

***Mapping Patient Involvement in Drug Coverage Recommendations:
Boundary Work in the Context of Canada's
Health Technology Assessment Agency***

**A thesis submitted in conformity with the requirements
for the degree of Doctor of Philosophy (Ph.D.)**

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Abstract

This dissertation investigates the Patient Input Program, the context from which it arose and the struggles that it evokes for rare disease patients. By drawing on the concepts of boundary work, boundary object, public involvement and needs talk, the dissertation explores a local application of patient involvement as a construction of social participation and site where the needs of rare disease patients are contested among the actors, groups and institutions involved in the Health Technology Assessment (HTA) network. A case study approach was chosen to explain the intrinsic aspects of the Patient Input Program, showing the shaping of the Program and its shaping effect on societal agents and knowledge forms, particularly with respect to rare disease patients. Their experience in the Patient Input Program provides insights on the complexities and controversies rooted within the program, and links more broadly to certain system inadequacies.

Acknowledgements

First, I would like to express my gratitude to my co-supervisors Kathleen Rodgers and Michael Orsini for their continuous guidance and support throughout my Ph.D. pursuit. Your poise and insights were reassuring and motivating in times of doubt. I am grateful for your energy and intellectual curiosity, which steered me through the perils of writing. Thanks are also due to the School of Sociological and Anthropological Studies for funding that made my Ph.D. experience possible. Besides my co-supervisors, I would like to thank the rest of my thesis committee: Prof. Julie Laplante, Prof. Jose Lopez, and Prof. Nathalie Burlone, for their thorough and constructive comments. Their research perspectives allowed me to refocus the research and achieve greater academic success.

I would like to thank those who have generously given their time to take part in this study and for sharing pieces of their history and personal journeys through the healthcare system. Your strength in overcoming life's hardships and your dedication have inspired me and given me courage to face the future.

I am also indebted to my husband and friends, who have kept me grounded throughout the years. Your sense of humour was therapeutic; your genuine interest was heartfelt. To my children Lexsie and Nica, perseverance and commitment lead to great achievements!

“Laughter is wine for the soul - laughter soft, or loud and deep, tinged through with seriousness - the hilarious declaration made by man that life is worth living.”

— Seán O'Casey

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List of Acronyms

aHUS	Atypical hemolytic-uremic syndrome
AAN	AIDS Action Now
BCAM	Breast Cancer Action Montreal
BOT	Boundary Object theory
CADTH	Canadian Agency for Drugs and Technologies in Health
CDEC	Canadian Drug Expert Committee
CORD	Canadian Organization for the Rare Disorders
CCOHTA	Canadian Coordinating Office for Health Technology Assessment
CSEMI	Comité scientifique permanent de l'évaluation des médicaments aux fins d'inscription
CDR	Common Drug Review
CED	Committee to Evaluate Drugs
CER	Cost-effectiveness research
CFF	Cystic Fibrosis Foundation
EUPATI	European Patients' Academy on Therapeutic Innovation
EBM	Evidence-based medicine
F/P/T	Federal, provincial and territorial
HDD	Hereditary Disease Foundation
HRQL	Health-related quality of life measures
HTA	Health Technology Assessments
HTAi	Health Technology Assessment international
ICERs	Incremental cost-effectiveness ratios
INESSS	Quebec's Institut national d'excellence en santé et en services sociaux
NBCC	National Breast Cancer Coalition
NCCS	National Coalition for Cancer Survivorship
NIH	National Institutes of Health
NICE	National Institute for Health and Clinical Excellence
NTSAD	National Tay-Sachs and Allied Diseases Association
OCAPI	Office of Consumer and Public Involvement
OECD	Organization for Economic Co-operation and Development
pCPA	Pan-Canadian Pharmaceutical Alliance
pCODR	Pan-Canadian Oncology Drug Review
PMPRB	Patented Medicines Review Board
pERC	pCODR Expert Review Committee
PXE	Pseudoxanthoma elasticum
QALYs	Quality-adjusted life years
RCT	Randomized controlled trials
RWD	Real-world data
SDM	Shared decision-making
SMC	Scottish Medicines Consortium
STS	Science and technology studies
TAG	Treatment Action Group
WHO	World Health Organization
6MWT	Six-minute walk distance test

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CHAPTER 1

INTRODUCTION

In recent months there has been significant media coverage regarding the struggles of Canadian rare disease patients to access essential, but cost-prohibitive drugs. A recent piece on CTV News, for instance, discussed access issues with respect to Spinraza, an expensive treatment for a rare neuromuscular disorder. The segment describes how a group of healthcare activists descended on Parliament Hill, “Carrying placards and chanting “Cure SMA” and “Spinraza for all,” to urge the federal government to help fund a live-saving therapy for their condition¹. CNN News similarly published a story on Procybsi, a drug to treat a life-threatening genetic disease that destroys the kidneys. The drug’s price hiked 3,000% and resulted in a cost of more than \$300,000 a year/per patient compared to its older version Cystagon, which has been accessed by patients through Health Canada’s compassionate use program². Ben Lobb, an Ontario conservative Member of Parliament, raised the issue in a House of Commons debate on March 20th, 2018. He discussed the case of eight-year-old Olivia who has been rejected by Health Canada twice for access to Cystagon because of the market availability of Proscysbi, and asked: “Why is the Liberal health minister making access to life-saving medications difficult for most vulnerable Canadians?”³. And finally, the Globe and Mail reported that the maker of Soliris, a drug for a rare blood disorder that tallies up to \$750,000 per year/per patient had been ordered to “slash the price of the medication in Canada and pay back millions of dollars in

¹ <https://www.ctvnews.ca/health/activists-urge-ottawa-to-fund-1-million-treatment-for-rare-disease-1.3851584>

² <http://www.cbc.ca/news/health/second-opinion-procysbi-cystagon-march10-1.4570152>

³ <https://openparliament.ca/debates/2018/3/20/ben-lobb-1/>

revenue.”⁴ As these stories demonstrate, in the context of today’s healthcare system, there is a close proximity between drug accessibility and drug affordability. Excessive drug pricing is regarded as a barrier to drug access. Not only does excessive drug pricing affect the rare disease community directly, but also the issue is compounded by other challenges faced by rare disease patients.

For the majority of rare disease patients, the journey to diagnosis and treatment is a long and uncertain one, as illustrated by the story of a rare disease patient named Robert. Robert is a husband and father of two young children⁵. After experiencing complete loss of kidney function, Robert needed multiple plasma pheresis treatments (as an in-patient and out-patient) and was on dialysis for over 13 years. He received an incorrect diagnosis of thrombotic thrombocytopenic purpura (TPP) in 2002, which led to a kidney transplant that was ultimately unsuccessful. Within 2 weeks of the surgery, the disease returned and destroyed the transplanted kidney donated by his wife. Although initially difficult to diagnose, over time, other disease indicators became more apparent. In 2010, Robert was finally correctly diagnosed with Atypical hemolytic-uremic syndrome (aHUS) after he and his wife became aware of a cousin in B.C. who had been diagnosed with atypical aHUS. This hereditary discovery led them to expert physicians in the field of genetics and genetic testing. The journey to final diagnosis took 9 years, a common occurrence with rare diseases. Unfortunately, the published evidence that was overlooked at the time pointed to the fact that failed kidney transplants were more common in patients with atypical aHUS, a disease caused by a combination of genetic mutations. Like most patients with debilitating diseases, everyday Robert suffered from the symptoms of his disease, which

⁴ <https://www.theglobeandmail.com/news/national/canadian-regulator-orders-price-cut-of-expensive-us-drug-soliris/article36418703/>

⁵ Names have been changed to protect the identity of the individual interviewed.

included extreme fatigue often leading to interruptions in daily life and the inability to work. He has not been able to work since 2006 and no longer has health insurance. Due to his disease, Robert has had several hospital stays. In acute periods, he has been hospitalized for up to 6 consecutive months. He has been unable to participate actively in family life and unable to travel due to dialysis 5 nights per week (8 hours at a time).

Robert's only chance to become healthy was to receive a second kidney transplant. To undergo the transplant, Robert has to be on the drug Soliris before and after the surgery. As noted earlier, Soliris' prohibitive costs make it one of the world's most costly drugs. To date, Soliris has only been covered on a case-by-case basis in Quebec and Ontario. In February 2015, the Globe and Mail reported that Ontario was expanding its funding of Soliris to a sub-class of patients with aHUS that included about 30 Ontarians⁶. At a healthcare system level, the drug costs amount to approximately \$21 million yearly for less than one percent of the population. In contrast, the Ontario Ministry of Health and Long-Term Care reports that each year, 2.8 million people receive \$3.8 billion in drug benefits from Ontario Public Drug Programs through the administration of five provincial drug plans, including the Ontario Drug Benefit (ODB) Program and the Trillium Drug Program. Yet Robert did not meet the clinical criteria for drug funding eligibility. He has been denied funding for Soliris, the only marketed drug that can stop the disease from being active. Now Robert and his wife fight with the Province to change the clinical criteria for eligibility, while Robert's health declines.

This snapshot of Robert's life reflects the complexities of living with a rare disease. Often there is a lack of information in the scientific community on the course of a disease in

⁶ <http://www.theglobeandmail.com/life/health-and-fitness/health/ontario-to-fund-expensive-drug-for-rare-life-threatening-illness/article23208907/>

individuals and on the therapies that could benefit patients. This results in a high level of uncertainty for patients whose needs are left unmet. Furthermore, the imbalance caused by the high cost of rare disease drugs and small number of affected patients creates cost conundrums for policy-makers and drug funders because drug coverage decisions are founded on the notion that healthcare costs must benefit the aggregate. In this way, decision-makers see “evidence-based coverage as an appropriate way to control the public drug plan’s costs while maintaining access to medicines of proven benefit” (Morgan et al. 2004: 270).

Unfortunately, Robert’s narrative has become more common in Canada’s healthcare system. For this reason, this dissertation focuses on the predicament of the rare disease community and their involvement in evidence-based policy-making because “reaching out and understanding the rare disease community are primary steps to identifying inadequacies in existing systems” (Soon et al. 2014: 2). Rare disease patients form a unique community of persons with particular health needs that are determined by the rarity and severity of their condition. They also share several other commonalities: patients in the rare disease community face high drug costs, treatment access challenges and express feelings of isolation in their diagnosis and frustration with the level of care they receive. On the other side of the coin, health budgets are under enormous pressure to respond to public demand for high-priced specialty drugs. This is especially true in the Canadian context, where rare disease patients are putting demands on politicians and policy-makers to gain recognition for their health needs against a backdrop of scarce healthcare resources. Due to the lack of a comprehensive pharmacare program under Canada’s public health insurance system (i.e., medicare), patients often incur high costs for diagnosis and treatment that can be financially devastating. Morgan and Daw (2012) contend, “If Canadian medicare is a source of national pride, then Canadian pharmacare should

be a source of national embarrassment” (Morgan and Daw 2012:16). They elaborate this idea as follows:

Medicare is a cornerstone of Canadian identity. It covers virtually all of the cost of medically necessary hospital care, diagnostic tests and physician services for every Canadian, regardless of age, income or province of residence. Canadians’ pride in this system derives from the values that it represents: most fundamentally, that access to healthcare should be based solely on need and that the cost of that care should be shared among all members of society. However, there is essentially no pharmacare in Canada’s medicare system. What we have is a varied and incomplete patchwork of federal, provincial and territorial drug plans that function alongside voluntary private insurance for those able to attain and afford it (Morgan and Daw 2012: 15).

Canada is the only country in the world with a universal health insurance system that excludes coverage of prescription drugs, even though these drugs can be life-saving or life-improving. The growth of prescription drugs was significant in the mid-1980s through the 1990s in a world of heightened technology, and peaked in 1997 when pharmaceuticals “became the second-largest component of healthcare spending in Canada” (Daw and Morgan 2012: 17). Not only does the growth of pharmaceutical drugs present hope for patients, it puts a great strain on the Canadian healthcare system, notably on public drug programs. As per Aitken et al. (2009),

The retrenchment of public drug benefits is occurring against a backdrop of dramatic changes in the global pharmaceutical marketplace. In particular, the pharmaceutical industry is in the midst of a transformation from the era of the blockbuster drug – developed and sold at moderate costs for large segments of the population – to the era of the niche-buster drug – developed for very specific population groups, often those with serious unmet health needs (in Daw and Morgan 2012: 19)

Some scholars argue that Canada’s precarious healthcare system is not capable of handling these pressures equitably and efficiently because the publicly funded drug system is faced with competing demands, while having limited healthcare dollars to satisfy the needs of its diverse citizenry.

This changing landscape coincides with the advent of a new evidence-based policy movement, which has given a prominent role to evidence and the notion of “quantification”: “what can be (easily) measured is 'what counts' (both literally and metaphorically)” (Clarence 2002: 2). In resource allocation, a form of state rationing emerged almost thirty years ago to alleviate the pressures on healthcare systems. Value-for-money and cost containment approaches were conceived within the federally mandated Common Drug Review process in order to provide drug coverage at the best available price using the best available evidence. The Common Drug Review process is managed by the Canadian Agency for Drugs and Technologies in Health (CADTH) and involves an evaluation of a drug’s value based on the availability of reliable synthesised research information, known as evidence-based medicine (EBM) and cost-effectiveness research (CER). While EBM emphasizes a hierarchical classification of evidence, CER promotes standard models of costing. The general assumption in health policy is that decision-making on principles of EBM and CER leads to fair and neutral spending practices. Ultimately, drug coverage recommendations are made by CADTH’s Canadian Drug Expert Committee (CDEC) to shape provincial and territorial formulary listings intended to be uniform across the country. These recommendations are said to be controversial because they may result in negative decisions and deny access for patients in need. Hence, the drug expert committee makes complex value judgements that involve arguments related to the authority of science, patients’ unmet needs and the public good. Inadvertently, rationing exercises lead to the prioritization of some patients’ needs at the expense of others, such as rare disease patients who are often marginalized in the healthcare system.

In this climate of public discontent and competing demands, CADTH can be viewed as breathing new life into evidence-based policy-making allowing patients groups to contribute,

through a formal public involvement process, knowledge and disease experiences about drugs under funding review. In May 2010, the Agency inaugurated a Patient Input Program as part of the Common Drug Review process “in response to requests from several key stakeholders”⁷. The Program was designed to give the public a greater say in drug coverage decision-making and overcome divisions between decision-makers and the public.

1.1 Goals and Relevance of Dissertation

The dissertation seeks to understand the social dimensions of the Patient Input Program and the set of interactions and expectations therein. Despite the democratization of evidence-based policy-making and the arguments for patient participation, the Program “should not be taken for granted” just because the system opens a window allowing the expression of patients’ views and experiences (Wait and Nolte 2006: 159). There is increasing reference to the involvement of citizens and patients in the allocation of healthcare resources but their influence as stakeholders in decision-making processes is under-studied. In addition, there is no agreed upon definition or term to describe the involvement of citizens and patients in the governance of health. For the purpose of this research study, the term “patient involvement” will be used to refer to the participation of patients by way of claims-making upon the state to resolve health matters. Regarding the growing interest for patient involvement, it is imperative that we ask how patients’ understanding of disease and their lived experiences are accounted for in evidence-based coverage. To build on the existing citizen engagement literature and science studies on engagement, I have formulated the following main research question: *what is the role of knowledge and disease experience in the Patient Input Program and how do they influence drug*

⁷ https://www.cadth.ca/media/pdf/2012_SECOR_Patient-Input-Review_e.pdf

coverage decisions? In addition to the main research question, three sub-questions determined the outline of the study design.

I. How was the Patient Input Program brought into practice in Canada?

II. How are claims crafted in the context of the HTA network?

III. How is patient feedback considered in CADTH's decision-making?

More precisely, the dissertation is interested in the establishment of the Patient Input Program, in its capacity to bind different knowledge forms and join different perspectives under one collective undertaking. Central to this investigation is the emergence of the public involvement concept and the means by which it was operationalized within CADTH. In this study, I examine CADTH's Patient Input Program, investigate claims-making in drug coverage decision-making, identify potential obstacles to the enhancement of patients' influence in drug coverage decisions, while providing possible avenues to overcome these obstacles.

This research is relevant to sociology for various reasons. It speaks to the ways in which disease communities mobilize as political actors, explains the process by which patients' needs are formulated into state demands, and shows how patient groups gain legitimacy in the realm of policy. The research reveals certain aspects of social marginalisation, and points to what counts as knowledge in engagement activities. The political relevance of the study is that it provides a case study example of social participation and the interplay of governmental institutions, patient groups, and political commitments. The research offers a holistic view of patient involvement in terms of the stakeholders and factors that contributed to the Patient Input Program, and the implications of the Program on end-users.

1.2 Application of Theoretical Models and Research Design

In order to situate patient involvement in the theoretical landscape, the research reviewed public policy theories on citizen engagement, which relate public involvement to citizenship and systems of governance. In the literature, public involvement is placed within political discourses and institutional practices. A sub-set of this scholarship casts a critical eye on public involvement in terms of power relations between policy-makers and citizens. I examined literature in the fields of sociology of knowledge and science and technology studies, which explores the role of lay individuals in scientific work and the contested nature of lay knowledge against standardized knowledge forms through the boundaries of knowledge domains. Furthermore, social policy studies guided the study framework in their ability to explain the knowledge claims that underlie patients' demands and those at the root of evidence-based policy-making.

In the next section of this chapter, I present the conceptual tools of the study framework, followed by a brief overview of the main empirical findings. The chapter ends with an outline of the dissertation.

1.2.1 Boundary Work and the Creation of Boundary Objects

Instead of viewing the Patient Input Program as a simple product of bureaucracy and engagement method, this dissertation demonstrates the generative power of the Patient Input Program using the concept of boundary object. By doing so, the analysis moves beyond the outward facing policy process, and brings to light the inward values, stakes and knowledge claims, that are at the heart of drug coverage decisions. On the one hand, I investigate the capacity of the Patient Input Program to bind different actors under a common drug funding exercise, on the premise that the Program works to enhance their interactions, fosters communication among them, and leads to improved decision outcomes. More broadly, the

dissertation explains how one mechanism of public involvement has entered the boundaries of evidence-based policy, producing a particular form of evidence while acquiring a certain epistemic standing in relation to other forms of evidence. On the other hand, the dissertation looks at ways in which scientific knowledge organizes drug coverage decision-making and excludes certain knowledge forms. Here, the concept of boundary work is used to explain the monopolization of scientific knowledge, and ways that expert knowledge fields succeed in establishing self-serving boundaries. I explain how CADTH engaged in boundary work and reveal certain rhetoric and design choices for the Patient Input Program that served to secure the Agency's position as an influential actor in drug coverage decision-making.

Together the concepts of boundary object and boundary work expose the ambiguous role of patients in the Patient Input Program and some of the nuances and complexities in the Program's adoption in the real-world setting. To this end, the dissertation demonstrates how various experts and non-experts engage in boundary interactions in spite of their diverging interests and explores whether the Patient Input Program functions as a positive or negative boundary object for the actors involved in its development and those involved in its maintenance. I will elaborate on these concepts in chapter 2 of the dissertation. The next section describes the various actors in CADTH's decision-making network.

1.2.2. CADTH and its Decision-Making Network

The Canadian Agency for Drugs and Technologies in Health (CADTH) is a privileged point of entry to study the role of knowledge and disease experience in evidence-based coverage. The Agency is at the intersection of government resource planning, parliament fiscal policies and decisions, patients' claims-making on the state, drug companies' price setting, and the general public's prioritization preferences. CADTH can be viewed as a complex knowledge producing

institution and the Common Drug Review a contentious multiplayer process and delicate balancing act between the divergent views about what should be funded why and for whom. In this regard, the study centers on five main groups of actors (i.e., HTA experts, the government of Canada, the pharmaceutical industry, the public and the rare disease community) who form part of the Health Technology Assessment (HTA) network and who contribute to the Common Drug Review (CDR). The dissertation contends that the Program functions as a boundary object as it mobilizes HTA experts, drug companies, the public and patient groups, and provides the means through which actors engage with different bodies of knowledge and perspectives.

The Patient Input Program

All actors taking part in CADTH's decision-making network participate directly or indirectly in drug coverage decision-making, whether by gathering patient input, interpreting the feedback and transforming it into something analyzable and comparable, or measuring and validating it against different evidence forms. As a construction of social participation, the enactment of the Program supposes the weaving of different groups who have different stakes, expectations and demands. Below, I describe in more detail the important actors involved in the Common Drug Review process.

The HTA Experts

HTA experts form a stakeholder group that plays a central role in drug coverage decision-making. This group encompasses program officials, program advisors and committee members. Their decision-making is strongly guided by institutional policies and processes, as well as a prioritization exercise based on a drug's potential clinical benefit, likelihood of adoption by prescribers and patients, and possible incremental costs or savings, among others. Through the institutionalization of CADTH and the operationalization of rationing and evidence-based

models (Menon and Stafinsky 2008; Facey et al. 2010; Gauvin et al. 2011; Gagnon et al. 2011, 2012; Kreis and Schmidt 2013), HTA experts actively participate in the reproduction of scientific knowledge, which in turn reinforces the scientific authority of the Agency over drug coverage matters.

Besides the HTA experts themselves, their institutional context plays an important role in steering their work priorities. CADTH is an independent not-for-profit organization. However, due to its reporting relationship to the government of Canada and funding ties to federal, provincial and territorial (F/P/T) governments, the Agency is accountable to the Government of Canada for the use of federal funding. The Deputy Ministers of Health from participating F/P/T governments elect CADTH's board members and Health Canada monitors the Agency's activities through advisory committees. The Agency is also part of an international HTA network, which inadvertently influences its scope of work.

The Government of Canada

The Government of Canada is an important actor who sets the state's political climate and main priorities through political processes, such as agenda-setting, striking committees and commissions, publishing House and Senate reports, as well as positioning the parliamentary budget. By setting public engagement as a key thematic element of healthcare, this institutional actor steers the work of federal departments and agencies. Parliament and governments play an instrumental role in the approval, administration, organization and coordination of healthcare funding, and the setting of engagement opportunities and best practices. Here, the social and political contexts in which engagement is embedded shapes the forms it takes (Aronson 1993; Contandriopoulos 2004; Tritter and McCallum 2006 and Martin 2008).

The Pharmaceutical Industry

The pharmaceutical and biotechnological industry constitutes a vital stakeholder in the HTA network. Several drug companies are strong lobbyists as they have a stake in drug policy-making. Their lobbying activities are recorded in the registry of registered lobbyists, which is maintained by the Canadian Office of the Commissioner of Lobbying. In Canada, the pharmaceutical sector is composed of innovator and generic drug companies who develop and manufacture prescription drugs and over-the-counter drugs based on standards of scientific evidence. Pharmaceutical drug companies are for-profit entities that rely on market forces. These companies often employ a range of persuasive mechanisms to increase their profit margins (Matheson 2008). Several drug companies lobby and influence drug policy-making in Canada. Since the 1990s, the pharmaceutical industry has transformed considerably given the shift from blockbuster drugs to niche-buster drugs for unmet needs (Aitken et al. 2009 in Daw and Morgan 2012). The industry plays an important role in innovation, treatment access and setting drug prices in Canada through negotiations with the provinces and territories and through the pan-Canadian Pharmaceutical Alliance (pCPA). A favorable listing recommendation can signify market exposure, influence more prescribing and generate quicker revenues for companies. In other words, drug coverage is a major component of their business model because drugs that are reimbursed by drug plans will likely be prescribed by physicians and consumed by patients.

The General Public

The public at large can be considered a stakeholder of the HTA network because citizens' taxes finance Canada's publicly funded healthcare system. Canadians voice their views on topics that are relevant to them through democratic processes and media outlets. The public encompasses various groups and individuals such as patients, who are not affiliated to disease

communities, parents and relatives, and healthcare providers. The public is also represented in CADTH's Common Drug Review: the drug expert committee includes two public representatives. Their dual role is to: i) represent the societal perspective, which is to ensure that Agency spends healthcare resources responsibly, and equitably, and ii) represent the patients' perspective and ensure that their views are accounted for in drug coverage decision-making. Public members contribute directly to funding decision-making; they may support or refute patients' claims-making alongside committee experts in the field of pharmacoeconomics and biomedical sciences. These members are volunteers, who have a voting power.

Increasingly, drug coverage decision-making involves the public in order to reflect social concerns and enhance public trust in government. The public's role increased in the 1990s at a time when governments saw the restructuring of hospitals and health services as an opportunity to share decisions regarding scarce resource allocations with the public (Abelson et al. 2002). Moreover, the creation of an Office of Consumer and Public Involvement (OCAPI) within the Ministry of Health in 2000 was perceived by some as marking a cultural shift in health policy-making (McGregor 2003). OCAPI, which was abolished in 2012, promoted an infrastructure for in-house public engagement expertise (McGregor 2003; Jones and Einsiedel 2011). The Office engaged in several public involvement activities in the 2000s and sought the public's views on many health related topics. However, these engagement activities did not include patients in a permanent and structured way. It was HTA bodies, namely CADTH and three provinces (B.C., Ontario and Quebec) that paved the way for the formal inclusion of patients in drug funding reviews. Thus, patients are only occasionally involved structurally in evidence-based policy and their actual influence on decision outcomes is not well documented.

