverage for a DRD may be sought. However, they typically begin with a review that follows the same process as drugs for more common conditions (i.e., the centralized review process), although specific submission requirements could differ (e.g., no need to submit a cost-effectiveness analysis). When drugs fail to receive a positive recommendation/decision, they are reconsidered by “safety net”-type programs. Eligibility criteria vary across countries, as do the decision options, which may be applied to individual patients or patient groups.

Conclusions: With few exceptions, countries have not created separate centralized review processes for DRDs. Instead, they have modified components of existing mechanisms and added safety nets.

Résumé

Introduction : Peu importe le type de système de santé ou le type de payeur, les décisions quant à la couverture des médicaments pour maladies rares (MMR) présentent tout un défi. Bien que ces médicaments constituent habituellement les seuls traitements pour certains états de santé évolutifs ou mettant en danger la vie, les données démontrant leurs avantages cliniques sont souvent limitées en raison de la petitesse des échantillons de patients et des coûts élevés. Ainsi, les décisions s'accompagnent d'incertitude et de risques. Au Canada, on montre un certain intérêt pour mettre au point une démarche de prise de décisions pancanadienne éclairée par l'expertise internationale.

Objectif : Développer un inventaire des politiques et processus touchant les décisions quant à la couverture des MMR dans le monde.

Méthode : Nous avons procédé à une revue systématique des documents publiés et non publiés

qui décrivent les politiques et procédures dans les 20 pays se classant en tête selon le produit intérieur brut. Les bases de données bibliographiques, l'Internet et les sites Web des gouvernements et des organismes d'évaluation des technologies de la santé de chaque pays ont été consultés. Deux chercheurs ont indépendamment recueilli les données et les ont tabulées pour permettre d'effectuer des analyses comparatives qualitatives. Nous avons demandé à des experts des politiques dans chacun des pays de réviser la précision et l'exhaustivité de l'information recueillie.

Résultats : Presque tous les pays sont dotés de multiples mécanismes par lesquels on peut obtenir une couverture pour les MMR. Cependant, cela commence habituellement par un examen qui suit les mêmes processus que dans le cas d'un médicament pour une maladie plus commune (c'est-à-dire un processus de révision centralisé), bien que les exigences pour soumettre un dossier peuvent être différentes (par exemple, il n'y a pas besoin de présenter une analyse du coût-efficacité). Si un médicament ne reçoit pas de recommandation ou décision positive, on l'aborde alors en fonction de programmes de type « filet de sécurité ». Les critères d'admissibilité varient d'un pays à l'autre, de même que les choix de décisions, lesquelles peuvent s'appliquer parfois à des patients individuels, parfois à des groupes de patients.

Conclusions : Sauf quelques exceptions, les pays n'ont pas créé de processus de révision distincts centralisés pour les MMR. Ils ont plutôt modifié les composantes des mécanismes déjà en place et ont ajouté des filets de sécurité.

Introduction

Most developed countries have instituted centralized review processes for making coverage/reimbursement recommendations or decisions around new therapies (Morgan et al. 2006, Stafinski et al. 2011). In Canada, they include the Common Drug Review (CDR) and the pan-Oncology Drug Review (pCODR), which make funding recommendations to a number of public drug programs (at federal, provincial and territorial levels). In general, these processes were designed for therapies that treat relatively common conditions. However, with the advent of promising drugs for rare diseases (DRDs), they are being challenged (Cote and Keating 2012; Simoens 2011; Stafinski et al. 2011), as such diseases affect small populations, and have limited clinical evidence, leading to considerable harm and benefit uncertainties. Additionally, treatment costs are often high, and while the budget impact for a single rare disease may be small, there are over 7,000 rare diseases (in the US) (National Institute of Health 2015), many with promising treatments on the horizon. Therefore, decision-makers need to balance access to treatments with health system sustainability. In an effort to achieve this, mechanisms for determining funding conditions for DRDs have been established in many countries. However, details of these, whether they are specific to DRDs and how they compare internationally, have not been systematically explored. This information is needed to inform the current policy debate around a possible national Canadian DRD formulary. Recently, concerns

from patients, providers and industry over the funding of some DRDs in Canada have heightened (Weeks 2014). This has resulted in a commitment from provincial and territorial health ministers to consider developing a national approach to managing access to DRDs (Alberta Public Affairs Bureau 2014; Goodman 2004).

Purpose and Objectives

The purpose of this paper is to determine the current landscape of reimbursement decision-making around DRDs, based on a review of processes in 20 OECD countries. Its objectives are:

1. To identify and systematically describe national-level reimbursement/coverage decision processes on DRDs in 20 OECD countries with socially insured healthcare systems.
2. To analyze and compare these processes across countries according to their scope, information requirements, decision criteria, stakeholder participation and decision options.

Methods

The top 20 OECD countries by gross domestic product per capita (in 2012) with socialized health insurance programs/universal healthcare were included. These countries were selected because they share similar competing healthcare demands and economic environments and provide coverage for some pharmaceuticals. They were: Australia (AUS), Austria (AUT), Belgium (BEL), Denmark (DEN), Finland (FIN), France (FRA), Germany (GER), Iceland (ICE), Ireland (IRE), Italy (ITA), Japan (JAP), Korea (KOR), Luxembourg (LUX), the Netherlands (NET), New Zealand (NZ), Norway (NOR), Spain (SPA), Swede (SWE), Switzerland (SWI) and the United Kingdom (UK), which includes England (ENG), Wales (WAL) and Scotland (SCO). The three UK jurisdictions were included, as they operate additional decision-making processes to those through the central UK National Health Service (NHS). To identify relevant published literature, bibliographic databases covering health and social science literature, including PubMed (MEDLINE), EMBASE and Web of Knowledge, were searched using a structured search strategy. The strategy combined controlled vocabulary terms, such as medical subject headings, with additional keywords and synonyms, such as “orphan drugs,” “rare diseases,” “decision-making” and “reimbursement” (see Appendix A at www.longwoods.com/content/24210 for the full strategy). To capture non-peer-reviewed, unpublished information, several grey literature sources were searched, including NHS Evidence, the Knowledge Utilization – Utilisation des Connaissances at Laval University, the New York Academy of Medicine Grey Literature Collection and the websites of rare diseases associations and ministries of health. Last, separate Google searches were conducted for each country using a set of standard keywords (“rare disease*” OR “rare disorder*” OR “orphan drug*” OR “ultra rare”) AND [decision* OR policy OR policies OR reimbursement OR economic* OR rationing OR access* OR fund* OR legislation OR catastrophic OR regular*]) combined with country name. For each search,

the first 300 “hits” were scanned. Please see “Appendix A1. Literature search strategy” at www.longwoods.com/content/24210.

A publication limit of 2004 or later was applied to increase the likelihood of identifying information reflecting current reimbursement processes. This period also included the points when reimbursement processes in many countries were first exposed to high-cost DRDs (United States Food and Drug Administration 2014). No language limits were placed. For non-English language information, translation software was used (Babylon® and GoogleTranslate®).

Information on each country was extracted independently by two researchers using a standard, pre-tested data extraction form, and included: country, reimbursement mechanisms and patient eligibility criteria, information and factors considered during the decision-making process, structure and membership of the decision-making committee and types of decision options available. To ensure the accuracy and comprehensiveness of the data collected, individuals from each country were contacted and asked to review their respective completed extraction forms. They were government policy makers or senior staff of agencies managing drug review processes in the country. The data were then summarized in tables to facilitate a qualitative comparative analysis. The qualitative analysis focused on 10 key policy elements (Table 1) identified by drug program decision-makers in Canada and abroad who are part of a Canadian Institutes of Health Research-funded team focusing on the development of policies around DRDs (University of Alberta, School of Public Health 2014). Drug plan managers from Canadian provinces were also consulted.

Results

Results, summarized in Tables B1 through B4, are presented by the policy element mentioned below (Please see Tables B1 through B4 at www.longwoods.com/content/24210).

TABLE 1. Summary of 10 key policy elements

Policy element	Description
Eligibility/scope	Characteristics of the disease or condition and the types of drugs that fall within the mandate of the review process
Patient population	Eligible patients and whether a process resulted in population or individual patient-level recommendations or decisions
Clinical evidence	Specific types of clinical evidence and study designs required by the review process
Cost data	Information on the cost implications of providing the drug
Cost-effectiveness	Requirements for economic evaluations
Patient input	Opportunities for patients and families to provide input into the review process
Review/decision-making participation	Review committee composition and terms of reference
Decision options	Range of reimbursement options available
Decision factors	Factors considered by the review committee
Transparency	Information on the review process and decisions available to the public

Eligibility and scope

Across the countries, two types of decision-making processes for DRDs were identified: (1) centralized drug reviews (CDRs), through which most new drugs considered for inclusion on the benefit list are reviewed, and (2) “safety net” programs, through which drugs unlicensed for the specific indication, failing to receive a positive CDR recommendation/decision or have yet to undergo CDR review are assessed (see Table B1 at www.longwoods.com/content/24210) (Garau et al. 2009; *Ministre des Affaires Sociale et de la santé* 2014; Ministry of Health, Welfare and Sport 2013; OrphaNews Europe 2009; Seoane-Vazquez et al. 2009). “Safety net” programs typically provide temporary reimbursement of a drug to individual patients. Drugs eligible for review through the CDR include all outpatient and, in some cases, in-patient drugs with market authorization/approval. “Safety net” programs exist in 13 countries (AUS, AUT, BEL, DEN, FRA, GER, IRE, ITA, NZ, NOR, SPA, SWI and the UK). However, except for the UK and BEL, these programs are not limited to DRDs, and eligible drugs typically also include those for “severe” conditions regardless of prevalence, which have no treatment alternatives (FRA, GER, SPA) or if alternatives have been insufficiently efficacious or have unacceptable side effects (NZ, NOR). “Severe” is commonly defined as “life-threatening” or “chronically debilitating” (AUS, JAP, KOR, NOR, SPA, SWE). In AUS and NZ, drugs must have already been reviewed by the CDR before they are eligible for consideration by a “safety net” program. There may also be further requirements. For coverage through AUS’s “safety net” program (“Life Saving Drugs Program” or LSDP), a drug must have failed to receive a positive recommendation based on unacceptable cost-effectiveness. In NZ, patients must first meet certain prerequisites before being considered for high-cost DRDs, through the Unusual Clinical Circumstances pathway. This pathway applies to patients with rare conditions, and requires that (i) the patient has reasonably tried and failed all alternative funded treatments, (ii) the patient is experiencing an indication or set of clinical circumstances that is so unusual that PHARMAC (the Pharmaceutical Agency in NZ that decides on behalf of District Health Boards which medicines will be subsidized) is unlikely to consider listing treatments for these on the Schedule and (iii) PHARMAC has not yet considered the treatment for the patient’s clinical circumstances, through the CDR (see Table B2 at www.longwoods.com/content/24210).

Seventeen of the countries have a formal national definition of DRDs. A majority (AUT, BEL, FIN, FRA, GER, IRE, ITA, LUX, the NET, SCO, SWI) use the European Union (EU) Regulation on Orphan Medicinal Products’ definition: “... intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating; the prevalence of the condition in the EU must not be more than 5 in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development; and no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition” (European Medicines

Agency 2014). Other countries have developed specific definitions, none of which are based solely on prevalence (AUS, JAP, KOR, NOR, SPA, SWE, the UK).

In a number of countries, “safety net programs” that include DRDs as well as other drugs involve reviews of requests for individual patients (AUT, DEN, FRA, ITA, SPA). By contrast, such programs in the UK and BEL are specific to DRDs only. The UK’s National Institute for Health and Care Excellence (NICE) has established the Highly Specialised Technology (HST) program, to which drugs are referred by the Secretary of State for Health, and must meet all of these criteria: “The target patient group for the technology in its licensed indication is so small that treatment will usually be concentrated in very few centres in the NHS; the target patient group is distinct for clinical reasons; the condition is chronic and severely disabling; the technology is expected to be used exclusively in the context of a highly specialized service; the technology is likely to have a high acquisition cost; the technology has the potential for life-long use; and the need for national commissioning of the technology is significant” (NICE 2013b). While “so small” is not explicitly defined, guidance around the criteria states that the number of patients affected in the UK should be fewer than 500. Decisions on drugs not selected for review through the HST program are left to the relevant budget holder (NHS England or one of the 211 local Clinical Commissioning Groups) (NICE 2013c).

Another exception is Belgium’s “Special Solidarity Fund” (SSF) comprising a “safety net” program exclusively for DRDs. Eligible DRDs are orphan drugs (as designated by the European Medicines Agency) that have yet to receive a positive recommendation from the Belgian CDR *and* meet at least one of the following criteria:

1. treat a rare disease requiring a specific physiopathological treatment,
2. treat a rare disease requiring a continuous and complex treatment,
3. treat chronically ill children,
4. involve innovative treatment techniques or
5. otherwise requires medical treatment abroad (Guillaume et al. 2010).

This program provides temporary funding. To obtain access to a drug through SSF, patients should have exhausted all other public or private reimbursement options at national, European or international levels. In several countries, existing CDR processes have been modified to accommodate DRDs and other high-cost drugs. ENG, SCO and WAL have each instituted “Patient Access Schemes,” which manufacturers or sponsors may propose as part of their CDR submission. These schemes facilitate patient access to expensive and typically innovative drugs that do not appear to offer significant benefits over existing treatments by improving their cost-effectiveness. This may be achieved through listing agreements or risk-sharing arrangements that incorporate outcomes guarantees realized within a defined period (Morel et al. 2013). Other countries have implemented “fast-track” mechanisms, where eligible drugs are prioritized by the CDR, bypassing the standard process in which drugs are reviewed

in the order received (e.g., FRA, the NET, SWI). In general, “fast-tracked” drugs are those that treat life-threatening conditions where no alternative treatment exists. Therefore, while eligibility may not include a prevalence condition, many DRDs meet these other criteria.

Patient population

In all countries, a DRD that receives a positive decision from the CDR is added to the list of publicly insured medicines. As with drugs for common diseases, the list specifies the groups of patients for whom the DRD will be provided. In contrast, decisions made within most “safety net” programs apply to an individual patient, whose prescribing physician has submitted a request for access on his/her behalf (AUT, BEL, DEN, FRA, GER, IRE, ITA, the NET, NOR, NZ, SPA) (see Table B1 at www.longwoods.com/content/24210). Some “safety net” programs also accept requests from other sources, including Centres of Excellence/Reference (virtual or real centres of a network of experts in research, treatment and care across the child-to-adult age spectrum) or patient organizations (FRA, ITA), manufacturers (AUS, GER, the NET, the UK), a pharmacist or pharmacy (FIN, the NET), the treating hospital (SPA) or a university (ITA) (see Table B1 at www.longwoods.com/content/24210). However, the decisions remain at the patient level. In the remaining countries (FRA, GER, ITA, SPA), “safety net” programs may provide access to groups of patients (see Table B1 at www.longwoods.com/content/24210).

EVIDENCE REQUIREMENTS

In all countries, funding requests for DRDs to the CDR must provide specific information that usually applies to all drugs. This includes: (1) indication and target population, (2) therapeutic claim, (3) net clinical benefit (safety, efficacy and effectiveness), (4) budget impact and (5) economic evaluation (usually a cost-effectiveness analysis) (see Table B3 at www.longwoods.com/content/24210). However, in NET, cost-effectiveness analyses are waived for DRDs (see Table B3 at www.longwoods.com/content/24210). All of the “safety net” programs require statements of medical justification and information on items (1), (2) and (3) above. In some programs, treatment protocols, e.g., treatment dose, duration or planned monitoring, must also be submitted (DEN, FRA, GER, SPA, the UK). Others require information demonstrating no appropriate alternative treatment (AUS, AUT, BEL, GER, the NET, NZ) or intent to submit an application for market authorization (FRA, GER).

Clinical evidence

In most countries, CDR submission guidelines indicate a *preference* for head-to-head randomized controlled trials (RCTs) for *all* drugs, with the drug being compared to current best practice or standard care based on hard clinical endpoints (as opposed to surrogate endpoints) (see Table B3 at www.longwoods.com/content/24210). However, no guideline specifies minimum evidentiary requirements/standards for determining clinical benefit. Moreover, most

indicate that all types and levels of evidence will be considered, including trials using surrogate endpoints, as well as unpublished and ongoing studies (AUS, AUT, BEL, the NET, NOR, SWE, the UK).

In contrast, all but two of the “safety net” programs have no formal clinical evidence requirements. ITA specifies data from Phase II/III clinical trials, and AUT requires at least one Phase III clinical trial to be in progress or completed.