The Rare Disease Community

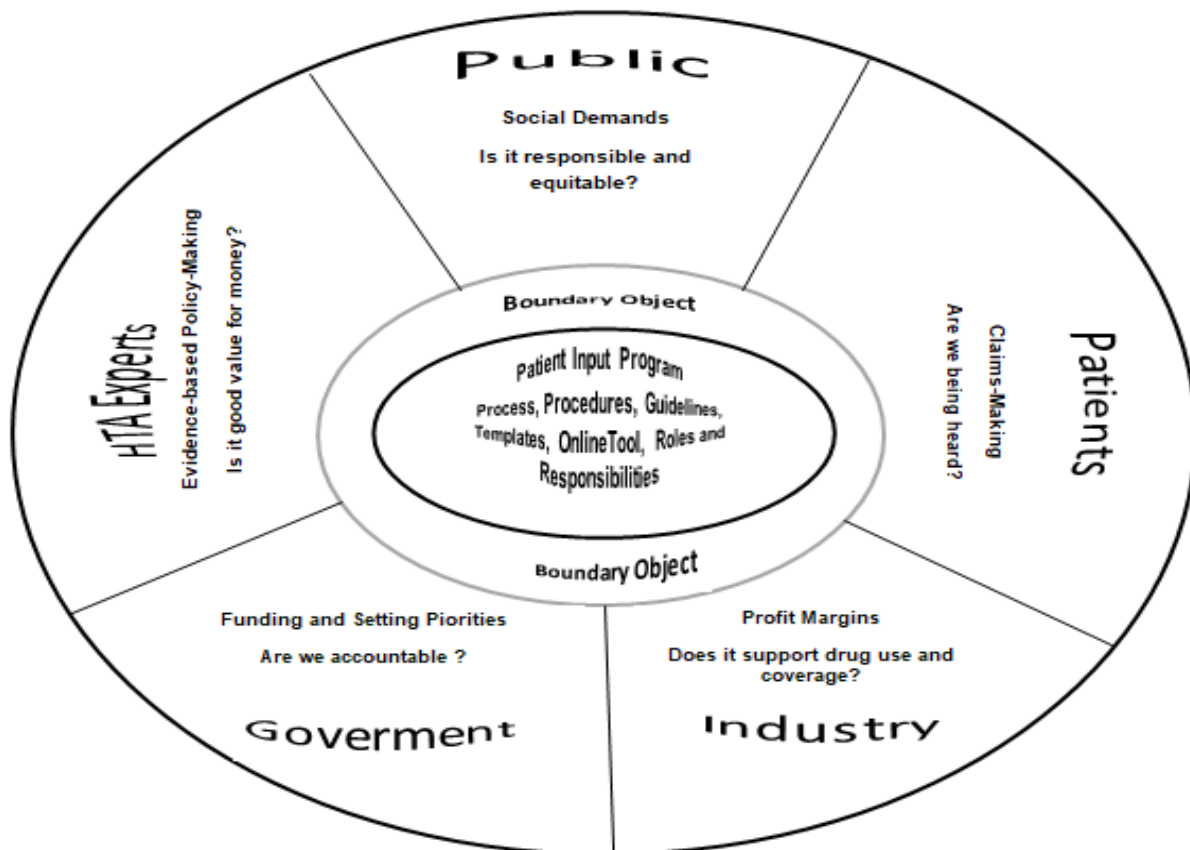
Patients and their organizations form a group of stakeholders that is of particular interest in this dissertation. This group includes patients and patient advocates, who are members of rare disease groups and/or part of the Canadian Organization for Rare Disorders (CORD). Although patients can be involved in a spectrum of engagement activities as end-users and target groups, their influence in evidence-based policy-making is not self-evident. In the case of rare disease patients, the dissertation conceives them as a vulnerable population. This statement echoes the views of Mascalzoni, Paradiso and Hansson (2014: 26), which advance that: “A patient affected by rare disease is as vulnerable as any other patient with regard to subjective experience of illness, but is actually more vulnerable in terms of healthcare availability and regulations that hamper healthcare research”. In the Canadian healthcare system, discourses on rare disease patients relate to health disparities. The rarity of the disease itself invokes claims to justice, it portrays a sense of invisibility in the face of statistical weakness of clinical studies, and it is ambiguous (Huyard 2009). Not only does a rare disease patient live with the personal consequences of his/her disease, but he/she also lives with the social consequences of that disease in terms of being subject to discrimination in the provision of healthcare services and health insurance.

As demonstrated above, the network of institutions and social groups encompass those who provide disease perspectives, influence policies and practices around patient input, as well as handle and manipulate patient input within decision-making frameworks. The HTA network comprises the totality of actors from different communities of practices and factors that determine drug coverage decisions. These communities of practice or social worlds refer to the membership of a social group, in which individuals conduct activities together. Over time, a

community of practice acquires shared lessons, conversations and expertise in a specific domain of activity (Bowker and Star 1996). A community of practice joins people together by conventions, language, practices and technologies. In other words, communities of practice can be described as groups having legitimacy and authority in a field of expertise and can have different audiences and pursue different tasks (Star et al. 1998). The actors forming part of the HTA network may have diverging interests and varying degrees of influence on drug coverage decision-making.

Figure 1.2 provides a portrait of the HTA decision-making network, the key actors, their stakes and the questions provoked by the Patient Input Program.

Figure 1.2 The HTA Decision-Making Network



It is in this context that the Patient Input Program has the potential to connect patients' claims-making to the drug coverage decision-making apparatus, and has the ability to open a dialogue among the various actors within the HTA network. To reiterate, the Patient Input Program becomes the interface by which different actors in the HTA network engage with certain bodies of knowledge and perspectives. In the following section, I discuss through the lens of citizen engagement the role envisaged for patients in drug coverage decision-making as knowledge producers in the same way as HTA experts.

1.2.3 The Many Faces of Public Involvement

The inclusion of patient groups in the governance of health does not necessarily imply the actual involvement and influence of patients in decision-making processes. In contemporary society, both scientific and health policy communities have embraced the concept of patient involvement and oftentimes, have provided the means to interaction with patients. Because of this interaction, patients contribute a form of evidence (though usually not regarded as factual and objective) that can support research, policies and programs. Within this evolving context of social participation, patient involvement appears as a new space of practice connecting decision-makers and patients who are end-users of healthcare services. The literature on the involvement of lay experts in health research and biomedical sciences has taught us about the relevance of patient participation in knowledge producing environments (Epstein 1995; Collins and Evans 2002; Boeva and Foster 2016). It also emphasized on the role of patients and health consumer groups in shaping health policy agendas (Jones, Baggott and Allsop 2004; Abma 2006), as well as biomedical policies and research (Oliver 1996; Shea et al. 2005; Terry et al. 2007; Rabeharisoa and Callon 2012; Landy et al. 2012). Here, lay expertise is understood as a new resource contested by biomedical experts (Caron-Flinterman et al. 2005, 2007), which also

contributes to health democracy (Akrich and Rabeharisoa 2012). Many scholars have explained the successes of high profile patient organizations, particularly cancer and HIV groups, including some rare disease communities, in the participation of healthcare, underlining their illness experiences (Anglin 1997, 1998; Roberts 2002; Klawiter 2004), while revealing certain institutional biases that limit their participation (Baggott et al. 2004). This scholarship stresses that integrating lay expertise in governmental/expert decision-making is not clear-cut because patients are not structurally involved as partners and because expert decision-making networks can be reductionist and specialized (Caron-Flinterman et al. 2007).

Patient involvement in evidence-based policy-making can be problematized in a similar fashion. Patients are generally invited to participate in engagement activities only on an adhoc basis and in a non-structured way. Although the Canadian experience in health policy is rich with examples of public engagement initiatives, few studies have examined how their disease experience and knowledge is incorporated in decision-making frameworks. This under-studied topic may be explained by the fact that engagement in health policy, like participation in biomedical policy and research, rarely entails the formal involvement of patients in decision-making processes. However, the year 2010 marked a turning point in institutional decision-making. Health Technology Assessment (HTA) bodies started providing permanent channels to include Canadians in their evidence-based decision-making processes. At the federal level, the Canadian Agency for Drugs and Technologies in Health (CADTH) produced the Patient Input Program, thereby creating an opening in the Common Drug Review for a feedback mechanism that allows patients to make pleas for drug coverage. Until recently, experts within CADTH made rationing decisions in the allocation of healthcare resources. A growing sub-set of public policy scholarship emerged around engagement methods in HTAs and the role of the public as

influential stakeholders in evidence-based policy (Abelson et al. 2002, 2007; Bridges and Jones 2007; Menon and Stafinsky 2008; Facey et al. 2010; Bombard et al. 2011; Gauvin et al. 2010; Gagnon et al. 2011, 2012; Kreis and Schmidt 2012).

In general, public policy works advance a notion of citizenship on the agency of citizens in state interventions or the design of services. I now discuss this form of citizenship through science and technology studies, which relate a new form of patient activism to redefined knowledge boundaries and reconfigured boundaries of health governance.

1.2.4 Redefined Knowledge Boundaries and Reconfigured Boundaries of Health Governance

Until the late 1980s and 1990s, disease organizations started to offer more than psychosocial benefits to patients. They gained disease knowledge that enabled them to infiltrate media, public policy and scientific circles. For example, AIDS and cancer activists succeeded in making their demands public through aggressive campaigning and resourceful networking with key actors in policy and scientific domains. These groups actively shape expert knowledge, which in turn is used to make demands on the state and sustain their claims to health equity. They work from within the boundaries of science and politics to assert their needs. In the words of Akrich et al. (2013), evidence-based activism “constitutes a powerful leverage which allows patients’ organizations to penetrate others’ territory, to redefine borders, to bring in new entities and new issues, so that the whole geography may be turned upside down in some cases” (Akrich et al. 2013: 13). This evolving landscape of patient activism includes the emergence of informed patient groups, who engage with bodies of knowledge and advance their claims on alternate understandings of science and disease that have the potential to conflict with that of dominant institutions of science. In this dissertation, patients’ alternative understandings of science and disease can be compared to the concept of situated knowledge used by Akrich and Rabeharisoa

(2012; 2016). Situated knowledge refers to a patient's ability to acquire a two-dimensional perspective on their disease, namely a mixture of experiential knowledge (which is described as patients' experience of a specific health condition) and medical-scientific expertise (understood as academic knowledge). To gain an alternative understanding of science and disease, patient groups assess their collective disease experience against the existing scientific evidence to further knowledge and understanding of their condition. Here, the construction of knowledge becomes a social endeavour and involves a process of weighting up academic knowledge on disease experiences:

This work on and with academic and experiential knowledge contributes to sorting out, ordering, and articulating an understanding of their conditions and the problems they induce, for themselves and for specialists. This reconfigures the epistemic network they make themselves part and parcel of, and results in politics of knowledge that eventually transforms the modalities of research, and in the production of new evidence for grounding health policies in their condition areas (Akrich et al. 2013: 2).

On this point, the dissertation is interested in how new forms of patient activism play out in drug coverage decisions founded on claims of scientificity, rationality and academic study (Marston and Watts 2003). As mentioned earlier, evidence-based policy is the driver in health resource allocation and is practiced on principles of evidence-based medicine and cost-effectiveness research. Since evidence-based policy is embraced and controlled by technocrats⁸, this way of knowing has the potential of privileging the knowledge of some over others. In this regard, claims-making by patient groups and health institutions has a scientific and political dimension.

⁸ Within this dissertation, a technocrat is defined as a scientist or other expert who is one of a group of similar people who have political power as well as technical knowledge (The Collins English Dictionary).

Further, attending to the claims-making of patients points to patient groups and the way they mobilize around disease identities, socialize their disease through membership, and make political demands for their unmet needs on public institutions. Social policy scholars have explained the politicization of needs in the realm of health policy, and revealed the contested nature of needs between those who determine needs and those who are claimants of needs (Fraser 1989; Aronson 1993; Tronto 2010). The dissertation discusses the ideological struggles and unequal power dynamics among different social agents in the determination of those needs (Fraser 1989). Not only does claims-making refer to ways that groups themselves frame and strategize their needs in the public eye, but it also refers to ways that elite groups attempt to reframe patients' needs into manipulable forms. In this regard, the Canadian Organization for Rare Disorders (CORD) was instrumental in formulating patients' needs into political demands against elite needs interpretations, influencing political agenda-setting and ministerial commitments, as well as shaping the space of practice between policy-makers and rare disease patients. CORD's presence and advocacy work made visible the struggle of rare disease patients in the political sphere. In turn, governments came to recognize the suffering of rare disease patients and prioritized their needs. This new interaction between rare disease patients and governments led to the creation of an Orphan Drug Framework aiming mainly to foster international collaboration in rare disease research and create hubs of domestic expertise on rare diseases. Hence this relationship can be viewed as a test bench for patient involvement processes. It is through this synergy that the dissertation begins to explore the Patient Input Program as a bridge between civil society, policy and science; a bridge that carries with it unspoken expectations from all sides of that relationship. Under the leadership of CORD, the needs of rare disease patients were successfully formulated into political demands, thus shifting their private

struggles onto the public agenda. It is therefore relevant to ask if the politicization of patients' needs in the rare disease community enhanced their role in the Patient Input Program?

1.3 Empirical Findings

To document an untold chapter of patient involvement, the research questions were explored empirically by scrutinizing CADTH's Patient Input Program as it passed from a policy goal to a patient involvement practice. CADTH provides a useful context for the study to the extent that it serves to investigate how governments interact with patient communities on matters of health, and how patients influence (or not) decision-making processes. To achieve triangulation, the case study drew on three forms of data, including public documents and archives, audio-visual materials, and interviews with 30 key informants, such as CADTH officials, public and scientific drug expert committee members, patient activists, patient organization administrators, and patients diagnosed with a rare disease. I now present the overall findings of my research and briefly discuss its theoretical contributions.

The main findings of my research indicate that:

- The Patient Input Program emerged from a distinct set of social transformations and pressures that pertain to both the local and global spheres, including the emergence of a new patienthood, healthcare reform, political willingness regarding issues of public interest, and rise of evidence-based policy-making.
- In health policy circles, patients are generally invited to participate in engagement activities only on an adhoc basis and in a non-structured way. Yet Health Technology Assessment (HTA) bodies prefer including patients in involvement structures. In spite of this structured involvement of patients in the Common Drug Review process, patients

remain as outsiders in drug coverage decision-making and as a result, have a ‘voice without influence’.

- Because patients are not included in the drug expert committee’s decision-making process and their voice is channelled through community leaders and public members, they have an unequal leverage power within the HTA network, which is also reflected in the determination of their unmet needs.
- Despite the politicization of the needs of rare disease patients through the work of CORD, in drug coverage decision-making, dominant needs interpretations appear to trivialize and depoliticize the personal and social consequences of living with a rare disease, and inadvertently push them in the private domain and outside the realm of public policy.
- On this point, the logics of scientificity, rationality and academic study that underpin evidence-based drug coverage reinforce a hegemonic medical understanding of disease solely represented by physical impairments, rather than the personal and social aspects that accompany the impairment.
- CADTH’s use of standardization methods, processes and representativeness to capture patients’ knowledge and disease experiences has the effect of delegitimizing patients’ input and further marginalizing vulnerable individuals and groups.

The dissertation argues that both the government and patient groups contributed to a new patienthood in the healthcare system. Governments transformed citizens from subjects of the state to individuals who take increasing responsibility for health-related decisions and exercise agency, while patient groups redefined knowledge boundaries and reconfigured boundaries of health governance through a new form of patient activism. Not only did governments come to

view patients as a specific form of citizenship, identified by their unique biologic characteristics and health needs, but patient groups contribute to evidence-based activism (Akrich and Rabeharisoa 2012) which advances a repertoire of patient voices that has both elements of biomedical knowledge and experiential knowledge. However, in drug coverage decision-making, the dissertation demonstrates that though CADTH recognizes the value of patient voices, the Agency engaged in boundary work and placed a social boundary between expert and non-expert knowledge in the HTA decision-making network, devaluing patients' knowledge and disease experience. Furthermore, the Agency blurred the lines between social values and patients' values by maintaining an obscure role of public members (i.e., not defined and not distinguished from patients), thus creating competing demands between the public's best interest and patients' best interest.

The dissertation also contends that the patient involvement concept has received broad support in various health domains and appears to foster a new relationship and compromise among actors within the HTA network. Yet at the local level the usefulness of the Patient Input Program is contested by certain actors since it creates challenges in terms of understanding the 'unconventional' form of evidence provided by patients. As a boundary object, the Patient Input Program gives the illusion that it reconciles the interests of all stakeholders within the HTA network and advances that drug coverage decisions are socially just and follow a reasonable and democratic evidence-based approach. I suggest that the Patient Input Program functions as a positive boundary object for governments and policy-makers in terms of accountability and social responsiveness, but functions as a negative boundary object for HTA experts who contest the legitimacy and credibility of patients' feedback, and patient groups who face institutional biases. In other words, the Patient Input Program is not optimally used by HTA experts and

patients because its relevance, validity and impact are limited. The findings suggest that patient groups have unequal status and resources, which inhibit their ability to satisfy CADTH's institutional expectations. Moreover, the dissertation emphasizes that the reduction of patient involvement to an indirect paper exercise does not enable the agency of patients as makers and shapers of healthcare planning and resource allocation.

The dissertation also explains how the personal struggles of rare disease patients seeped into public policy because of CORD's ability to formulate patients' needs into political demands against elite needs interpretations. By doing so, CORD reframed patients' needs into a discourse about treatment access. Despite this political visibility, I contend that dominant needs interpretations can have the effect of deflecting attention from important political questions that pertain to the bigger problems, including excessive drug pricing and over-restrictive clinical criteria to qualify for drug coverage. Hence, the politicization of patients' needs by CORD did not enhance the role of rare disease patients in the Patient Input Program because evidence-based policy-making adopts a narrow view of what counts as valid knowledge and privileges technocrats and policy-makers as knowledge producers.

Although the Patient Input Program can be viewed as an inhibitor to meaningful and influential involvement of patients, the dissertation suggests that failed attempts at patient involvement can motivate patients to think of innovative ways to influence the system, such as the Right to Try movement led by patients with Amyotrophic lateral sclerosis (ALS). These patients by-passed all established involvement mechanisms to steer public attention to the issue of accessing experimental drugs. Their actions trigger investigations on ways that patient groups can contribute to policy-making without formal structures of engagement and outside formal solicited involvement processes.

1.4 Organization of the Dissertation

The dissertation is organized into seven chapters. Following this Introduction, chapter 2 provides a detailed review of the Patient Involvement literature, including public policy works related to citizen engagement. The review also explores the literature from the lens of sociology of knowledge and boundary object theory, which has become a central analytic framework in the field of science and technology studies. The chapter contains both theoretical considerations and empirical evidence relating to disease communities, knowledge boundaries and public engagement. Chapter 3 outlines the research design, describes the case study and outlines the methods of data collection. Chapter 4 provides a historical overview of patient involvement and the enabling conditions that led to the inauguration of the Patient Input Program. Chapter 5 explores the circulation of needs discourses as they relate to patients, communities of practice and institutions of knowledge production, while chapter 6 explores the weaving of individuals and stakes in the shaping of patient involvement practices. Finally, chapter 7 summarizes the findings, discusses the study's limitations and challenges, and reflects on the implications of the findings for policy-makers and for future research.

CHAPTER 2

CURRENT UNDERSTANDINGS OF PATIENT INVOLVEMENT

2.1 Introduction

In order to begin to address the central inquiry of the dissertation on the role of knowledge and disease experience in the Patient Input Program and how they influence drug coverage decisions, this chapter outlines the literature on patient involvement and the contribution of patients in decision-making apparatus. Although the body of literature is not always cohesive, it provides lines of inquiries that help us understand the role of patients in involvement activities and the nature of patients' health claims in today's evidence-based society. As previously mentioned, within the last decade the Canadian Agency for Drugs and Technologies in Health (CADTH) began considering patient-based values in the drug coverage decision-making process. The Patient Input Program offers a permanent channel for patient groups to make health claims by voicing "their needs, wants, aspirations, as well as hopes and fears" with respect to needed drug therapies (Bridges and Jones 2007: 33). To that end, through a social process of participation, patients produce a repertoire of patient voices derived from their individual and collective health needs.

To begin, this chapter conceptualizes patients' experiential repertoire by turning to the scholarships on illness identities, claims-making and lay expertise. Secondly, a special focus will be given to the boundary work and boundary object literature due to the intersecting corridors of different knowledge forms and perspectives within the Patient Input Program. Finally, the literature on citizen engagement will be discussed in terms of the democratic nature of social participation. Three main objectives structure the literature review: i) characterizing patients in engagement processes; ii) understanding claims with attention to knowledge boundaries between

science and society, and experts and laypersons; and, iii) providing conceptual clarity with respect to the aims and function of patient involvement in evidence-based policy. The literature was drawn from a broad range of disciplines and serves to help situate the framework of this study.

2.2 Illness Identities and Collective Mobilization

The concept of illness has been thoroughly examined by medical sociologists. Some topics of interest are the notion of illness careers (Gerhardt 1990; Corin 1998), illness as a somatic and symbolic construction (Nichter 1981; Darghowth et al. 2006), and illness as a lived experience (Moss and Dyck 2002). Several scholars have also argued that illness identities have emerged around novel technologies and therapies, which have the effect of mobilizing patients and allowing them to interact with biomedicine. Not only do new forms of patient citizenship materialize, but new categorizations are created of people who are carriers of genetic diseases (Wheling 2011) and/or people who may be future users of medical therapies (Clarke et al. 2000).

Historically, medical sociology has focussed on the hegemonic power of the biomedical paradigm, conceived as a reductionist disease theory through the medicalization (Illich 1975; Armstrong 1995) and pharmaceuticalization (Conrad 1992) of society. As per Clarke et al. (2000), pivotal changes such as infrastructure changes, the creation of a managed care system, heterogeneous modes of information production, as well as privatization, decentralization and globalization, led to a shift in medical paradigms. In this new biomedicalization model, medical technologies and pharmaceuticals are conceived as vehicles of meaning, markers of change in social relations and practices, as well as political tools to govern and reshape human bodies and lives. While some science and technology studies scholars examine the impact of technologies on patients' narratives (Browner 1999; Roberts 2004; Rapp 2007), others study how new

technologies, namely genetic research, create new social and ethical practices that are supported by a new way of thinking in terms of risk management (Rabinow 1999). This new way of thinking has emerged on a molecular vision in which life is dissected and manipulated by technology; these techniques and discursive practices program knowledge and assumptions about human beings. The 1990s were a time of scientific and technological changes, including significant advances in genetics, which resulted in the Human Genome Project. For Canadian scholars Bouffard (2000) and Robitaille (2008), the ideological backdrop of the Human Genome Project is one of perfecting humanity through the use of medical technologies on the alleged unfitness of human beings (e.g., the absence of body parts). This underlying assumption serves to legitimize research aimed at adapting humans to the modern world. That said, the introduction of genetics in the social sphere not only allows society to change the fate of human beings, but also changes the rules that define human identity, control reproduction and determine the rights of citizens. Rabinow (1996) refers to this process as biosociality, a new form of collective identity that is built from the human genome and dictated by social desires.

The socialization of patients and diseases is a key element of biosociality, which refers to the formation of illness identities around new categories of citizens based on “corporeal vulnerability, somatic suffering, and genetic risk and susceptibility” (in Rose and Novas 2004: 444). Rabinow (1996) uses the term “biosocial groupings” to describe collectivities that form around a biological conception of a shared disease identity. Hence, individual patients associate with a disease identity and create a relationship with their condition. For instance, in the area of rare diseases, patients develop a specific language around disease prevalence, disease complexity and disease inheritance (i.e., an individualizing process). They also unify under a shared disease identity to advance their health needs in the public healthcare system (i.e., a collectivizing

process). These newly formed biosocial collectivities are sometimes specified in emerging forms of genetic or biological citizenship: “we have seen the rise of new kinds of patients’ groups and individuals, who increasingly define their citizenship in terms of their rights (and obligations) to life, health and cure (Rabinow and Rose 2006: 203). The concept of biological citizenship was first introduced by Petryna in 2002 and later in 2004 by Rose and Novas (2004) to explain that in the contemporary age of biomedicine, a new form of citizenship is manifesting around citizens’ injured biologies and is the basis for social membership and staking claims (Petryna 2004: 261). As demonstrated by Petryna in the case of sufferers of the Chernobyl disaster, biological citizenship is viewed by individuals as a desirable category because it can be tied to financial security, social inclusion and survival. In this way, illness identities trigger new forms of citizenship. For example, Nguyen et al. (2007) use the concept of therapeutic citizenship to describe the political claims of belonging to a disease community and accessing drug therapies for AIDS patients. Seen in this light, the concept of biological citizenship refers to notions of triage, membership and material gains. It is used to further examine how patients gain recognition of their disease identity in scientific, regulatory and policy settings through both individualizing and collectivizing processes.

Clarke et al. (2000) further build on Rabinow’s concept of biosociality by contending that illness identities do not simply arise from biomedical classifications and genetic conditions. Rather, illness identities are negotiated and contested among lay and expert actors. They are negotiated in heterogeneous ways (Wheling 2011). In this regard, Rabearisoa and Callon’s (2002) study of patients with muscular dystrophy is exemplary in demonstrating the formation of a collective identity created by l’Association des paralysées de France around the aspects of being handicap and having a defective gene. On the one hand, the association socialized patients

with muscular dystrophy as political actors and scientific contributors. On the other, the association mobilized different social actors and created a sphere of solidarity around the general recognition of their humanity, thereby educating society and alleviating obstacles to their mobility. The scientific and political dimensions of patient and disease socialization encompass new collaborative models, relations and strategic alliances among those who participate in biomedicine. Here, medical sociology and science and technology studies have demonstrated the agency of medical technologies, but also the formation of patients' identities through collective mobilization.

Modes of collective mobilization provide patients the ability to change the social configuration among lay individuals and experts in various health domains, as well as change the locus of control over health. Medical sociology and anthropology have demonstrated how patient groups not only center their efforts on materiality in terms of cures and therapies, but they can also change the distribution of resources in decision-making processes. Best (2013) argues that patient groups in the U.S. were able through advocacy activities to secure direct benefits in the form of increased medical research funding: they produced direct benefits for their constituents. Moreover, patient groups were able to transform the categories and meanings that shaped the politics of medical research funding. By making claims based on dollars per death, patient groups encouraged policy-makers to use mortality as a metric to commensurate diseases. These claims led to a cultural change in the funding distribution "shifting money toward high-mortality diseases and away from stigmatized diseases" (Best 2013: 795). In other words, patient groups develop disease identities and disease knowledge through mobilization and political action. This can have the effect of redefining the politics of scientific knowledge. For example, Anglin's study (1998) illustrated how activists in the area of breast cancer provided an alternative reading

of breast cancer thereby redefining cancer etiology and prevention through the lens of environmental pollution. This process entailed recognizing a “different science” that offered women hope of improved prognosis. In addition, it took “a willingness to understand and contest the alignments of power that produce environmental toxins, and science, with its bias towards the status quo, as part of everyday life under transnational capitalism” (Anglin 1998: 202).

As demonstrated by the literature above, the role of patients and their interaction with biomedicine is well established as patient groups have reshaped the normative contexts of biomedical research and their futures (Novas 2006). Yet the distinctiveness of patients as a stakeholder group is underdeveloped in the public policy scholarship. In general, scholars use the term “public” to describe end-users of involvement. For example, Kreis and Schmidt (2012) use the term public “as the broadest generic term to include engagement of individual citizens, patients, consumers, laypeople or formal or informal representatives of groups of these” (Kreis and Schmidt 2012: 91). Although there is recognition in the literature that there is a diversity of publics in engagement activities, few scholars have attempted to differentiate these different public audiences (Facey et al. 2010; Gagnon et al. 2011; Menon and Stafinsky 2011). The following section explores the notion of public engagement audiences.