Cost data

Most CDRs require budget impact analyses (BIAs) for all drugs, regardless of their indication (AUS, BEL, DEN, FIN, FRA, ICE, IRE, ITA, KOR, the NET, NOR, SPA) (see Table B3 at www.longwoods.com/content/24210). The BIAs must incorporate the prevalence and incidence of the disease and the potential number of patients, and comply with the CDR’s methodological guidelines. Almost all CDRs require information on both the cost and price of the treatment (AUS, FRA, IRE, ITA, LUX, the NET, NZ, SPA, SWE, SWI, WAL, the UK). In five of these, price information on comparable reimbursed drugs must be provided (AUT, ICE, ITA, LUX, SWI), and in three, price comparisons with other European jurisdictions must also be made (AUT, ITA, LUX). In countries where reimbursement decision-making and pricing may occur simultaneously, sales forecasts for the first year and usually three years after approval are required (AUS, FIN, ICE, NOR). In contrast, most “safety net” programs do not require cost information (AUT, DEN, FIN, FRA, GER, ITA, the NET, NOR, NZ, SCO, SPA, the UK, WAL). The exceptions are the LSDP (AUS) and SSF (BEL). Information demonstrating an unreasonable financial burden of treatment to the patient must be submitted.

Cost-effectiveness

In the majority of countries, *all* drugs considered by the CDR must provide an economic evaluation (AUS, AUT, FIN, ICE, IRE, JAP, NOR, NZ, SCO, SWE, SWI, the UK, WAL) (see Table A2 at www.longwoods.com/content/24210). Some state a preference for cost-utility analyses (AUS, IRE, NZ), others prefer cost-effectiveness analyses (ITA, SWI) and yet others accept either type (JAP, SCO, SWE). Most of these specify that it must be based on a comparison to available alternative treatments (AUS, AUT, FIN, ICE, SCO, SWE, the UK, WAL). In some countries, the perspective for the evaluation is also specified: societal (FIN, IRE, SWE), payer (AUS) or both (NOR). However, several countries do not require an economic evaluation for drugs with indications for which no alternative treatment exists (GER, ITA, KOR, the NET). Further, in the NET, CDRs waive economic evaluations specifically for DRDs. In GER, DRDs with annual sales below €50 million per year are exempted from cost-benefit assessments (Fulda 2011; Holtorf et al. 2009).

Patient input

Most CDR processes provide opportunities for input from patients/families; this applies to all drugs (including DRDs) (see Table B2 at www.longwoods.com/content/24210) (Danish Health and Medicines Authority 2012; Taruscio et al. 2011). This often consists of submissions in which patients or patient organizations provide prescribed information on their experiences with the condition/disease (AUS, FRA, GER, JAP, KOR, NZ, SCO, SWI, the UK, WAL). These are usually considered alongside the technical assessment of clinical and economic implications by the review/decision-making committee. In some countries (AUS, GER, JAP, KOR, SCO, WAL), that committee includes a patient representative (a further opportunity for patient input) (see Table B3 at www.longwoods.com/content/24210). Except for the Scottish Medicines Consortium (SMC) in Scotland, none of the CDR processes appear to have established separate opportunities for DRDs. The SMC's Patient and Clinician Engagement process was created solely for the review of end-of-life treatments and DRDs. It involves a meeting of patient representatives and healthcare professionals with relevant expertise to gather information on the benefits of a medicine, specifically around impact on patients' quality of life (Scottish Medicines Consortium 2014b). This information is subsequently presented to the review/decision-making committee during its meeting. Please see "Table B2. Information requirements of reimbursement review processes" at www.longwoods.com/content/24210.

Opportunities for patient input in "safety net" programs are limited. None have implemented patient submission processes comparable to those of CDRs. This may be, in part, because these are usually considered on a case-by-case basis (AUT, BEL, DEN, FRA, GER, IRE, ITA, the NET, NOR, NZ, SPA, the UK). Only two countries accept requests directly from patients or patient representatives (AUS, ITA).

Review/Decision-making participation

In all of the countries where DRDs are included in the CDR process, reimbursement recommendations or decisions are made by a review committee. These committees are appointed, range from 5 to 28 members, and represent multiple stakeholder groups (see Table B3 at www.longwoods.com/content/24210) (Nagae 2011; Office Federal de la Santé Publique 2013). All committees include physicians, healthcare providers or clinical experts. In some countries, members also include pharmacists/pharmacologists (AUS, BEL, FIN, ITA, JAP, LUX, the NET, SCO, SWI), social insurance representatives (AUT, BEL, ENG, FIN, GER, KOR, LUX, SWI), government representatives (AUT, BEL, FIN, FRA, LUX, the NET), health economists/economic experts (AUS, FRA, JAP, the NET, WAL), patient representatives (AUS, ENG, GER, KOR, SCO, SWI, WAL) or drug industry representatives (AUS, ENG, SCO, SWI, WAL). Please see "Table B3. Elements of processes through which reimbursement recommendations or decisions on DRDs are formulated" at www.longwoods.com/content/24210.

In “safety net” programs, adjudication may be by separate committees in the same organization as the CDR (AUS, DEN, FRA, ITA, NZ, the UK), or in a different organization (often the national pharmaceutical regulatory body) (FIN, GER, the NET, NOR, SPA), or a senior-level medical officer or group of physicians representing the payer (e.g., sickness funds) (AUT, BEL, respectively) (see Table B3 at www.longwoods.com/content/24210). In Belgium, the group of physicians consists of experts in rare diseases from the College of Medical Doctors for Orphan Drugs.

Decision options

In almost all of the countries, the CDRs consider, at a minimum, three options: “provide,” “do not provide” or “provide with conditions” (i.e., restrict to certain prescribing physicians, facilities or patients) (see Table B1 at www.longwoods.com/content/24210). In some countries (BEL, FRA, the UK), an option for interim provision of treatment with additional data collection to address existing evidence uncertainties to make a definitive recommendation or decision (“provide with data collection”) is available. In countries where all drugs receiving market authorization must be reimbursed (ITA, GER), the decision options comprise negotiations on price and patient sub-populations for whom the drug will be offered (see Table B1 at www.longwoods.com/content/24210). Therefore, all decisions are “provide with conditions”. For all “safety net” programs, decision options are limited to: “do not provide” or “provide with conditions”. “Conditions” include restrictions on prescribing to certain physicians, centres and/or patients who fulfill specific clinical criteria and enrolment in a registry to facilitate ongoing monitoring of patients receiving the drug (e.g., the UK). These decisions typically provide temporary funding only, and patients (through their physicians) must apply for renewal (BEL, DEN, FRA, GER, ITA, NZ, SPA, the UK). Renewal may require that a patient demonstrate improvement in certain pre-defined outcomes (FRA, the UK). In most countries, “provide with conditions” decisions are implemented through “managed access programs” or “patient access programs”.

Factors considered by review committees

Factors considered by at least two countries with CDRs that review DRDs are summarized in Table 2 (see Table 2 at www.longwoods.com/content/24210). They include, in decreasing order of frequency: clinical benefit or effectiveness (17 CDRs), value for money or cost-effectiveness (13 CDRs), affordability or budget impact (13 CDRs), disease severity or burden (12 CDRs), clinical need (12 CDRs), availability of alternative treatments (10 CDRs), safety or benefit-harm ratio (8 CDRs), therapeutic value (6 CDRs), price and level of reimbursement in other jurisdictions (6 CDRs), quality of and uncertainty in the evidence (5 CDRs), impact on public health (3 CDRs), innovativeness of the drug (4 CDRs), experience with the drug or extent of current use (2 CDRs) and the ethical principle of solidarity (2 CDRs). Some countries specify ethical principles used in their decision-making such as “rules-of-rescue” (AUS) or human value (SWE). In most countries, the CDRs have not defined a

threshold incremental cost-effectiveness ratio (ICER) (AUS, FIN, IRE, ITA, JAP, KOR, NZ, SWE, SWI, WAL). SCO and the UK are exceptions, with a formal ICER threshold of £30,000 per quality-adjusted life year gained (QALY). But this is waived for specific drugs, including those for ultra-rare diseases (with fewer than 500 patients in the UK). While few countries use fixed ICER thresholds, several identify informal thresholds (e.g., €45,000 per QALY [IRE] and NOK 500,000 per QALY [NOR]; Coughlan et al. 2009; Gothesen et al. 2013). Drugs with ICERs below thresholds would generally be considered cost-effective. In some countries, thresholds are either not applied (the NET) or are applied flexibly (KOR) for DRDs. Please see “Table 2. Factors/criteria most commonly used by centralized drug review committees considering DRDs” at www.longwoods.com/content/24210.

All “safety net” programs consider (see Table B3 at www.longwoods.com/content/24210): medical necessity or unmet clinical need arising from the lack of suitable alternative treatments (see Table B3 at www.longwoods.com/content/24210). In addition, most require some indication of clinical benefit (safety, efficacy and/or effectiveness) (AUS, BEL, DEN, FRA, ITA, NOR, NZ, the UK). Less common factors considered by programs include: patient’s financial burden (AUS, BEL, NOR), cost-effectiveness (although no ICER threshold is specified) (AUS, AUT, NOR, NZ, WAL), cost or budget impact (AUS, AUT, FRA, IRE, NZ, the UK), plans for additional studies (the UK), innovativeness (the UK), impact on existing services (AUT, FRA, the UK) and clinical plausibility and appropriateness (SCO, the UK, WAL). NZ is the only country that utilizes the same criteria for “safety net” decision-making as in the CDR.

Transparency

In 10 countries, key findings by the CDR, final recommendations/decisions and rationale for the recommendations/decisions are publicly available (AUS, DEN, FRA, GER, IRE, ITA, the NET, SCO, SWE, SWI, the UK), and are summarized and posted on the CDR’s website (see Table B4 at www.longwoods.com/content/24210) (Mossialos et al. 2008). In some countries, the full evaluation report and minutes of review committee meetings are also made public (AUS, FRA, the NET, SWE, SWI). Please see “Table B4. Public accountability and decision implementation considerations” at www.longwoods.com/content/24210.

In contrast, publicly available information on specific DRDs considered by “safety-net” programs is scarce. Rationale supporting a closed process includes the fact that most “safety-net” programs are offered at the individual patient level; if information is made public, patient confidentiality may be violated. Additional rationale relates to protecting commercial interests of the manufacturers/sponsors.

Interpretation

This paper presents a comparative analysis of national reimbursement processes for DRDs in 20 OECD countries on 10 key policy relevant elements. While in Canada, there are two CDR processes (the CDR for non-cancer drugs and the pan-Canadian Oncology Drug

Review for cancer drugs), their mandates are limited to the formulation of recommendations. Reimbursement decisions remain with provincial and territorial drug plans. Further, the review processes for DRDs are the same as those for other drugs. In general, DRDs reviewed through the CDR processes have fared poorly, and subsequent reimbursement decisions across the jurisdictions have varied (Menon et al. 2015).

Lessons that might be learned from other countries facing similar competing demands for health correlate to the overall process itself, the role of rarity (or prevalence), evidence uncertainties and costs. First, although all of these countries have established CDR processes for outpatient and, in some cases, in-patient drugs, none, except for the UK, have created a separate CDR process for DRDs. This may be explained by the explicit use of ICER threshold ranges by CDRs for non-DRDs in the UK that drugs for ultra-rare diseases would invariably fail to meet. Its separate process recognizes the need to assess “value” differently. In Canada, new drugs (including DRDs) being considered for reimbursement through the provincial and territorial drug benefit plans must first be assessed by the CDR (non-oncology drugs) or the pan-Canadian Oncology Drug Review (oncology drugs), both of which issue a funding recommendation. The absence of a separate centralized review process for DRDs may be attributable to the fact that decision-making still rests with the individual provincial and territorial drug plans. In four provinces (Alberta, British Columbia, New Brunswick and Ontario), “safety net” programs for some DRDs have been established.

Second, although many countries have adopted a definition of “rarity,” none have implemented DRD reimbursement eligibility criteria based on prevalence alone. Typically, the DRD must be indicated for a life-threatening or chronically debilitating condition for which there are no other treatment alternatives. Therefore, not all DRDs will meet these criteria. Internationally, healthcare systems have recognized that DRDs comprise treatments for complex, often severe, diseases with poor prognoses and limited treatment options. They do not represent salami-slicing, in which small sub-groups of patients within a more prevalent disease are identified and then classified as a DRD to gain access to special concessions, including pricing.

The third issue relates to evidence uncertainties, which are inherent in the area of rare diseases, and arise because of small patient number, heterogeneity of disease and, in many cases, limited understanding of the natural course of the disease (and, therefore, of relevant outcomes of an intervention). These uncertainties often challenge generally accepted methodological standards in health technology assessment and translate to increased risk in decision-making. It has been argued that the large uncertainties associated with DRDs have resulted in fewer positive funding decisions, when compared to non-DRDs. As demonstrated in this review, “safety net” programs have emerged as a means of providing coverage for DRDs that “fail” the standard CDR process. They may also include “managed access programs” (MAPs), whereby a DRD may be funded with the condition that an identified evidence gap

be filled through additional data collection. MAPs continue to generate significant interest from decision-makers in many countries and may be the way of the future for any new and innovative health technology which might appear very promising but requires definitive proof before widespread adoption.

Fourth, the per patient cost of most DRDs is extremely high. Numerous DRDs cost in excess of \$500,000 per patient per year, and there appears to be little indication that they will drop over time. Some governments have chosen to deal with “high-cost” technologies, regardless of their indication, as a specific class for review purposes. The argument often made is that as rare diseases affect small populations, the budget impact of a single DRD in a jurisdiction would be relatively small, despite the high individual price. However, it is anticipated that governments will soon need to contend with a long list of DRDs. Risk-sharing agreements with companies may be the only feasible alternative to ensure that there is appropriate control of DRDs when they are funded.

Conclusion

This review shows that DRDs are an issue for governments worldwide. Most continue to use pre-existing CDR processes, and when that fails, resort to “safety net” programs or modified decision criteria. When considering the experiences of other countries, it is important to keep in mind, differences in healthcare systems, which may limit their applicability to the Canadian context. However, international experience may yield some options in Canada for the development of a pan-Canadian approach to the funding of DRDs.

Limitations

This review relied on publicly available information. Therefore, it is possible that relevant information may have been missed. In addition, while the search for grey literature included non-English language documents, the search for published, peer-reviewed literature was limited to papers appearing in English. Translation software was used to convert non-English language articles to English. To the extent possible, policy experts from non-English-speaking countries were consulted to validate the information collected. However, it is possible that some information may have been missed.

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References

- Alberta Public Affairs Bureau. 2014. *Provinces and Territories Talk Health Care*. PR Newswire (Sep 30). Retrieved March 20, 2015. <<http://www.prnewswire.com/news-releases/provinces-and-territories-talk-health-care-277671231.html>>.
- Anell, A. and U. Persson. 2005. "Reimbursement and Clinical Guidance for Pharmaceuticals in Sweden: Do Health-Economic Evaluations Support Decision Making?" *The European Journal of Health Economics* 6(3): 274–79.
- Arthur Cox. 2013. *European Lawyer Conference: Distribution and Marketing of Drugs 2013 – Irish Chapter*. Retrieved March 20, 2015. <<http://www.arthurcox.com/publications/european-lawyer-reference-distribution-and-marketing-of-drugs-2013-irish-chapter/>>.
- Arthur Cox. 2012. *The IPHA Agreement 2012*. Retrieved March 20, 2015. <<http://www.arthurcox.com/updates/the-ipha-agreement-2012/>>.
- Australian Government Department of Health (AGDH). 2009. *The Review of the Life Saving Drugs Programme (LSDP)*. Retrieved March 20, 2015. <<http://www.health.gov.au/internet/ministers/publishing.nsf/Content/health-mediarel-yr2014-dutton030.htm>>.
- Australian Government Department of Health (AGDH). 2010a. *Application for Treatment through the Life Saving Drugs Programme (LSDP)*. Retrieved March 20, 2015. <<http://www.health.gov.au/LSDP>>.
- Australian Government Department of Health (AGDH). 2010b. *Australian Statistics on Medicine*. Retrieved March 20, 2015. <<http://www.pbs.gov.au/info/statistics/asm/asm-2010>>.
- Australian Government Department of Health (AGDH). 2011. *Administrative Requirements for Proposals to Fund a Drug through the Life Savings Drug Program*. Retrieved March 20, 2015. <<http://www.health.gov.au/internet/main/publishing.nsf/Content/lspd-criteria>>.
- Australian Government Department of Health (AGDH). 2013a. *Highly Specialised Drugs*. Canberra: Department of Human Services. Retrieved March 20, 2015. <<http://www.medicareaustralia.gov.au/provider/pbs/highly-specialised-drugs/>>.
- Australian Government Department of Health (AGDH). 2013b. *Life Saving Drugs Program Criteria and Conditions*. Retrieved March 20, 2015. <<http://www.health.gov.au/internet/main/publishing.nsf/Content/lspd-criteria>>.
- Australian Government Department of Health (AGDH). 2013c. *Obtaining Consumer Comments on Submissions to the Pharmaceutical Benefits Advisory Committee*. Retrieved March 20, 2015. <<http://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/pbac-consumer-comments>>.
- Australian Government Solicitor. 2013. *Deed of Agreement between the Commonwealth of Australia and [company] in Relation to the Provision of [drug] through the Life Saving Drugs Program: Template*. Retrieved March 20, 2015. <<http://www.health.gov.au/LSDP>>.
- Austrian Federation of Social Insurance Institutions. 2007. *Rules of Procedure for Publishing the Code of Reimbursement According to 351g ASVG*. Retrieved March 20, 2015. <<http://apps.who.int/medicinedocs/documents/s17144e/s17144e.pdf>>.
- All Wales Medicines Strategy Group (AWMSG). 2012a. *Members of the Patient Access Scheme Wales Group*. Retrieved March 20, 2015. <<http://www.awmsg.org/docs/awmsg/appraisaldocs/inforandforms/Patient%20Access%20Scheme%20Wales%20Group%20members.pdf>>.
- All Wales Medicines Strategy Group (AWMSG). 2012b. *Wales Patient Access Scheme (WPAS) Process Guidance*. Retrieved March 20, 2015. <<http://www.awmsg.org/docs/awmsg/appraisaldocs/inforandforms/Wales%20Patient%20A>>.