2.2.1 Public Engagement Audiences

Science and technology studies have shown how the public, namely patients, have become participants in the biomedical enterprise by articulating different forms of knowledge and engaging in the production of scientific knowledge (Caron-Flinterman et al. 2005; Rabinow 1996; Wheling 2011; Akrich and Rabeharisoa 2012 and Akrich 2016). Public policy scholarship by contrast examined the public as influential stakeholders in deliberative processes and evidence-based policy-making (Baggott et al. 2003, 2004; Abelson et al. 2002, 2007; Gagnon et

al. 2011, 2012; Gauvin et al. 2010). First, public policy scholars contend that the notion of public is ambiguous.

The nature of the public is an elusive facet of public involvement. At no point do we find agreement in the literature or in interviews about who the public is or ought to be. Neither do we find agreement on the most appropriate terms with which to define the public (Gauvin et al. 2010: 1522).

Gagnon et al. (2011) provide preliminary insight on two broad categorizations of publics in engagement activities. The first category includes citizens and representatives of citizens who usually have fragmented interests and who provide a societal or lay perspective. This public is not affiliated with organizations with a financial or professional interest in the healthcare system and have the democratic task of ensuring correspondence of the government decisions to the public's wishes (Martin 2008). The second category includes patients or caregivers whose perspective is directly affected by a health condition, and who may be affiliated with organizations that have financial or professional interests in the healthcare system (i.e., patient groups or health consumer groups). Often, this public is perceived as biased because they represent patient and service user groups. In the context of health technology assessments (HTAs), citizens provide input into the relative value of a technology in comparison to other therapies under consideration for funding, whereas patients as users of health services provide insights into the value of a technology through their direct experience with the condition for which the therapy is used (Menon and Stafinsky 2011). That is, "patients have a legitimate personal interest in the technology they wish to receive, while citizens share broader long-term interests in the health system themselves (as voters, taxpayers, and members of the community) and the welfare of their fellow citizens" (Facey et al. 2010: 75). A patient can provide views about living with the illness/condition or the intended and unintended consequences of a

technology whereas citizens have an interest in the efficiency and fairness of the health system (Facey et al. 2010). Citizens apply their priorities and patients draw on their experience (Tritter 2009). Here, public engagement reflects a tension between collectivism and individualism. On the one hand, citizens represent society and speak for the collective. On the other, patients represent their interests, values and preferences. The use of the term “citizen” as a catch-all term for any participant who wishes a voice in healthcare decisions is a conceptual oversight because different publics (including patients and patient groups) have different bases of legitimacy, statistical representation and political efficacy (Contandriopoulos 2004). Different publics also have different stakes as suggested by Abelson et al. (2007), who offer a distinct conceptualization of stakeholders and the public.

Although often used interchangeably, the terms “stakeholders” and “the public” are not the same thing. Stakeholders, as the term suggests, are parties that have a ‘stake’ (self-interest in terms of resources, power, etc.) in a given issue (e.g., professional, consumer advocacy groups and pharmaceutical companies). Technically, the public also holds a stake on many issues, but representing the public’s interest incorporates a much broader, diffused and fragmented set of interests that are not easily mobilized (Abelson et al. 2007: 43).

For these scholars, the notion of public is socially constructed and can be manipulated by those with strong interests. “Stakeholder involvement presented as public involvement gives greater voice to professionals and industry interests than to citizens and patients. When ‘public’ and ‘stakeholders’ both sit at the table, inequalities in their powers of persuasion must be overcome if the public perspective is to have any force” (Abelson et al. 2007: 44). To this end, the public can be characterized as diverse and having different degrees of influence in policy-making.

As users of healthcare services and recipients of medical treatments, citizens and patients can be considered as key stakeholders in involvement initiatives. They too have stakes in a given issue. On this point, patient organizations have succeeded to mobilize people and resources in response to illness, suffering and health inequities. Public involvement allows affected stakeholders, through participatory practices, to share their experiential knowledge. As alluded by Baggott et al. (2004), patient organizations have power resources, namely mobilizing media support and seeking alliances with more powerful interests. The authors raise questions about the powers of patient organizations, and differentiate between groups who are insiders and those who are outsiders. They argue for public involvement in policy processes “to curb the monopoly powers of health professionals and to build public support for particular health policies” (Baggott et al. 2004: 753). Public policy studies point to the power dynamic between professionals and the public through the strategies of healthcare consumer groups. Yet through this scholarship, the role of patients in involvement initiatives and the distinction between societal views and patient values have not been fully fleshed out. I now turn to science studies on engagement to further clarify the contribution of patients in health policy-making.

2.2.2 Lay Expertise and Lived Experience

For their part, science studies on engagement have taught us that lay actors have a role alongside expert actors. This scholarship examines who can claim to be an expert in relation to others who interact in scientific work. Some scholars looked at expertise in technology design and production to illustrate how recent library developments, aimed at increasing technical literacy, brings non-experts and experts in a dialogue (Boeva and Foster 2016), while others have shown the contested production of expert biomedical knowledge and work of AIDS activists as non-experts in changing scientific practice (Epstein 1995). For Collins and Evans (2002), non-

experts contribute lay expertise, which is described as experience-based expertise (not validated by degrees and certifications). In this way, science studies scholarship on engagement explores who has the power to legitimize or bring different forms of expertise into decision-making processes.

As previously remarked, several social scientists have offered critical insights about the potential for laypersons to challenge dominant paradigms in science. As the literature established, the 21st century patient can make health claims based on their biological characteristics. Originally, the social science literature contrasted expert claims, based on universalist knowledge, against lay people's situated understandings, grounded on experience of local conditions (Weiner 2009: 256). However, rather than starting with the basic assumptions of binary differences between knowledge types, some science and technology studies explored the relation between biomedical expertise and patients' insights from an epistemological lens (Caron-Flinterman et al. 2005; Rabinow 1996; Wheling 2011; Akrich and Rabeharisoa 2012 and Akrich 2016). Caron-Flinterman et al. (2005) describe biomedical knowledge and patient knowledge as hybrid formations: a fusion of experiential knowledge, propositional knowledge and practical knowledge. First, the authors view experiential knowledge "as the often implicit, lived experiences of individual patients with their bodies and their illnesses as well as with care and cure" (Caron-Flinterman et al. 2005: 2576). Experiential knowledge is confirmed by sharing experiences with other patients whereas propositional knowledge is gained through a patient's familiarity with their bodies, disease and treatment, as well as with their social context. Patients acquire practical knowledge in terms of their physical and mental coping strategies. Caron-Flinterman et al. (2005) argue that:

Both experiential knowledge of patients and biomedical knowledge of scientists comprise the same three types of knowledge (i.e.,

experiential knowledge, propositional knowledge and practical knowledge), but their distribution and order of genesis differ. While biomedical knowledge concerns external objects and is mainly acquired through detached and impersonal study and observation, patients' experiential knowledge concerns the personal situation and is acquired through personal and bodily experiences with the disease (Caron-Flinterman et al. 2005: 2577).

In this respect, patients' experiential knowledge can complement the biomedical knowledge of professionals. The contribution of science and technology studies is to abandon: i) the opposition between knowledge of science and knowledge of society, and ii) the distinction between objectivity and subjectivity because both forms of knowledge require an assembly of objective and subjective knowing. This scholarship has demonstrated the potential for other knowledge forms, notably that of patients, to break knowledge boundaries and bring resistance to mainstream science. In the context of the Patient Input Program, this would suppose a breakdown in the hierarchy of knowledge, a complementarity between various forms of knowledge and a willingness of individuals involved to acknowledge difference (Tritter and McCallum 2006: 164).

In their critique of scientific objectivity, feminist science and technology studies explore the social construction of knowledge in terms of who knows and how one can know (Haraway 1988, Harding 1992). For instance, Harding (1992) contends that a person's social situation enables or limits what one can know. Furthermore, feminist scholars point to the ways in which beliefs achieve knowledge status. For her part, Haraway (1988) conceives patients' beliefs as situated knowledge defined in terms of partial views joined into communities (Haraway 1988: 590). Here, situated knowledge is a process of achieved perception and empowerment can be viewed as "elevating people's knowledge, in particular their situated and communal knowledge (Haraway 1991 in Goodson and Phillimore 2012: 92). This research tells us that patients'

knowledge can also encompass knowledge of relevant scientific fields and knowledge of how science works institutionally and culturally. Defining patients' knowledge as a two-dimensional perspective therefore blurs the lines between patients as lay individuals and experts in the roles they play. Not only can patients provide knowledge about their lived experiences, but they also can contribute to scientific knowledge. Thus, patients have the capacity to bind knowledge forms and move away from being passive receivers of information. Seen in this light, it becomes possible to view knowledge as an endeavour of collaboration and co-construction. Knowledge is a co-discovery among social agents and a blending of partial knowledge forms. Interestingly, these works further spell out what patients as stakeholders of healthcare can contribute to health decision-making in terms of their agency. Feminist scholars (Haraway 1988, Harding 1992) suggest that the social construction of scientific knowledge entails a negotiation process among individuals rather than a simple transfer of knowledge between them (the experts and lay individuals). They further examine knowledge as an act of knowing the world through interactions that generate socio-political differences. Akrich and Rabeharisoa (2012) further build on these conceptualisations of experiential knowledge by stressing the political dimension of knowledge articulation. They contend that patient organizations engage in political strategizing when they articulate lay expertise as a hybrid form of experiential expertise and medico-scientific expertise, targeted at both lay and expert audiences. The authors refer to a new form of evidence-based activism (Akrich and Rabeharisoa 2012: 6). By doing so, patient organizations strengthen their capacity for action in the governance of health, while putting pressure on those who govern the healthcare system.

The capacity of patient organizations to articulate knowledge forms that engage or intervene in dominant biomedical discourses cannot be understood without an account of

biomedical knowledge production and the boundaries of what counts as legitimate evidence. In the next section, I explore the scholarship on boundaries to explain how new forms of knowledge are produced through the interactions of experts and lay individuals.

2.3 The Boundaries of Scientific Knowledge

In recent years, the idea of “boundaries” has come to play a key role in important new lines of scholarship across the social sciences. It has been associated with research on cognition, social and collective identity, commensuration, census categories, cultural capital, cultural membership, racial and ethnic group positioning, hegemonic masculinity, professional jurisdictions, scientific controversies, group rights, immigration, and contentious politics, to mention only some of the most visible examples (Lamont and Molnar 2002).

As per Lamont and Molnar (2002), the renewed interest in boundaries builds on a well-established tradition of boundary work embedded in classical social theory (i.e., Marx’s class boundaries and Weber’s ethnic groups). These scholars distinguish symbolic boundaries from social boundaries. The former are described as conceptual distinctions made by social actors to classify objects and people for example, and to mark social differences. The latter are described as the objectified forms of social differences in terms of unequal resources and social opportunities. Lamont and Molnar highlight trends in the scholarship on boundary work and topics that were explored using the concept of boundaries. These include the forming of social and collective identity through processes of differentiation; the creation of social class boundaries and the reproduction of inequality; the production of racial and ethnic classification and the formation of intergroup boundaries; as well as the creation of gender boundaries through gender socialization and reproduction and the effect of stigmatization. In addition, the literature on professions used the concept of boundaries to understand how professions came to be distinguished from one another, while literature on the science of knowledge focusses on social

boundaries between experts and non-experts, science from non-science, and disciplines between themselves. Within this scholarship, there are two streams in boundary work. On the one hand, scholars emphasize the importance of boundaries for the constructing of scientific communities and disciplines (Gieryn 1983; Jasanoff 1996). On the other hand, scholars stress the importance of bridging boundaries through the construction of interfaces such as boundary objects (Star and Griesemer 1989; Star and Bowker 1996).

First, sociology of science has looked at the enterprise of science through professionalization and institutionalization while sociology of knowledge has analysed the content of scientific claims. Both schools of thought provide an understanding of the professional authority of science as a reference framework to view the world. Mertonian sociology of science underlines the professional authority of science by the powerful norms that guide scientists' behaviours, such as standards of professional competence and accountability, rules for the diffusion of scientific knowledge, codes of professional conduct and the discovery of truth by means of theoretical and empirical investigations. Merton refers to the "ethos of science" as an "affectively toned complex of values and norms", which is binding on scientists (Merton 1973: 269). Sociology of knowledge attends to the framing and description of science and the process of negotiation between different knowledge forms. Recent sociological studies of science and technologies have been instrumental in characterising scientific knowledge as a technological and medical reference framework that legitimizes science as a credible knowledge-making institution.

Under the rubric of social constructivism, sociology of knowledge and actor-network theory are often grouped together and thought to have a family resemblance to one another (Bloor 1999). Although both paradigms share a commonality, since they analyse knowledge

claims (and controversies therein) by different social groups, Bloor (1999) asserts that they have fundamental differences: sociology of knowledge makes a distinction between society and accounts or beliefs about nature, whereas actor-network theory does not. Rather than studying how nature and society are constituted and co-produced, sociology of knowledge scholars study the different interpretations of science and how these are framed. For instance, Mannheim's sociology of knowledge (1954) refers to contrasting modes of thought and the collective process that underpins these different interpretations. As per Bloor (1999), scientists are always responding to nature (by making selections and approximations) through their shared conventions and institutionalised concepts (Bloor 1999: 7). The "strong programme" in the sociology of knowledge (Collins 1983; Bloor 1999), which calls for empirical research in the construction of knowledge in various contexts, treats all knowledge claims as social constructions. It follows that "explanations for the genesis of knowledge, acceptance and rejection of knowledge claims are sought in the domain of the social" (Pinch and Bijker 1984: 401). From this relativist viewpoint, symmetry and impartiality are methodological principles of choice. For Bloor (1999), the aim of the sociological enquiry "is into the character and causes of knowledge, or what passes as knowledge, and not (in general) into objects which knowledge is meant to be about" (Bloor 1999: 87). The sociology of knowledge is therefore concerned with the content of scientific ideas, theories and experiments. Bloor, among others (Knorr-Cetina 1985; Gingras 1995), have criticized Latour's micro level analysis through the actor-network theory for several reasons. According to these authors, Latour rejects the existing culture that social agents and researchers themselves are grounded in (Bloor 1999); he reduces the social and scientific life to a form of political power struggle (Knorr-Cetina 1985); and, he does not distinguish explanatory factors and recognize the structural constraints on scientific practices

(Gingras 1995). In this regard, Latour rejects the pre-existence of nature and society; he rejects the notion that social factors explain scientific work and that the socio economic context affects ways social artefacts are defined. This discussion highlights the opposing principles within the social constructivist worldview. To remedy these internal disputes, Pinch and Bijker (1985) advance a convincing hybrid program that integrates both strands under the labels of the social construction of technology (SCOT) and the Empirical Programme of Relativism (EPOR). Their approach begins by defining social groups and their character, describing the function of the social artefact (i.e., human creation) with respect to each group, and explaining how these groups perceive problems and possible solutions with respect to the “thing-ness” they are creating (term used in Star 2010: 603). This model demonstrates that: i) flexibility exists in interpretations around artefacts and in the design of artefacts; ii) social mechanisms are involved in a process of negotiation and consensus building around the artefact; and, iii) the artefact is linked to the wider sociopolitical milieu as exemplified by the invention of the Penny-Farthing Bicycle. Here, the authors’ analysis of the invention of the seventieth century bicycle revealed the conflicting views among different social groups around the technological requirements for the bicycle, the various safety solutions, and the moral considerations (e.g., the idea of women wearing slacks). Although Pinch and Bijker demonstrate the multi-directional character of technological development, they do not explain how certain knowledge forms are accepted or rejected in this process of negotiation and how certain knowledge forms evolve into a legitimate body of knowledge.

The sociology of science has provided a unique account of the supremacy of science as a powerful professional authority and distinct intellectual discipline. Gieryn’s notion of boundary work (1983) was instrumental in this area to demonstrate “the ambiguous, flexible, historically

changing, and contextually variable, internally inconsistent and sometimes disputed boundaries of science”, which serve to demark science from non-science (Gieryn 1983: 792). For example, scientists like Tindall in Victorian England succeeded in expanding the authority of science authority and resources against the religious authority of the clergy and the civil authority of technical and practical engineers by emphasising the characteristics of science as practical, empirical, skeptical and objective. Tindall argued that science was driven by logic and rationality and was the underpinning of technology because scientists discover unseen technological processes by way of observations and chains of reasoning. Tindall was able to advance an agenda of pure science by drawing the boundary of science around the discovery of truth, free of moral impulses and self-serving interests (Gieryn 1983: 785). Gieryn also demonstrated that boundary work allowed Scottish anatomists to draw the boundary of science in order to exclude other non-scientific claims. To this end, the author explained the ideological and disciplinary battle between anatomists and phrenologists like Spurzheim, who fought for legitimacy of phrenology (study of the brain as a measure of the power of mental faculty) in academia. Characterized by anatomists as a layman’s pseudo-science, in the end, phrenology lost its quest for professional authority due to its political and emotional ambitions (Gieryn 1983: 788). Here, boundary disputes led to the monopolization of scientific expertise, and the exclusion of non-scientific expertise.

Instead of explaining the professional authority of science through the moral and strategic impulses of scientists who make efforts to preserve or expand their authority by way of scientific claims as did theorists of ideology, sociology of science can explain how a profession expands its authority into domains claimed by other professions while highlighting ideological and disciplinary contrasts. Jasanoff’s (1996) study of the interplay of science and policy in risk

carcinogen regulation was exemplary in showing how the effort to demark science and policy through regulatory discourse was a politically charged process. Further, sociology of science can explain how the professional authority of science can monopolize professional authority and resources by excluding rivals. It can also explain how scientists can protect their professional autonomy by dissociating science as an intellectual endeavour, and science as means to exploit non-scientific applications of scientific knowledge, as was the case in military technology (Gieryn 1983: 790). More specifically, through science and technology studies and the analytical framework of social constructivism, the scientific enterprise can be deconstructed to better illuminate the autonomy of science, the underpinnings of scientific assertions as well as scientific controversies (Jasanoff 1996: 273).

Because boundary work highlights the symbolic and social boundaries that define a field of expertise, it can provide insights into the production of knowledge and its effects of marginalisation. For example, Bowker and Star (1996) explain this process of exclusion and neglect of certain knowledge forms through the establishment of classification systems. These authors provide “a good map and working compass” to grasp how classification systems and their categories organize social life (Bowker and Star 1996: 286). They first define a classification system as “a set of boxes, metaphorical or not, into which things can be put in order to then do some kind of work - bureaucratic or knowledge production” (Bowker and Star 1996: 2). By setting the boundaries of categories through classification schemes (e.g., the category of hysteria, dead bird specimens and nurses’ work processes), communities of practice in the scientific world develop a common standard language, they defend their professional autonomy and enter various social spaces. Classification systems set social and cultural expectations and ties individuals to a set of work practices, knowledge claims and organizational

routines. Bowker and Star advance that classification schemes as historical and political artefacts can set the conditions for the coordination of social action, but they can also set the conditions for the separation of knowledge forms, valorizing some and silencing others. These authors conceive power as an effect or outcome of a social process and not as a cause. In this school of thought, power reveals itself through the power dynamics within the networks of collaborative agents. Through the lens of classification systems, knowledge can be conceptualized as constructed and legitimized narratives or readings of artefacts; these narratives and readings are contested and validated through social processes. Power is therefore about whose narrative or reading is imposed on others. Here, boundary work serves to monopolize the professional authority of privileged knowledge-producers. This monopolization of scientific authority is plausible in the context of the Patient Input Program because health technology assessments (HTA) stress the importance of rationality and scientific evidence. In this vein, I highlight some of the literature that insists on the constitutive role of expert knowledge in health matters, including evidence-based policy-making.

2.3.1 Evidence-Based Policy-Making

As per Nutley et al. (2000), there has been a surge of interest in evidence-based policy in the last two decades and the beginning of a new interface between policy-makers and knowledge producers. Although policy-makers need to gain access to a range of evidence forms to design and implement policies and other forms of intervention, the authors contend that policy-makers tend to privilege quantitative research methods (i.e., randomized controlled trials (RCTs), large-scale surveys), and are weaker on qualitative research methods (i.e., meanings that individuals give to context). For their part, Marston and Watts (2003) explain the appeal of evidence-based policy by its epistemic values:

The concept of evidence-based policy has an intuitive, common sense logic, which partly explains how it has become naturalised in a diverse range of policy settings. The term acts as a catch phrase for ‘scientific’, scholarly’, and ‘rationality’, which taken together can be understood as an attempt to modernise policy-making and professionalise human service practice (Marston and Watts 2003: 144).

The authors (2003) ask important questions with respect to evidence-based policy-making. What kinds of evidence do policy-makers advocate? What are their conceptions of evidence? Do these conceptions privilege certain forms of methods and knowledge over others? They argue that knowledge operates hierarchically and that lay knowledge ranks lower because elite policy-makers have greater access and authority in decision-making processes than do members of the public or service users. Evidence-based policy-making is not an objective exercise, though efforts are made by policy-makers to objectify knowledge. This line of thinking is consistent with that of Jasanoff (2011), who argues that policy knowledge rests on the epistemic achievement of objectivity (Jasanoff 2011: 335). To this end, evidence-based policy-making adopts a narrow view of what counts as valid knowledge based on conventional scientific methods.

With this in mind, scholars have investigated the scientific basis of knowledge systems and paradigms in healthcare (Michel 2002; Pope 2003; Timmermans and Kolker 2004; Sacristán 2012). Their analyses provide conceptual insights about the evidence models that drive HTA systems. As previously mentioned, evidence-based medicine (EBM) and cost-effectiveness research (CER) form the basis of rationing decisions. They are employed in drug coverage recommendations to maximise health results in the general population (Sacristán 2012). As domains of expertise, EBM and CER are intertwined and have the power to exercise influence on health spending practices. They have become the cornerstone of health policy as they contribute

to the standardization of healthcare, the development of evidence-based guidelines and best practices for health spending. EBM and CER carry with them a promise of demarcating credible science from ethical judgements and political motivations. For these reasons, it is important to look at how social scientists have explained the nature of these scientific paradigms to contextualize rationing strategies in resource allocation.

To begin the analysis, it is relevant to grasp the rise of HTA methodologies through the social science literature. Drummond et al. (2008) suggest that the trend in HTA methodologies was triggered by the increased societal concerns about balancing strained healthcare budgets while preserving and enhancing access to high quality medical care. For his part, Sacristán (2012) speaks to the role of quantitative data in the field of epidemiology and the centrality of social, political, cultural, and economic circumstances that can influence health. To this end, EBM and CER lead to new forms of knowledge based on values of cost-containment, resource prioritization, societal preferences and responsiveness to the needs of the aggregate. This statement supports the idea that knowledge is socially and culturally constructed as a means to an end. In the context of resource allocation, the means are scientific rationality and technical competence and the end is a fair distribution of health resources. Undeniably, the construction of expert knowledge produces a hierarchy of knowledge in which lay knowledge has a different status. EBM is described as a “new scientism” that first infiltrated the domain of medicine (Klein 1996 in Samanta and Samanta 2005). In the field of medicine, EBM is a dominant practice comprising a specific configuration, scientific method and generic way of doing science in terms of drug development (i.e., formulating research questions and producing research results) and clinical practice (i.e., basing clinical decisions and drug prescribing on the best available evidence). Scholarship in medical sociology identifies EBM as a reform movement

organized to ensure that a particular form of evidence (i.e., randomized controlled trials (RCTs)) was incorporated into medicine (Pope 2003). That is, EBM challenges the foundation of medical practice and has the potential to distort the doctor-patient interaction, with the doctor no longer an advocate but an agent of the healthcare system and the patient a consumer (Michel 2002). As per Michel (2007), “at such a system level, the physician becomes a double agent because one person’s health is another person’s health costs” (Michel 2007: 149). In short, EBM promotes the use of methods to generate and synthesize evidence. This entails an exercise of triage to filter credible evidence from less credible evidence in an effort to make decisions on the best research information. For proponents of this doctrine, the premise is that quantitative methods with their structure and rigid techniques rank higher than qualitative methods. Through the work of Archie Cochrane and his 1972 landmark publication, *Effectiveness and efficiency: random reflections on health services*, EBM revolutionized healthcare systems (Stevens et al. 2003). In the spirit of Cochrane’s vision of EBM, the Cochrane Collaboration was established in 1993 as a non-profit international group mandated to foster well-informed, evidence-based decision-making. The Cochrane Collaboration maintains a database of systematic reviews of EBM reports (i.e., the effects of health interventions) and a register of controlled trials⁹.

Again, EBM introduces a hierarchical classification of knowledge. As per Knaapen (2014), EBM places the systematic review and RCT at the top of the evidence totem pole. On the one hand, by using methods to reduce bias and produce reliable results, systematic reviews attempt “to identify, appraise and synthesize all the empirical evidence that meets pre-specified eligibility criteria to answer a given research question” (See Section 1.2 in the Cochrane

⁹ <http://www.medicinenet.com/script/main/art.asp?articlekey=39532>

Handbook for Systematic Reviews of Interventions)¹⁰. On the other hand, by using methods to reduce allocation bias and produce generalizable results, the RCT assigns participants to different study groups to receive one of several clinical interventions (e.g., the standard of comparison or control, which may be a standard practice, a placebo (sugar pill), or no intervention at all) in order to compare treatment outcomes. RCTs are usually conducted by large pharmaceutical companies and involve large numbers of participants. They are quantitative studies that are used to generate the evidence base for medical decision-making. The success of EBM has nevertheless attracted criticism from scholars who contend that this approach marginalizes many kinds of knowledge and expertise, such as pathophysiological knowledge, ethical principles, clinical skills, and patient's values/preferences (Knaapen 2014: 826). Knaapen points to other critiques of EBM in terms of neglecting patient diversity and favoring industry-sponsored clinical trials. As a population-based approach, EBM presents a privileged perspective of knowledge (Haraway 1988). As a community of practice, EBM proponents have rallied behind the façade of EBM to set social and cultural expectations in the health domain and set work practices and organizational routines to ensure maintenance of EBM in social life.

It is clear that formal scientific methods are now cemented in the medical field and shape evidence-based policy. The work I highlight now helps to unveil EBM methods and more broadly the foundations of EBM as a form of expert knowledge. Through an epistemological analysis, medical sociologists explain the role of EBM and its constitutive nature. As previously discussed, EBM is described as a shift in the scientific basis for medical knowledge due to the introduction of epidemiology, population health and the randomized clinical trial. As per Timmermans and Kolker (2004), “instead of opening up a few corpses, medical students might

¹⁰ <http://www.cochranelibrary.com/about/about-cochrane-systematic-reviews.html>

need to crunch a few numbers to learn about medicine” (Timmermans and Kolker 2004: 184). These scholars also describe EBM as a shift in the knowledge base to third parties (i.e., research institutions and academics) who impose their interests on medical practice through the establishment of clinical practice guidelines. These interests are seen as conflicting with the task of physicians to treat patients using their best clinical judgement. Other shifts stemming from EBM include a change in focus of funding priorities and change in locus of medical knowledge from the individual to the aggregate level. It becomes therefore obvious that these shifts in knowledge to rules of evidence and the synthesis of quantitative data leave little room for other forms of knowledge, notably patients’ disease insights and experience. The perceived notion underlying this hierarchical approach to knowledge is that other forms of knowledge (e.g., non-expert knowledge or lay knowledge) lack scientific rigor and validity because they do not meet methodological standards of objectivity. Facey et al. (2010) clearly reinforce the general view that non-expert knowledge, namely patients’ perspective are less reliable and credible than standard data collection methods in quantitative research. Their study suggests that patient input can be incorporated into HTA assessments if the robustness of the evidence on patients’ perspective is strengthened. The authors suggest that this can be achieved by conducting systematic reviews of qualitative studies on patients’ perspectives following pre-defined criteria, generating quantitative data on patients’ perspective via survey questionnaires, and assessing and synthesising the studies.

The reviewed works demonstrate that EBM as a form of expert knowledge has the power to reconfigure relationships between patients, healthcare providers and other individuals and organizations. As well, it places value on rationalist practices over individual health experiences. Thus, EBM can be viewed as a classification system (along the lines of Bowker and Star 1996),

that is, a box that organizes knowledge production. EBM, in its constitutive nature, takes for granted the diversity of needs within patient populations and hides the contested nature of knowledge and the conditions of its production.

The other facet of rationing decision-making is the cost-effectiveness ratio. As per Bowker and Star (1996), it can be argued that both EBM and CER “knit together” to form the texture of the HTA space (Bowker and Star 1996: 286). Within the debate on the role of evidence in healthcare decision-making, scholars have looked at value-for-money approaches in HTA allocation decisions. These works have highlighted the methodological challenges with CER. They have focussed attention on the underpinnings of the HTA framework and the scientific claims that serve to legitimize government intervention and resource allocations. First, it is widely recognized that the goal of CER is to make the most economically sound decisions on the most effective therapies in an environment of limited resources. CER measures the net cost or efficiency of the drug compared to other drugs and non-drug alternatives. Evidently, the wide use of CER has become a trend in healthcare. Centres of expertise in this domain are proliferating at a steady pace. Some examples include the U.S. Agency for Healthcare Policy Research (AHRQ), the U.S. Patient Centered Outcomes Research Institute (PCORI), the U.S. Institute of Medicine, the United Kingdom's National Institute for Clinical Excellence (NICE), Germany's Institute for Quality and Efficiency in Healthcare (IQWiG) and Sweden's Pharmaceutical Benefits Board (LFB). These organizations all have different degrees of interactions with local and national governments (Patel et al. 2015). Fundamentally, “CER affects which drugs are listed on the formularies of nations and hence are completely reimbursed by the government” (Patel et al. 2015: 585). HTA methodologies may use cost-effectiveness analysis, including the use of incremental cost-effectiveness ratios (ICERs) and quality-adjusted

life years (QALYs). These economic evaluations follow rigorous analytical methods. They must start with a relevant and well-defined study question based on the overarching investigation: is the therapy good value-for-money? CER assesses the efficiency of alternatives based on health outcomes and measured by the added cost per unit of the benefit or outcome, usually on a long-term basis. Health outcomes and more particularly quality-of-life outcomes are frequently measured as life-years gained (LYGs). There are different types of cost evaluation methods, such as cost-minimization analysis (CMA), cost-benefit analysis (CBA), and cost-consequence analysis (CCA). The use of these methods varies among nations (Jakubiak-Lasocka and Jakubczyk 2014). Based on an international comparison of 13 HTA established countries including Canada, Jakubiak-Lasocka and Jakubczyk (2014) conclude that cost-utility analysis (CUA) is preferred overall. CADTH's 2006 *Guidelines for the Economic Evaluation of Health Technologies* defines a CUA as the evaluation of treatment outcomes based on health-related preferences, largely expressed as QALYs gained (refers to length of life). As per CADTH (2006), CUA "permits decision makers to make broad comparisons across different conditions and interventions" and "facilitates the allocation of resources based on maximizing health gains" (CADTH 2006: 13).

The use of cost-effectiveness research (CER), its methods and measuring instruments are not without attack. Even CADTH cautions that the use of CUA to measure preferences often produces different scores for the same health state. Further, QALYs are not always comparable across all conditions (e.g., chronic versus acute conditions) and across patients (e.g., patients with greater life expectancy versus patients with shorter life expectancy). If EBM has the effect of transforming relationships among social agents in the web of healthcare, we can conclude in parallel that CER has the effect of discriminating against certain patient populations. The

following works raise critical issues in the application of CER and reveal certain theoretical challenges (Neumann and Johannesson 1994; Garber and Phelps 1997). For example, an existing alternative for the purpose of comparison may be unknown (this is likely the case for rare disease drugs); effectiveness may vary among patient groups and individual patients; and, patients may value health outcomes differently. Gaining a year of life may be different for patients living with a chronic disability than patients with a completely healthy year of life (Neumann and Johannesson 1994). Other outcomes such as functionality, degree of disability, and emotional health may also influence the way a patient values a year of life gained. Other issues with the application of CER include difficulties in measuring indirect costs due to variances in periods of treatment incapacities and measuring future medical costs if life is extended because of positive treatment responses (Garber and Phelps 1997). Garber and Phelps (1997) also raise the question of age bias because potential increase in life expectancy may be limited in older persons. As per Avorn (1984), cost-effectiveness methods “embody a set of hidden value assumptions that virtually guarantee an anti-geriatric bias to their purportedly objective data” (in Garber and Phelps 1997: 5). Thus, a key aspect of this discussion is that the intertwining of EBM and CER into a HTA classification and standardization system does not appear to be contested by public debate despite the intellectual curiosity of a few academics. The literature demonstrates that EBM and CER generate a privileged perspective of knowledge, which may lead to HTA practices that are discriminating. In this regard, expert knowledge in the field of HTAs is only a partial view of the healthcare landscape, of patients and of drug treatments, that is built on population-based preferences. Consequently, EBM and CER can have a silencing effect on marginal populations and other knowledge forms. In sum, scholars in the social sciences and other scientific disciplines have adopted the general insight inherited from

the sociology of knowledge that the way of knowing the world is affected by social structural positions in society in terms of privileged knowledge. Whereas sociology of knowledge furthers our understanding of expert knowledge forms, sociology of science provides insights on the ways that scientific authority becomes a credible knowledge-making enterprise through boundary work. Boundary work also demonstrates how expert knowledge is defined in contrast to non-expert knowledge. As per Gieryn (1983), “boundary-work is a sociological parallel to the familiar literary device of the foil. Just as readers come to know Holmes better through contrast to his foil Watson, so does the public better learn about science through contrasts to non-science” (Gieryn 1983: 791).

2.3.2 Boundary Objects

In addition to providing an analytical framework to understand the boundaries of scientific knowledge, social constructivism also provides conceptual tools such as boundary objects. These have been used in sociology to provide insights on the ability of certain social artefacts to succeed in crossing the boundaries or divides created by professional authorities. Boundary objects can produce a common ground for dialogue among actors. Here, boundary objects are not only conceptualized as markers of difference but can facilitate knowledge production. Bowker and Star (1996) coined the term “boundary object” to describe these interfaces that work to develop and maintain coherence across social worlds. Boundary objects have the effect of blurring the demarcation between social, policy and scientific boundaries. For Bowker and Star (1996) boundary objects can act both as interfaces facilitating knowledge production and as markers of differences. First, it is important to recognize that boundary objects have elasticity; they can be representations, things, activities or ideas among others, and they arise from cooperative work arrangements among communities of practice. As noted in the

introductory chapter, these communities of practice or social worlds¹¹ refer to the membership of a social group, in which individuals conduct activities together. Through time, a community of practice acquires shared lessons, conversations and expertise in a specific domain of activity. To borrow Bowker and Star's example, one is not born a violinist, but gradually becomes a member of the violin playing community of practice through learning and experience. In other words, boundary work serves to trace symbolic and social boundaries to delineate a field of expertise or community of practice and to delineate what is and what is not a component or characteristic thereof. As this section demonstrates, scholarship in science and technology studies further explores how certain social constructions become boundary objects, crossing previously erected barriers and allowing different research communities to draw from them. Notwithstanding the potential forms that they may take (e.g., repositories, ideal types, methods of communication), boundary objects have blurry contours whose "materiality derives from action, not from a sense of prefabricated stuff or "thing-ness" (Star 2010: 603). Boundary objects are ill structured, but when necessary, the object is worked on by local groups, who maintain its vague identity as a common object, while making it more specific and tailored to local use within a community of practice. In other words, boundary objects play a critical role in developing and maintaining coherence among communities of practice.

To account for the passage of certain social artefacts into different disciplinary processes and communities of practices, various scholars have used the metaphor of boundary objects to stress the constitutive relationships between machines (science) and people (society). For instance, the concept of boundary object was applied in the context of health (Fujimura 1992 (cancer and cancer research); Prout 1996 (the metered dose inhaler); Novek 2002 (computerized

¹¹ Bowker and Star appear to use these terms interchangeably.

drug dispensing equipment); and Fox 2011 (antiseptic and aseptic techniques). In these studies, boundary objects explain the diffusion of an innovative technology or in some cases, the limitations of a technology. Such is the case in Allen's (2014) study of clinical governance in UK hospitals. Due to nurses' lack of specialized skills and physicians' limited involvement in the implementation of integrated care pathways (ICPs), their value as a workable boundary object is hindered. Allen contends that the translation of the concept of clinical governance into ICPs resulted in a positive boundary object for nurses who work to standardize practices, but may have become a negative boundary object for doctors who question the credibility and quality of ICPs. The author expands on the notion of boundary objects-in-use, introduced by Levina and Vaast (2005), to distinguish social artefacts that may not be useful or relevant in boundary interactions, and therefore are not systematically adopted by all intended users. "To become boundary objects-in-use, artefacts have to be locally useful (i.e., be meaningfully and usefully incorporated into practices of diverse fields) and must have a common identity across fields" (Levina and Vaast 2005: 341).

Social science scholars also creatively used the concept of boundary objects to frame the prescription (Cooper 2011) and categorization of rare diseases (Huyard 2009) through different actors. Huyard (2009) suggests that the rare disease category displays very distinct local uses that all converge towards a simple statistical definition, serving "as a tool for voicing patients' unmet needs and transforming the statistical weakness of each individual disease into larger groups with political visibility" (Huyard 2009: 468). For patients, a rare disease means unmet needs. For doctors, because of their closeness to the disease and disease specialties, diseases are not rare, but thought of in practical terms. For example, doctors may have concerns with the small numbers of patients and the standards for research and development. For market-based

pharmaceutical companies, a rare disease means non-profitability and for the U.S. Food and Drug Administration (FDA), a rare disease triggers a prevalence-based regulatory mechanism tied to financial incentives and accountability. Here, as a boundary object, the rare disease classification was constructed within domains of regulatory policy and is used as a tool for innovation in biomedical research. For Cooper (2011), the medical prescription (i.e., the physician's script authorizing a patient to be provided a drug by a pharmacist) evolved as an object of social control, labour division, and patient safety. The author suggests that the medical prescription takes different forms in different healthcare systems and among prescriber groups. Further, it carries with it different meanings. For patients, the script is evidence of consultation or a means of empowerment, whereas for doctors it is an object of decision-making, a symbol of concern, or symbol of medical power, authority and legitimisation of the sick role. The prescription is therefore accepted as an obligatory point of passage for prescribers and users.

As the scholarship has shown, boundary objects are useful hermeneutic tools to explain how actors interact, and how new knowledge is produced and diffused, as well as how the interests of various groups of actors collectively shape an object or concept. Boundary objects can facilitate interaction and dialogue among expert and non-experts, but they can also emphasize differences among them. Finally, another important feature of boundary objects is that they can incentivize or disincentivize participation in a collective undertaking. All these notions are relevant to the study of the Patient Input Program in terms of understanding who is involved, what is being pursued, what is at stake, and how the multiple interests and knowledge perspectives are reconciled. The next section of this chapter discusses what public policy studies have taught us to date about public involvement to further contextualize the democratic ideals that plead for the participation of citizens in health policy decision-making.

2.4 Public Involvement and Government Decision-Making

The literature on citizen engagement (or public involvement) is motivated by the nature of citizen engagement, the use of engagement methods and the definition and role of the public. It crosses both public policy and social policy scholarship, covers a broad spectrum of citizen engagement topics, and offers important conceptual tools to frame the discussion henceforth.

To date, scholars have not reached a consensus on the definition of citizen engagement (Contandriopoulos 2004; Li 2013; Tritter 2009) despite the steady increase of publications. As a body of literature, citizen engagement “is difficult to capture; there is a lack of a common structure, no common terminology, and much of what is known is published in grey literature” (van Thiel and Stolk 2013: 8.5-3). It is generally recognized that Arnstein’s (1969) typology of citizen participation paved the way for scholars in the public involvement literature. Arnstein inspired public policy researchers to examine citizen engagement either to expand or reinvent the participation framework (Tritter 2009; Messina and Grainger 2012). Public policy theory investigates the political motivations underlying public involvement and the impact of democratic ideals of citizen engagement. Moreover, the literature attempts to anchor citizen engagement in the social and political context in which it is embedded. Here, citizen engagement is explored as a social practice and as a means to shift an imbalance of power from policy decision makers to citizens (Aronson 1993; Contandriopoulos 2004; Tritter and McCallum 2006 and Martin 2008) who are viewed as “today’s empowered public” (Caron-Flinterman et al. 2007). Approaching patient involvement from a public policy perspective allows a better understanding of its aim and function as a route to democracy (Contandriopoulos 2004). Hence patient involvement is no longer solely bound to medical decision-making, but also to public policy-making, notably health policy.

2.4.1 Notions of Democracy, Citizenship and Power

Because public involvement in the 21st century is at the nexus of civil society, science and policy, the literature shows that it encapsulates notions of political responsiveness, evidence-based decision-making and social demands. In the current literatures on citizen engagement, there is no common understanding of patients as a distinct public in involvement initiatives. Furthermore, several authors have noted gaps between theory and practices of involvement (Abelson et al. 2002, 2007; Bridges and Jones 2007; Bombard et al. 2011; Gauvin et al. 2011; Gagnon et al. 2011, 2012; Kreis and Schmidt 2013). This section explores how citizen engagement is conceptualized in public policy research and how it relates to notions of democracy, citizenship and power. In this regard, I review how social scientists have approached the study of civil society's interaction with policy processes to build a conceptual understanding of patient involvement practices.

To start understanding the nature of citizen engagement (used interchangeably with public involvement), public policy theories offer conceptual tools to encourage researchers to explore public involvement as a democratic process and as a form of struggle for dominance and power. The concept of empowerment is central in these debates as scholars make efforts to emphasise the nuances of citizen participation in terms of the different kinds of power relations it triggers. Whether the studies centre on social and symbolic aspects of citizen participation, or whether they focus on the relations between governments and citizens, scholars in the evolving field of public involvement advance a conceptualization of public involvement that goes beyond a one-size-fits-all model (Messina and Grainger 2012). Here, public policy theories insist that public involvement is related to the exercise of power and the ability of citizens to overturn

government decision-making. The work that is highlighted now helps expose the democratizing claims of citizen engagement and its efforts to redistribute power.

To begin, Arnstein's ladder of citizen participation must be further analysed. It comprises eight levels of involvement and adopts a hierarchical approach that frames citizen participation as a power struggle between government officials and community activists. The ladder's first rung begins with non-participation and moves to genuine participation, partnerships, power delegation and finally, citizen control. Thus, the ultimate achievement of participation is seen as a seizure of power by citizens in the decision-making process. The correlation between the ladder's upper levels and the increasing levels of citizen power in institutional decision-making stirred debate in the literature. Tritter and McCallum (2006) criticize Arnstein's claim that citizen participation equates to citizen power and that the single outcome of participation is a transfer of power between citizens and policy-makers. In Arnstein's framework, power is seen as an object that can be owned, lost or delegated. Tritter and McCallum (2006) depart from this notion of power and emphasize the structures and processes that are legitimized by both participants and policy-makers. The authors conceive a model that is respectful of diversity in which participation is empowering and enabling in a way that offers, "safeguards to protect the rights of people with rare diseases, provide space for people with dissenting views, or those for whom services need to be tailored differently" (Tritter and McCallum 2006: 163). This process of empowerment is obtained through an iterative process of participation that is shaped in the interactions among the social actors involved. Consequently, the realization of empowerment depends upon human capital and is facilitated by public policy responsive to the public "that not only accepts, but actively uses their involvement" (Bridges and Jones 2007: 33). In this public involvement model, the capacity of individuals to act independently and to make choices is

pivotal: “social actors must have agency and the ability to shape the methods used for their involvement; these may change over time” (Tritter and McCallum 2006: 157).

More importantly, the social policy literature on citizen engagement reveals what is hidden behind the façade of empowerment. Despite the democratizing claims of citizen engagement, its efforts to redistribute power and the rhetoric on empowerment, in fact people have little say and control over policies and practices that affect their lives (Aronson 1993). The discourse on empowerment can therefore “provide a cloak under which powerful actors obscure their continuing exercise of power (Mayo and Anastacio 1999 in Callaghan and Wistow 2006). Citizen engagement simply becomes a product of bureaucracy in the administration of engagement activities. Based on her findings, Aronson (1993) explains this democratic flaw in engagement activities as a gap between institutional and consumer perspectives created by a clash in vocabularies between the public (in terms of everyday experiences) and the government (in terms of bureaucratic language and conventions) around needs interpretations. This feature of engagement is particularly important because patients engage in health democracy to communicate their health needs. Aronson (1993) studied the accounts of elderly women about their needs. She revealed that elderly women desired choice, control, security and companionship (among others), while the official needs discourse of policy stressed their need to be independent in the community. For elderly women, independence in the community was more accurately understood as dependence on the support of female kin or paid service providers (Aronson 1993: 76). Fraser’s (1989) concept of needs talk can therefore be used to determine the contested nature of patients’ needs in public involvement, as well as the different degrees of influence and legitimacy of some groups, and the different vocabularies that groups use in the determination of their needs (Aronson 1993). It provides a granular examination of needs on the

premise that an official policy discourse on needs exists and is largely unchallenged. Through the politicization of needs interpretations, it becomes possible to understand how individual needs, which are in the private domain, become legitimized as genuine political issues and subject to government intervention. Hence, social policy studies highlight the role of policy in the construction and legitimization of needs. A needs talk approach fosters a critical reading of discourses about needs, which are contested among individuals and agents in institutions of power. By shifting the research lens to implicit needs interpretations and “making them explicit and visible, renders them disputable and enables us to consider how people are positioned in the process of talk about needs” (Aronson 1993: 76). As noted by Tronto (2010), patients’ needs are tied to the ability of dominant groups to interpret and influence them. Studies of citizen engagement therefore reveal the social and political dimensions of patients’ needs. Indeed, a discussion on the determination of these needs takes into account interpretations of needs and discourses about needs from the claimants’ perspectives and others who have interests in the satisfaction of these needs and resulting consequences. Attending to needs interpretations also exposes a different facet of public involvement and gets to the core of the power dynamics within the healthcare system.

Beyond the doctor-patient interaction, public policy works allow us to conceptualize patient involvement as a quest for social justice and citizens’ right to be engaged in governmental policy decision-making. Hence, the study of the Patient Input Program must consider the political dimension of engagement. In this way, social policy theory can help unveil layers of involvement in terms of potential misunderstandings and divisions among the social agents who participate in patient involvement initiatives and debate about the needs of citizens. Hambleton (1988) stresses that public involvement initiatives fail to resolve “the gap between

them and us - between those who plan and provide services and those targeted by them” (in Aronson 1993: 367). Taken together, these works are useful to grasp the relationship between the determination of needs and systems of governance and expose notions of legitimacy and authority in the determination of these needs. Furthermore, the underlying premise of this scholarship is that responsiveness of health institutions to citizens’ needs is not guaranteed and is a question of government policy and political interest.

Because needs may be translated into claims and asserted as demands for social justice, which entail the right to be included with dignity in the social organization (Dean 2008: 8), needs are embedded in notions of citizenship and democracy. In other words, needs (which are individual and differentiated) and rights (which are universal) stand in a dynamic relationship to each other (Lister 2003). From the perspective of rare disease sufferers, Huyard (2009) places these demands under the rubric of moral claims defined as common aspirations of fair treatment. These moral claims form a discourse about patients’ rights to disclosure about diagnosis that is realistic but humane; to appropriate guidance regarding daily life; and, to the freedom of association to disease-related groups for combatting feelings of isolation. For Tritter (2009), this notion of rights was created by the rise of patients’ choice in treatment decisions and the rise of patient-centered care, which gave way to direct democracy and patients’ participatory rights. In public policy theory, this collective mode of engagement in claims-making on the state is conceived as a route to democracy.

In addition to the principle of democracy, public policy authors emphasize that the policy discourse on public involvement relates to notions of citizenry and representativeness. For example, Contandriopoulos (2004) presents representation (statistical representation or organized groups as a source of legitimacy) and objectification (process of objectifying a social object or

social agents to gain political efficacy) as key components of the democratic process relating to public participation. Participation in this case can reflect struggles of legitimacy of different groups in the appropriation of their needs. In contrast, other works caution that the use of certain democratic principles of public involvement can lead to misrepresentation of communities. For Martin (2008), the criterion of representativeness produces community representatives that act on behalf of the collective. These community representatives need to “know and can make knowable their constituencies” (Martin 2008: 49). The danger is that the collective interest is voiced at the expense of non-active citizens, who live at the margins of society. In their critique of Arnstein’s model of citizen participation, Tritter and McCallum (2006) refer to this control of the elite citizenry as a tyranny of the majority, which has the effect of satisfying the needs of some individuals more than others (Tritter and McCallum 2006: 162). It can be argued that a form of elitism takes place (Martin 2008). Abelson et al. (2002) explain this form of elitism based on their study of Quebec’s Regional Health and Services Boards (RHSC). The authors suggest that RHSCs most commonly use public hearings to consult on health matters, but participants in these hearings are typically drawn from interest groups active in the health and social services fields and limited by the board’s view of which groups have an interest in a particular issue. As Martin (2008) states: “The average citizen can get involved as long as they are organized in groups that set forth a representation for the interests of clients, recipients or users of health” (Martin 2008: 85). In other words, the more efficient a representative system gets, the less grassroots participation will be (Contandriopoulos 2004) because it has the potential to drift further from the interests of marginal individuals who are not able to advocate for their best interest. Although citizen engagement is embedded in notion of citizenship and democracy, it would be erroneous to conclude that citizen engagement is necessarily inclusive of

its citizenry and allows equal opportunities for citizens to assert their rights to healthcare. The literature on citizen engagement further informs the conceptualization of patient involvement as it unveils key aspects of a democratic model of engagement that can lead to elitism and marginalisation. I turn now to a subset of public policy literature. This scholarship points to the forming of another model of involvement in the context of health technology assessment (HTA) agencies (Abelson et al. 2002, 2007; Bridges and Jones 2007; Menon and Stafinsky 2008; Facey et al. 2010; Bombard et al. 2011; Gauvin et al. 2011; Gagnon et al. 2011, 2012; Kreis and Schmidt 2013). These works are concerned with engagement methods and the place of patients in HTA decision-making.

2.4.2 Engagement Methods in Health Technology Assessments (HTAs)

The literature on Health Technology Assessments (HTAs)¹² conceives engagement methods as policy instruments that enable HTA agencies to interact with citizens. Although patient involvement, public engagement, public involvement, public and patient participation as well as patients' perspectives are used interchangeably in the literature, HTA agencies have adopted the term "patient input" as the preferred term to describe the interaction between HTA decision-makers and lay individuals in terms of seeking patients' insights on drug coverage recommendations. This scholarship is limited but is gradually growing. Several Canadian scholars have examined patient input processes in HTAs for over a decade now. The scholarly works generally follow two pathways: some authors explore the use of involvement in drug funding recommendations, while others propose conceptual models to incorporate patients in HTA decision-making. To begin, Bridges and Jones (2007) describe the inception of HTAs

¹² The term health technology assessment has its origin in the 1970s in the U.S., where health technologies were among a number of technologies that Congress saw the need to evaluate in an environment plagued by a shortage of reliable evidence and cost pressures on health services (Stevens, Milne and Burls 2003).

(dating back to 1990 in Canada) as a movement emerging from the “uncontrolled diffusion of costly medical equipment and the more recent desire to control the growth in drug costs” (Bridges and Jones 2007: 31). More specifically, along the lines of Menon and Stafinsky (2011) and Gauvin et al. (2010), HTAs are described as a sequential set of activities in coverage decision-making, including identification of technologies, HTA priority setting, conduct of HTA, review of HTA results and formulation of recommendations, implementation of listing recommendations, and dissemination of recommendations and HTA findings. The role of HTAs is to consider all the intended and potential unintended consequences of drugs while assessing the economic impact of coverage on fixed health budgets. To reduce the burden of health expenditures on taxpayers, HTA agencies such as CADTH, are tasked with promoting the deployment of health resources in an efficient and accountable manner.