A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada? Insights from Across the Ponds

All Wales Medicines Strategy Group (AWMSG). 2013. *AWMSG Policy Relating to Ultra-Orphan Medicines*. Penarth: Author. Retrieved March 20, 2015. <<http://www.awmsg.org/docs/awmsg/appraisaldocs/inforandforms/AWMSG%20policy%20relating%20to%20ultra-orphan%20medicine.pdf.ccess%20Scheme%20process%20guidance.pdf>>.

All Wales Medicines Strategy Group (AWMSG). 2014. All Appraisal Documents. Penarth: Author. Retrieved March 20, 2015. <http://www.awmsg.org/industry_alldocs.html>.

Ayme, S. and C. Rodwell. 2012. *State of the Art of Rare Disease Activities in the Netherlands*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <http://www.eucerd.eu/?post_type=document&p=1513>.

Ayme, S. and C. Rodwell. 2013a. *2013 Report on the State of the Art of Rare Disease Activities in Europe. Part II: Key Developments in the Field of Rare Diseases in Europe in 2012*. Paris: European Union Committee of Experts on Rare Diseases (EUCERD). Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesII.pdf>>.

Ayme, S. and C. Rodwell. 2013b. *State of the Art of Rare Disease Activities in Austria. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesAT.pdf>>.

Ayme, S. and C. Rodwell. 2013c. *State of the Art of Rare Disease Activities in Finland. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesFI.pdf>>.

Ayme, S. and C. Rodwell. 2013d. *State of the Art of Rare Disease Activities in France. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesFR.pdf>>.

Ayme, S. and C. Rodwell. 2013e. *State of the Art of Rare Disease Activities in Germany. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesDE.pdf>>.

Ayme, S. and C. Rodwell. 2013f. *State of the Art of Rare Disease Activities in Italy. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesIT.pdf>>.

Ayme, S. and C. Rodwell. 2013g. *State of the Art of Rare Disease Activities in Luxembourg. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesLU.pdf>>.

Ayme, S. and C. Rodwell. 2013h. *State of the Art of Rare Disease Activities in Sweden. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesSE.pdf>>.

Ayme, S. and C. Rodwell. 2013i. *State of the Art of Rare Disease Activities in Switzerland. 2013 Report on the State of the Art of Rare Disease Activities in Europe*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesCH.pdf>>.

Ayme, S. and C. Rodwell. 2013j. *State of the Art of Rare Disease Activities in the United Kingdom*. Paris: EUCERD: European Union Committee of Experts on Rare Diseases. Retrieved March 20, 2015. <<http://www.eucerd.eu/upload/file/Reports/2013ReportStateofArtRDActivitiesUK.pdf>>.

- Bakowska, M., R. Berekmeri-Varro, H. Biro, S. Brandt, A. Bruyndonckx, and A. Casanueva et al. 2011. *Pricing and Reimbursement Handbook* (1st ed.). Baker & McKenzie. Retrieved March 20, 2015. <<http://www.bakermckenzie.com/files/Uploads/Documents/Germany/LifeSciences/EuropeanPricingReimbursement.pdf>>.
- Barham, L. 2012. *Orphan Medicines: Special Treatment Required?* London: 2020health.org. Retrieved March 20, 2015. <<http://www.2020health.org/2020health/Publications/publications-2012/Orphan-Drugs.html>>.
- Belogy, C. 2012. *The Compassionate Use of Medicinal Products. An Example: the French ATU System. Off Label Use in France*. Paris: Agence Française de Sécurité Sanitaire des Produits de Santé. Retrieved March 20, 2015. <<http://media.drugwonks.com/media/attachments/5007f6242017a84dfc000006.pdf?1342699044>>.
- The Best Practice Advocacy Centre New Zealand (BPAC). 2013a. "Accessing Funded Medicines in New Zealand." BPAC Magazine (21): 43–05. Retrieved March 20, 2015. <http://www.bpac.org.nz/magazine/2009/june/docs/bpj21_fundedmedicine_pages_43-45.pdf>.
- Blankart, C.R., T. Stargardt and J. Schreyogg. 2011. "Availability of and Access to Orphan Drugs: An International Comparison of Pharmaceutical Treatments for Pulmonary Arterial Hypertension, Fabry Disease, Hereditary Angioedema and Chronic Myeloid Leukaemia." *Pharmacoeconomics* 29(1): 63–82.
- Bogaert, P. and G. Klasa. 2009. *Belgium*. In *Cross-Border Handbooks* (pp. 121–32). Brussels: Practical Law Company.
- Bucholz, P. 2009. *Austria – Pharmaceutical*. *ISPOR Global Health Care Systems Road Map*. Lawrenceville (NJ): International Society for Pharmacoeconomics and Outcomes Research (ISPOR). <<http://www.ispor.org/htaroadmaps/Austria.asp>>.
- Caisse nationale de santé. 2003. *Union des caisses de maladie: Demande d'inscription d'un médicament sur la liste positive des médicaments pris en charge par l'assurance maladie au Grand Duché de Luxembourg*. Luxembourg: Caisse nationale de santé (CNS d'Gesondheetskeess). <<http://www.cns.lu/files/listepos/DemEnrMed.pdf>>.
- Caisse nationale de santé. 2011. *Dispositions législatives et statutaires*. Luxembourg: Caisse nationale de santé (CNS d'Gesondheetskeess). <<http://www.cns.lu/prestataires/?m=55-41-0&p=69>>.
- Chicoye, A., A. Chhabra, S. Holmstrom and N. Mehin. 2009. *ISPOR Global Health Care Systems Road Map: France – Pharmaceutical*. <<http://www.ispor.org/htaroadmaps/france.asp>>.
- Cote, A. and B. Keating. 2012. "What Is Wrong with Orphan Drug Policies?" *Value in Health: The Journal of the International Society for Pharmacoeconomics and Outcomes Research* 15(8): 1185–91. doi: 10.1016/j.jval.2012.09.004.
- Coughlan, JJ., D. Fortescue-Webb, R. Heaney, G. Judge, L. Tilso, M. Barry et al. 2009. *ISPOR Global Health Care Systems Road Map Ireland – Pharmaceutical*. <<http://www.ispor.org/HTARoadMaps/Ireland.asp>>.
- Danish Health and Medicines Authority. 2012. *Reimbursement of Medicines*. <<http://sundhedsstyrelsen.dk/en/medicines/reimbursement>>.
- Davidova, J., L. Mergaert, C. Fostier, I. Cleemput, F. Hulstaert and S. Simoens. 2008. "Pricing and Reimbursement of Pharmaceuticals in the Czech Republic and Sweden". *Pharmacy World and Science* 30(1): 57–64. doi:10.1007/s11096-007-9141-z.
- Denis, A., Mergaert, L., Fostier, C., Cleemput, I., Hulstaert, F. and Simoens, S. 2011. "Critical Assessment of Belgian Reimbursement Dossiers of Orphan Drugs". *Pharmacoeconomics* 29(10): 883–93. doi:<http://dx.doi.org/10.2165/11585980-000000000-00000>.
- Denis, A., S. Simoens, C. Fostier, L. Mergaert and I. Cleemput. 2009. *Policies for Orphan Diseases and Orphan Drugs*. KCE Reports 112C. Brussels: Belgian Health Care Knowledge Centre (KCE). <http://kce.fgov.be/sites/default/files/page_documents/d20091027332.pdf>.
- European Medicines Agency. 2014. *Orphan Designation*. <http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp>.
- Federal Ministry of Health. 2010. *Ordinance on the Placing on the Market of Unauthorized Medicinal Products for Compassionate Use*. Berlin, Germany. <http://www.bfarm.de/SharedDocs/Downloads/EN/Drugs/licensing/clinicalTrials/compUse/AMHV_en.pdf?__blob=publicationFile&v=2>.
- Federal Office of Public Health. 2013. *Frequently Asked Questions (FAQ) about Benefits*. Berne, Switzerland: Federal Office of Public Health. <<http://www.bag.admin.ch/themen/krankenversicherung/00263/00264/?lang=en>>.

A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada? Insights from Across the Ponds

- Festoy, H., L. M. Yu, T. Aanes, A.H. Ognoy and T. Gregersen. 2011. "PHIS Pharma Profile: Norway (Version 1 ed.)." Pharmaceutical Health Information System (PHIS). <<http://www.legemiddelverket.no/English/the-norwegian-health-care-system-and-pharmaceutical-system/Documents/PHIS%20Pharma%20profile.pdf>; http://www.stm.fi/c/document_library/get_file?folderId=71804&name=DLFE-26224.pdf>.
- Finland Ministry of Social Affairs and Health. 2013. *Pharmaceuticals Pricing Board*. <<http://www.stm.fi/en/ministry/boards/pharmaboard/frontpage>>.
- Folino-Gallo, P., S. Montilla, M. Bruzzone and N. Martini. 2008. "Pricing and Reimbursement of Pharmaceuticals in Italy". *European Journal of Health Economics* 9(3): 305–10.
- Fulda, C.B. 2011. "A revolution of Reimbursement in Germany." Jones Day Publications. <http://www.jonesday.com/revolution_of_reimbursement/>.
- Garau, M. and J. Mestre-Ferrandiz. 2009. "Access Mechanisms for Orphan Drugs: A Comparative Study of Selected European Countries". OHE Briefing; No. 52. London: Office of Health Economics. <<http://www.ohe.org/publications/article/access-mechanisms-for-orphan-drugs-26.cfm>>.
- German Federal Ministry of Health. 2010. "Ordinance on the Placing on the Market of Unauthorised Medicinal Products for Compassionate Use". <https://www.bundesgesundheitsministerium.de/fileadmin/dateien/Downloads/Gesetze_und_Verordnungen/GuV/M/Medicinal_Products_for_Compassionate_Use_14_07_2010.pdf>.
- Goodman, L.A. 2014, October 5. *Call Growing Louder for National Prescription Drug Plan*. *Globe and Mail*. <<http://www.theglobeandmail.com/news/politics/call-growing-louder-for-national-prescription-drug-plan/article20937357/>>.
- Government of the Netherlands: Ministry of Health, Welfare and Sport, W. a. S. 2013. *Strategy of the Netherlands in the Field of Rare Diseases*. <<http://www.government.nl/documents-and-publications/reports/2013/01/24/strategy-of-the-netherlands-in-the-field-of-rare-diseases.html>>.
- Gøthesen, O., J. Slover, L. Havelin, J.E. Askildsen, H. Malchau and O. Furnes. 2013. "An Economic Model to Evaluate Cost-Effectiveness of Computer Assisted Knee Replacement Surgery in Norway". *BMC Musculoskeletal Disorders* 14(202). <<http://www.biomedcentral.com/1471-2474/14/202>>.
- Guillaume, P., I. Moldenaers, S. Bulte, H. Debruyne, S. Devriese, L. Kohn et al. 2010. *Optimisation of the Operational Processes of the Special Solidarity Fund*. (KCE Report No. 133C). Retrieved from the Belgian Health Care Knowledge Centre website: <https://kce.fgov.be/sites/default/files/page_documents/kce_133c_special_solidarity_fund.pdf>.
- Haga, A. 2007. "Priority Decisions – The Authority Challenge". *ISPOR Connections: The Official News & Technical Journal of the International Society for Pharmacoeconomics and Outcomes Research*. <<http://www.ispor.org/news/articles/april07/authority.asp>>.
- Haga, A. and J.M. Sverre. 2002. "Pricing and Reimbursement of Pharmaceuticals in Norway". *European Journal of Health Economics* 3(3): 215–20.
- Hallinen, T. and E.J. Soini. 2011. "The Impact of the Pharmaceutical Pricing System on Cost-Effectiveness Results: Finnish Analysis". *Open Pharmacoeconomics & Health Economics Journal* 3, 6–10. <<http://benthamopen.com/contents/pdf/TOPHARMEJ/TOPHARMEJ-3-6.pdf>>.
- Health Service Executive. 2006. *National Shared Services Primary Care Reimbursement Service: Information and Administrative Arrangements for Pharmacists*. Dublin: Ireland. National Shared Services – Primary Care Reimbursement Service. <http://www.hse.ie/eng/Staff/PCRS/Contractor_Handbooks/PCRS_Handbook_for_Pharmacists.pdf>.
- Heron, L., S. Laursen, J. Costello, C. Anderl and J. Knosp. 2012. "Gaining Reimbursement of Orphan Products in Europe: Challenges Due to Wide Variations in Evidence Requirements and Processes [abstract]". *Value in Health* 15(7): A308. doi:<http://dx.doi.org/10.1016/j.jval.2012.08.652>. <http://www.santen.eu/eu/news/Documents/ISPOR_Reimbursement%20of%20Orphan%20Products%20in%20EU_Poster_fer_final.pdf>.
- Holtorf, AP, K. Matuszewski, M. Nuijten and C. Vauth. 2009. *ISPOR Global Health Care Systems Road Map, Germany – Pharmaceutical*. <<http://www.ispor.org/htaroadmaps/Germany.asp>>.
- Icelandic Medicine Pricing and Reimbursement Committee. 2011a. *Icelandic Medicine Pricing and Reimbursement Committee*. <http://www.lgn.is/gogn/icelandic_medicine_pricing_and_reimbursement_committee_english_may_2014.pdf>.

- Icelandic Medicine Pricing and Reimbursement Committee. 2011b. *Icelandic Medicine Pricing and Reimbursement Committee: Flow Chart – Pharmaceutical System in Iceland in the In- and Out-Patient Sector* [poster]. <http://whocc.goeg.at/Literaturliste/Dokumente/CountryInformationPosters/IS_PHIS_poster_Rome.pdf>.
- Icelandic Medicine Pricing and Reimbursement Committee. 2013. *Forms*. <<http://www.lgn.is/?pageid=5>>.
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR). 2014. *Pharmacoeconomic Guidelines around the World: Finland*. <<http://www.ispor.org/peguidelines/index.asp>>.
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR). 2007. *ISPOR Global Health Care Systems Road Map – the Netherlands – Reimbursement Process*. <<http://www.ispor.org/htaroadmaps/netherlands.asp>>.
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR). 2011. *Switzerland – Pharmaceuticals*. <<http://www.ispor.org/htaroadmaps/SwitzerlandPH.asp>>.
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR). 2013. *Norwegian Guidelines for Pharmacoeconomic Analysis in Connection with Applications for Reimbursement*. <http://www.ispor.org/peguidelines/source/Norwegian_guidelines.pdf>.
- Kela. 2013. *How to Appeal Against a Decision*. <<http://www.kela.fi/web/en/how-to-appeal-a-decision7>>.
- Liu, G.G., T. Fukuda, C. Lee, V. Chen, Z. Qiang and I. Kamae. 2009. "Evidence-Based Decision-Making on Medical Technologies in China, Japan, and Singapore". *Value in Health* 12(Suppl 3): S12–17.
- Mabbott, V., M. Robinson, A. Segrave and Q. Brennan. 2012. "Australian Statistics on Medicines". Canberra: Australian Government Department of Health and Ageing. <<http://www.pbs.gov.au/statistics/asm-2010>>.
- Martikainen, J. and S. Rajaniemi. 2002. *Drug Reimbursement Systems in EU Member States, Iceland and Norway: Social Security and Health (Report No.54)*. Helsinki: The Social Insurance Institution. <https://helda.helsinki.fi/bitstream/handle/10138/13932/Drug_reimbursement.pdf?sequence=1>.
- Ministère des Affaires Sociales et de la Santé. 2014. *Autorisation temporaire d'utilisation (ATU)*. <<http://www.sante.gouv.fr/autorisation-temporaire-d-utilisation-atu.html>>.
- Ministry of Welfare. 2011. *Icelandic Medicine Pricing and Reimbursement Committee*. Reykjavik, Iceland. <www.lgn.is/index.php?pageid=62>.
- Ministry of Health, Welfare and Sport. 2013. *Strategy of the Netherlands in the Field of Rare Diseases*. <<http://www.government.nl/documents-and-publications/reports/2013/01/24/strategy-of-the-netherlands-in-the-field-of-rare-diseases.html>>.
- Moller, P.K. 2003. "Pricing and Reimbursement of Drugs in Denmark". *European Journal of Health Economics* 4(1): 60–65.
- Morel, T., F. Arickx, G. Befrits, P. Siviero, C. van der Meijden, E. Xoxi et al. 2013. "Reconciling Uncertainty of Costs and Outcomes with the Need for Access to Orphan Medicinal Products: A Comparative Study of Managed Entry Agreements across Seven European Countries". *Orphanet Journal of Rare Diseases* 8:198. <<http://www.ojrd.com/content/8/1/198>>.
- Morgan, S.G., M. McMahon, C. Mitton, E. Roughead, R. Kirk, P. Kanavos and D. Menon. 2006. "Centralized Drug Review Processes in Australia, Canada, New Zealand, and the United Kingdom". *Health Affairs (Millwood)* 25(2): 337–47. <<http://content.healthaffairs.org/content/25/2/337.long>>.
- Mossialos, E. and D. Srivastava. 2008. *Pharmaceutical Policies in Finland: Challenges and Opportunities (Observatory studies series No. 10)*. Copenhagen: European Observatory on Health Systems and Policies/Finland Ministry of Social Affairs and Health. <http://www.euro.who.int/__data/assets/pdf_file/0020/80651/E91239.pdf>.
- Moise, P. and E. Docteur. 2007. *Pharmaceutical Pricing and Reimbursement Policies in Sweden (OECD Health Working Papers No. 28)*. Paris: Organisation for Economic Co-operation and Development (OECD). <<http://www.oecd.org/sweden/40699881.pdf>>.
- Nagae, T. 2012. *The Payer/Reimbursement Environment in Japan: Present and Future*. Global Forum Inform DIA. <<http://www.diahome.org/~media/15404CBD3C934077AD32285E2D58DC14.ashx>>.
- Natz, A. and M.G. Campion. 2012. "Pricing and Reimbursement of Innovative Pharmaceuticals in France and the New Healthcare Reform". *Farmeconomia* 13(2): 47–58. <<http://www.natz-law.com/en/wp-content/uploads/sites/3/2013/04/Natz-Campion-PR-France.pdf>>.

A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada? Insights from Across the Ponds

National Centre for Pharmacoeconomics. 2010. *Guidelines for the Economic Evaluation of Health Technologies in Ireland*. Version 1.3. Dublin. <<http://www.hiqa.ie/search/node/guidelines%20for%20economic%20evaluation%20of%20health%20technologies>>.

National Centre for Pharmacoeconomics. 2013a. *Process Flowchart*. Dublin: National Centre for Pharmacoeconomics (NCPE). <<http://www.ncpe.ie/submission-process/process-flochart/>>.

National Centre for Pharmacoeconomic. 2013b. *Guidance on the Reporting Format and Layout of Pharmacoeconomic Submissions to the National Centre for Pharmacoeconomics (Version 1.3 ed.)*. <<http://www.ncpe.ie/wp-content/uploads/2013/02/Submission-template-v1-3-01-10-2013-3.pdf>>.

Ngorsuraches, S., W. Meng, B.Y. Kim and V. Kulsomboon. 2012. "Drug Reimbursement Decision-Making in Thailand, China, and South Korea". *Value in Health: the Journal of the International Society for Pharmacoeconomics and Outcomes Research* 15(1 Suppl): S120–S125.

NICE. 2009a. *Consultation on Proposed 'Innovation Pass'*. London: National Prescribing Centre (NPC). <<http://www.npc.nhs.uk/rapidreview/?p=898%20-%20sthash.PCyTfguA.dpuf>>.

NICE. 2009b. "Process for Advising on the Feasibility of Implementing a Patient Access Scheme". Interim. London: National Institute for Health and Care Excellence (NICE). Patient Access Schemes Liaison Unit <<http://www.nice.org.uk/Media/Default/About/what-we-do/PASLU/PASLU-process-guide.pdf>>.

NICE. 2013a. *Developing NICE Highly Specialised Technologies*. London: National Institute for Health and Care Excellence. <<http://www.nice.org.uk/proxy/?sourceurl=http://www.nice.org.uk/aboutnice/howwework/devhst/developingnicehighlyspecialisedtechnologies.jsp>>.

NICE. 2013b. *Highly Specialised Technologies Interim Methods and Process Statements*. <<http://www.nice.org.uk/media/4AF/0F/HighlySpecialisedTechnologiesInterimMethodsAndProcessStatements.pdf>>.

NICE. 2013c. *NICE and the NHS*. <http://www.nice.org.uk/aboutnice/whatwedo/niceandthenhs/nice_and_the_nhs.jsp>.

NICE. 2013d. *Patient Access Schemes Liaison Unit*. London: National Institute for Health and Care Excellence (NICE). <<http://www.nice.org.uk/about/what-we-do/patient-access-schemes-liaison-unit>>.

NICE. 2013e. "Patient Access Schemes Liaison Unit". *PASLU Proposal Template Standard Scheme*. London: National Institute for Health and Care Excellence (NICE). <<http://www.nice.org.uk/about/what-we-do/patient-access-schemes-liaison-unit>>.

NICE. 2013f. Specification for Manufacturer/Sponsor Submission of Evidence. <<https://www.nice.org.uk/proxy/?sourceUrl=http%3a%2f%2fwww.nice.org.uk%2faboutnice%2fhowwework%2fdevnicetech%2fspecificationformanufacturersponsorssubmissionofevidence.jsp%3fd0media%3d1%26mid%3d97E2DC44-19B9-E0B5-D4F73F1D-B153AA86>>.

NICE. 2014a. *Guide to the Technology Appraisal and Highly Specialised Technologies Appeal Process*. <<http://www.nice.org.uk/article/PMG18/chapter/Foreword>>.

NICE. 2014b. *Highly Specialised Technologies Evaluation Committee*. <<http://www.nice.org.uk/Get-Involved/Meetings-in-public/Highly-Specialised-Technologies-Evaluation-Committee>>.

NICE. 2014c. *NICE Technology Appraisal Guidance*. <<http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-technology-appraisal-guidance>>.

NICE. 2014d. *Technology Appraisal and Highly Specialised Technologies Appeals*. <<http://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-technology-appraisal-guidance/Technology-appraisal-and-Highly-specialised-technologies-appeals>>.

Niezen, M., A. de Bont, E. Stolk, A. Eyck, L. Niessen and H. Niessen. 2007. "Conditional Reimbursement within the Dutch Drug Policy". *Health Policy* 84(1): 39–50. doi:doi:10.1016/j.healthpol.2006.11.005.

Norwegian Medicines Agency. 2013. *About Norwegian Medicines Agency*. Oslo: Statens Legemiddelverk / Norwegian Medicines Agency. <<http://www.legemiddelverket.no/English/about-norwegian-medicines-agency/Sider/default.aspx>>.

NZORD. 2013. "A Definition of Rare Disorders". <http://www.nzord.org.nz/introduction/a_definition_of_rare_disorders>.

- Office Fédéral de la Santé. 2013. *Instructions concernant la liste des spécialités*. Berne, Switzerland. <<http://www.bag.admin.ch/themen/krankenversicherung/06492/07568/?lang=fr>>.
- Office of the Attorney General. 2004. Health Act, 2004. Irish Statute Book. Dublin: Ireland. Office of the Attorney General. <<http://www.irishstatutebook.ie/2004/en/act/pub/0042/index.html>>.
- Orphanet. 2009, September 9. *Spotlight on ... The Norwegian Way ... to Managing Rare Disorders*. OrphaNews Europe: Newsletter of the European Union Committee of Experts on Rare Diseases. <<http://www.orpha.net/actor/EuropaNews/2009/090930.html>>.
- Orphanet. 2014. *Orphan Drugs in Japan*. <http://www.orpha.net/consor/cgi-bin/Education_AboutOrphanDrugs.php?lng=EN&stapage=ST_EDUCATION_EDUCATION_ABOUTORPHANDRUGS_JAP>.
- OrphaNews Europe. 2009. *Spotlight on ... The Norwegian Way ... to Managing Rare Disorders*. <<http://www.orpha.net/actor/EuropaNews/2009/090930.html>>.
- Paris, V. and E. Docteur. 2007. *Pharmaceutical Pricing and Reimbursement Policies in Switzerland (OECD Health Working Papers No. 27)*. Paris: Organisation for Economic Co-operation and Development (OECD). <http://www.oecd-ilibrary.org/social-issues-migration-health/pharmaceutical-pricing-and-reimbursement-policies-in-switzerland_136157357151>.
- PBAC. 2013a. *The Listing Steps: A Step-by-Step Guide to Listing on the PBS*. Canberra, Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/info/industry/listing/listing-steps>>.
- PBAC. 2013b. *The Listing Steps. Step 5: Communication of PBAC Outcomes*. Canberra: Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/info/industry/listing/listing-steps/e-communication-pbac-outcomes>>.
- PBS. 2013c. *Obtaining Consumer Comments on Submissions to the Pharmaceutical Benefits Advisory Committee meetings*. Canberra: Australian: Department of Health, Pharmaceutical Benefits Scheme (PBS). <<http://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/pbac-consumer-comments>>.
- PBAC. 2008a. *Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee*. Version 4.3. Canberra, Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/pbs/industry/listing/elements/pbac-guidelines>>.
- PBAC. 2008b. "Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee". Part 1. Section 1: Role of Pharmaceutical Benefits Advisory Committee. Part 1.4 Processing Submissions. Version 4.3. Canberra, Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.ispor.org/peguidelines/source/Australia-Guidelines-for-preparing-submissions-to-the-Pharmaceutical-Benefits-Advisory-Committee-2008.pdfPharmaceutical>>.
- PBAC. 2008c. "Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee". Part 1. Section 3: Processing Submissions. Version 4.3. Canberra, Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/info/industry/listing/elements/pbac-guidelines/a-part-1/section-1>>.
- PBAC. 2008d. "Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee". Part 2. Section F: Options to Present Additional Relevant Information (Version 4.4 ed.). Canberra, Australia: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbac.pbs.gov.au/content/information/printable-files/pbacg-book.pdf>>.
- Pelen, F. 2000. "Reimbursement and Pricing of Drugs in France: An Increasingly Complex System". *HEPAC (Health Economics in Prevention and Care)* 0(0): 20–23.
- PHARMAC. 2010. *Guidelines for Funding Applications to PHARMAC* (Amended in 2015 ed.). <<http://pharmac.govt.nz/2010/02/11/Guidelines%20for%20Suppliers%20Submissions.pdf>>.
- PHARMAC. 2011a. *Named Patient Pharmaceutical Assessment (Exceptional Circumstances) Policy*. <<http://www.pharmac.health.nz/tools-resources/forms/named-patient-pharmaceutical-assessment-nppa-forms>>.
- PHARMAC. 2011b. *Switzerland – Pharmaceuticals*. <<http://www.ispor.org/htaroadmaps/SwitzerlandPH.asp>>.
- PHARMAC. 2012. *Named Patient Pharmaceutical Assessment (NPPA): Questions and Answers for Clinicians*. <http://www.pharmac.health.nz/ckeditor_assets/attachments/209/nppa_clinician_info_sheet.pdf>.
- PHARMAC. 2013a. *Access to Treatments That Aren't on the Lists (NPPA)*. <<http://www.pharmac.health.nz/tools-resources/forms/named-patient-pharmaceutical-assessment-nppa-forms>>.

A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada? Insights from Across the Ponds

- PHARMAC. 2013b. *Norwegian Guidelines for Pharmacoeconomic Analysis in Connection with Applications for Reimbursement*. http://www.ispor.org/peguidelines/source/Norwegian_guidelines.pdf.
- PHARMAC. 2013c. *Access to Treatments That Aren't on the Lists (NPPA)*. <<http://www.pharmac.health.nz/tools-resources/forms/named-patient-pharmaceutical-assessment-nppa-forms>>.
- PHARMAC. 2013d. *Medicinal Products and Health Care in Austria: Facts & Figures 2013*. [Vienna]: PHARMIG. <http://www.pharmig.at/uploads/Daten_Fakten_engl_13_9546_DE.pdf>.
- Pharmaceutical Benefits Advisory Committee. 2008a. *Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee (Version 4.3 ed.)*. Canberra: Australia. Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/pbs/industry/listing/elements/pbac-guidelines>>.
- Pharmaceutical Benefits Advisory Committee. 2008b. *Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee*. Part 1. Section 1: Role of the Pharmaceutical Benefits Advisory Committee (Version 4.3 ed.). Canberra: Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/info/industry/listing/elements/pbac-guidelines/a-part-1>>.
- Pharmaceutical Benefits Advisory Committee. 2008c. *Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee*. Part 1. Section 3: Types of submissions (Version 4.3 ed.). Canberra: Australia. Pharmaceutical Benefits Advisory Committee (PBAC). <<http://www.pbs.gov.au/info/industry/listing/elements/pbac-guidelines/a-part-1/section-3>>.
- Pharmaceuticals Pricing Board. 2011. *Application for Reimbursement Status and Wholesale Price for a Medicinal Product Subject to Special Licence*. Helsinki, Finland. <http://www.stm.fi/c/document_library/get_file?folderId=71804&name=DLFE-26224.pdf>.
- Pharmaceuticals Pricing Board. 2013. *Application for Reimbursement Status and Wholesale Price for a Medicinal Product Subject to Marketing Authorization*. Form ML 1.1.2013. Helsinki, Finland. <http://www.stm.fi/c/document_library/get_file?folderId=6809157&name=DLFE-25829.pdf>.
- Scottish Medicines Consortium. 2007. *Orphan Drugs*. <http://www.scottishmedicines.org.uk/About_SMC/Policy_statements/Orphan_Drugs>.
- Scottish Medicines Consortium. 2013a. *Patient Access Scheme Assessment Group (PASAG)*. Industry Guidance (Version 8 ed.). <http://www.scottishmedicines.org.uk/files/PAS/PASAG_Industry_Guidance_v8_0813.pdf>.
- Scottish Medicines Consortium. 2013b. *Templates/Guidance for Submission*. <http://www.scottishmedicines.org.uk/Submission_Process/Submission_Guidance_and_Templates_for_Industry/Templates-Guidance-for-Submission/Templates-Guidance-for-Submission>.
- Scottish Medicines Consortium. 2014a. *Meeting Registration*. <http://www.scottishmedicines.org.uk/About_SMC/Meetings_Minutes/meetingRegistration?iru=M3I5CMJJRF>.
- Scottish Medicines Consortium. 2014b. *PACE – Patient and Clinician Engagement*. <https://www.scottishmedicines.org.uk/files/PACE/PACE_factsheet_FINAL.pdf>.
- Scottish Medicines Consortium. 2014c. *PACE – Patient and Clinical Engagement Overview Document*. <http://www.scottishmedicines.org.uk/files/new_medicines_review/PACE_Overview_Document_May_2014.pdf>.
- Seoane-Vazquez, E., H. Middlehoven, J.M. Barrios and S. Szeinbach. 2009. *ISPOR: Global Health Care Systems Road Map – Spain – Pharmaceutical*. <<http://www.ispor.org/htaroadmaps/spain.asp>>.
- Simoens, S. 2011. *Pricing and Reimbursement of Orphan Drugs: the Need for More Transparency*. *Orphanet Journal of Rare Diseases* 6:42. <<http://www.ojrd.com/content/6/1/42>>.
- Stafinski, T., D. Menon, D. Philippon and C. McCabe. 2011. "Health Technology Funding Decision-Making Processes Around the World: the Same Yet Different". *Pharmacoeconomics* 29(6): 475–95.
- State Government of Victoria. 2013. *Commonwealth/State Highly Specialised Drugs Program Guidelines*. <<http://www.health.vic.gov.au/hsdp/>>.
- Stolk, E.A., A. de Bont, A. van Halteren, R.J., Bijlmer and M.J. Poley. 2009. "Role of Health Technology Assessment in Shaping the Benefits Package in the Netherlands". *Expert Review of Pharmacoeconomics and Outcomes Research* 9(1): 85–94. <<http://informahealthcare.com/doi/pdf/10.1586/14737167.9.1.85>>.
- Office fédéral de la santé publique. 2013. *Instructions concernant la liste des spécialités (LS)*. Berne: Switzerland. <<http://www.bag.admin.ch/themen/krankenversicherung/06492/07568/index.html?lang=fr>>.

Taruscio, D., F. Capozzoli and C. Frank. 2011. "Rare Diseases and Orphan Drugs". *Annali dell'Istituto Superiore di Sanità* 47(1): 83–93. <<http://www.iss.it/publ/anna/2011/1/47183.pdf>>.