Taking a societal perspective (looking at the population as a whole) coupled with a methodological approach that requires validity, reliability and generalizability, HTAs have been driven by evidence-based medicine (EBM), cost-effectiveness research (CER) and societal preferences (perhaps health state preferences as well), and less by individual patient preferences. In the case of CADTH, the drug expert committee (CDEC) makes drug coverage recommendations and the provinces and territories (P/Ts) make the final coverage decisions. Nevertheless, federal drug coverage recommendations are controversial in nature because they determine whether a specific drug should be covered by P/T formularies (i.e., lists of prescription drugs that are reimbursed by drug benefit programs) or delisted from these formularies. Constrained health budgets, political pressures and moral obligations (Abelson et al. 2007) further shape listing recommendations. Listing recommendations also have a shaping effect on drug pricing, drug prescribing and drug adherence because they affect governments, insurance

companies, drug manufacturers, physicians and patients alike. In this context, patient input is not only seen as a widespread policy instrument to interact with affected stakeholders, but also to inform controversial coverage recommendations (Kreis and Schmidt 2013). Gagnon et al. (2011) define this type of involvement “as the passive and active procedures used by an HTA agency to interact with the public and its representatives (i.e., citizens, groups representing citizens, patients, and service users, and groups representing patients and service users)” (Gagnon et al. 2011: 44). Although the idea of patient input is well recognized by HTA agencies worldwide, many authors have revealed the variances in HTA practices among HTA-producing organizations and the gaps between theory and practice. Kreis and Schmidt (2012) elaborate this idea as follows:

While there is a powerful theoretical groundswell calling for engagement as a *prima facie* good thing, in an evolving field there is not yet a consensus about on exactly what grounds and in what form engagement should be carried out in practice (Kreis and Schmidt 2012: 117).

Some informants involved in international HTAs affirm that because the discipline of HTAs is new and theoretically immature, that the absence of a theoretical foundation generates uncertainty about the purpose and methods of public involvement (Gauvin et al. 2010).

Given this knowledge gap, Menon and Stafinsky (2008) demonstrated ways that public involvement can be practiced to set priorities in HTAs by using a citizens’ jury. In their study, citizens were able to generate a ranked list of criteria for priority setting in HTAs. The absence of a gold standard of involvement and the inconsistencies among HTA agencies in involvement activities and formats have also led public policy scholars to conceive conceptual frameworks in efforts to assist policy-makers in the HTA system. Abelson et al. (2007) advance an involvement matrix that maps HTA activities onto opportunities for public involvement. The authors affirm,

“Understanding how to combine the two could affect the many goals of each” (Abelson et al. 2007: 38). The model connects the goals of involvement (priority setting, development of eligibility criteria and assessment of technology), to involvement models (direct representation, ad-hoc involvement or institutionalised involvement) and methods (e.g., surveys, focus groups, deliberative participation), to policy outcomes (answerability, citizen engagement, sanction or appeals), while linking to appropriate publics (who? how many? how diverse? and how will they be selected?). While scholars provide a roadmap for involvement that guides policy-makers in determining the why, who and how of involvement initiatives, they have only written about the distinctiveness of patients and the public on the periphery. They do not speak to ways that patients’ values can be incorporated into HTA decision-making, nor do they explore the level of influence of patients in the HTA process.

In this regard, Gagnon et al. (2011) posit that the concept of patient input is well engrained in health policy domains; however, there is no clear “know-how” on ways in which patient insights can be translated into evidence used by HTA agencies. Because HTAs involve various activities previously described as sequential, Gagnon et al. (2012) explain the benefits of using different methods of involvement at different stages of the HTA process. They suggest that direct participation at the beginning and end of the HTA process is more relevant to patients. For example, patients can be members of working groups tasked with setting HTA priorities and contributing to final HTA reports and recommendations. In contrast, patient consultation methods may be more pertinent during the HTA evaluation to assess the potential impact of a technology on end-users through the collection of patients’ views. The notion that an HTA system must be patient-based is emphasised by Bridges and Jones (2007) who developed a patient-based HTA model focussed on six key stages empirically classified into two broad areas:

i) questions HTAs attempt to address, and ii) processes HTA agencies adopt. A more patient-oriented HTA system must address questions that are relevant to patients. What is the problem as interpreted by patients? Is the HTA relevant to patients? Is the decision respectful of patients' preferences? To achieve a more patient-oriented HTA system, the works advance that patients must be engaged in all aspects of the HTA process. As well, the system must build upon doctor-patient partnerships and empower the patient. Here, "patient empowerment is the process of enabling individuals to have control over their own health" (Bridges and Jones 2007: 33). In general, authors focus on ways that the system can facilitate involvement, rather than on what can hamper the inclusion of patients and their experiential knowledge into HTA decision-making.

2.5 Conclusion

The chapter discussed the works of social theorists and other academics in the field of sociology of knowledge and science and technology studies who debate about knowledge claims. These scholars designed important conceptual understandings of knowledge forms, the ways they are constructed collectively, and the power dynamics that are involved. The chapter also turned to science studies on engagement and the sociology of science, which offered useful understandings of patient involvement in terms of characterizing patients as stakeholders in the healthcare system, insisting on the organizing principles of knowledge, and exploring the legitimacy and marginalisation effects of privileged knowledge forms through boundary work. Under this lens, social agents shape knowledge and knowledge has a shaping effect on their practices. For its part, scholarship on public policy and social policy informed the conceptualization of patient involvement and contributed insights to the democratizing claims of involvement and its perverse effects, the role of policy in the legitimization of patients' needs,

and the use of involvement as a means to achieve evidence-based policy-making. This discussion pointed to the interplay between patient involvement, needs discourses and institutions of knowledge production. This is especially interesting in the context of rare diseases, as illustrated by Robert's journey with aHUS, because the needs of rare disease patients are more complex in terms of their disease particularities and the associated costs of their therapies. These needs are highly politicized and attempts to re-frame them may have the effect of threatening their legitimacy.

The literature review also highlighted gaps in the scholarship, including limited attention to the actual involvement of patients and their level of influence in health policy decision-making. In addition, it leaves readers with unanswered questions about the institutionalization of involvement in evidenced-based policy and the role of patients' input in decision-making processes. Research to date does not tell us how patient involvement was constructed at the micro-levels where it was established, nor does it identify who participated in this phenomenon and what stakes were at play. Although the scholarship on patient involvement is still in its infancy and draws heavily on public policy theory, the works discussed in this section enabled me to identify key dimensions of patient involvement and understand the role of science and policy in the determination and legitimization of claims in drug coverage decision-making. More importantly, the literature pointed to interesting avenues of research utilizing boundary work to explain the monopolization of scientific knowledge, and ways that expert knowledge fields succeed in establishing self-serving boundaries (whether social or symbolic). These boundaries have the effect of organizing social processes and excluding certain knowledge forms. To this end, a multidisciplinary scholarship has shown that boundary objects emerge from boundary setting and collective work. The use of boundary work and boundary object concepts is

promising in demonstrating how various experts and non-experts engage in boundary interactions in spite of their diverging interests, and how concepts flow into real-world practices and are adopted (or not) by intended users.

In the following chapter, I will present the framework of study and discuss certain methodological considerations.

CHAPTER 3

THEORETICAL FRAMEWORK AND METHODOLOGY

3.1 Introduction

As stated in the introductory chapter, little is known about the emergence of patient involvement in evidence-based decision-making. In addition, the influence of patients in this process and the way their disease experience and knowledge is incorporated in decision-making frameworks is under-studied. Who are the patients targeted in involvement initiatives? What do they provide in terms of feedback? Are there any obstacles to this process? What happens to their feedback once submitted to the institution? How are drug coverage decisions made? What claims drive patients' demands? And how are they reconciled with evidence-based policy claims?

This aim of this chapter is to present the research objectives and explain how the research design and methodology will assist to generate research findings.

3.1.1 Research Objectives

Within this framework of study, I have formulated the following main research question: *what is the role of knowledge and disease experience in the Patient Input Program and how do they influence drug coverage decisions?* In order to answer this question, I have formulated three sub-questions in relation to the overall study objective:

- I. How was the Patient Input Program brought into practice in Canada?*
- II. How are claims crafted in the context of the HTA network?*
- III. How is patient feedback considered in CADTH's decision-making?*

These three sub-questions determined the outline of the study design, which can be divided into three parts as well. The first findings chapter (Chapter 4) will focus on the emergence and

establishment of patient involvement in practice. The question will provide a lens on the institutionalization of patient involvement, the role of the HTA network, and the enabling conditions that explain the structured involvement of patients in drug coverage decision-making. The second findings chapter (Chapter 5) will centre on the politicization of patients' needs in current evidence-based drug coverage, the circulation of discourses about patients' needs, and the role of the Canadian Organization for Rare Disorders (CORD) in framing the suffering of rare disease patients. The question will serve to characterize patients, their claims and the nature of drug coverage decision-making. In the third findings chapter (Chapter 6), the Patient Input Program will be scrutinized in terms of its role as a boundary object and its effect on individuals and knowledge forms. The question will seek to understand the value of patient's input and ways this input is accounted for in drug coverage decision-making.

The following section develops a framework for the study drawing on the concepts of boundary work, boundary object, public involvement and needs talk.

3.1.2 Boundary Work in Resource Allocation and the Place of Evidence-Based Activism

I will draw on the concepts of public involvement, boundary work and the notion of evidence-based activism to further grasp the inter-relationship between science, new forms of patient activism and social participation. The concepts will provide insights on the emergence of patient involvement practices, the role of the HTA network, and the factors that explain whether the structured involvement of patients has allowed patient voices to influence drug coverage decision-making. As previously discussed, boundary work originated in the sociology of science scholarship and was introduced by Gieryn (1983) to explain how scientists use discursive practices to draw and redraw the boundaries of science. These works provide new avenues to study the construction of expert repertoires and disciplinary disputes. Boundary work serves

scientific aspirations of professional authority against other expert claims outside the parameters of an expert field of knowledge. Because HTA knowledge bases stress the importance of rationality and evidence models in health domains, I have already characterized HTA decision-makers as privileged knowledge-producers. Only by looking at the enactment of CADTH as a centre of policy and scientific authority can we begin to understand how patient involvement entered the realm of scientific knowledge and public policy, and how the Agency managed to limit what counts as knowledge and limit who can be involved in decision-making processes. Here, boundary work will be employed to reveal certain rhetoric and design choices of CADTH in the Patient Input Program to secure its position as an influential actor in drug coverage decision-making.

The notion of evidence-based activism (Akrich and Rabeharisoa 2012) will also be useful to provide insights on patients' knowledge contribution in evidence-based policy-making. Evidence-based activism refers to the active role of patient organizations in the articulation of expertise, which in turn is used to make demands on the state and sustain their claims-making. These patient organizations work from within the boundaries of science and politics to assert their needs. By looking at patients' knowledge contribution, we can determine the epistemic nature and status of patients' voice within the HTA network. As remarked earlier, some patient organizations exercised influence in the politics of disease and health governance. Gradually, patient groups started mobilizing people and resources, and demanding institutional changes to address injustices with respect to healthcare services and health research agendas. The previous chapter has demonstrated cases where patients' alternate understandings of science and disease have influenced biomedical decision-making processes. Here, the dissertation is interested in the

nature of lay knowledge in drug coverage decision-making, the stakes at play, and the way evidence-based activism plays out in drug coverage decisions.

3.1.3 The Politicization of Claims in the HTA Network

Because the Patient Input Program involves boundary interactions between experts and non-experts and between patients' needs and existing models of biomedical sciences and pharmacoeconomics, drug coverage decision-making reflects multiple interests and involves claims-making by both patient groups and technocrats. Foremost, the Common Drug Review promotes evidence-based claims on evidence hierarchies (Marston and Watts 2003), in which scientific paradigms determine thinking on drug coverage matters and standard methods of knowledge production direct the work of HTA experts. Through principles of evidence-based medicine (EBM) and cost-effectiveness research (CER), evidence-based policy claims fair and neutral spending practices. In an earlier chapter, I explained that the task of evidence-based policy-making is to measure and objectively quantify evidence. Attending to the politicization of claims in the HTA network therefore points to the scientific underpinnings of drug reimbursement decisions. As domains of expertise, EBM and CER are intertwined and have the power to exercise influence on health spending practices.

A claims-making approach will attend to both the elements of evidence-based policy and patients' demands in the context of drug coverage matters. It points to patient groups and the way they mobilize around disease identities, socialize their disease through membership, and make political demands for their unmet needs on public institutions. On this matter, the research will examine the determination and legitimization of patients' needs and their articulation into political claims.

These needs can be politically contested. They can be skewed and can reflect the interests of groups and social relations among them. The premise is that various needs interpretations circulate in the social sphere, including controlling interpretations that have the effect of silencing others. Seen in this light, the needs of patients are constructed, discursively interpreted, negotiated and politicized from below by consumer groups and from above by experts. This process leads to the articulation and transformation of needs into objects of state interventions. The concept of needs talk will add an important dimension to the study of patient involvement to capture the ways that society talks about patients' needs and the politics at play behind the talks (Fraser 1989). It will be used to explain how needs of rare disease patients are brought to light in the political discourse.

3.1.4 The Social Construction of the Patient Input Program

Conceptualizing the Patient Input Program as a boundary object assists in tracing the path of emergence of the patient involvement concept, how it was anchored as a social practice, and how it is socially constructed within the HTA network. Scholars who have used the concept of boundary object have explored repositories, technologies and social processes, among others, in terms of mobilizing engagement of participants from disparate disciplines and traditions, who are joined together with a singular focus. As per the examples discussed in an earlier chapter, the use of boundary object theory can explain interactions and relationships within collaborative work arrangements, and the emergence of new practices and knowledge forms. Within the HTA network, the concept of boundary object was used as a hermeneutic tool to examine the Common Drug Review (CDR) as a multiplayer process and examine the role of the Patient Input Program in enhancing or hampering the involvement of patients in drug coverage decision-making. The dissertation will seek to expose the inward values, stakes and knowledge claims, which are at the

heart of drug coverage decisions, and explicate ways in which scientific knowledge organizes drug coverage decision-making and excludes certain knowledge forms. The dissertation is concerned with the capacity of the Patient Input Program to bind different actors under a common drug funding exercise, on the premise that the Program works to enhance their interactions, fosters communication among them, and leads to improved decision outcomes.

Figure 3.1 demonstrates the elements of the study framework, including the individual, organizational and societal factors that have the potential to influence the course and shape of the patient involvement concept into real-world practice, as well as the corresponding discourses around the patient involvement phenomenon and the needs of rare disease patients.

Figure 3.1 Study Framework



To reiterate, the boundary object concept will be used to reveal some of the nuances and complexities in the Program's adoption in the real-world setting and to explore whether the Patient Input Program functions as a positive or negative boundary object for the actors involved in its development and those involved in its maintenance. In the following section, I examine the concept of boundary object and discuss some of the criticism aimed against the approach and its relevance to the study at hand.

3.1.5 Boundary Objects as a Sociological Concept

As discussed in the previous chapter, the concept of boundary object was developed by Star and Griesemer and has inspired a multidisciplinary scholarship. Scholars have pointed out that the concept has developed over time. Researchers have adapted its use to explain various

components of our healthcare system. Within this discussion, I examine in more detail the key foundational principles, and analyse their resulting strengths and weaknesses.

Since the 1990s, science and technologies studies scholarship has turned its lens on medical technologies, pharmaceuticals and biomedical research as highlighted earlier. At the same time, the site of sociological inquiry has moved to new points of social interactions (i.e., hospital, operating room, pharmacy, and clinic). Through fieldwork techniques, science and technology research looked at medical technologies as vehicles of meaning, markers of change in social relations, producers of new social practices, producers of power relations, political tools to govern people, or entities that reshape human bodies and lives. Classical science and technology studies sought to understand the construction of scientific facts and the hidden discourses of science and technologies. Latour and Woolgar were the pioneers of these works with their actor network concept (1979) alongside Sharon Traweek (1992). By infiltrating a laboratory, Latour identified the technical and social dimensions of science through the construction of the scientific facts. Using anthropological methodologies to explain the underpinnings of scientific work, the authors generated a new tradition of ethnographic work. Latour's theory of the actor network allowed him to follow the actors in various settings by inserting himself into the scientific environment and rebuilding an ordered account of the scientific fact. For her part, Traweek (1992) studied international collaborative research that was conducted in Japan. She also aimed to reconstitute the history of a laboratory in the Japanese science city of Tsukuba Kenkyu Gakuen Toshi through a thorough analysis of historical government files and more importantly, interviews with citizens, bureaucrats, politicians, and other community leaders. Together, these authors focussed on knowledge making through fieldwork while emphasising the meanings that actors give to their observations and experiences. In response to the network-

building concept of Latour and Woolgar (1979), Star and Griesemer (1989) conceptualized the boundary object theory with key concepts such as boundary objects and standardization methods (although scholars less frequently utilize the latter). Their approach maintained a historical focus while providing critical insights on ways collective work arrangements can be achieved among diverse actors. In the following sub-section, I discuss in more detail the concepts that underlie boundary object theory.

The concept of boundary object is used as a metaphor and part of the grounded theory model in the sense that it emerges and is constructed as an element of theory-building for authors in their confrontation with field materials (Trompette and Vinck 2009: 4). On this point, by use of archives, Star and Griesemer (1989) traced the history of the Museum of Vertebrate Zoology at the University of California, from the viewpoints of different participants. These included those of professional scientists (who collect specimens for ecological and evolutionary purposes), trappers (for whom specimens were a coin of exchange), amateur naturalists (who promoted the preservation of nature), university administrators (who sought prestige and recognition), and the museum administrator (who sought to educate the public about conservation and zoological research). This case study demonstrated that a process of reconciliation of meanings is necessary to allow cooperation among various actors (Star and Griesemer 1989: 388).

Boundary object theory advances the notion that diverse interests are at play in scientific work and that a labour-intensive process of cooperation among participants across different communities of practice is necessary to reconcile these interests. The concept of communities of

practice¹³ is not central in this analytical framework. Within the context of scientific collaborative work, communities of practice intersect and may lead to the creation of new scientific knowledge. Based on the model of Latour (1988), each group within a work arrangement engages in simultaneous translations of interests and/or concerns to maintain the integrity of the other audiences in order to retain them as allies. For instance, scientific endeavours are often collaborative ventures in which “scientists and other actors contributing to science translate, negotiate, debate, triangulate and simplify in order to work together” (Star and Griesemer 1989: 389). In this model, the concerns of the non-scientist are translated into those of the scientist. For Star and Griesemer, the translation process across communities of practice needs to be understood from multiple viewpoints (in line with Hughes’ 1971 description of the ecology of institutions). In contrast to Latour’s story-telling approach, which privileges one perspective, Star and Griesemer argue that although one perspective can be fully fleshed out, there are several obligatory passage points that are negotiated with several allies – meaning that multiple translations can occur within and outside the collaborative endeavour. Once an obligatory passage point is established, the authors affirm that participants need to defend it against other translations threatening to displace it. As demonstrated by the success of the Museum of Vertebrate Zoology at the University of California, a system was established in which diverse allies could participate concurrently in heterogeneous work to build a museum. While Star and Griesemer (1989) state that the central cooperative task of communities of practice, “which share the same space but different perspectives is the translation of each other’s’ perspectives” (Star and Griesemer 1989: 412), this success is explained by two contributing factors: methods of standardization and boundary objects.

¹³ This sociological term was first coined by Anselm Strauss (1978b) (in Star, Bowker and Neumann. 1998).

Methods of standardization are employed to manage work across communities of practice. In the case of the museum, standards of collection and curation were introduced to simplify the heterogeneous work of diverse allies. The goals of this standardization were three-fold: to ensure the integrity of the information collected; to render the information collected analyzable for present and future use; and, to build a centre of authority following set scientific requirements. Methods of standardization imply that each ally is willing to conform to information-gathering standards. These methods of standardization become obligatory passage points. If they wanted money or scientific recognition, collectors working for the museum had to adhere to prescribed methods, which became a common ground among allies to be able to work while maintaining some autonomy.

Boundary Objects serve to develop and maintain coherence across intersecting communities. They are internally heterogeneous; they have multiple memberships and act as bridges or anchors. The construction of boundary objects only occurs when the work of communities of practice coincides and when conflicting concerns are satisfied through reconciliation rather than consensus. Boundary objects cross multiple communities of practice. They can gain acceptance, legitimacy and authority when they go unquestioned and infer higher claims of naturalness; a perfectly fitting belief that has no anchored history. Further, Star and Griesemer (1989) convincingly advance that the acceptance or legitimacy of the boundary object will depend on a process of naturalisation that has the effect of uprooting knowledge from its historical context. In their own words, this process of naturalization secures the object within the community of practice, obscures “the contingent and historical circumstances of its birth” and has the effect of sinking “into the community's routinely forgotten memory” (Star and Griesemer 1989: 299).

In Star and Griesemer's words (1989), boundary objects "adapt to local needs" within communities of practice but "maintain a common identity across sites" (Star and Griesemer 1989: 393). The authors suggest four forms that these objects might take, based on particular forms of action and cooperation:

- Repositories: these objects are indexed in a standardized fashion and can be referenced (such as a library or museum) to deal with a group-specific problem without the need to directly negotiate differences in purpose among people of different communities;
- Ideal types: they are symbolic objects that do not have local details (or belong to a community of practice) but bear attributes from all domains and serve as a roadmap for all parties (e.g., the concept of species, which is adaptable due to its vague nature);
- Coincident boundaries: These common objects have the same boundaries but different internal contexts and are employed as common referents (e.g., maps of California defined as roadmaps versus life zones) ;
- Standardized forms: These objects are devised as methods of common communication across dispersed work groups such as forms to fill out.

Bowker and Star (1996) also introduced the notion of boundary infrastructures as regimes and networks of boundary objects. The authors stipulate that classification systems, which are part of Western bureaucracy, can be viewed as a boundary infrastructure. Although the dissertation has established earlier that the intertwining of evidence-based medicine (EBM) and cost-effectiveness research (CER) has the effect of producing a HTA classification and standardization system, it does not advance that the HTA network is a boundary infrastructure. The HTA network is small-scale and does not involve a comprehensive classification scheme,

multiple communities and work tasks, as well as heterogeneous sources of information (as conceptualized by Bowker and Star 1996).

In this dissertation, I will employ the concept of boundary object to explore the Patient Input Program as a construction of social participation that is time- and locally- dependent. The Patient Input Program is a case example of the structured inclusion of patients in an expert-led process, namely the drug coverage decision-making process. The dissertation applies the concept of boundary object to the study of the Patient Input Program and argues that it is relevant because the Program straddles different social worlds, and presents itself as a reconciliation of contradictory knowledge forms, perspectives and needs. By looking at the Program as a boundary object, we are confronted to the complexities and intricacies of this bureaucratic process, and can expose the values, stakes and knowledge claims within. We can also consider the hierarchies and imbalances of power within, and explore whether the Program leads to a tangible sense of satisfaction or disappointment for patients.

3.1.6 Critiques and Limitations of a Boundary Object Approach

Originally, boundary object theory was used to understand collaborative work in the field of science. Although this framework of study has provided insights into the work of scientists, it has also generated criticism from scholars for becoming a generic concept decontextualized from its origin in science and technology studies: “in recent years a disturbing trend has emerged: the concept of boundary objects has become a catch-all for several constructs” (Lee 2007: 335). Star (2010) responded to this criticism by clarifying that boundary objects take on particular forms of action and cooperation. In other words, boundary objects refer to work arrangements whether visible or invisible. The concept has also been criticised for being too abstract (Fujimura 1992). In this line of thinking, Fujimura (1992) argues, “While boundary objects can promote

translation for the purpose of winning allies, they can also allow others to resist translation and to construct other facts” (Fujimura 1992: 175). For Lee (2007), boundary object theory does not consider internal negotiations that emerge from collaborative processes among communities of practice. The negotiating process involved in the adoption of boundary objects is explored by Fox (2011) who concludes that technologies (as boundary objects) are not necessarily objects of consensus building. Fox discusses the rejection of Lister’s 19th century antiseptic technologies and the uncontested adoption of asepsis into surgery. While Lister’s convictions were based on germ theory and the belief that post-surgical infections were caused by germs and microbes in a patient’s wounds, his views challenged the dominant humoral theory that associated infection with environmental circumstances, including poor diet, bad habits and poverty. Not only did Lister’s technologies try to overturn a theory rooted in two millennia of medical scholarship but his techniques also had the unintentional consequences of characterizing the surgeon as a “corrupter of sterility rather than a healer of disease” (Fox 2011: 12). Fox (2011) explains that despite carrying negative connotations, Lister’s antiseptic techniques “were in effect inhibitory boundary objects for promoting surgical sterility” (Fox 2011: 15). According to Lee and Fujimura, it is this process of negotiation that is absent from the boundary object theory. Yet some scholars have built on Star and Griesemer’s theory by demonstrating the messiness of scientific work. In her study, Henderson (1994) demonstrates that the world of engineering design is one of negotiation, collaboration and potential conflict because design work is based on visual representations that organize and structure discussions, but also that enlist and constrain participation in the design process. Negotiation can therefore lead to transforming the understandings of others and in doing so, generating a richer understanding of the product that is produced by the collective and problems the collective faces (Bechky 2003). For Bechky (2003)

and Henderson (1994), the emphasis is on problem solving between heterogeneous communities of practice working towards a common goal: developing a product. In their studies, boundary objects are sketches, drawings and prototypes used in communities with differences in their language, loci of practice and their conceptualisation of the product. These studies discuss the tacit knowledge specific to each community and the process to overcome barriers in understanding and communication.

In fact, these works show us that collaborative work is not linear but rather complex. Thus, collaborative work can “push the boundaries rather than merely sailing across them” (Lee 2007: 307). Boundary objects are not therefore stable and can be contested within and across communities of practice. In 2010, Star clarified that boundary objects move in cycles and can experience breaches and result in the generation of new boundary objects: “we live in a world where the battles and dramas between the formal and informal, the ill-structured and the well-structured, the standardized and the wild, are being continuously fought” (Star 2010: 614). Despite this clarification, Lee and Fujimura argue that within Star’s theoretical framework, an uncomfortable separation exists between the boundary objects and the socially negotiated processes that give them meaning. Evans (2009) brings to light this exact process of negotiation with his study of American sociology. He theorizes that actors can reengineer/redefine a boundary object so that it does not fulfill its functions. That way, actors can limit or eliminate the usefulness of that boundary object, by effectively drawing a new boundary as given by the example of the American Journal of Sociology. Evans contends that sociology was able to replace religion as source of credible science at a time of transformation from Baconian empiricism to positivism in late 19th century. He suggests that the success of sociologists was due to their ability to construct a sociological audience by creating an official sociological

journal, thereby securing a subscriber base of professionals and establishing information requirements. This resulted in the exclusion of religious reformers who could not satisfy the requirements of the journal. Sociologists demarked their disciplinary boundaries by defining their desired sociological public with whom they wanted to engage in American sociology during the credibility crisis among sciences. In Evans study, boundary objects can bridge different publics but can also work to exclude certain publics. Here, we can view boundary objects as interfaces that can facilitate knowledge production and diffusion or restrict knowledge production by denying access to certain groups. Another example was demonstrated by Allen (2014) in her study of the passage of the clinical governance concept into a local application, that of Integrated Care Pathways (ICPs). As discussed earlier, ICPs became positive boundary objects for some, and negative boundary objects for others.

On another point of criticism, Lee also questions the concept of standardization as a condition of collaborative work. In her view, methods of standardization do not necessarily exist within a web of collaboration as demonstrated in her study of the design of a travelling exhibit about wild and domestic dogs. She concluded that collaborative work arrangements can be disorderly processes. Thus, boundary objects do not always “pass cleanly and unproblematically between communities of practice... differences between participant’s unique “life-worlds” lead to misunderstandings, conflict, and uncertainty and participants appear to contest, or challenge, each other’s contributions (Lee 2007: 316). Fujimura went a step further by merging Star and Griesemer’s boundary object concept (which looks at the convergence of different viewpoints within collective processes) and Latour’s fact stabilization theory (which focusses on the negotiation processes among actors) into a new concept coined standardized packages to study how cancer and cancer research is defined. Fujimura elaborates a complex conceptual

framework, which reunites both boundary objects (i.e., genes, cancer, and cancer genes in proto-oncogene theory) and standardized methods (i.e., recombinant DNA technologies, probes, sequence information) to explain how they produce a new representation of cancer derived from heterogeneous viewpoints and approaches, including oncogene theory, evolutionary biology, molecular biology and medical genetics (Fujimura 1992: 170). Her study revealed how different laboratories in different fields of research came together under a single network to explain the causes of cancer.

In this regard, Fujimura points to another limitation of Star's approach in terms of its story-telling perspective and the constraints regarding the availability of information to build a narrative. That is, "whose story gets told depends on whose life is recorded in more detail" (Fujimura 1992: 172). Star and Griesemer framed their story in terms of the organizational and management work done by the main characters in building the museum. In contrast, Fujimura framed her story in terms of ways that many different worlds adapted the theory-method package in their line of work (i.e., coupling of the oncogene theory and recombinant DNA with molecular biology techniques), while simultaneously introducing novelty. Despite this limitation in Star and Griesemer's story-telling approach, it can be argued that new trends in research, combining ethnographic methods to content-analysis methods, are well suited to bring to light the multiple perspectives of actors in the various networks, such as interview techniques and participatory observation (see Bechky 2003 and Henderson 1994). In the next section, I discuss the study design and methodology.

3.2 Study Design

To fulfill the goals of the dissertation, a case study approach was chosen of the Patient Input Program housed within the Canadian Agency for Drugs and Technologies in Health

(CADTH). The case study satisfies the three tenets of the qualitative method: “describing, understanding and explaining” (Tellis 1997: 2). Because case studies are multi-perspectival analyses and insist on interactions among systems of action (rather than an individual or group of individuals) (Tellis 1997: 6), the case study was deemed the most relevant method to describe and understand the intrinsic aspects of the Patient Input Program and how it functions (Berg 2004). This involved an analysis of the enabling conditions and influences that shaped the Program and its shaping effect on rare disease patients and different knowledge forms. This case study is intended as a window on the complexity of diverging interests in the HTA network. It serves to explore the relations between the emergence of patient involvement, the context from which it arose and the struggles that it evokes. Although the boundary object approach does not presume the primacy of one viewpoint over another and treats the interaction between different communities of practice with equal importance, I chose to foreground the perspectives and experiences of rare disease patients, which are not always prominent in scientific discourses. The use of rare disease patients can be viewed as a deviant case (Teddlie and Yu 2007). By focussing on the particularity and distinctiveness of rare disease patients, the case study provided more information about the patient input process that may not have been visible using typical disease communities. I envisioned that their experience in engagement activities would provide insights on some of the complexities and controversies embedded within these processes, and would link more broadly to certain system inadequacies. As remarked earlier in the introductory chapter, rare disease patients have different socio-political realities based on their disease identities and health needs, and generally feel marginalized in the healthcare system because their needs are not being fulfilled. Although the use of a deviant case limits the way I can generalize the findings, it can also “provide interesting contrasts with other cases, thereby allowing for

comparability across those cases” (Teddlie and Yu 2007: 81). This particularity and distinctiveness was explored in the context of this study. Rare disease patients have not gained the same level of visibility as cancer and AIDS patients. They are the invisible face of human suffering.

3.3 Gaining Access and Study Sample

Initially, my intention was to interview scientists within Health Canada to seek their perspectives on patient feedback mechanism and the role of patient input, which was being piloted, in the context of regulatory science. Although I did obtain approval to use Health Canada as a site of enquiry by the Research Ethics Board (REB) of Health Canada and the Public Health Agency of Canada, senior officials within the Health Products Food Branch had concerns about privacy and as such, I was not permitted to interview scientific staff. Despite not being able to freely speak to scientists within a research framework, I was able to gain insights through informal conversations with scientific colleagues on my research topic throughout the research endeavour. The Canadian Agency for Drugs and Technologies in Health (CADTH) was much more receptive to the study. After receiving approval from the Ethics Committee at the University of Ottawa in February 2015, I contacted CADTH to inform staff of the study objectives and to request their participation in the study. My objective was to interview bureaucrats employed by CADTH and technocrats appointed to the drug expert committee who have direct experience with the Patient Input Program to seek their perspectives about the program, experience with patient input, and insights about government-stakeholder relations. I also planned to interview rare disease patients and activists about their experience with the Patient Input Program and their perspectives about the patient involvement agenda put forward by CADTH and more broadly, the Government of Canada. I believed that the information

collected from the interviews would best capture an untold story of patient involvement, which I would then assess against the established patient involvement literature.

It is important to note, that although CADTH was eager to participate in the research, staff imposed certain limitations. For example, I was not able to interview the newly hired qualitative researcher, nor could I name the public members or reach them directly. In addition, staff did not provide any internal (unpublished) documents. The material provided was available on the CADTH website.

In terms of recruitment process, I used a snowball sampling approach (Berg 2004). This involved identifying a key individual employed by CADTH and a key leader in the rare disease community (i.e., President and Chief Executive Officer (CEO) of the Canadian Organization For Rare Disorders (CORD)), who provided the names of other potential participants and groups of interests. To expedite the recruitment process, I provided CADTH recruitment text that could be circulated within the organization (Appendix A). Subsequently, two key individuals agreed to being interviewed. These individuals also provided the names of other informants including a senior official at CADTH (who wanted to remain anonymous) and members of the drug expert committee. In addition to establishing links with other key individuals, one of the primary informants shared presentation slides, audio-visual materials, CADTH material and web links to contextualize the case study. A total of 5 individuals employed by CADTH were interviewed.

The same recruitment process was followed with the President and CEO of CORD. The study poster was posted on CORD's website and Facebook page (Appendix B). I was also directed to CORD's list of members/affiliate members and was given permission to contact the organizations, which totalled approximately 100 organizations. Out of the 100 organizations,

some organizations were no longer active, or did not answer the call for participation. In total, 22 individuals from various disease organizations and health charities contacted me by email or telephone to schedule an interview. Through these research participants, new leads were provided that directed me to 3 key informants within CADTH's pan-Canadian Oncology Drug Review process (pCODR).

3.4 Data Collection

To piece together an untold story of patient involvement, the dissertation triangulated three main types of evidence on patient involvement: i) theoretical discussions and empirical findings from the published literature: ii) documents in the grey literature, including documents published on government of Canada websites; and, iii) interviews with individuals involved in CADTH's Patient Input Program.

3.4.1 Literature and Public Documents

I performed a search of the academic literature relating to patient advocacy, patient (or public) involvement and citizen engagement. Several sources were used including, bibliographic databases (i.e., MEDLINE, Sociological Abstracts, CAIRN, ProQuest Research, among others), the Research Index of Canadian Research and Government Information, citation indexes such as the Web of Science, the Canadian Dissertations and Theses Database, and Google Scholar. The documents retrieved included published articles or studies within the social science, political science and health science fields and specifically, in the sociology domain. A structured search strategy was developed that combined controlled vocabulary terms (e.g., "health technology assessments", "patient involvement", "patient engagement", "public engagement" and "patient input") with free text terms related to Health Technology Assessments (HTAs) (e.g., "rare

disease”, “orphan drugs”). The search strategy was applied to all the databases available through the Ottawa University Library. No time limit was applied to the publication dates.

I also performed a search of the grey literature using the Google search engine with the same search terms identified in the search strategy. This approach allowed me to retrieve several useful documents, including federal and provincial government publications, Parliament reports, committee proceedings and testimonies, CADTH documents, and news articles. Over 25 publicly available sources were reviewed, such as documents on the Health Services Restructuring Commission, the Ontario Ministry of Health’s Public Dialogue, Ontario Legislation, Parliament Committee and Senate Reports, international HTA reports, patient input guidelines and templates (produced by HTA international), as well as CADTH’s patient input guidelines, submission templates, Common Drug Review (CDR) guidelines and material from CADTH’s past Symposiums. In addition, several websites were scanned from governments, international drug regulators, the pharmaceutical industry and third-sector organizations on relevant patient involvement information and programs.

More specifically, I reviewed program and process related documents on CADTH’s website. I also viewed CADTH videos in which key individuals (whom I interviewed as well) discuss the patient input initiative. CADTH webinars and lectures were also referenced to gain a broad understanding of CADTH’s role in the healthcare system. Based on a preliminary analysis of the search sources, two interview guides were developed: one targeting CADTH staff (Appendix C) and one for patients (Appendix D). Three main themes were used to direct the interviews: procedural knowledge about the Patient Input Program, experience with patient input, and insights about government-stakeholder relations. The questions aimed to capture the different nature of their respective roles and involvement in the Patient Input Program.

Flexibility was applied in the case of patient groups given the fact that not all groups were active members of CODR. Groups had different levels of familiarity and experience with the Patient Input Program.

3.4.2 Qualitative Interviews

In addition to the use of documents and audio-visual materials, interviews with key stakeholders involved in the patient input process allowed me to uncover the perceptions, perspectives and practices of various groups who shape drug coverage decision-making. Research participants were employed by either CADTH or members of rare disease patient groups in Canada, the majority as members of CODR. In total, semi-structured interviews were conducted with 30 individuals with various backgrounds and expertise, including patients, patient advocates, staff within health charities, CADTH and pCODR staff, and members of their drug expert committees (CDEC and pERC) (Appendix H). These individuals had various disease experiences and backgrounds in health advocacy, managing patient groups, providing support services to patients and their families, clinical practice, government policy and program implementation, academia, and basic science research. Interviewing research participants from both CADTH and the patient community allowed me to unveil the different perspectives on the topic.

Interviews were scheduled between June to September 2015 and varied in duration from 20 minutes to 75 minutes. Due to the geographical locations of participants across Canada, only 4 interviews were conducted in person and the rest were conducted by telephone and recorded on an audio-tape. The project synopsis was also available to individuals seeking further information about the research objectives. Through a consent form (Appendices E and F), key informants were given information about the study and the ethical considerations associated with study

participation. Participants were also given the choice to remain anonymous. Before the scheduled interviews, participants were asked to sign and return the consent form.

A semi-structured approach was taken for the interviews to allow me to explore certain key areas in all interviews, which were adapted to the community of individuals interviewed. For example, in all interviews with officials of CADTH, CADTH's pan-Canadian Oncology Drug Review and drug expert committee members, I asked the research participants to discuss the triggers for the inclusion of patients in HTA decision-making, the role of patients' feedback in these decisions and the perceived challenges. In interviews with patients and patient advocates, I asked research participants to discuss their involvement in health advocacy, their familiarity or experience with the Patient Input Program, and the perceived challenges. Certain individuals were sent advance interview questions in preparation for the interview and others were sent follow-up questions when necessary.

During the interview process, I was truly touched by the respondents' openness and generosity. Some patients and patient advocates discussed the difficulties of living with a rare disease and their life struggles, which includes losing loved ones and suffering on a daily basis. Their genuine responses made the research possible. As well, I was grateful for the frank and informative insights that others working under the CADTH umbrella were able to provide. The patient involvement narrative could not have come together without their contributions. Below I shortly describe data analysis. Further details can be found within the different chapters.

3.5 Data Analysis and Validation Strategies

For the purpose of this qualitative study, data analysis and data validation occurred iteratively. Since the investigation concerned the interactions among people, groups and

institutions involved in the patient involvement enterprise, it was important to weave the various voices and sources together in a narrative that responded to the research questions. I approached the content-analysis of the material collected following both an inductive and deductive method (Berg 2004; Creswell 2016).

The development of inductive categories allows researchers to link or ground these categories to the data from which they derive. Certainly it is reasonable to suggest that insights and general questions about research derive from previous experience with the study phenomena. This may represent personal experience, scholarly experience (having read about it), or previous research undertaken to examine the matter. Researchers, similarly, draw on these experiences in order to propose tentative comparisons that assist in creating various deductions. Experience thus underpins both inductive and deductive reasoning (Berg 2004: 246).

All tape-recorded interviews were transcribed and imported into QDA Minor for analysis. This qualitative data management software allowed me to manually code the interview data based on a coding scheme. To begin the analysis, I first randomly selected a few transcripts, read them attentively and created three broad classifications, which I identified as disease-related, patient-related and HTA-related. I then coded common ideas (i.e., categories) that emerged from the interviews. Thirdly, these were weighted against themes that were identified in the literature and public documents, which served to further organize and sort the content of the material. These themes included illness identities, patients' experiential repertoire, the role of evidence, enabling factors of patient involvement, and obstacles hampering patients' participation. Lastly, inter-connections were made between the categories and themes, which allowed me to explain the patient involvement story.

Figure 3.5 demonstrates the inter-connections between the categories that emerged from the interviews and the themes that were identified in the literature and public documents.

Figure 3.5 Coding Scheme

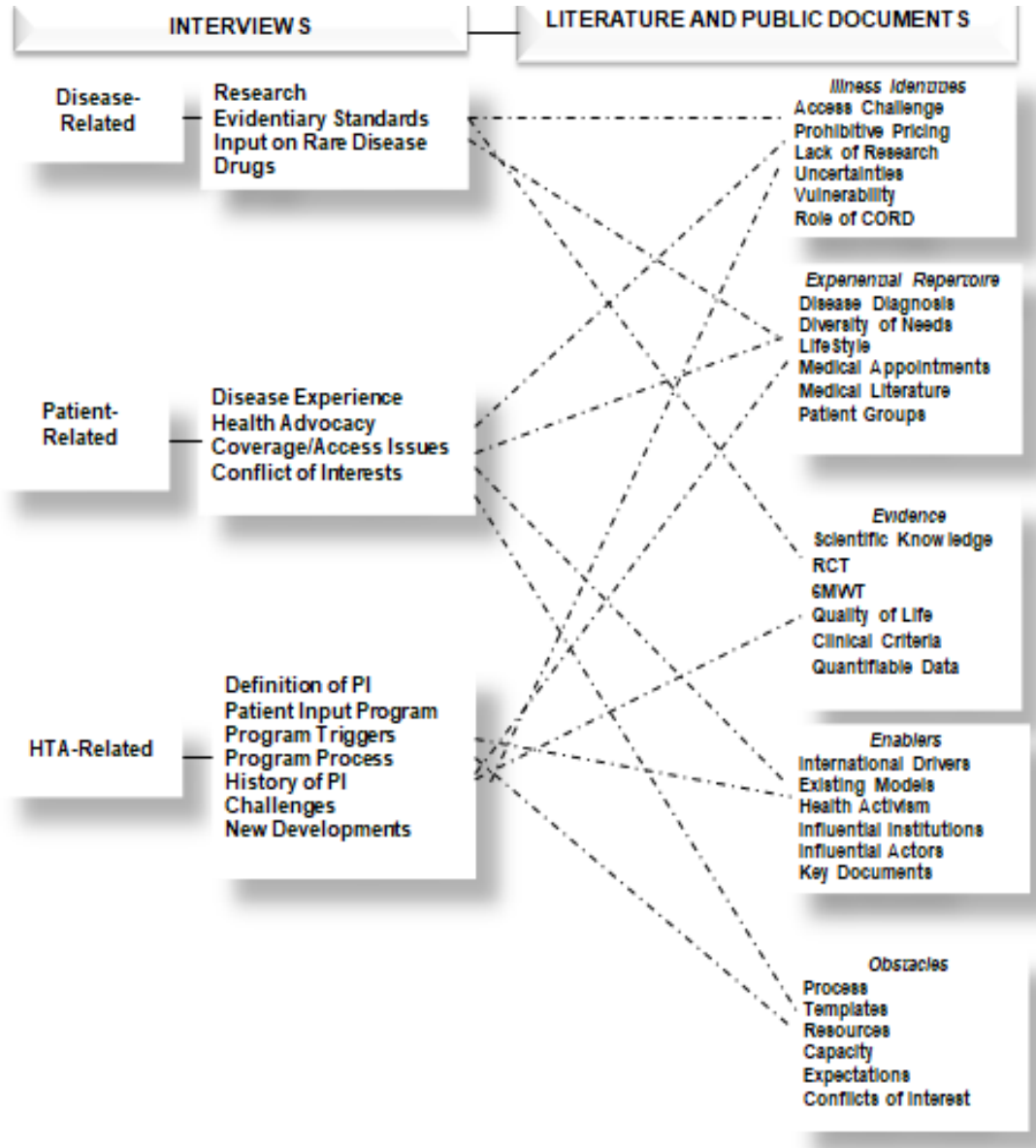


Table 3.5 presents the common themes and categories in a tabular format. These groupings of information stimulated the analysis and generated major findings.

Table 3.5 Themes and Information Groupings

Illness Identities	Experiential Repertoire	Evidence	Enablers	Obstacles
Access Challenge	Disease Diagnosis	Scientific Knowledge	International Drivers (iHTA)	Process
Prohibitive Pricing	Diversity of Needs	RCT	Existing Models	Templates
Lack of Research	Life Style	6MWT	Health Activism	Resources
Uncertainties	Medical Appointments	Quality of Life	Influential Institutions	Capacity
Vulnerability	Medical Literature	Clinical Criteria	Influential Actors	Expectations
Role of CORD	Patient Groups	Quantifiable Data	Key Documents	Conflicts of Interest

By use of a triangulation approach, which combined content-analysis of the interviews and documents from the published literature and grey literature, I was able to produce an account of the shaping of the Patient Input Program and its shaping effect, which I organized into three chapters. Firstly, based on the various enabling conditions that were identified, a discussion was initiated on the institutionalization of patient involvement in the HTA network, and the enabling factors that led to the structured involvement of patients in drug coverage decision-making. The interviews pointed to important leads involving key actors, organizations, key events and periods, which were verified by a search of document sources. To anchor the findings in the academic literature, I used scholarships on engagement, lay expertise, and boundary work.

Some themes were combined and cross-analysed, and subsequently led to a discussion around claims-making. For example, the themes “illness identities”, “experiential knowledge” and “evidence” were combined and triggered analysis around the characterization of patients, their claims and the nature of drug coverage decision-making. To make sense of these inter-

relationships, I consulted the literature on the politics of needs and evidence-based policy-making. It was important to establish a basis for understanding patients' needs in the field of rare diseases and show how they relate to discourses about patient involvement and the HTA evidence models that underlie drug coverage recommendations.

Finally, a discussion focussed on the many obstacles to patient participation in drug coverage decisions. The use of the boundary object concept enabled me to look at the Patient Input Program from concept to practice, describe the different actors who are part of the HTA network, determine their role and stakes in the Common Drug Review Process, and argue whether the Program acts as a positive or negative boundary object for those involved. In the follow section, I explain ways I applied reflexivity and sought trustworthiness in the research study.

3.6 Trustworthiness and Reflexivity

To protect the confidentiality and anonymity of research participants, I provided them with assurance that the information shared would remain strictly confidential and would be used only for the dissertation, publication in scientific journals or meeting presentations. In some cases, names have been changed to protect the identity of the individual interviewed. Confidentiality of participants was protected at all times (during and after the research endeavor). Although their names were not used in the published dissertation or associated materials, their organization and position was revealed (unless the research participant did not grant me permission). Participants were also asked for their permission to be audiotaped during the interview to make sure that conversations were recorded accurately. Participants could still be interviewed even if they decided not to be taped. I also informed participants that the information collected (i.e., interview transcripts and my notes) would be kept in a secure manner in a locked

filing cabinet and kept on an encrypted memory stick. Participation was voluntary as participants were under no obligation to participate and could withdraw from the study at any time and/or refuse to answer any questions, without suffering any negative consequences.

To validate the research findings further, a summary of the discussion points was developed based on the transcribed interviews and sent to participants providing them with the opportunity to make edits or revise the information for accuracy. Out of the 30 individuals interviewed, a total of 12 individuals responded and provided editorial changes and/or clarifications to my written summaries.

Throughout the data analysis phases, I continuously reviewed the research questions, reflected on them and refined them as the data revealed insights. I also referred to the interview transcripts and summaries to ensure that relevant information was not overlooked accidentally. The dissertation writing was therefore an iterative process between the public records of patient involvement and interview findings. In discussing the research findings, quotes were provided to illustrate major points while protecting confidentiality of participants. Quotes only identified participants by title or group membership.

As part of demonstrating the trustworthiness of the research, my co-supervisors served as a sounding board and performed external checks throughout the data collection and writing process.

During this writing process, I also attempted to build in reflexivity by considering my dual role as researcher and public servant with the Department of Health. I had to reflect on my own knowledge base, training and assumptions about government-stakeholder relations because of the bureaucratic culture I have been grounded in since the beginning of my work life, over 15

years ago. To begin, I did not conduct an ethnography of patient involvement, as is common among conventional science and technology studies scholars; I did not immerse myself in the work of CADTH nor did I do participant observation. However, my insider status as a policy-maker in regulatory science gives me firsthand experience with the negotiation process that is involved between scientific knowledge and political power. In my line of work, policy-making includes groups who advance science, client groups who are the targets of policy decisions, and elite groups who manage and make decisions. My experience as a public servant is not negligible. It provides me insights on the policy-making machinery. At the same time, I recognize that my experience is also narrow; I have always worked as a policy analyst nestled in the comfort of my cubicle, removed from the real-world setting and from patients' experience with the healthcare system. The works of Jasanoff (1996, 2011) and Marston and Watts (2003) on the biases of the policy-making machinery were revealing on this front and triggered self-examination about the nature of health policy and the institutional culture I have been immersed in. This hindsight enabled me to understand why I have a tendency to write in the third person and become invisible in the dissertation, unconsciously feeling the pressure of objectivity.

Because of this process of reflexivity, the research process alerted me to the consequences of program implementation on the groups whose lives may be impacted, and challenged my taken-for-granted assumptions about patient groups. For instance, based on my limited knowledge of patient groups and my readings of certain theoretical works, I assumed that patient groups were homogeneous in nature. My empirical findings suggested otherwise. Although patients can join patient groups under one common goal, their health needs, values and preferences can vary significantly. Patient groups also prioritize the needs of some at the expense of others. Further, not all patients desire to be organized and represented by a patient group.

There are various reasons why patients choose not to join groups. I had to readjust my thinking as evidence of this diversity mounted.

Another perceived notion was about the culture of secrecy within institutional settings. Having been immersed in a bureaucratic organization that has been plagued with secrecy, I expected the same from CADTH. Although CADTH is a non-government organization, it does not appear to be at arm's length from the federal government, as people might believe. Because of its funding relationship and mandated commitments and processes, CADTH has a direct relationship with the federal government. I was pleasantly surprised by the support of CADTH officials towards the research endeavour and the transparency they demonstrated throughout the process, even with certain restrictions they imposed on my study.

Regarding the process of reflexivity, it does not only address my roles as a research and public servant, but it includes thinking critically about the research process, the outcomes of the data and analysis (Zevallos 2010). On this point, the research enabled me to understand “the real-world constraints” of public engagement programs (Marks and Russell 2015) and my role as a social scientist in providing public engagement opportunities in policy-making. My research intention was not simply to criticize a process and show the hidden stakes and power imbalances (Latour 2004). Through the research process, I sought to learn about patients’ experiences, and think of ways that sociological work can assist policy-makers in facilitating involvement that is more meaningful. Zevallos (2010) argues that sociology students should be socialized to think more about the applications of their skills to policy problem solving, while considering the groups affected by the policies. For example, I suggest that qualitative researchers may have a role on drug expert committees and evidence-based policy.

I also came to the realisation that my academic journey in political science and social work has also shaped my “worldview” in terms of my critical reading of the Patient Input Program and my interest in the principles of equality and respect for individuals who are disadvantaged in society. These values and educational experiences influence the research project because “all writing is “positioned” and within a stance...how we write is a reflection of our own interpretation based on the cultural, social, gender, class, and personal politics that we bring to research” (Creswell 2016:179). The most educational aspect of my journey through the research process was to listen to patients and getting to know their experiences. Throughout my career as a public servant, I have always wondered who the patients we serve are. Where is the patient voice? Although I work in a health department mandated to help Canadians attain the best quality health, until now, I had no direct contact with patients. Because of my academic and personal biography and the fact that I am coming to qualitative research for the first time, I was perhaps overly enthusiastic about the patients I interviewed and focussed particularly on the accuracy of their narratives, without accounting for the way I saw and heard during the data analysis phase (Mautner and Doucet 2003). For instance, I seem to relate more to patients who spoke about their sick children given my own experience of motherhood. As Mautner and Doucet (2003) point out, some researchers privilege one perspective based on their epistemological standpoint. In my case, I generally had a tendency to privilege the accounts of patients’ and promote the voice of marginalized patients. Despite the challenges presented by my personal narratives in the research process, the experience I have gained from my research will forever inspire me and will inform my work for years to come.

3.7 Conclusion

In summary, the study framework is informed by boundary object theory with a focus on the politicization of claims. To peel the layers of the Patient Input Program, it was necessary to understand the HTA network as a field of competing communities of practice and examine the discourses on patients' needs in the area of rare diseases. This required studying the social dimensions of patient involvement, the process of claims-making, and the formation of knowledge boundaries. To understand how patient involvement infiltrated the HTA network, it was necessary to look at established evidence models and ways different communities of practice interpret patients' needs. This led to an examination of power dynamics among the players in the HTA network. By making visible the challenges of patient groups concerned with rare diseases, it is then possible to see the struggles of patients in dominant social institutions. In line with the ontological and epistemological principles of social constructivism, the research strategy and methodology attempted to account for the social phenomenon of patient involvement through documents and actors themselves as they unfolded in the raw findings cumulated. Furthermore, the research takes on a reflexivity approach that considers my position as a researcher and public servant as well as the relationship between the data gathered from research participants and my interpretation of the findings. In describing my trajectory and discussing preconceived notions, I have provided context for this research. Further, the efforts to validate the research data and foster trustworthiness towards research participants demonstrate my willingness to co-produce knowledge about patient involvement with the research participants who share the final story. As per Guba and Lincoln (1994), "constructivism...sees knowledge as created in interaction among investigator and respondent" (Guba and Lincoln 1994: 111). In doing so, the dissertation

supports social justice issues and values the lives and experiences of marginalized groups. The following three chapters present the study's major findings and arguments.