TLV. 2012a. *Guide for Companies When Applying for Subsidies and Pricing for Pharmaceutical Products (Version 2.0 ed.)*. Stockholm: Tandvards-Och Lakemedelsformansverket (TLV). <<http://www.tlv.se/Upload/English/ENG-guide-for-companies.pdf>>.

TLV. 2012b. *Pricing and Reimbursement of Medicines*. Stockholm: Tandvards-Och Lakemedelsformansverket (TLV). <<http://www.tlv.se/In-English/medicines-new/pricing-and-reimbursement-of-medicines/>>.

UK Department of Health. 2009. *A Consultation on Proposals for an Innovation Pass Pilot*. <http://webarchive.nationalarchives.gov.uk/+/www.dh.gov.uk/en/Consultations/Closedconsultations/DH_109236?PageOperation=email>.

United States Food and Drug Administration. 2014. *Developing Products for Rare Diseases & Conditions*. Silver Spring: Office of Orphan Products Development. <<http://www.fda.gov/forindustry/DevelopingProductsforRareDiseasesConditions/default.htm>>.

University of Alberta. School of Public Health. Health Technology & Policy Unit. 2014. *PRISM: Promoting Rare-Disease Innovations through Sustainable Mechanisms*. <www.prismfive.org>.

von Stokar, T., J. Fliedner and A. Vettori. 2013. *Equal and Secured Access to Cancer Drugs for Off-Label Use: Summary of the Final Report*. Berne, Switzerland: Swiss Cancer League. <http://assets.krebsliga.ch/downloads/130527_summary_off_label_use_infras_e.pdf>.

Weeks, C. 2014. *Canada Lags Behind on Rare Disease Drugs*. *Globe & Mail* (Feb 27). <<http://www.theglobeandmail.com/life/health-and-fitness/health/canada-lags-behind-on-rare-disease-drugs/article17137268/>>.

Whitfield, K., K.H. Huemer, D. Winter, S. Thirstrup, C. Libersa, B. Barraud, et al. 2010. *Compassionate Use of Interventions: Results of a European Clinical Research Infrastructures Network (ECRIN) Survey of Ten European Countries*. *Trials*, 11, 104. <<http://www.trialsjournal.com/content/11/1/104>>.



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Estimating Nursing Wage Bill in Canada and Breaking Down the Growth Rate: 2000 to 2010

Estimation de la masse salariale de la main-d'œuvre infirmière au Canada et ventilation du taux de croissance : de 2000 à 2010



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Abstract

Even though the nursing professional category (registered nurses [RNs] and licensed practical nurses) made up about one-third of the Canadian health professionals, no study exists about their wage bill, the composition and growth rate of this wage bill. This paper attempts to fill this gap by estimating the nursing wage bill in the Canadian provinces and breaking down the growth rate for the 2000–2010 period, using the 2001 Census and the 2011 National Household Survey. Total wage bill for the nursing professional category in Canada was estimated at \$20.1 billion (\$17.3 billion for RNs), which suggests that it is as substantial as net physician remuneration. The average annual growth rate of this wage bill was 6.6% for RNs. This increase was mainly driven by real (inflation-adjusted) wage per hour, which was 3.0%, suggesting the existence of a “health premium” of 1.7 percentage points during the study period.

Résumé

Bien que la catégorie des infirmières professionnelles (infirmières autorisées et infirmières auxiliaires autorisées) représente près du tiers des professionnels de la santé au Canada, il n'existe aucune étude sur la masse salariale de ce groupe, ni sur la composition ou le taux de croissance de cette masse salariale. Cet article tente de combler cette lacune en estimant la masse salariale de la main-d'œuvre infirmière selon les provinces du Canada et en ventilant le taux de croissance pour la période entre 2000 et 2010 au moyen du Recensement de 2001 et de l'Enquête nationale auprès des ménages de 2011. Le total de la masse salariale de la main-d'œuvre infirmière au Canada était estimé à 20,1 milliards de dollars (17,3 milliards pour les infirmières autorisées), ce qui laisse entendre qu'elle est aussi importante que la rémunération nette des médecins. Le taux de croissance annuel de cette masse salariale était de 6,6 % pour les infirmières autorisées. Cette croissance était principalement motivée par le salaire horaire réel (corrigeé selon l'inflation), qui était de 3,0 %, ce qui suggère l'existence d'une « prime de santé » de l'ordre de 1,7 points de pourcentage au cours de la période de l'étude.

Introduction

During the past 10 years, healthcare spending growth has gained increasing attention by policy makers in Canada and other industrialized countries because of its rising trends (CIHI 2010; Hartwig and Sturm 2014). Given that health is a labour-intensive sector, compensation is expected to be an important component of this trend. While physician compensation has been extensively studied (Ariste forthcoming; Barer and Evans 1983; CIHI 2006, 2010, 2014; Contandriopoulos and Perroux 2013), it is not the case for nurses, in spite of the fact that they made up about one-third of the Canadian health professionals. In fact, nurses are an important entity in the Canadian healthcare system. They include essentially registered nurses (RNs, including registered psychiatric nurses in the four Western provinces) and licensed practical nurses (LPNs – the applicable term in Ontario is RPNs: registered practical nurses)¹ and were 354,910 in 2010 in Canada (CIHI 2011). They typically work in hospitals, long-term care facilities and clinics and are paid mainly from public funds. Numerous studies have been done on the number of nurses, their characteristics, employment status, hours worked as well as on retention factors (Baumann et al. 2010; O'Brien-Pallas et al. 2004; Zeytinoglu et al. 2006). Also, some studies on nurses' wages exist (Buhr 2006; Vujicic 2003). However, to our knowledge, there is no study that looks at the level of, trend in and decomposition of wage bills for nurses in Canada. The present paper uses the Census and the National Household Survey (NHS) from Statistics Canada to address this gap in the literature. Therefore, the objective of this study is to estimate the nursing wage bill in 2010, to examine the trends during the 2000–2010 period and to better understand the different components of this wage bill growth.

Being able to distinguish between the effects of unit cost (wage per hour) and magnitude of volume on nursing wage bill can help policy makers and health system administrators to

better understand the costs of providing healthcare in Canada. In fact, the policy implication of a growing wage bill is different depending on which component of the bill is growing. If the wage per hour is the main cost driver, this means that we are paying more, but not necessarily getting more nursing throughput (more nurses and/or more worked hours). On the other hand, if the volume or throughput is the main driver, this might not be a bad thing because we are potentially getting more services. The following key questions are addressed:

- What is the total wage bill for RNs and LPNs in Canada in 2010?
- What is the growth rate of this wage bill during the past decade and which component was mainly responsible for this growth rate?

This study estimates growth in average hourly wage per hour for nurses and compares it with that in the quantity of nurse labour (number of nurses and average number of hours worked) on one hand. On the other hand, growth in average hourly wage per hour for nurses is also compared with that in average hourly wage per hour for other workers. This allows us to assess whether (1) wage per hour was the main cost driver of the nursing wage bill and (2) the nursing sector has benefited from a wage premium compared to the other sectors of the economy. These policy issues are not studied in the Canadian literature. Hence, this paper attempts to fill this gap. The rest of the paper is organized as follows: The second section presents the data and methodology. The results are presented and interpreted in the third section, followed by the conclusion in the fourth section.

Data and Methodology

Data sources include the 2001 Census and the 2011 NHS from Statistics Canada (2003, 2011, 2013). The Census included a short- and a long-form questionnaire, both mandatory before 2011. However, the long-form questionnaire has been made optional, via the 2011 NHS. The Census and NHS data contain detailed information on average annual income of personnel from different professional categories, including nurses.

Moreover, the regulated nursing occupational category has not changed between the 2001 and 2011 surveys. It refers to three nursing groups: head nurses and supervisors (D111), RNs (D112) and LPNs (D233).² Besides, NHS survey respondents were also given the choice to give Statistics Canada permission to use income information available in their income tax files instead of answering the multiple-part income question. Almost three-quarters (73.2%) of respondents to the NHS gave permission to use income information available on their income tax files. The item response rate for total wages and salaries after retrieval of tax data was 59.2% in the 2011 NHS, compared to 79.3% in the 2006 Census (Statistics Canada 2011). The average annual income was finally determined based on both self-reported³ and linked tax data.⁴

In spite of the fact that response rate for income was generally lower in the NHS compared to the Census, the quality of the income estimates in the 2011 NHS is deemed at least

as substantial as the 2001 Census, where response rate was higher but permission to use the income information available in income tax files was not asked. Consequently, all the 2001 income data were self-reported. Overall, income estimates for the nursing occupational category from the 2011 NHS remain comparable to those of the 2001 Census.

Total nursing wage bill can be defined as average income per nurse times the number of nurses:

$$\text{Tot Wage Bill} = \frac{\text{Income}}{\text{Nurse}} * \text{Nbr of Nurses} \quad (1)$$

From a public policy perspective, it is more insightful to further break down total annual wage bill, as we can more fully understand the factors influencing changes in the estimated spending on nurses. Average annual income per nurse can be decomposed into wage per hour and number of hours worked in one year:

$$\text{Tot Wage Bill} = \frac{\text{Wage}}{\text{Hr}} * \text{Nbr of Hours} * 52 * \text{Nbr of Nurses} \quad (2)$$

The term “Nbr of Hours” refers to *average weekly hours worked per nurse*. Actual hours were used.⁵ Both full-time and part-time nurses were included. Hourly and/or weekly wages are calculated together with the working hours per week.

Equation (2) can be further divided to also take into account the population:

$$\text{Tot Wage Bill} = \frac{\text{Wage}}{\text{Hr}} * \text{Nbr of Hours} * 52 * \frac{\text{Nbr of Nurses}}{\text{Pop}} * \text{Pop} \quad (3)$$

Total wage bill is expressed as the product of wage per hour times the number of hours worked per year per nurse times the number of nurses per habitant times the number of habitants. Using the natural logarithms of the terms in equation (3) permits to approximate the annual rate of change in total wage bill as the sum of the annual rates of change in wage per hour, number of hours worked and number of nurses per thousand and population. Note that this nominal (without adjusting for inflation) annual rate of change in wage per hour has been further decomposed into change in general inflation and change in real (after adjusting for inflation) wage per hour. The general inflation rate was calculated using the Consumer Price Index. This adds a fifth component in the breakdown of the total wage bill, which gives a framework relatively similar to the one used in the Canadian Institute for Health Information (CIHI 2010) healthcare cost driver study, except for the population aging factor, which is not included in the framework of this study.⁶

Total compensation is estimated by the product of the average annual employment income and the total number of workers in each of the three nursing groups. The overall total compensation is the sum of the three nursing groups. A weighted average hourly wage is also estimated for these groups. Then, total compensation growth is broken down into general inflation, population growth and growth in wage per hour above and beyond general inflation,

in number of weekly hours worked and in number of nurses per thousand. The first two components (general inflation and population growth) are macroeconomic factors not amenable to policy change within the health sector. Note that the quantity of services, which is a key factor in assessing cost drivers, is not explicitly measured in this paper. It is proxied by the quantity of labour (number of nurses per thousand population and average number of weekly hours), by implicitly assuming that more throughput turns into more services. Finally, it should be noted that the data elements from the NHS and the Census were obtained via a custom-run request to Statistics Canada, and not directly via the public-use microfile of these surveys.

For triangulation purposes, the administrative Nursing Database (NDB) from the CIHI has been used for the count of nurses. Besides, the Labour Force Survey (LFS) from Statistics Canada and the Canadian Federation of Nurses Union document (CFNU 2011a) have been used to compare average hourly wage of nursing professionals. Also, the Survey of Employment, Payroll and Hours (SEPH), an administrative and industry-based data source from Statistics Canada, has been used to compare average hourly wage of all workers. Wherever possible, estimated total wage bill was compared to figures from provincial health ministries.

Results

Level of wage bill

In 2010, the total wage bill in Canada was estimated at \$20.1 billion for all regulated nurses, \$17.3 billion for front-line RNs and \$2.2 billion for LPNs (Table 1). The difference (\$0.6 billion) represents the wage bill for head nurses and supervisors. Spending (wage bill) for regulated nurses in Canada represents about 73% of total physician spending. In fact, the latter was estimated at \$27.4 billion in 2010 (CIHI 2013). This includes overhead costs (administrative charges, nurse salaries, rent, etc.), which can represent from 24 to 31% of gross physician remuneration (CMA 2010). This suggests that the wage bill for regulated nurses is as important as net physician remuneration and is not trivial.⁷ Not surprisingly, the two most populous provinces (Ontario and Quebec) were responsible for more than half (56%) of the total wage bill for nurses in Canada.

TABLE 1. Estimated total wage bill for nurses, wage per hour, number of weekly hours worked and number of nurses per thousand, 2010

	Total wage bill (\$ million)			Wage per hour (\$)		Number of weekly hours worked		Number of nurses per thousand	
	All nurses	RN*	LPN	RN*	LPN	RN*	LPN	RN*	LPN
NL	447.0	363.4	79.2	35.8	23.1	32.9	35.4	11.4	3.6
PEI	104.4	90.2	N/A	31.2	N/A	31.9	N/A	12.3	N/A
NS	652.0	545.2	88.9	32.3	20.9	34.0	34.3	10.1	2.5
NB	555.2	468.5	78.0	33.0	20.6	33.8	32.9	10.7	2.9
QC	4,075.2	3,278.2	565.8	32.1	21.5	30.8	30.6	8.1	2.1
ON	7,319.5	6,455.7	635.8	35.1	23.8	34.0	33.3	7.9	1.2
MB	867.4	770.0	80.8	35.5	23.7	32.4	31.7	10.5	1.7
SK	852.7	756.1	79.4	38.9	23.8	33.2	33.8	10.7	1.8
AB	2,552.3	2,231.1	270.2	39.0	25.0	31.6	32.7	9.3	1.7
BC	2,600.7	2,263.9	260.4	35.3	24.3	32.2	32.0	8.6	1.4
Canada	20,104.1	17,289.3	2,158.1	35.0	23.1	32.6	32.4	8.6	1.6

*Front-line registered nurses.

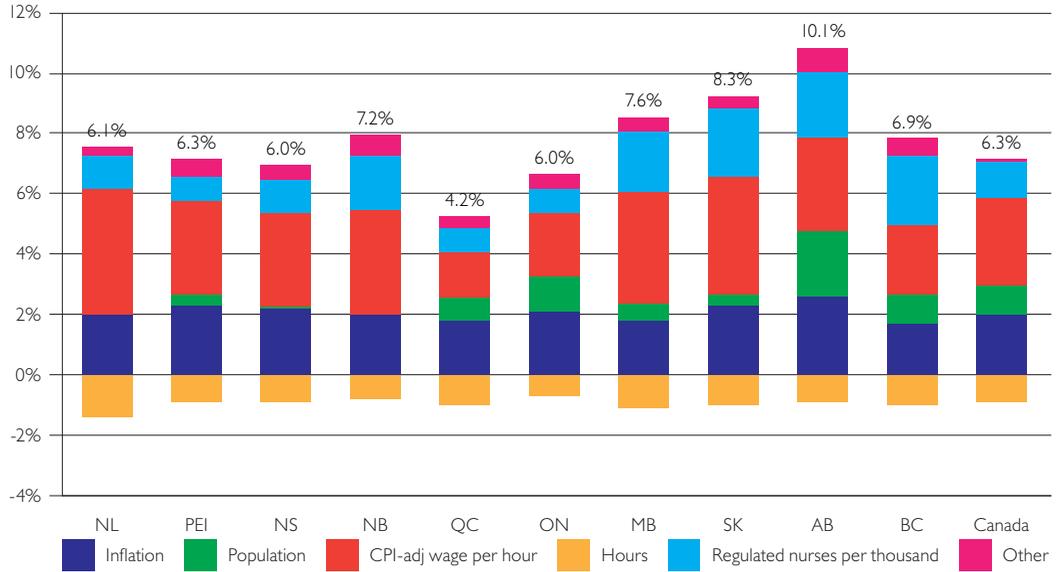
N/A = not available (data for LPN in PEI are not available due to sample size issue).

Average wage per hour in 2010 in Canada was estimated at \$35.0 for RNs and \$23.1 for LPNs. RN wage per hour was higher in Alberta and Saskatchewan and lower in PEI and Quebec. Average number of hours worked per week in 2010 in Canada was virtually the same for RNs and LPNs, 32.6 and 32.4, respectively. The pattern is basically similar in the provinces, except in Newfoundland-Labrador, where LPNs tend to work a bit more hours than RNs. There is not much variability in the number of hours worked among provinces, even though Quebec RNs tend to work a bit fewer hours (30.8 compared to the high of 34.0 in Ontario). Finally, the number of front-line RNs per thousand population was 8.6 at the Canada level, with a high of 12.3 in PEI and a low of 7.9 in Ontario. LPNs at the national level averaged 1.6 per thousand population, with a high of 3.6 in Newfoundland-Labrador and a low of 1.2 in Ontario.