CHAPTER 4

MODELS AND RHETORICS OF PATIENT INVOLVEMENT

This chapter elaborates on the emergence and establishment of patient involvement practices in drug coverage decision-making in Canada. As part of this discussion, the role of the Health Technology Assessment (HTA) network and the factors that led to the structured involvement of patients are investigated and described. The findings demonstrate that the Patient Input Program is an offspring of various social conditions. More specifically, through the setting of boundaries between expert knowledge and lay perspectives, the Canadian Agency for Drugs and Technologies in Health (CADTH) promotes what counts as credible knowledge in the HTA decision-making network. Despite the structural inclusion of patients in a feedback mechanism within the Common Drug Review process, CADTH prevents patients from structurally influencing the drug expert committee given the fact that patients are not voting members of the committee. Thus, CADTH's logics of scientificity, rationality and academic study prevail over patient considerations, as the findings will demonstrate.

Data were collected by means of a literature search, scanning of policy documents, and semi-structured interviews with relevant participants, who commented on the history of the Patient Input Program. The chapter sought to explain how the Patient Input Program was brought into practice in Canada. First, the chapter focusses on the path of emergence of patient involvement through different spaces and discourses. The second section describes the enabling conditions that led to the structural involvement of patients in drug coverage decision-making, while the final section discusses the results and presents concluding remarks.

4.1 Emergence of Patient Involvement Practices

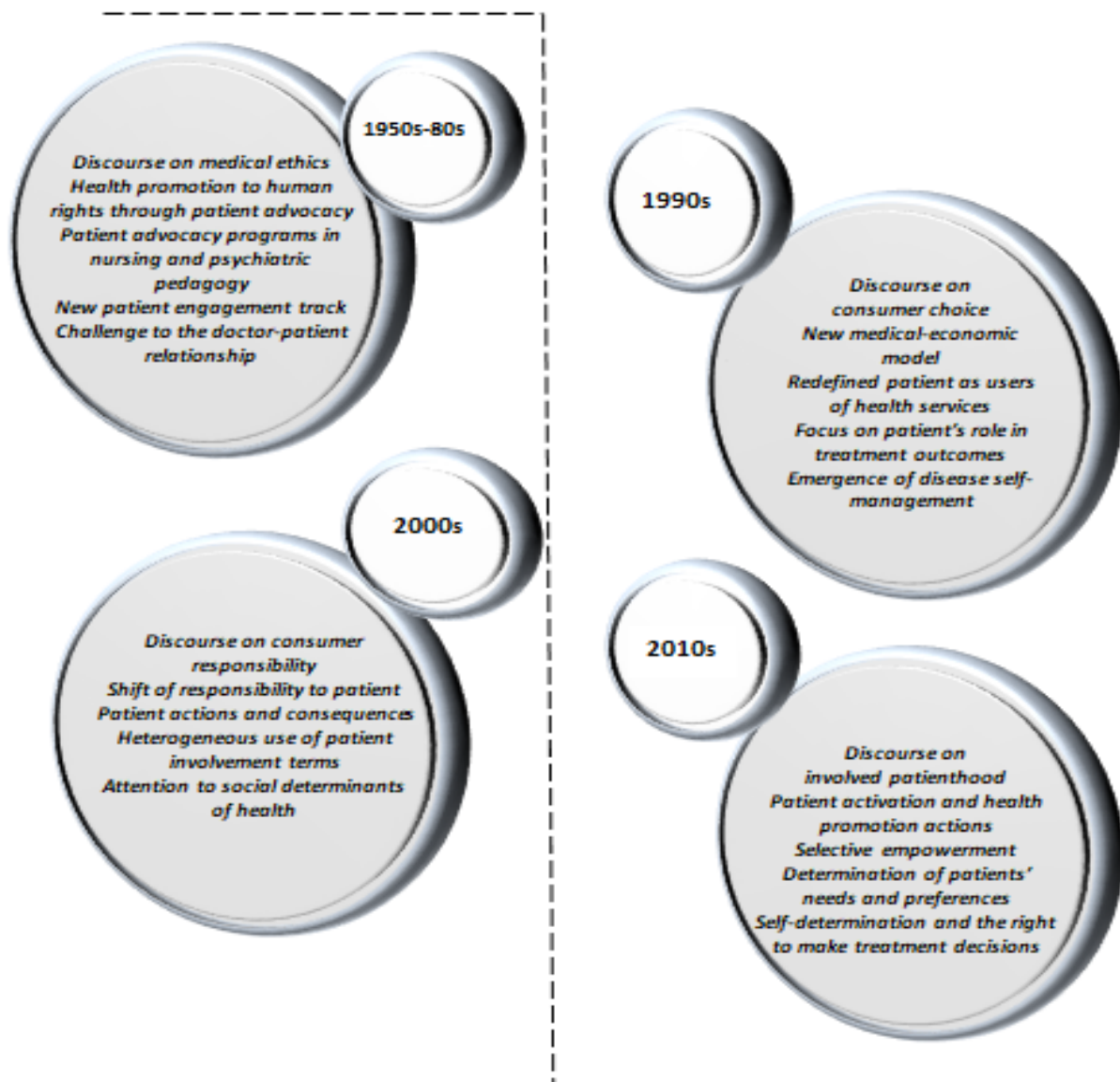
To account for an untold chapter of patient involvement, a review of scholarly journals was performed with the terms “patient involvement” and “patient advocacy” in their titles. Considering that the term “patient input” is not commonly used outside the context of HTAs, it was important to look more broadly at these terms due to their frequent use and proximity. From this search of the literature, 708 records were found spanning the years between 1971 and 2016. In general, the volume of peer-reviewed publications was scarce in the academic journals in the 1970s and peaked between 2009 and 2016 in which 446 studies were published. Starting in the 1980s, publications relating to patient involvement slowly formed an interdisciplinary literature on the engagement of patients in healthcare decisions. This growth of the literature culminated in 2015 with the launch of a patient involvement journal. From this scanning of the literature, I contend that the concept of patient involvement emanated from notions of patient advocacy in domains pertaining to nursing and community involvement, psychiatric and mental health services, prosthetic dentistry, and healthcare services such as in-patient and out-patient care, cancer care (namely breast cancer), palliative care, occupational therapy, surgery and pain relief management. Hence, patient involvement began as an extension of patient advocacy and was enabled by a rights discourse that rests on a central premise: patients and their needs are embedded at the heart of the healthcare system. The inclusion of patients in health-related decisions therefore emerged in healthcare practices and led to redefining the doctor-patient relationship in an effort to correct an imbalance of power between those who seek medical treatments and those who offer medical services. More recently, patient involvement in the organization of healthcare refers to discourses on patients’ needs in terms of service delivery, consumer responsibility and self-advocacy. Gradually a shift can be observed from the

involvement of patients in medical decisions to the involvement of patient groups in broader healthcare system needs.

It can be argued that patient involvement embarked on a distinct course than patient advocacy, occupying different spaces and reflecting different discourses and practices. In the healthcare setting, patient involvement is analogous to patient empowerment in terms of a shared decision-making model and symmetrical relationship between patients and their healthcare providers. In public policy, patient involvement has become a democratic principle added to the health policy toolbox. It carries claims of democratization and redistribution of power among citizens (Aronson 1993; Contandriopoulos 2004; Tritter and McCallum 2006 and Martin 2008). Within Health Technology Assessment (HTA) circles, patient involvement is an evolving practice as HTA agencies have turned the policy goals of involvement into patient input platforms. As previously noted in an earlier chapter, a growing HTA scholarship (Abelson et al. 2002, 2007; Bridges and Jones 2007; Menon and Stafinsky 2008; Facey et al. 2010; Bombard et al. 2011; Gauvin et al. 2010; Gagnon et al. 2011, 2012; Kreis and Schmidt 2012) has provided insights on engagement methods and the place of patients in HTA decision-making. Here, patient involvement programs are used as policy instruments to gauge public acceptance for resource allocation recommendations. The following section describes in more detail the emergence of patient involvement within the span of four decades, and shows how the concept of patient involvement crossed multiple spheres of social life.

Figure 4.1 illustrates how the patient involvement phenomenon emerged from social change and produced social change. Here the findings tell a great deal about the shape and the shaping of social phenomenon.

Figure 4.1 The Course of Patient Involvement



4.1.1 The Advancement of Patients' Rights

To begin, the findings reveal a shift in healthcare in the 1970s and 1980s from health promotion to a human rights approach. This shift marks a significant social transformation that stems from patient advocacy programs in nursing and psychiatric pedagogy. In the 1970s and 1980s the concept of patient advocacy received wide coverage in the nursing literature (Robinson 1985). It was identified as a crucial function of nursing in geriatrics (Stilwell 1976) and low-income communities (Kosik 1972). The nurse's role was seen not only as healthcare provider but as defender of patient rights and an enabler of independence. This period was also distinguished for the general increase in mental health advocacy programs, which were viewed as safeguards for psychiatric patients either needing legal representation or faced with involuntary hospitalization (Perlin 1982; Turner et al. 1984). Turner et al. (1984) discuss Canada's first province-wide system of human rights advocacy for psychiatric patients in terms of conceptualisation, design and implementation. In their evaluation of the program's successes, the authors comment on the nature of the healthcare system "where the natural goal is to promote health, rather than rights" (Turner et al. 1984: 349). In the nursing and psychiatry literature, the concept of patient advocacy is directly linked to human dignity and the right to justice for patient populations that are perceived as vulnerable. The works insist that patient advocacy is a task-oriented approach (based on readings of Fitzpatrick 1981; Bradley et al. 1982) that has an element of empowerment and self-help through education and knowledge. Although the literature promotes more safeguards to protect patients in the healthcare setting, Bradley et al. (1982) posit that few attempts have been made to alter the traditionally passive role of patients. Thus, patient advocacy and human rights may be intimately connected but these concepts do not provide an understanding of patients' role in the medical encounter.

Greenfield et al. (1985) introduced the concept of patient involvement as it relates to patients' engagement in medical care. Their work presented a new aspect of empowerment that focused on ways that patients can influence the patient-healthcare provider relationship. For example, the authors use the patient's medical record and accompanying algorithm to identify decision points relevant to patients' care. They suggest prepping patients to influence the doctor-patient interactions at follow up visits. For his part, Weiss (1986) proposed the use of a health contract between the patient and health professional to set the parameters of the relationship and patients' expectations, including active participation in the discussion and treatments tailored to their values and lifestyles. Patients' health outcomes are now a critical component of the medical encounter. Increasingly in the 1980s, research in the fields of medicine, surgery and dentistry studied how patient involvement can directly impact patients' health outcomes. Some authors focussed on patient satisfaction and noted a correlation between patient involvement, satisfaction and culturally and socially endorsed expectations (Larsson et al. 1989) while others demonstrated that patient satisfaction and acceptance of a certain therapy was attributed to their involvement in the process, such as the case of denture selection (Hirsch et al. 1972). Moreover, in diabetes treatment, patient education researchers showed the positive outcomes of active involvement in patient education strategies. The strategies involved patient rehearsal, feedback and correctives on treatment plans, periodic review of disease management, and constant motivational follow up (Hanson and Pichert 1985). These works emphasise a new role for patients in the medical encounter. Here, a conceptual change appears in the medical literature. Through involvement processes, vulnerable patients can become empowered patients since they can influence the dynamic and outcome of the interaction with their physicians in order to access adequate health services and treatments.

This discussion leads to an understanding of patient involvement that is distinct from patient advocacy. Patient advocacy in this period is discussed in the context of patient rights and the patient's best interest, whereas patient involvement related to notions of patients' engagement in health decisions, and more generally to the role of individuals participating in the interaction between patients and healthcare providers.

4.1.2 End-users and the Choice of Treatment

In the 1990s, the volume of publications on patient advocacy and patient involvement doubled and the approaches continued to follow separate pathways. On the one hand, patient advocacy remains at the forefront of the nursing literature. Discussions focused on the risks associated with patient advocacy from the nurse's perspective (described as whistle blowing by Andersen 1990) and the ethical considerations they take into account to protect patient rights, which sometimes conflict with medical practice (Wiseman 1990; Sellin 1995; Love 1995). On the other hand, patient involvement highlights the role of patients in their treatment success. For example, patient involvement is regarded as a tool for increased success in the psychiatric patient's rehabilitation post-hospitalization (Nightengale 1990). Works in the medical literature, including the discipline of medical ethics, also discussed the role of patients in contributing to the effectiveness of patient education programs and disease self-management (Cicutto et al. 1999) as well as cancer pain management and cancer support services (Calder et al. 1993). Hence, patient involvement refers to a patient's capacity to direct their healthcare.

The growing importance of patients' involvement in healthcare is illustrated by works that proposed ways to measure patients' level of engagement in doctor-patient interactions. Health communication research, for instance, demonstrated the benefits of an active patient model that promotes participative decision-making. In this model, AIDS patients are encouraged

to be assertive towards their healthcare and willing to be mindfully nonadherent to recommended treatment when alternative treatments are in their best interest (Brashers et al. 1999). Street et al. (1995) also put emphasis on choice in their study of the impact of pre-consultation materials on the selection of early treatments for breast cancer patients.

So far, the literature on patient involvement has been useful to decipher the trajectory of patient involvement in terms of patients' behaviours, decision-making processes and relationships with their healthcare provider. It does not however explore the shaping effect of social context on the patient involvement phenomenon. Although one can view patient involvement as a social product created by society, and a force shaping relationships therein, one must recognize that patient involvement emerges from the social and political ground we stand on. In this regard, an important subset of this literature is dedicated to the negative impact of the new medical-economic reality in the U.S., which is driven by managed-care and third party reimbursement. These consequences are seen on women's health and well-being (American College of Obstetricians and Gynecologists 1996), brain injury rehabilitation (Banya 1999), and organ transplantation (Weston and Lauria 1996). The medical literature reveals the consequence of low-resource settings on patient care and points to trends of commercialism and entrepreneurship in healthcare (Flynn 1995). In response to the health inequities produced by resource deficiencies, there is a general societal consensus that the position of patients as "users or consumers of medical service" must be strengthened (Forster 1998: 155). Forster (1998) refers to a new inventory of terms in the healthcare setting such as, user involvement, consumerism, community participation, advocacy, and empowerment. These terms share the same conviction that a correction of the imbalance of power between professionals and recipients of healthcare services will primarily come from a bottoms-up approach which in turn, will reinforce the

patient's place within the healthcare encounter. In the 1990s, the works expose a discourse of choice in the context of consumerism that redefines the role of the patient as a consumer of services. Patients are now viewed as empowered users with service expectations.

This review of the patient involvement literature suggests that patient advocacy and patient involvement have different interests. While patient advocacy stresses human dignity, rights to justice, empowerment and self-help for vulnerable populations, patient involvement fosters active engagement in medical care and treatment decisions through an altered doctor-patient relationship in which patients are empowered users of medical services. While patient advocacy serves as a go-between for patients, their family members and healthcare professionals, patient involvement places the patient at the centre of their own healthcare.

4.1.3 Consumer Responsibility and Shared Decision-Making

In the 2000s, over 170 publications were made available in the scientific literature on the topic of patient advocacy and patient involvement that point to co-existing discourses with respect to patients' role in their healthcare. Here, we see a shift in the research as American, European and Australian scholars from nursing and medical communities attempt to conceptualize patient advocacy and patient involvement. Hyland (2002) discusses the ethical and legal roots of patient advocacy in relation to the principle of "patient autonomy" whereas Balwin (2003) looks at patient advocacy through nursing attributes and concludes that patient advocacy is a combination of essential helping strategies (i.e. valuing, apprising and interceding). In a similar vein, American scholars Bu and Jezewski (2007) developed a theory by means of a systematic review of the literature to guide nursing research and practice. They conclude that the core attributes of patient advocacy are safeguarding patient autonomy, acting on behalf of patients and championing social justice in the provision of healthcare. These works offer critical

insights that provide an understanding of patient advocacy as a unidirectional relationship between guardians of care and vulnerable patients. Scholars also examine patient advocacy from a cultural perspective in terms of its practical application in nations where the nursing culture differs, such as Japan (Davis et al. 2003), Iran (Negarandeh et al. 2006) and Ireland (O'Connor and Kelly 2005). In these studies, patient advocacy is described as a western concept stemming from the civil rights movement and rise of consumerism in the U.S.

In the medical literature, certain trends are apparent with respect to patient involvement. Through the literature search, a shift was identified to the term “patient engagement” with respect to disease self-management and patient responsiveness. More generally, scholars argue that healthcare consumerism has shifted more healthcare responsibilities to patients as consumers. In this regard, a new discourse on consumer responsibility begins to surface and now becomes “a patient’s deliberate effort to work toward recovery by participating fully in their rehabilitation therapies” (Lequerica et al. 2009: 753). While the patient involvement literature of the 1970s and 1980s focussed on the patient-doctor interaction, ideally as a symmetrical relationship that entails a negotiation of expectations, the patient involvement literature of the 1990s and more so in the 2000s attributes the leading role to patients themselves. At the turn of the millennium, scholars consider ways that patients’ actions can improve or worsen health outcomes. For instance, Bourbeau (2008) conceptualizes a self-management program for patients with chronic obstructive pulmonary disease (COPD) that involves acquiring self-treatment skills and sustained personal performance in relation to the expected outcomes of care. In other words, patients must evaluate their actions and understand the consequences that result from them. In this model, not only is the doctor-patient relationship described as a partnership in need of

continuous interactions and communications, but it also refers to a shift in responsibility to patients.

To explain health responsiveness, researchers also analysed patient behaviours within the doctor-patient relationship through a set of influential factors. Protheroe et al. (2008) discuss the role of information sharing tailored to the type of condition and illness stage as a means to influence a patient's behaviour in self-care for chronic conditions. For his part, Howe (2006) argues that inter-professional partnerships with patients have a role in influencing patients' participation and responsiveness in safety-related behaviours that prevent accidental or preventable harms and injuries produced by medical care. In contrast, Davis et al. (2003) conclude that participation and health responsiveness varies among patients based on demographics, disease severity, level of health literacy, emotions, relationship with care givers and the care setting. On the other hand, researchers have looked at ways to measure patient engagement in treatment decisions through the behaviours of physicians and patients themselves. By introducing an OPTION (Observing Patient Involvement) Scale as a means to score the extent to which clinicians involve patients in the decision-making process, Elwyn et al. (2003) revealed the lack of partnership between physicians and patients within the medical encounter in the mental health context and a very limited degree of patient participation. For their part, Geest et al. (2005) demonstrated the absence of a consensus among physicians and patients on preferred engagement methods with elderly patients, such as patient satisfaction questionnaires and patient information leaflets. Preferences varied from seven countries studied. Lester et al. (2006) go a step further and conclude that there is resistance to patient involvement on the part of some health providers in mental health services who share "negative patient stereotypes of irrationality, poor intellect, and bad time-keeping, which influenced their perception of

patients' capabilities to make informed choices" (Lester et al. 2006: 419). Researchers have also proposed tools to improve patient engagement in the patient-healthcare provider interaction. One such tool is the 38-item Patient Perspective Survey (PPS) introduced by Laerum et al. (2004), which guides physicians in the management of patients with complex health conditions and centers on patient perspectives and patient participation. The purpose of the PPS is to engage patients and physicians in a more efficient, and open communication approach and to encourage patient participation in determining coping mechanisms and solving health problems related to their disorder. These works highlight the relationship between patient involvement in decision-making, the nature of the patient-healthcare provider interaction and success of treatment outcomes. The selected works also demonstrate that little is known about the extent to which healthcare professionals involve patients in decisions that affect their lives.

Hence, the patient involvement literature in the 2000s reveals the circulation of two underpinning discourses in the healthcare system, one of consumer responsibility and the other of shared decision-making. Within these discourses, the concept of power relations is debated with respect to the decision-making process during the medical encounter. A few authors in the fields of communication, education, psychology and political science have explored the decision-making process within healthcare settings. For example, Kennedy and Rogers (2002) examine the use and influence of information materials in the management of ulcerative colitis. Instead of pointing to decision aids, Martin et al. (2003) looked at the effectiveness of the medical interaction. They recommend more information sharing between the physician and patient. Martin's term "physician-facilitated involvement" is appropriate to describe the role of physicians in facilitating patient involvement. In other words, patient involvement cannot materialize if providers are not able to shift the locus of power to patients in a way that

empowers them. In the context of HIV/AIDS, scholars have explored patient involvement in terms of passive versus active involvement in treatment decisions and refer to the growing number of empowered HIV/AIDS patients (Marellich et al. 2002). Conversely, Kremer et al. (2007) show that there is a high level of uncertainty relating to treatment decisions involving antiretroviral drugs (ARTs) and speak to decisional conflicts and post-decision regrets. Here, the view is that patients are not in ideal decision-making roles. UK political scientist, Andrew Thompson (2007) developed a taxonomy of patient-desired involvement and identified five levels of involvement that range from no-involvement to autonomous decision-making in relation to the desired involvement of patients and physicians. The authors distinguish a collaborative model of shared decision-making between patients and physicians and describe patient participation in terms of “a degree of transfer of power from the professional to the patient in the form of increased knowledge, control and responsibility” (Thompson 2007: 1308).

The works that have been discussed further expose conceptual differences between patient advocacy and patient involvement. Patient advocacy refers to notions of patient autonomy, helping attributes and guardianship, whereas patient involvement refers to notions of personal performance, health responsiveness, patient’s needs and preferences as well as shared decision-making. Moreover, the literature points to the heterogeneous use of terms to define patient involvement such as patient participation, patient partnerships, patient centeredness, patient responsiveness and shared decision-making (Wensing and Baker 2003). Regardless of these differences, Wensing and Baker (2003) posit that all concepts “share the fundamental idea the patients’ needs and preferences influence the process of healthcare provision and its organization” (Wensing and Baker 2003: 62). Furthermore, the aims of patient involvement include a transfer of power between the patient and healthcare professional and consider the

social determinants of health that underlie health inequities as elaborated by Davies et al. (2003). In this period, the research tells us to be mindful of disparities in healthcare, diversity in patient communities and the unequal health status of patients.

In an effort to promote standardized patient engagement methods, scholars in the interdisciplinary field of health policy, investigated how best to design and implement patient engagement models, and emphasise the social factors that create obstacles in successful patient engagement initiatives. Canadian scholar Kreindler (2009) recommends best practices for patient involvement in health services while focussing on methods and outputs that generate different results. She concludes that a successful patient involvement strategy should seek to promote a sense of shared ownership by ensuring the genuine participation of all stakeholders, including patients and front-line staff in decision-making. Gagliardi et al. (2008) concluded, based on an exploratory study of physicians' attitudes towards patient participation in the selection of performance indicators, that patient interest, health professional attitudes, and the lack of insight on appropriate patient involvement methods may be limiting patient participation in healthcare programming. For Smith et al. (2009), patients' level of education and health literacy correspond to varied meanings of patient involvement, and consequently, a patient's ability to be involved. To explain patient involvement in the context of pain management with elders, Borders et al. (2005) highlight other factors such as the presence (or absence) of health coverage and the presence (or absence) of a personal doctor. These factors may lead to fewer healthcare services for certain elders and more suffering.

4.1.4 Self-Advocacy and Patients' Needs

From 2010 to 2016, the number of publications relating to patient involvement increased to 446. In this decade, the medical literature dominates the literature on patient involvement and

patient advocacy. The use of the terms “patient involvement” and “patient engagement” appear to be used interchangeably but a trend in the literature appears around patients’ needs and the impact of the patient involvement agenda. Scholars continue to study how to improve patient engagement in medical decision-making and improve treatment adherence through the doctor-patient relationship (Holzmueller et al. 2012; Flickinger et al. 2013). The concept of shared decision-making (SDM) officially takes shape in literature and is described as a medical decision model where patients and doctors participate in sharing information and values and where patients play an active role in making healthcare decisions. Yet, even with this attention to the principles of SDM, there remains a lack of clear guidance on how to accomplish SDM in routine practice.

Some scholars explored tools and strategies to enhance SDM and improve disease self-management with the use of mobile-enabled devices in blood pressure remote monitoring programs (Agboola et al. 2013), use of social media in educating oncology patients (Thompson et al. 2012) and use of multimedia resources in improving diagnosis and treatment of depression (Kravitz et al. 2013). Thus, health responsiveness remains at the forefront of these discussions. In this context, the term “patient activation” is introduced as it related to a patient’s need for knowledge, skill, motivation, and confidence to manage their health and participate in their healthcare (Hibbard and Greene 2013; Hibbarb, Greene and Overton, 2013; Hibbarb, Mhoney, Stock and Tusler 2007 in Mayer 2014). Patient activation and self-advocacy are used interchangeably to describe a patient’s “health-promoting actions” (term used by Wasson and Coleman 2014). This type of engagement “focuses on the behaviours that patient and families do in support of their health and healthcare as opposed to behaviours coming from the healthcare system” (Shonce et al. 2014: 851). The Willow Breast Cancer Support Canada (2010) describes

self-advocacy as patients’ taking an active role in their treatment to make sure they get the support and care they need. “It’s about asking questions and getting answers” (Willow Breast Cancer Support Canada 2010: 5 in Sinding et al. 2012: 410). The discourse on patient activation (or self-advocacy) implies that the quality and success of a patient’s healthcare is contingent on the patient’s willingness to take charge of their health situation. Here, patients are responsible for their health and can manage their health needs. As well, patients are expected to have an educated understanding of their health needs. These last conclusions lead me to relate patient involvement to a focus on patients’ needs.

This investigation of the literatures on patient advocacy and patient involvement has allowed me to delineate a patient involvement model which encompasses elements of a new healthcare tenet, which is characterized by patients’ self-determination and right to make decisions regarding treatment.

Table 4.1 presents a side-by-side comparison of the patient advocacy and patient involvement models.