Average Annual Growth in Total Wage Bill and Decomposition

This section of the paper focuses on growth in the nursing wage bill for the period 2000–2010. Figure 1 presents the average annual growth and breaking down of the total wage bill for regulated nurses, by jurisdiction.

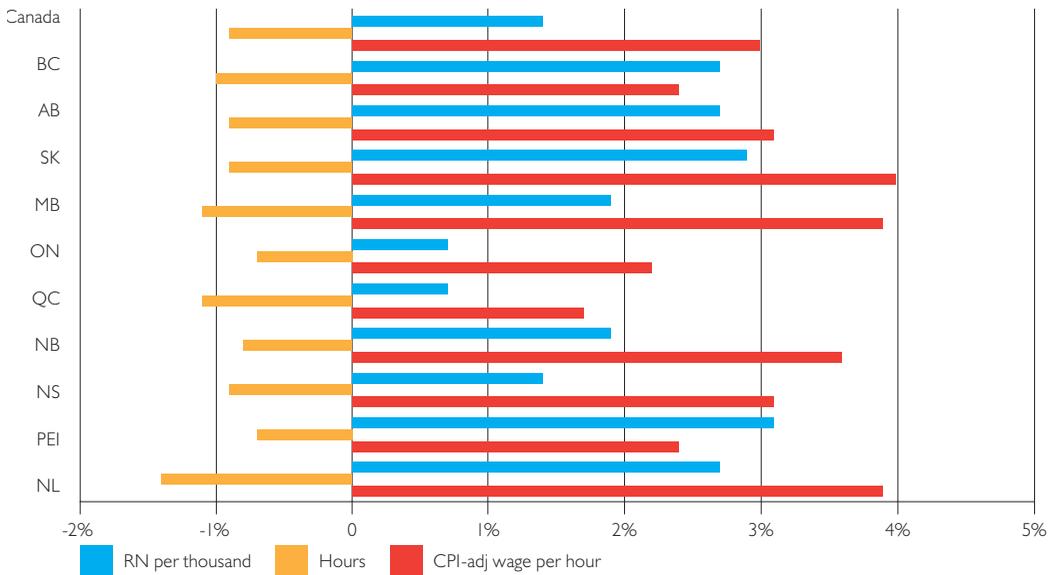
FIGURE 1. Average annual growth in the total wage bill of regulated nurses and decomposition, by jurisdiction: 2000–2010



Total wage bill for regulated nurses (the nursing professional category as a whole) in Canada was estimated at \$10.9 billion in 2000. Since then, it grew at an average annual rate of 6.3% to reach \$20.1 billion in 2010. Among the provinces, the average annual growth rate varied from a high of 10.1% (Alberta) to a low of 4.2% (Quebec). The key policy question is what were the cost drivers of the total nursing wage bill in each jurisdiction? Decomposition of this nursing wage bill helps address this question. After removing general inflation and population growth, which, at the Canada level, were 2.0% and 1.0%, respectively, the adjusted wage bill for regulated nurses still grew at 3.3% at the Canada level. This growth was mainly driven by real wage per hour (hourly wage growth above and beyond general inflation), which rose at an average annual rate of 2.9%. Growth in the number of regulated nurses per thousand (1.2%) contributed also to the growth in the total wage bill. However, this was partially offset by growth in average number of weekly hours worked, which was rather negative (-0.9%), suggesting that professionals in the nursing category worked less time in 2010 than in 2000. The same trend was observed in the general economy, even though to a lesser extent (-0.3%). It seems to be a paradox given the perceived shortage of nurses in Canada during this period and evidence of nurse overtime and overwork (O'Brien-Pallas et al. 2004), as well as the higher percentage of RNs employed full-time – 52.0% in 2000 versus 58.0% in 2010.⁸ Absenteeism could be perceived as being partly responsible for the decline in worked hours. In fact, 9% of public-sector full-time healthcare nurses were absent each week in 2010 owing to own illness or disability, nearly twice the rate of all other occupations (CFNU 2011b). However, it should be noted that this rate of absenteeism had not changed much during the 2000s,⁹ which suggests looking for reasons elsewhere. Seemingly, the aging of the nursing workforce could be partly responsible for the increase in real wage per hour. As a matter

of fact, the percentage of RNs aged 50 and over in the workforce grew from 29.0% in 2000 to 40.3% in 2010 (CIHI 2011).¹⁰ Assuming that age is correlated with tenure, more senior nurses mean higher hourly costs. It also means more vacation time. So, the aging factor could be partly responsible for both the increase in hourly rate and the decrease in weekly hours worked. There could be other potential explanations.¹¹ At the provincial level, the pattern is similar. Real wage growth was also the main driver of the increase in nurse wage bill (high of 4.2% in Newfoundland-Labrador to low of 1.5% in Quebec), followed by growth in number of nurses per thousand (led by the Western provinces with growth of 2.0% and higher). Number of weekly hours worked also dropped in each province; the range was narrow around the Canada average (-1.3% in Newfoundland-Labrador to -0.7% in Ontario). The component “Other” is a residual that may capture factors such as changes in nursing workload (patient severity) and policy interventions. It is worth investigating how this decomposition looks like for the specific cases of RNs and LPNs. Figure 2 depicts the situation for RNs. Given that general inflation and population growth are macroeconomic factors that do not change with the professional category, they are not reproduced in the figures thereafter.

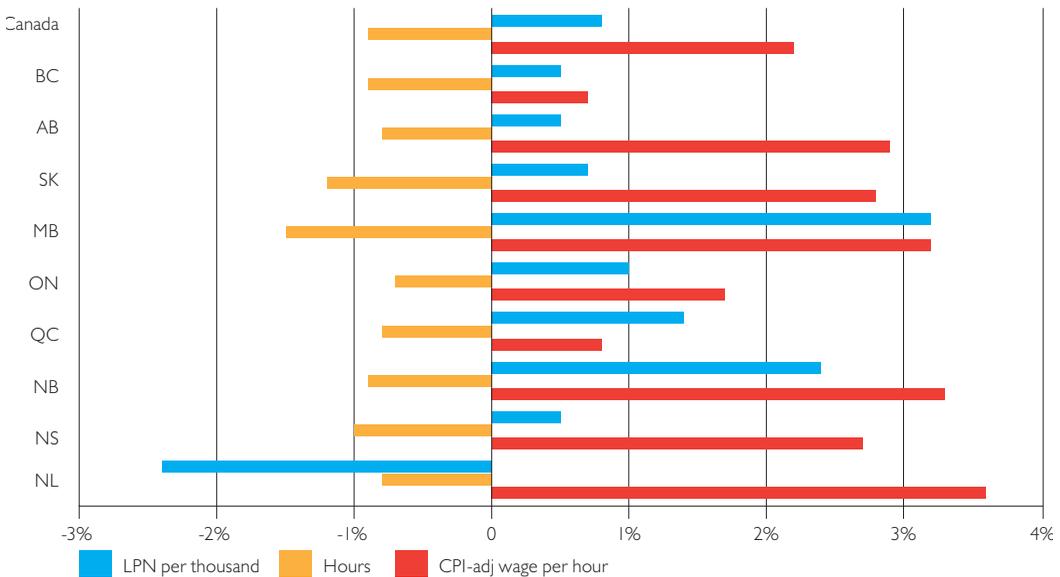
FIGURE 2. Average annual growth in real wage per hour (\$2002), number of hours worked and number of RN per thousand, by jurisdiction, RN: 2000–2010



At the Canada level, inflation and population-adjusted wage bill for RNs grew at 3.6%. *Once again, this growth was mainly driven by real wage per hour, which rose at an average annual rate close to 3.0%.* Growth in the number of RNs per thousand (1.4%) contributed also to the growth in wage bill, while growth in average number of hours worked per week was rather negative (-0.9%), suggesting that RNs worked less time in 2010 than in 2000. It is not surprising that at the Canada level, the findings for RNs are similar to those for the nursing category as a whole, given that RNs represent the vast majority of this category (more than

80%). At the provincial level, the pattern is similar: *real wage per hour was the main factor driving RN wage bill in each province, except in PEI and BC, where number of RN per thousand was the main factor.* As previously noted, the policy implication of a growing wage bill is different depending on which component of the bill is growing (see the Introduction). This suggests that, except for PEI and BC, the other provinces are paying more without getting significantly more RN throughput. As for the LPN wage bill, the situation is presented in Figure 3.

FIGURE 3. Average annual growth in real wage per hour (\$2002), number of hours worked and number of LPN per thousand: by jurisdiction: LPN: 2000–2010



At the Canada level, total wage bill for LPNs grew at an average annual rate of 5.2%, which is 1.4 percentage points less than RNs. *After taking into account general inflation and population growth, the 2.2% increase in LPN wage bill was totally driven by real wage per hour.* Growth in the number of LPNs per thousand (0.8%) was offset by decrease in number of hours worked by about the same percentage (-0.9%). This suggests that, as in the case of RNs, LPNs also worked less time in 2010 than in 2000 and by the same proportion.

At the provincial level, the pattern is pretty much similar: real wage per hour was either the only factor or the main factor driving LPN wage bill in each province, except in Quebec, where number of LPN per thousand was the main factor. Therefore, the provinces are paying more without getting significantly more LPN throughput, except for Quebec. Number of weekly hours worked dropped in each single province. Note that for Newfoundland-Labrador, both number of hours worked and number of LPN per thousand have dropped, suggesting that this province has been substituting RN for LPN. Numbers for PEI are not presented in this figure, owing to sample size issue.

Data Triangulation and Discussion

Because of its large sample size and its link to administrative tax data, the 2011 NHS is a rich database capable of generating reliable estimates of average hourly wages, average number of hours worked and number of professionals. Still, it is worth assessing how it compares to other data sources. We have found that the count of regulated nurses in the NHS is close to the administrative CIHI (2011) NDB (with only 0.7% more in the NHS).

As for the hourly wage, the LFS represents another data source to which the NHS findings are compared. Average hourly wage for RNs in Canada in 2010 was estimated at \$35.0 in the NHS and \$33.0 in the LFS; a difference of 6.1%. Note that NHS data include overtime, while LFS data do not. The CFNU (2011a) document offers also a basis for comparison of hourly rates of general-duty RNs and LPNs. However, it is not a direct comparison because only the range is available in this CFNU document and the rates are as of July 1, 2011. Having said that, it is still possible to use this source for comparison purposes: the minimum (maximum) can be interpreted as a lower (upper) bound. It is important to note that for both RNs and LPNs, the average hourly rate from the NHS generally fell within the CFNU range. Comparison of estimated total wage bill to figures from provincial health ministries was most exhaustive in Quebec, where figures for nurses in both the public and the private sectors were obtained. The discrepancy from the two sources was only 0.5%.

Moreover, results based on the NHS and the SEPH (for the general worker) suggest that, during the past decade, RNs benefited from a “wage premium” estimated at 1.7 percentage points compared to the general worker. In the case of LPNs, the “wage premium” was estimated at 0.9 percentage points. So, *the “wage premium” was more substantial for RNs than for LPNs*. The fact that the pattern is the same and the results tend to converge independently of the data sources suggests that the estimates are robust. One limitation of the paper is that the 2000 income data are exclusively self-reported, while for 2010, they are based on a combination of self-reported and tax data. This could introduce some bias. However, comparison with LFS data (exclusively self-reported in 2000 and 2010) suggests that the bias is not substantial. With the LFS, real wage per hour for RNs is estimated to grow at an average annual rate of 2.2%, which still suggests the existence of a wage premium. Another limitation of the study is that it does not address the issue of nurse workload or output. However, it is still a step that contributes to the existing literature. The results can be used later in conjunction with other clinical data sources to shed light on the issue of value for money in the nursing professional category. Indeed, future studies that address the issue of nurse compensation and workload or output are required. Then, decision-makers can rely on a more comprehensive setting for policy interventions.

Conclusion

At the Canada level, total wage bill for the nursing professional category in Canada was estimated at \$20.1 billion (\$17.3 billion for RNs), which suggests that it is as substantial as net physician remuneration. The average annual growth rate of this wage bill was 6.3%

(6.6% for RNs). This increase was mainly driven by real wage per hour (3.0% above and beyond general inflation in the case of RNs). This finding is similar to physician cost driver studies, which found that unit price was the main driver of physician compensation in Canada for the 1998–2008 period (CIHI 2010) or in Quebec (Contandriopoulos and Perroux 2013) for the 2007–2011 period. It should be noted that real wage per hour in the general economy has grown only at an average annual rate of 1.3% between 2000 and 2010: which suggests the existence of a “health premium” of 1.7 percentage points for RNs. The “health premium” was lower for LPNs. While the number of nurses per thousand has contributed to the increase in the total wage bill, it was partially offset by the reduction in the numbers of hours worked per week. At the provincial level, the pattern is generally similar. Real wage per hour was the main factor driving RN wage bill in each province, except in PEI and BC, where number of RN per thousand was the main factor. In the case of LPN, real wage per hour was either the only factor or the main factor driving wage bill in each province, except in Quebec, where number of LPN per thousand was the main factor. Pending a more comprehensive analysis that includes nursing workload and output, this finding suggests that in most provinces, governments are paying more without necessarily getting more nursing throughput.

Disclaimer

The views expressed in this paper are those of the authors and do not represent the opinions of their employing organizations.

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Notes

1. In this study, the term LPN is used to designate both licensed practical nurses and registered practical nurses.
2. This study does not include the nurses-aides, orderlies and patient service associates (D312), which are unregulated.
3. Based on the set of questions: During the year ending December 31, 2010, did this person receive any income from the following sources?
4. Personal e-mail communication with Statistics Canada.
5. Number of weekly hours was derived from the survey question: *Last week (or during the week of Sunday, May 1, 2011 to Saturday, May 7, 2011), how many hours did this person spend working for pay or in self-employment?*
6. Estimating the impact of population aging on nurse expenditures in Canada is difficult because data on nurse spending by age category do not exist.
7. The purpose of the comparison is to show the relative importance of the nurse wage bill in the public healthcare system. Medical services in Canada are privately delivered but also publicly funded.

8. These numbers are obtained from a special CIHI data request where full-time is set as defined by the employer (generally, 35 hours of work and more per week). The same trend is observed when the NHS or the LFS is used – full-time defined as 30 hours of work and more per week. For example, Figure 8 in Laberge et Montmarquette (2009) uses the LFS to show an increased trend in the proportion of nurses working full-time between 1997 and 2007 in Quebec, Ontario and the rest of Canada.
9. CFNU (2011b) reports a weekly absenteeism rate of 8.1% for all nurses in the public sector in 2002 and 2010, with fluctuation of around 1 percentage point. Likewise, Laberge and Montmarquette (2009) report only a 2 percentage point increase in the absenteeism rate of nurses between 1997 and 2007. Depression was a significant determinant of absenteeism for both RNs and LPNs, namely, in the hospital setting (Basu and Rajbhandary 2010).
10. The figure for 2000 was obtained through a special CIHI data request.
11. For example, given that this professional category is highly unionized (Uppal 2011) with relatively difficult working conditions, employers and unions might come to mutual agreements during collective bargaining to improve nurse working conditions as a retention strategy and thus reduce the number of hours worked per week while still securing high increases in wage rate.

References

- Ariste, R. forthcoming. "Fee-Schedule Increases in Canada: Implication for Service Volumes among Family and Specialist Physicians." *Journal of Health and Human Services Administration*.
- Barer, M.L. and R.G. Evans. 1983. "Prices, Proxies and Productivity: An Historical Analysis of Hospital and Medical Care in Canada." In W.E. Diewert and C. Montmarquette, eds., *Price Level Measurement: Proceedings from a Conference Sponsored by Statistics Canada*, Ottawa, ON.
- Basu, K. and S. Rajbhandary. 2010. "Working Conditions of Nurses and Absenteeism: Is There a Relationship? An Empirical Analysis Using National Survey of the Work and Health of Nurses." *Health Policy* 87: 152–59.
- Baumann, A., M. CreaArsenio, D. Idriss-Wheeler, M. Hunsberger and J. Blythe. 2010. "Health Human Resource Series Number 23. A MadeinLHIN Solution: Identifying Local Needs in 70% Full-Time Nurse Employment." Central West LHIN 5 [Internet]. Hamilton, ON: Nursing Health Services Research Unit. Retrieved June 2010. <<http://www.nhsru.com>>.
- Buhr K. 2006. *Three Essays on the Labour Market for Registered Nurses in Canada*. PhD thesis. Carleton University, Available at ProQuest.
- CFNU. 2011a. *Overview of Key Nursing Contract Provisions*. Retrieved from the Canadian Federation of Nurses Union on February 27, 2014. <http://www.nursesunions.ca/sites/default/files/2011.07.contract_comparison_e_0.pdf>.
- CFNU. 2011b. *Trends in Own Illness or Disability-Related Absenteeism and Overtime among Publicly-Employed Registered Nurses: Quick Facts*. Prepared for the Canadian Federation of Nurses Unions by Informetrica Limited. Ottawa, ON.
- Canadian Institute for Health Information (CIHI). 2006. *Average Payment per Physician Report, Fee-for-Service Physicians in Canada, 2004–2005*. Ottawa, ON: Canadian Institute for Health Information.
- Canadian Institute for Health Information (CIHI). 2010. *Healthcare Cost Drivers – the Facts*. Ottawa, ON: Canadian Institute for Health Information.

- Canadian Institute for Health Information (CIHI). 2011. *Regulated Nurses: Canadian Trends, 2006–2010*. Ottawa, ON: Canadian Institute for Health Information.
- Canadian Institute for Health Information (CIHI). 2013. *National Health Expenditures Trends, 1975–2013*. Ottawa, ON: Canadian Institute for Health Information.
- Canadian Institute for Health Information (CIHI). 2014. *Physicians in Canada, 2013: Summary Report*. Ottawa, ON: Canadian Institute for Health Information.
- Canadian Medical Association (CMA). 2010. *National Physician Survey, 2010*. Canadian Medical Association (Communication with the Workforce Research Unit). Statistics, reports and links on physician resources can be found at: <<http://cma.ca/c3pr>>.
- Contandriopoulos, D. and M. Perroux. 2013. "Fee Increases and Target Income Hypothesis: Data from Quebec on Physicians' Compensation and Service Volumes." *Healthcare Policy* 9(2): 30–35. DOI:10.12927/hcpol.2013.23613.
- Hartwig, J. and J.E. Sturm. 2014. "Robust Determinants of Health Care Expenditure Growth." *Applied Economics* 46(36): 4455–74. DOI:10.1080/00036846.2014.964829.
- Laberge M. and C. Montmarquette. 2009. *Portraits des conditions de pratique et de la pénurie des effectifs infirmiers au Québec*. CIRANO. Rapport de projet 2009 RP-01. Montreal, QC: CIRANO.
- O'Brien-Pallas, L., G.T. Murphy, H. Laschinger, S. White, S. Wang and C. McCulloch. 2004. "Enquête auprès des infirmières et infirmiers des trois groupes professionnels au Canada." In *Dans le contexte du projet : Construire l'avenir : une stratégie intégrée pour les ressources humaines infirmières au Canada*. Ottawa, ON: Société de l'étude sectorielle sur les soins infirmiers.
- Statistics Canada. 2003. *2001 Census Handbook*. Catalogue No. 92-379-XIE. Ottawa, ON: Author.
- Statistics Canada. 2011. *Income Reference Guide. National Household Survey*. Analytical document. Catalogue no. 99-014-X2011006. Ottawa, ON: Author.
- Statistics Canada. 2013. *National Household Survey Dictionary, 2011*. Catalogue no. 99-000-X2011001. Ottawa, ON: Author.
- Uppal, S. 2011. "Unionization, 2011." Perspectives on Labour and Income. Statistics Canada. Catalog No. 75-001-X. Retrieved April 15, 2015. <<http://www.statcan.gc.ca/pub/75-001-x/2011004/article/11579-eng.pdf>>.
- Vujcic, M. 2003. "Recent Trends in the Nursing Labour Market in Canada." PhD thesis, University of British Columbia, Department of Economics.
- Zeytinoglu, I.U., M. Denton, S. Davies, A. Baumann, J. Blythe and L. Boos. 2006. "Retaining Nurses in Their Employing Hospitals and in the Profession: Effects of Job Preference, Unpaid Overtime, Importance of Earnings and Stress." *Health Policy* 79(1): 57–72.

The Impact of Private Insurance Coverage on Prescription Drug Use in Ontario, Canada

Impact de l'assurance médicaments privée sur l'utilisation des médicaments délivrés sur ordonnance en Ontario, Canada



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Abstract

Canadians obtain prescription drug coverage through a patchwork of public insurance, private benefit plans and out-of-pocket payments. Prior evidence suggests that insurance coverage, in general, leads to higher utilization rates of essential medicines; it is unclear whether individuals with private insurance have better access to medicines.

Using data from the 2008 Canadian Community Health Survey, we identified cohorts from Ontario who reported having been diagnosed by a physician with asthma, high blood pressure or diabetes. Using propensity score stratification techniques, we compared drug utilization of individuals holding private insurance with that of individuals holding either public insurance (for those aged over 65 years) or no insurance (aged under 65 years).

In five out of six comparisons, individuals with private insurance were more likely to take prescribed drugs than those without. Raw differences in the percentage of patients taking medicines ranged from 0.1 to 8.1%.

Ontarians with chronic conditions holding private drug insurance are more likely to use prescription drugs than those who do not. Whether these inequities result in health outcome differences remains unknown.

Résumé

La couverture pour les médicaments sur ordonnance au Canada provient d'une mosaïque d'assurances publiques, de régimes d'assurance privés et de déboursements par les particuliers. Des données antérieures suggèrent qu'en général, la couverture offerte par les assurances mène à des taux plus élevés d'utilisation des médicaments essentiels; cependant, on ne sait pas dans quelle mesure les personnes qui ont une assurance privée jouissent d'un meilleur accès aux médicaments.

À l'aide des données provenant de l'Enquête sur la santé dans les collectivités canadiennes de 2008, nous avons repéré des groupes de patients ontariens qui ont indiqué avoir reçu un diagnostic médical pour l'asthme, l'hypertension artérielle ou le diabète. Au moyen des techniques de stratification des coefficients de propension, nous avons comparé l'utilisation des médicaments chez, d'une part, les personnes détenant une assurance privée et, d'autre part, celles qui détiennent une assurance publique (pour les 65 ans et plus) ou aucune assurance (pour les moins de 65 ans).

Dans cinq des six comparaisons, les personnes qui ont une assurance privée étaient plus susceptibles de prendre des médicaments sur ordonnance que ceux qui n'ont pas une telle assurance. Les différences brutes dans les pourcentages de patients qui prennent des médicaments variaient de 0,1 à 8,1 %.

Les Ontariens aux prises avec un état chronique et qui détiennent une assurance médicaments privée sont plus susceptibles d'utiliser des médicaments sur ordonnance que ceux qui n'ont pas une telle assurance. On ne sait pas encore si ces iniquités donnent lieu à des différences dans les résultats cliniques.

Introduction

Background

Unlike all other developed countries with universal healthcare insurance schemes, Canada does not provide universal outpatient prescription drug coverage (Morgan et al. 2013). Rather, Canadians receive prescription drug coverage from a patchwork of different insurance plans, including both public and private plans (Daw and Morgan 2012). Several provinces have age-based drug coverage programs that typically cover the elderly, while others have programs that provide income-based coverage based on deductibles that are a portion of household income (Daw and Morgan 2012). The majority of prescription drug coverage comes from employer-sponsored drug benefits, which are the primary source of drug coverage for about 60% of the population (Marshall 2003). The remainder of the population is either covered by public plans or is uninsured (Fraser Group/Tristat Resources 2002). In 2012, public plans paid for approximately 44% of drug expenditures in Canada, private insurance paid for 35% and out-of-pocket payments made up the remaining 20% (CIHI 2013).

Much previous work, both in Canada and elsewhere, suggests that the presence and type of insurance coverage – and the resulting lower out-of-pocket costs for patients – is associated with higher utilization of essential medicines (Adams et al. 2001; Joyce et al. 2002; Soumerai 2004; Tamblyn et al. 2001). For example, Goldman and Zheng (2007) synthesized international evidence on the relationship between costs associated with drug benefits and the use of prescription drugs. They found that increased cost sharing (for example, copayments and deductibles) was consistently associated with lower rates of drug use, while patients with diabetes demonstrated that higher cost sharing is associated with an increased use of medical services (Goldman and Zheng 2007). How this impacts drug utilization in Canada, however, is comparatively unstudied. Given the unique stature among other developed nations that provide universal medicare, as being the only nation to also not provide pharmacare, Canada offers an opportunity to study the impact of differences in pharmaceutical coverage without being confounded by differences in out-of-pocket payments required for hospital and physician coverage. To our knowledge, only one study has explicitly examined the relationship between private drug insurance coverage and drug utilization in Canada, which found that seniors in the province of Ontario who held private insurance in addition to the publicly provided insurance had higher use of prescription drugs (Allin et al. 2013).

What remains less clear is how private health insurance impacts drug use in systems with both public and private coverage. Canada provides an ideal setting in which to test such relationships, as some public coverage co-exists with private insurance that generally offers more extensive coverage (Kratzer et al. 2013). About 60% of Canadians have private insurance, generally as a benefit of employment (Law et al. 2014). To date, research on this topic has been limited. Some evidence suggests that Canadians with private health insurance for prescription drugs have fewer barriers to accessing healthcare than those without. For example, prior

studies have found that holding private drug coverage was associated with a greater likelihood in accessing physician care and lower patient reports of unmet healthcare needs (Allin and Hurley 2009; Hanley 2009; Stabile 2001). That is to say that despite physician and hospital services being universally publicly provided, the presence of private pharmaceutical drug coverage impacted their use.

While the above-mentioned studies provide an indication that privately insured individuals use more prescription drugs than other Canadians, selection bias remains a significant concern. For instance, we know that higher-income individuals are more likely to hold private drug insurance (Dewa et al. 2005). In contrast, those holding public insurance or those who are uninsured are more likely to have a lower income, be older and use fewer prescription drugs (Dewa et al. 2005; Fang et al. 2008; Millar 1999). This is important because individual characteristics, such as chronic conditions, education, age and income, have all been shown to impact drug utilization (Law et al. 2012; Osterberg and Blaschke 2005). Past studies have used regression techniques to attempt to control for these factors, but it remains unclear how well they have performed. Further, prior work on this topic has aggregated drug use across all therapeutic classes, leaving open the possibility that some differences that have been found were the result of uncontrolled differences in individuals' underlying health conditions (Allin and Hurley 2009; Millar 1999). To investigate the impact of private insurance more robustly, we used propensity score techniques to study the difference in prescription drug use for three cohorts of individuals with and without private drug coverage for three common chronic conditions: asthma, high blood pressure and diabetes.

Methods

Study context

In 2008, Ontario's 12.9 million residents (Government of Canada 2012) received comprehensive hospital and physician coverage with no patient charges from the Ministry of Health and Long-Term Care. In contrast, only a portion of residents received prescription drug benefits from Ontario's six drug programs that pay a portion of the cost of their drug charges. Five of the six public programs covered either specific disease groups (e.g., cystic fibrosis, Gaucher's disease and thalassaemia) or specific drugs (e.g., verteporfin, zidovudine and clozapine) (Government of Ontario 2013). The largest program, the Ontario Drug Benefit program (ODB), covered residents who met at least one of the following criteria: aged 65 years or older, lived in a long-term care home, were enrolled in a home care program, had high drug costs relative to their household income (through the Trillium Drug Program) or who received social assistance.

The ODB formulary included around 3,800 drug products and an additional 850 products which required the treating physician to seek special approval from the drug plan. Seniors (aged 65 years and older) with a yearly net income of \$16,018 or more for a single

person and \$24,175 or more for a couple, paid a \$100 deductible and then up to \$6.11 per prescription as copayment. Those seniors with net income lower than the figures given above for singles and couples paid up to \$2 as a copayment for each prescription (Government of Ontario 2013). The majority of residents aged under 65 years were eligible for public coverage through the Trillium Program. Residents on the Trillium Program paid an annual deductible based on their household income, normally set at 4% of the household's total net income. This deductible was paid in four equal payments over the year. After the deductible was reached, the Trillium Program beneficiaries paid up to \$2 as copayment for each prescription (Government of Ontario 2013). In practice, many pharmacies in the province waived fees for those who were required to pay the \$2 copayment for both the ODB and the Trillium Program plan members.

Data sources

We used 2008 data from Statistics Canada's annual Canadian Community Health Survey (CCHS), which is the most recent year in which questions on drug coverage were included. The CCHS is a sample survey with a cross-sectional design, and it is collected through telephone interviews using computer-assisted interviewing. Responding to the survey is voluntary and data are collected directly from survey respondents. The CCHS collects information related to health status, healthcare utilization and socio-demographic information for the Canadian population. In 2008, Ontario purchased a supplementary module that included a question asking whether respondents had public, employer-sponsored or private drug coverage (please see Appendix 3 for the relevant questions from the CCHS, at www.longwoods.com/content/24212). For our analysis, we have grouped the latter two together, as they are both forms of private insurance. To investigate the relationship between drug coverage and the use of medications for chronic conditions, we analyzed questions that were asked specifically of respondents in Ontario who reported they had been diagnosed by a physician with one of the following three chronic conditions: asthma, high blood pressure or diabetes. These three chronic disease categories were selected because survey questions about diagnosis were followed up with questions about taking prescription drugs to treat. We believe that these conditions are important to consider because they are not only highly prevalent (MOHLTC 2007) but are also commonly treated with prescription drugs.

Study cohorts

First, we used three cohorts of respondents who reported having been diagnosed by a physician with one of three common chronic conditions: asthma, high blood pressure and diabetes. Within each of these three cohorts, we stratified our analysis into two age strata: individuals aged under 65 years and individuals aged 65 years and over. We used weighted samples provided by the CCHS. The total sample size for all three cohorts was 2,161,311. Table 1 demonstrates the weighted sample size for each cohort. For the under-65 years' age stratum,

we compared individuals with private coverage and individuals who reported having no coverage. We excluded those with public coverage in this age cohort because, by qualifying for public coverage, they were either low income (social assistance recipients) or had very high drug expenditures relative to their household income and were, therefore, not comparable to those with private insurance. For the 65 years and over stratum, we compared those with private coverage and those with public coverage, as all Ontario residents aged 65 years and over automatically qualify for universal public coverage.

TABLE 1. Weighted sample sizes of respondent disease cohorts

Cohort	Age strata	Weighted sample size
Asthma	under 65	494,628
	65+	60,622
High blood pressure	under 65	744,770
	65+	371,438
Diabetes	under 65	318,107
	65+	171,746
Total		2,161,311

Statistical analysis

We investigated the relationship between holding private drug coverage and the use of medications for chronic conditions. In non-randomized study designs, the treated and control groups may have large differences in both their observed and unobserved covariates, and these differences can lead to biased estimates of treatment effects. The propensity score method can be used to balance the observable covariates between the groups with the hope of reducing this bias (Rubin 1997). Using a sample survey, however, it is also necessary to incorporate survey weights into the analysis (Zanutto et al. 2005). Therefore, we applied a propensity score method based on stratification that allows the incorporation of survey weights into estimates of the treatment effect (Zanutto 2006). We incorporated the survey weights after the propensity score sub-classification to make population-level inferences, and then used the adjusted weights as the normalized weights to estimate the average outcome.

In our study, the main determinant of interest was an individual’s specific type of drug coverage. We needed to consider baseline covariates strongly associated with the use of medications and plausibly associated with having drug coverage, and we used propensity score stratification models to compare the prevalence of drug usage between drug coverage groups after accounting for baseline covariates. As we know from existing literature, individuals with and without private drug coverage are quite divergent on many characteristics (Dewa et al. 2005). We chose to use propensity score stratification over traditional regression approaches because it bases the results on comparisons between cohorts with similar observed characteristics.

In our first step, we calculated a propensity score for each respondent which reflected their probability of having drug coverage using logistic regression. In the model, we included the baseline covariates of age, sex, marital status, current residence, immigration status, household size, income, education, employment status, chronic conditions and health status measures of the number of pre-existing conditions (see Appendix 2, Tables 7–12 at www.longwoods.com/content/24212). Note, in the model for the 65 years and over cohort diagnosed with asthma, we were required to drop the household size variable to reach convergence when performing the regression. This issue, while a frequent problem in estimating logistic regression models, did not occur in any of the other five models.

We followed recommended practice and created five strata of equal size, as this number of stratum has been shown to remove approximately 90% of bias in prior studies (Cochran 1968). Subsequently, we confirmed that the observed characteristics of the groups with and without private drug coverage were balanced within each of the three cohorts (Rosenbaum and Rubin 1984). We evaluated the extent to which the balance of the distribution of covariates for two groups was similar within each stratum using statistical tests with respect to the measuring scale of selected covariates (recognizing discrete, count and continuous outcomes) (Stampf 2011). P-values from these tests were applied to data before and after stratification to test the extent to which the propensity score model increased comparability between the two drug coverage groups within each stratum for all baseline covariates.

We estimated the probability of using drugs within each stratum and also estimated the overall probability as a weighted average. Finally, we used an indirect post-stratification adjustment to the final survey weights to estimate an overall survey-weighted probability in the overall population (Zanutto et al. 2005). To obtain the adjusted odds ratio for respondents with one drug coverage compared with those with another after adjusting for the probability of having drug coverage, we used a survey-weighted logistic regression model with the propensity score quintile serving as a summary confounder variable (Hahs-Vaughn and Onwuegbuzie 2006). All reported results are from weighted samples. In this model, the drug coverage variable is used as the independent variable, along with four of the five dummy-coded strata (see Appendix 2, Tables 7–12 at www.longwoods.com/content/24212). All analyses were performed using SAS, Version 9.2.

Results

Of the 21,991 respondents from Ontario, 12,833 reported holding employer-sponsored drug coverage. Overall, the use of propensity score stratification limited the extent to which each stratum differ based on these estimated differences in observed covariates (see Appendix 1, Tables 1–6 at www.longwoods.com/content/24212).

Under 65 years

All our models of the patients under 65 years of age compared those who reported holding private drug against the population that reported no coverage. This latter group would all be

potentially eligible for the high-deductible public coverage of the Trillium Program. These results consistently showed that the individuals with private drug coverage had higher use of prescription drugs for their chronic conditions than those without.

ASTHMA

Patients with asthma in the under 65 years' cohort had a different likelihood of using prescribed drugs depending on whether they held private drug coverage. Table 2 shows the estimates of the effect of having drug coverage on the prevalence of drug usage when comparing groups with private coverage and groups with no coverage. Patients with asthma in the under 65 years' cohort had a different likelihood of using prescribed drugs depending on whether they held private drug coverage. The overall survey-weighted adjusted probability estimates indicate a modest difference in the prevalence of drug usage between these two groups (77.7% vs. 70.9%). We found that individuals with private drug coverage had 1.5 times greater odds of having used prescription drugs to treat asthma than those without (OR = 1.50; 95% CI [1.47, 1.53]).

TABLE 2. Drug utilization prevalence for respondents under the age of 65 years

Under 65 years	Private vs. none, prevalence			Private vs. none, odds ratio
		vs.		
Asthma	77.7%	vs.	70.9%	OR = 1.499 [1.471, 1.528]
High blood pressure	82.2%	vs.	76.1%	OR = 1.526 [1.504, 1.548]
Diabetes	79.3%	vs.	71.2%	OR = 1.158 [1.129, 1.186]

HIGH BLOOD PRESSURE

The prevalence of drug usage among individuals aged under 65 years with high blood pressure between individuals with private coverage and those without is shown in Table 2. Overall, survey-weighted probability estimates of drug usage for individuals with private insurance were moderately higher at 82.2% compared to 76.1% for individuals with only public coverage. Similar to the results for asthma, we found that individuals with private coverage had 1.5 times higher odds of having used prescribed drugs to treat high blood pressure than individuals with no coverage (OR = 1.53, 95% CI [1.50, 1.55]).

DIABETES

The prevalence of drug usage for individuals aged under 65 years with diabetes differed between those with private coverage and those without (79.3% and 71.2%, respectively). As shown in Table 2, after adjusting for quintile of propensity score, we found that individuals with private coverage had 1.2 times higher odds of having taken their prescribed diabetes drugs than individuals without (OR = 1.16, 95% CI [1.13, 1.19]).

Individuals aged 65 years and older

All patients in the cohorts of patients aged 65 years and older held some form of drug coverage: all comparisons are between the group that reported holding private drug coverage (those who would also be eligible for the ODB coverage) and the group with just public coverage. In total, 603,806 patients were aged 65 and older, representing 28% of the total sample.

ASTHMA

In the over-65 years' cohort that reported physician-diagnosed asthma, drug usage varied between the group who reported holding private coverage and the group with only public coverage. Table 3 shows the overall survey-weighted probability estimates of drug usage between these two groups, with 92.5% patients with private coverage using prescribed drugs compared to 86.5% patients on the public plan. In adjusted models, cohort members with private coverage had 2.1 times higher odds of having used prescribed asthma drugs than those with public coverage (OR = 2.12; 95% CI [1.98, 2.26]).

TABLE 3. Drug utilization prevalence for respondents aged 65+ years

65+ years	Private vs. public, prevalence			Private vs. public, odds ratio
Asthma	92.5%	vs.	86.5%	OR = 2.119 [1.984, 2.263]
High blood pressure	97.3%	vs.	97.4%	OR = 0.946 [0.880, 1.005]
Diabetes	88.9%	vs.	84.1%	OR = 1.327 [1.276, 1.380]

HIGH BLOOD PRESSURE

The type of drug coverage does not appear to impact drug usage for individuals aged 65 years and older with high blood pressure. This finding is in contrast to that of every other cohort we studied. As indicated in Table 3, the overall survey-weighted probability estimates for individuals with private coverage is 97.3%, whereas the prevalence of drug usage for individuals with public coverage was strikingly similar at 97.4%. The likelihood of an individual with private insurance taking their prescribed high blood pressure drugs in adjusted results was not statistically different from individuals who had only public coverage (OR = 0.95; 95% CI [0.88, 1.01]).

DIABETES

The prevalence of drug usage to treat diabetes, as demonstrated in Table 3, was higher among individuals with private coverage than among those with public insurance (88.9% and 84.1%, respectively). Similar to the other cohorts in our study, respondents with diabetes who had private coverage were more likely to take their prescribed medications than those with public coverage based on adjusted results (OR = 1.33 [1.28, 1.38]).

Discussion

Unlike universal coverage for hospital and physician services, there is significant variation in the coverage that Ontarians have to prescription drug insurance coverage. We found that these differences in coverage are associated with significant differences in drug utilization for individuals with three very common chronic conditions. Our results indicate that Ontarians with chronic conditions who are enrolled in a private benefits' plan are more likely to take the associated medications than those with public coverage or no drug coverage at all in five of our six cohorts. This study is consistent with previous results that have shown private coverage to increase drug utilization in those aged over 65 years (Allin et al. 2013). Further, it extends these results and shows that the differences are even larger for those aged under 65 years. These differences reflect inequities in access to medicines based on insurance coverage and also likely result in differences in health outcomes between individuals with and without private coverage.

Distinctly identifying the importance of insurance coverage on impacting access to medicines makes an important contribution to our understanding of how to reduce barriers to access. Prior studies in this area have identified several individual characteristics that impact access, such as age, chronic conditions, education and income (Osterberg and Blaschke 2005; Tamblyn et al. 2014). Insurance coverage type, unlike the other aforementioned characteristics, is very policy amenable. For example, policy makers may not be able to directly change the education or income level of individuals, but they do have the capacity to modify the availability and comprehensiveness of public insurance coverage plans. This could include lower deductibles and copayments that patients are required to pay out-of-pocket.

Comparing individuals reporting private plans with those reporting either public coverage or being uninsured reveals some important differences. With the exception of individuals aged 65 years and over with high blood pressure, all the chronic condition cohorts we studied showed that private drug coverage is linked to a greater likelihood of taking one or more of the associated prescribed medications for that condition. There are two key differences in the structure of private and public plans: the first being the size of the out-of-pocket payments paid by patients and the second is that public drug plans have a strict formulary, while most private plans do not (Kratzer et al. 2013). Given that the uninsured do not have any formulary, it seems less likely that this aspect of plan structure is a barrier to care and is not a determining factor in private plan patients having a greater likelihood of taking their medications.

This study suggests that the identified differences in the prevalence and the likelihood of taking prescription drugs likely result from how the availability of private insurance (or lack thereof) impacts the out-of-pocket payments that would be required of patients. This, of course, varies between our two age cohorts. Individuals aged under 65 years who do not have prescription drug coverage would be required to pay for all of their prescriptions out of their own pocket up to the Trillium Drug Program deductible (typically, 4% of household income).

In contrast, those with private coverage typically pay a \$25–50 deductible and some copayment (most typically 20% coinsurance) (Kratzer et al. 2013). In the case of individuals aged 65 years and over, the public ODB plan would require patients to make a small copayment for covered medicines. In this case, private plans normally pay most, if not all, of these copayment amounts, making prescriptions systematically less expensive for this group.

Limitations

There are limitations to this study worth noting. As with any survey, there is the potential for recall bias by participants. This may be true for patients who hold private drug coverage but do not use it. However, any such misreporting would likely have biased our results toward the null. There are potentially unobserved covariates that therefore have not been controlled for by the propensity score stratification. The survey did not provide information on type of employer or whether an individual was employed full or part-time, both of which are predictive of holding private employer-based insurance. Survey questions did not ask about adherence to medicines. We could only study the disease groups that questions were asked about in the CCHS survey; thus, we cannot be sure that our findings hold across other disease groups. We also could not assess whether the medicines prescribed to patients were included on the provincial formulary. Finally, the survey only measured self-reported drug use; however, our findings are consistent with those of the prior research that used administrative claims data in Ontario (Allin et al. 2013). It is impossible to know if our propensity score-matching techniques eliminated all residual confounding factors. However, as we know that the holders of private insurance are typically wealthier and healthier than those who do not, this likely would only act to make our comparisons conservative in nature.

Conclusion

Our results are highly suggestive that inequities in access to prescription drugs result from differences in access to prescription drug coverage. Overall, we found that individuals with private prescription drug coverage are more likely to take prescribed medications. This finding was consistent across five of the six groups we analyzed that encompassed three different highly prevalent chronic conditions. In particular, we found that for Ontarians aged under 65 years, private drug coverage was a major determinant in drug use for individuals with three major chronic conditions. This narrowing of the differences likely represents the impact of the universal public drug benefit for those aged over 65 years. Overall, however, our study indicates that Ontarians who have private drug insurance have greater access to prescription drugs than those who do not.

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References

- Adams, A.S., S.B. Soumerai and D. Ross-Degnan. 2001. “Use of Antihypertensive Drugs by Medicare Enrollees: Does Type of Drug Coverage Matter?” *Health Affairs* 20(1): 276–86.
- Allin, S. and J. Hurley. 2009. “Inequity in Publicly Funded Physician Care: What is the Role of Private Prescription Drug Insurance?” *Health Economics* 18(10): 1218–32. doi:10.1002/hec.1428.
- Allin, S., M.R. Law and A. Laporte. 2013. “How Does Complementary Private Prescription Drug Insurance Coverage Affect Seniors’ Use of Publicly Funded Medications?” *Health Policy* 110(2–3): 147–55. doi:10.1016/j.healthpol.2013.02.010.
- Canadian Institute for Health Information (CIHI). 2013. *Drug Expenditure in Canada, 1985 to 2012*. Ottawa, ON: Author. <https://secure.cihi.ca/free_products/Drug_Expenditure_2013_EN.pdf>
- Cochran, W.G. 1968. “The Effectiveness of Adjustment by Subclassification in Removing Bias in Observational Studies.” *Biometrics* 24(2): 295–313.
- Daw, J.R. and S.G. Morgan. 2012. “Stitching the Gaps in the Canadian Public Drug Coverage Patchwork? A Review of Provincial Pharmacare Policy Changes from 2000 to 2010.” *Health Policy* 104(1): 19–26. doi:10.1016/j.healthpol.2011.08.015.
- Dewa, C.S., J.S. Hoch and L. Steele. 2005. “Prescription Drug Benefits and Canada’s Uninsured.” *International Journal of Law and Psychiatry* 28(5): 496–513. doi:10.1016/j.ijlp.2005.08.003.
- Fang, H., M.P. Keane and D. Silverman. 2008. “Sources of Advantageous Selection: Evidence from the Medigap Insurance Market.” *Journal of Political Economy* 116(2) 303–50. doi:10.1086/587623.
- Fraser Group/Tristar Resources. 2002. *Drug Expense Coverage in the Canadian Population: Protection from Severe Drug Expenses* (p. 55). Retrieved April 15, 2015. <http://www.frasergroup.com/downloads/severe_drug_e.pdf>.
- Goldman, J.G.F. and Y. Zheng. 2007. “Prescription Drug Cost Sharing: Associations with Medication and Medical Utilization and Spending and Health.” *JAMA* 298(1): 61–69. doi:10.1001/jama.298.1.61.
- Government of Canada, S. C. (2012, September 27). *Population by Year, by Province and Territory*. Retrieved November 4, 2013. <<http://www.statcan.gc.ca/tables-tableaux/sum-som/l01/cst01/demo02a-eng.htm>>.
- Government of Ontario, M. of H. and L.-T. C. (n.d.-a). *Ontario Public Drug Programs – Public Information – MOHLTC*. Retrieved September 23, 2013. <[http://www.health.gov.on.ca/en/public/programs/drugs/programs/programs/programs.aspx](http://www.health.gov.on.ca/en/public/programs/drugs/programs/programs.aspx)>.
- Government of Ontario, M. of H. and L.-T. C. (n.d.-b). *Trillium Drug Program – Public Information – MOHLTC*. Retrieved September 23, 2013. <http://www.health.gov.on.ca/en/public/programs/drugs/programs/odb/opdp_trillium.aspx>.

- Hahs-Vaughn, D.L. and A.J. Onwuegbuzie. 2006. "Estimating and Using Propensity Score Analysis with Complex Samples." *The Journal of Experimental Education* 75(1): 31–65. doi:10.3200/JEXE.75.1.31-65.
- Hanley, G.E. 2009. "Prescription Drug Insurance and Unmet Need for Health Care: A Cross-Sectional Analysis." *Open Medicine* 3(3).
- Joyce, G.F., J.J. Escarce, M.D. Solomon and D.P. Goldman. 2002. "Employer Drug Benefit Plans and Spending on Prescription Drugs." *JAMA* 288(14).
- Kratzer, J., K. McGrail, E. Strumpf and M.R. Law. 2013. "Cost-Control Mechanisms in Canadian Private Drug Plans." *Healthcare Policy* 9(1): 35-43. doi:10.12927/hcpol.2013.23479.
- Law, M.R., L. Cheng, I.A. Dhalla, D. Heard and S.G. Morgan. 2012. "The Effect of Cost on Adherence to Prescription Medications in Canada." *Canadian Medical Association Journal* 184(3): 297–302. doi:10.1503/cmaj.111270.
- Law, M.R., J. Kratzer and I.A. Dhalla. 2014. "The Increasing Inefficiency of Private Health Insurance in Canada." *Canadian Medical Association Journal* 186(12): e470–e474.
- Marshall, K. 2003. "Benefits of the Job." *Perspectives on Labour and Income* 4(5): 5–12.
- Millar, W.J. 1999. Disparities in Prescription Drug Insurance Coverage. Health Reports/Statistics Canada, Canadian Centre for Health Information = Rappports sur la santé/Statistique Canada, Centre canadien d'information sur la santé 10(4) : 11–31(ENG); 9–30(FRE).
- Ministry of Health and Long-Term Care (MOHLTC). 2007. (May). *Preventing and Managing Chronic Disease: Ontario's Framework*. Retrieved April 5, 2015. <http://www.health.gov.on.ca/en/pro/programs/cdpm/pdf/framework_full.pdf>.
- Morgan, S.G., J.R. Daw and M.R. Law. 2013. *Rethinking Pharmacare in Canada*. Toronto, Canada: CD Howe Institute.
- Osterberg, L. and T. Blaschke. 2005. "Adherence to Medication." *The New England Journal of Medicine* 353(5): 487–97. doi:10.1056/NEJMra050100.
- Rosenbaum, P.R. and D.B. Rubin. 1984. "Reducing Bias in Observational Studies Using Subclassification on the Propensity Score." *Journal of the American Statistical Association* 79(387): 516–24. doi:10.2307/2288398.
- Rubin, D.B. 1997. "Estimating Causal Effects from Large Data Sets Using Propensity Scores." *Annals of Internal Medicine* 127(8 Part 2): 757–63. doi:10.1059/0003-4819-127-8_Part_2-199710151-00064.
- Soumerai, S.B. 2004. "Benefits and Risks of Increasing Restrictions on Access to Costly Drugs in Medicaid." *Health Affairs* 23(1).
- Stabile, M. 2001. "Private Insurance Subsidies and Public Health Care Markets: Evidence from Canada." *The Canadian Journal of Economics/Revue canadienne d'économique* 34(4): 921–42.
- Stampf, S. 2011. *Propensity Score Based Data Analysis Using Nonrandom*. Retrieved April 5, 2015. <<http://130.203.133.150/viewdoc/summary?doi=10.1.1.190.8730>>.
- Tamblyn, R., T. Egualé, A. Huang, N. Winslade and P. Doran. 2014. "The Incidence and Determinants of Primary Nonadherence with Prescribed Medication in Primary Care: A Cohort Study Primary Nonadherence with Prescribed Medication in Primary Care." *Annals of Internal Medicine* 160(7): 441–50. doi:10.7326/M13-1705.
- Tamblyn, R., R. Laprise, J.A. Hanley, M. Abrahamowicz, S. Scott, N. Mayo et al. 2001. "Adverse Events Associated with Prescription Drug Cost-Sharing among Poor and Elderly Persons." *JAMA* 285(4): 421–9.
- Zanutto, E. 2006. "A Comparison of Propensity Score and Linear Regression Analysis of Complex Survey Data." *Journal of Data Science* 4(1): 67–91.
- Zanutto, E., B. Lu and R. Hornik. 2005. "Using Propensity Score Subclassification for Multiple Treatment Doses to Evaluate a National Antidrug Media Campaign." *Journal of Educational and Behavioral Statistics* 30(1): 59–73. doi:10.3102/10769986030001059.

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