Table 4.1 Patient Involvement: A New Healthcare Tenet

PATIENT ADVOCACY	PATIENT INVOLVEMENT
Human Dignity Rights to Justice Empowerment/Self-Care Vulnerable Patients Patient Autonomy Helping Attributes Guardianship	Engagement in Medical Care Active Role in the Doctor-Patient Interaction Personal Performance Health Responsiveness Patients’ Needs and Preferences Transfer of Power Shared Ownership Self-Advocacy

As this scoping of the literature has shown, the intent of patient involvement was to forge a place for patients within the doctor-patient relationship allowing them to assert their needs; patient involvement was bound to medical decision-making. Yet patient involvement entered the sphere of health policy and gradually surfaced as a means to transform policy-making into a bottoms-up approach. Through social participation, citizens and patients began voicing their needs in public forums and putting pressure on the state. I now investigate this new citizen-state relationship in health policy.

4.2 Institutionalization of Patient Involvement

In this section, I analyse the individual, organizational and societal factors that influenced the course and shape of patient involvement in Canada. In the Canadian setting, there are several examples of structured and non-structured ways of including patients in health policy. However, the inclusion of patients in formal or informal ways does not appear to enhance patients' influence in policy-making. I examine more closely patient involvement through the lens of provincial and federal bodies, international agencies, and patient activism.

4.2.1 Provincial Ministries of Health

The literature suggests that the gradual uptake of involvement initiatives in health policy can be explained by the broader role of government in responding to a legitimacy crisis leading up to the health services restructuring in the 1990s. As per Church et al. (2002), since the reorganization of Canada's health system, citizens have become increasingly critical of governments' unresponsiveness and unaccountability. For Menon and Stakinsky (2008), this public outcry made the call for greater public involvement and accountability in healthcare decision-making clearer than ever in Canada. The authors identified a trend of eroding

confidence in the Canadian healthcare system in which “citizens are increasingly questioning decisions that involve funding some services and not others” (Menon and Stakinsky 2008: 283).

Ontario has a history of structured and non-structured involvement of the public in resource allocation. In 1973, the province established sixteen District Health Councils (DHCs) to provide advice to the central Ministry of Health about the local needs of its constituents. DHCs had dual accountability to the Ministry as the funder and the communities they served. Their function was to advise the Minister of Health and make recommendations on the allocation of resources to meet health needs (Collins 1999). They were made up of 15-19 volunteer citizens from local government, the healthcare system and the public in the geographical area they served. The instatement of DHCs is exemplary of the structured inclusion of patients. In Ontario, citizens represented forty percent of the DHC’s membership. DHCs had the most explicit mandates for incorporating public input into health systems decision-making (Padfield 2003). Abelson (2001) describes DHCs as enablers of participation in that they were required to “develop strategies to assure and enhance public participation in all parts of the planning process” (Association of District Health Councils of Ontario 1994, p.59 in Abelson 2001: 787). For over 20 years, certain DHCs in Ontario (i.e., Hamilton-Wentworth and Ottawa-Carleton) built strong relationships to their communities (Abelson 2001)¹⁴.

In 1996, Ontario’s Health Services Restructuring Commission (HSRC) was established by the Government of Ontario to restructure hospitals. As part of the HSRC’s mandate, a public relations strategy was adopted by communities to consider the views of end-users. HSRC recognized that citizens as users of healthcare services have substantial stakes in the reform. The

¹⁴ In 2005, DHCs were disbanded in light of the creation of Local Health Integration Networks (LHINs). These networks plan, integrate and fund local healthcare with a mandate of improving access and patient experience, <http://www.lhins.on.ca/>

Commission's strategy aimed to correct certain misconceptions circulating in the public. For example, a generalized view was that the reform would result in service cuts and job losses. Thus, HSRC was tasked with creating a vision to ensure validity of the restructuring plan, clarify and justify the reform's outcome, and provide a context for generating public and provider feedback. This vision included building blocks for the future health system; a patient-centred system focussed on the total health needs of a defined population. Throughout its mandate, the HSRC continuously engaged the public using various engagement methods as part of a consultation on the government's restructuring of the health system and the creation of an integrated system of healthcare delivery.

The HSRC was determined — to the extent possible — to communicate directly with Ontarians through news media and other communication vehicles. Commissioners were open to interviews with the media and meetings with editorial boards and key reporters were actively sought. The Chair, Commissioners, and Commission staff, in total, gave thousands of interviews. In addition, (as noted earlier) the public process that the HSRC followed for hospital restructuring included a call for written submissions in each community visited, face-to-face meetings with key stakeholders, release of Notices of Intention to Issue Directions, a 30-day appeal period following the issuing of Notices, and then the release of the final Directions. (Health Services Restructuring Commission 2000: 19)

These engagement initiatives supported the HSRC's vision of a health system in which healthcare providers work together toward the common purpose of meeting the publicly set goals, objectives, policies and priorities. As well, HSRC's vision supported the notion that healthcare delivery should reflect community and regional differences in healthcare needs. To ensure a continuum of engagement, Ontario's Ministry of Health maintained its agenda of engagement. For example, in 2001, Ontario conducted a province-wide Angus Reid opinion poll through a mailed survey that was sent to over four million households inviting feedback about the healthcare system. The responses (representing 10% of the provinces' households) indicated

that Ontarians, in particular, those with recent experience of long-term care, emergency rooms, and mental health services, perceived a decline in the quality of healthcare available in their community. As reported in the 2002 *Public Dialogue Report* commissioned by the Ministry of Health in Ontario, the public appeared to have concerns in three areas. These included “shortages of staff or overworked staff in key service areas such as hospitals, home care, and long-term care facilities; waiting times in emergency rooms and diagnostic testing facilities; and concerns about poor service, and to a lesser extent unfriendly and/or rude staff, at hospitals, home care services, and long-term care facilities” (The Strategic Council 2002: 22). Here, the provincial Ministry of Health appears to use public involvement to obtain buy-in from citizens for the restructuring of healthcare services. Interestingly, although the Ministry made efforts to reassure the public that the reform would not have the effect of cutting services and employment, upon completion of the HSRC’s 4 year mandate in making decisions about hospitals in the public interest, the public still perceived hospitals as inadequately staffed and patients not well-served by healthcare providers.

To this day, the Government of Ontario continues to provide opportunities for its citizens to be heard and allows individuals to submit input on a variety of issues. Recent public consultations were held on various topics (e.g., Ontario's First Patient Ombudsman, medical assistance in dying and end-of-life decisions) and policy documents (e.g., *Patients First: A Proposal to Strengthen Patient-Centred Healthcare* and *Developing Ontario’s Dementia Strategy: A Discussion Paper*). Notwithstanding the inclusion of the public in engagement activities, there is little evidence that citizens actually influence decision-making in health policy. Due to the centralized nature of health governance within Ontario’s Ministry of Health and Long Term Care, and the influence of large, powerful groups in government policy and resources allocation, citizens are low in the pecking order and their influence less noticeable.

For example, the Ontario Medical Association, the Ontario Hospital Association and the large academic teaching hospitals in particular have been highly effective in being able to influence government policy and resource allocation. While there is an informal ‘pecking order’ and a continuous ‘jockeying’ for influence, these large powerful groups are consistently influential; the influence of others is less consistent. In evolutionary terms, little has happened in Ontario to change this dynamic (Elson 2006: 11).

More recently, Ontario’s Bill 102 (*Transparent Drug System for Patients Act 2006*) recognized the importance of meeting patients' needs and involving them in a meaningful way into the province’s drug coverage decision-making process, which is under the purview of the Ministry of Health and Long-Term Care Act. This legislative commitment was also reiterated in the 2009 *Ontario Drug Benefit Act*, which states that the public drug system should aim to meet the needs of Ontarians, as patients, consumers and taxpayers by involving them in a meaningful way. Although the legislation does not speak to the mechanics of patient involvement processes, it led to changes in the composition of Ontario’s drug expert committee (i.e., Committee to Evaluate Drugs (CED)), with the addition of two patient members. In contrast to Quebec's Institut national d'excellence en santé et en services sociaux (INESSS) who appointed citizen members to its scientific committees, Ontario appointed patients on its decision-making committee. The Executive Director of the pan-Canadian Oncology Drug Review (pCODR) felt that this level of involvement was necessary. “Patients should be engaged in the process and allowed to sit at the table where drugs are being reviewed” (Participant #26). In addition to the structured inclusion of patients in decision-making processes, Ontario's Public Drug Program established in April 2010 a submission process for patient’s feedback (i.e., Patient Evidence Submissions). Another distinction among the provincial programs is that B.C.’s Pharmacare Your Voice Program recognizes that patients and patient groups are different publics, and subsequently permits patients who are not part of organized groups to make individual

submissions¹⁵, while Ontario only allows patients or caregivers to submit feedback on their reviews through an advocacy group registered on their website. The Ministry maintains a public listing of registered advocacy groups. Although the Ministry states that submission of patient evidence is not limited to the advocacy groups listed on their website and that the Ministry does not endorse any specific group, it does encourage advocacy groups who are not on the list to register by completing the Advocacy Registration Form¹⁶. Once the Ministry receives patient evidence submissions, information is collated, reviewed and presented by patient members of the drug expert committee. During the committee's discussions, patient members present societal values and patient perspectives. Final funding decisions take into consideration committee deliberations, which are based on clinical evidence, economic data, and public interest.

This discussion demonstrates the formation of a patient voice in health policy and drug coverage decision-making. The unique repertoire of patient experiences and views are now funneled through institutional processes and formats such as, boards, committees, working groups, surveys and submissions. Moreover, patients now have a seat at the table on Ontario's drug expert committee due to a legislative change that mandated the role of patients in decision-making processes. The findings also highlight some tensions within the health governance system and the relative influence of different stakeholders in evidence-based policy-making, particularly in resource allocation.

¹⁵B.C. defines a patient group is defined as: A not-for-profit entity whose primary purpose is to advocate for the patients it represents. Organizations may have other purposes, but patient advocacy must be a primary goal. For example, an organization of healthcare professionals that works with a particular patient group but does not actively advocate for them on an ongoing basis is not considered as a patient group”,

<http://www2.gov.bc.ca/gov/content/health/health-drug-coverage/pharmacare-for-bc-residents/what-we-cover/drug-coverage/drug-review-process-results/your-voice/caregiver-and-patient-group-eligibility-requirements>

¹⁶ http://www.health.gov.on.ca/en/pro/programs/drugs/patient_evidence/registered_advocacy_groups.aspx, accessed on January 31, 2017

4.2.2 Federal Ministry of Health

Abelson et al. (2002) argue that citizens gained a say in health policy in the 1990s at a time when governments saw the restructuring of hospitals and health services as an opportunity for communities to share rationing of scarce resource allocations with the public, and more specifically with patients affected by the reforms. It can also be argued that the creation of Health Canada's Office of Consumer and Public Involvement (OCAPI) marked a turning point in health policy-making in 2000. McGregor (2003) describes the inception of OCAPI as a cultural shift in the Department of Health. Following this bureaucratic novelty, several opportunities for citizen engagement in public policy-making were given to inform policy agendas¹⁷. Starting in 2000, public consultations were held on several topics including mental health, direct-to-consumer advertising, complementary and alternative health, obesity prevention, children's environmental health, silicone gel-filled breast implants, Cox-2 non-steroidal anti-inflammatory drugs, food and consumer safety, and more generally on topics related to the transparency of the food and health product review and regulatory process, among others. In this context, the establishment of OCAPI was seen as a genuine response by government to the "public call for a citizen-focused culture for the federal government's role in health" (McGregor 2003: 168).

OCAPI was established to encourage public involvement in Health Canada's Health Products and Food Branch (HPFB)'s priority setting, programs and policy decisions and to promote an infrastructure for in-house public engagement expertise (McGregor 2003; Jones and Einsiedel 2011). The Office engaged in several public involvement activities in the 2000s. These ranged from workshops, dialogues, advisory committees, and focus groups, mail outs for feedback, information sessions, public meetings, surveys, technical consultations, web posting

¹⁷ Canadian Policy Research Networks (2008), Handbook on Citizen Engagement: Beyond Consultation , http://www.cprn.org/documents/49583_EN.pdf.

and roundtable discussions. At the time, Health Canada had two Citizen Engagement Champions to support the Department's commitment to public engagement initiatives. Although its initial work was small-scale, OCAPI developed as a large-scale program and gained horizontal responsibility throughout HPFB by developing materials to support consultation initiatives (Jones and Einsiedel 2011). In 2000, OCAPI published the *Policy Toolkit for Public Involvement in Decision-making*, which provides a roadmap to public involvement, including principles, guidelines and information for the effective involvement of citizens in government decision-making on health issues. The toolkit attributes the rise of public engagement to four trends: the globalisation of health and new standards of public involvement set by the World Trade Organization (WTO); the transition to a knowledge-based society (i.e., evidence-based policy that includes citizens); a new social environment marked by a more demanding citizenship; and, a new fiscal environment of constrained budgets.

Government decision makers – both elected officials and public servants – are expected more than ever to discharge, and seen to discharge, their responsibility to effectively engage citizens, to listen, and to be accountable to citizens in explaining how citizens' views have been considered in the decision-making process. The input of citizens as individuals is increasingly being sought as governments recognize that the current decisions being made on major social policy issues, particularly healthcare, are not purely technical in nature, and therefore in the realm of experts. Current issues touch our values and could benefit from citizens' views and priorities¹⁸.

The premise underscoring Health Canada's public engagement discourse is that all citizens have an equal opportunity to participate in public policy; citizens do not have to draw on technical or expert knowledge to be engaged; and, public involvement will narrow the divide between the mistrusting public and decision makers. As already remarked, some stakeholders in the health governance system have high levels of influence on policy-making (Elson 2006);

¹⁸ http://www.hc-sc.gc.ca/ahc-asc/alt_formats/pacrb-dgapcr/pdf/public-consult/2000decision-eng.pdf

some groups are insiders while others are outsiders (Baggott et al. 2004). Abelson et al. (2007) argue that public involvement privileges the interest of professionals and industry and less on the interests of citizens and patients. Hence, the public engagement discourse highlighted above supports the argument that engagement can falsely promote a sense of citizen empowerment (Aronson 1993; Mayo and Anastacio 1999 in Callaghan and Wistow 2006). To reiterate though, there is no obvious correlation between the inclusion of citizens and patients in the prioritization of health matters (whether structured or non-structured), and their influence in decision-making processes. Although citizens and patients are invited to contribute to health policy-making, in the consultations listed above, there is no public record of their feedback and how it was considered in the matters at hand. Indeed, when analysing more closely OCAPI's engagement format, we conclude that it is far from what Arnstein (1969) conceived as a means for citizens to overturn decision-making. In fact, the goals of public involvement appear conflated.

Second, Health Canada's statement on public involvement also reinforces the misconception regarding lay knowledge. As we have seen, lay actors are capable of articulating different forms of knowledge, including technical knowledge. By downplaying the technical nature of health policy-making, governments further draw the line between experts and non-experts. That is, lay individuals do not have the specialized knowledge to contribute to expert policy-making. Still their views and priorities are welcomed as a means to complement the knowledge of policy-makers in terms of providing a wider social perspective. In this regard, there is an inherent contradiction between the goals of evidence-based policy-making in terms of scientificity, rationality and academic study (Marston and Watts 2003), and the potential non-technical contribution of citizens and patients. In this public involvement framework, the role of patient participation is less obvious. Indeed, if policy knowledge rests on the epistemic

achievement of objectivity (Jasanoff 2011), then there is little room for other forms of knowledge to co-exist.

Third, Health Canada's position on public involvement can be seen as an attempt to reassure the public of the government's commitment to citizen engagement, which is consistent with the inherited political climate of the late 1990s: a climate of partnerships among governments and civil society. For instance, on February 4, 1999, the Prime Minister and Premiers (except Quebec) signed the Social Union Framework Agreement, which made a commitment to "ensure effective mechanisms for Canadians to participate in developing social priorities and reviewing outcomes" (Canadian Centre for Management Development 1999: 5). During the October 12, 1999 Speech from the Throne, the government highlighted its commitment to "enter into a national accord with the voluntary sector" by establishing principles to guide the relationship between the voluntary sector and the federal government. In addition, the 1999 Federal Budget announcement of \$12.9 billion in total investments toward healthcare highlighted the importance of public involvement and federal, provincial, territorial (F/P/T) and Aboriginal collaboration as new initiatives were implemented. Building on the federal commitments in the 1999 Budget, the F/P/T Health Ministers and Deputy Ministers identified the issues of health information and accountability as priorities for collaborative work. The Government of Canada also made a policy commitment to ensure that the concerns and interests of the public were taken into account in the formulation and implementation of government policies and programs. The "Consultation" section of all Memorandums to Cabinet now highlights how the public was consulted and how its views were considered. Here, the emerging policy discourse on public involvement remains concealed and reveals a tension between the

rights of end-users of healthcare services versus the rights of the public to an accountable, transparent and open decision-making (Li 2013).

Despite the lack of clear public involvement goals, between 2001 and 2003, the number of annual public involvement activities (reported by OCAPI) increased from 10 to 79 (Health Products and Food Branch Public Involvement Framework 2005). While paper-based methods increased within this period, from 40% to 62%, interestingly, in-person methods decreased from 60% to 38%. This transformation in public engagement methods may be explained by new digital engagement trends. According to the federal government,

digital engagement has changed the nature and scope of how the government consults with the public. The use of social media channels and online consultation tools has provided new ways for Canadians to participate in and learn about health issues and to interact with the Government of Canada”. (Health Canada and the Public Health Agency of Canada 2016: 4).

In this new digital environment of engagement, one can easily conclude that citizens have less influence since they have no ability to shape engagement methods and no direct interaction with policy-makers. This trend in public engagement methods raises questions about the meaningfulness of public involvement and what counts as knowledge. For example, a recent court case in July 2017 sided with Inuit residents of Baffin Island who were not consulted adequately by the National Energy Board on the testing of potential off-shore oil and gas programs¹⁹. The court referred to the Government’s ‘duty to consult’ thoroughly. Hence, this discussion leads to an interrogation on the choice of consultation methods and what is considered meaningful engagement.

From the outset, there seems to have been a sincere attempt by OCAPI to move towards a more inclusive and interactive participatory model for the Branch’s regulated products, such as

¹⁹ www.rcinet.ca/en/2017/07/26/supreme-court-landmark-decisions-on-development-indigenous-rights/

pharmaceuticals and medical devices. In this regard, the creation of OCAPI was labeled as “revolutionary in the Canadian federal regulatory context” because it established “a mechanism to integrate certain forms of citizen knowledge into regulatory decision-making” (Graham and Jones 2010 and Jones and Graham 2009 in Jones and Einsiedel 2011: 660). OCAPI developed an infrastructure and best practices with its 2007 *Policy on Public Input* that set out a predictable process for HPFB to identify situations where its decision-making would benefit from public input. One of the tenants of the position paper on public input is making decisions in the public interest.

To inform its decision-making, the Health Products and Food Branch may seek public input at any time when it is reviewing a regulated product or class of products. In this policy, the term “public input” means input from external individuals and organizations other than the sponsors and manufacturers that are researching, producing, or marketing the regulated product (Health Products and Food Branch 2007: 5).

More recently in June 2016, Health Canada and the Public Health Agency of Canada published *Guidelines on Engagement*, which introduce a definition and continuum of public engagement. The Guidelines are much more detailed and comprehensive than the 2007 Policy and propose a definition of public engagement that involves an interaction among government and the public. Public engagement is defined as:

Planned two-way discussions with individuals, organizations, or groups, external to the Government of Canada, designed to gather input, clarify information and foster understanding among those interested and affected by an issue, decision or action and to better inform Health Canada and the Public Health Agency of Canada’s decision-making (Health Canada and Public Health Agency of Canada 2016: 4).

The health agencies provide the following meaning to the various activities of public engagement:

A range of public engagement levels in issues of public interest to inform decision-making. The Guidelines include four levels which increase in the amount of engagement that the public has in decision-making, with inform at the lowest level, then increasing from listen, discuss and then to dialogue, at the highest level of engagement. The Continuum helps to identify the level of engagement needed to meet the objectives. The levels are not intended to be sequential (Health Canada and Public Health Agency of Canada 2016: 29).

Despite government's effort to forge a relationship with citizens, the guidelines make it clear that that the concept of "empowerment" is not within the scope of public engagement and decision-making rests with policy-makers, making the relationship asymmetrical.

A level of engagement that is referred to as "empower" or "partner" is not reflected in this Continuum. The goal of empowerment and partnerships is to share decision-making. The Guidelines focus on public engagement where, although there is the opportunity for involvement and influence in decision-making, the final decision rests with HC or PHAC (Health Canada and Public Health Agency of Canada 2016: 5).

As per earlier discussions, the notion of empowerment emerged in the 1950s in the context of patient advocacy, which stressed human dignity, rights to justice and self-help for vulnerable populations. In the 1990s, empowerment was connected to patients' active engagement in medical decision-making and their ability to influence treatment outcomes. Hence, the federal government's position is that empowerment is not an aspect of public engagement in health policy. Despite the emphasis on the rights of citizens to be involved and to shape health policy, the underlying message is that the public does not have a degree of influence over decision-making processes. This premise supports the statement made by Aronson (1993) that there is little evidence to show whether these citizen engagement undertakings truly empower citizens. In fact, to use Aronson's words, people have little say and control over government policies. By making it clear that public involvement is distinct from the notion of

empowerment, it is conceivable that the Ministry of Health acted to protect its authority in health matters by monopolizing policy decision-making.

In short, the millennium was inscribed by public involvement commitments from various levels of the governance system, the rise of digital engagement methods and the building of an expert niche in public involvement practices through the work of OCAPI. Here, engagement becomes a democratic principle added to the health policy toolbox. Yet Budget 2012 announced the closure of OCAPI. Unfortunately, there are only a handful of records pertaining to OCAPI; information on its accomplishments is very scarce making OCAPI's legacy unknown. Even though Canada gained experience with public involvement, since OCAPI's closure, there is no model of public engagement or gold standard across government departments and agencies (Abelson 2007). It appears that very little debate took place about public involvement, its purpose and the process by which it was integrated in health policy. Moreover, there is no clear distinction between different publics in engagement activities. Citizens and patients form one group of constituents defined as the public. In this regard, the dissertation argues that the use of the term "citizen" to designate the public involvement audience served to blur the lines between social values and patients' values, thus creating competing demands between the public's best interests and patients' best interests.

Seen in this light, government officials facilitate public involvement under the pretense of democracy, at the same time as they reframe evidence-based policy-making into an exercise that even the layperson with very basic knowledge of science and policy is able to comprehend. Taken at face value, citizens are encouraged to work within the boundaries of health policy. But as the literature has demonstrated, only well-organized, knowledgeable and influential groups have succeeded in entering spaces of expert knowledge.

4.2.3 The Government of Canada

As previously established in the public policy scholarship, citizen engagement is conceived as a route to democracy; a quest for social justice and citizens' right to be engaged in government policy decision-making. The Canadian landscape is rich with examples of citizen engagement. Some examples that were discussed include consultations held by Ontario's Health Services Restructuring Commission, District Health Councils, and Ministry of Health and Long-Term Care, and Health Canada. In 2002, Parliament launched its first-ever online consultation on the Canada Pension Plan Disability (CPP-D) after constituents flagged their concerns to their parliamentarians. The Sub-Committee on the Status of Persons with Disabilities chaired by Carolyn Bennett who was a strong public engagement advocate, paved the way for e-democracy methods by using a website for information-sharing with the public, an online consultation for soliciting comments from 1,700 Canadians, and holding in tandem, subcommittee hearings with an array of witnesses. Participants were invited to debate the committee's findings and final recommendations. The views of the public were also sought on the work of Parliament's Commission on the Future of Healthcare in Canada and the Standing Senate Committee on Social Affairs, Science and Technology.

The Prime Minister under the leadership of Roy J. Romanow established the *Commission on the Future of Healthcare in Canada* (also referred to as the Romanow Commission or Romanow Report) in April 2001. The Commission was mandated to review medicare, engage Canadians in a national dialogue on its future, and make recommendations to enhance the system's quality and sustainability. The Commissioner's work led to the publication of a 2002 Report in which public input was put forward as a proposed health covenant for Canadians. The underlying message promoted the idea that public participation is important to ensure a viable,

responsive and effective healthcare system. Later in 2007, the Standing Committee on Health tabled the Report, *Prescription Drugs - Part I The Common Drug Review: An F/P/T Process* (also referred to as the Common Drug Review (CDR) Report) in the House of Commons. Joy Smith, who was a Member of Parliament member under the Conservative government, chaired the Committee. The Report presented recommendations to improve the Common Drug Review process. The Health Committee's CDR Report called on the federal government to work with provincial and territorial (P/T) governments to improve the openness and transparency of the Common Drug Review as a means to foster greater public trust in the process. This time, the Committee recommended that the federal government work with its P/T counterparts to increase the current level of public involvement in the Common Drug Review through public attendance at open CDEC meetings and the creation of a multi-stakeholder public advisory body. As per the Report, this advisory body would include healthcare providers and patients and would be tasked with recommending treatment access for life-threatening or serious rare disorders, based on scientific standards and social values. Furthermore, the Report recommended that the Common Drug Review develop a separate process for the review of rare disease drugs and for innovative or first-in-class drugs. In other words, Parliament set the broad expectation for policy-makers to include public involvement in healthcare matters and more specifically, to include as an integral part of the Health Technology Assessment (HTA) system more public involvement opportunities. Through Parliament (i.e., the House of Commons and Senate), the Government of Canada promoted public involvement through political processes such as agenda-setting, establishing the Romanow Commission and putting forward recommendations for government officials in the CDR Report. Even so, there is no further discussion within the Romanow Report and the CDR Report on the nature and form that public involvement should take, nor do they

discuss output in terms of outcomes of public involvement efforts. Here, the democratic political system in Canada was favorable to public engagement initiatives by setting public engagement as a key thematic element of healthcare (Aronson 1993; Contandriopoulos 2004; Tritter and McCallum 2006 and Martin 2008).

Interestingly, Canada's history of involvement shows a gradual uptake of involvement initiatives by health governments starting in the early 1970s. Taken together, these initiatives form an informal niche of expertise on involvement practices, and reveal a relation between government decision-making processes and citizens' health needs. Since early 2000s, scholars argued for the heightened interest of the public in decision-making and scrutiny over how decisions are made and by whom. Involvement of the public in health matters became the expression of citizenship and was deemed fundamental to democracy. To this end, government institutions attempted to gain public trust by gauging citizens' views and preferences on proposed policy approaches in various health domains. Usually these public engagement methods are highly technocratic in nature (Abelson et al. 2007) and formal in structure, such as advisory committees, dialogue sessions and web-based consultations, among others. The main purpose of engagement is to provide information and gather information from health constituents. The strategic and bureaucratic drivers behind engagement carry with them the promise of advancing more equitable and accountable decision-making. This further supports the notion that public engagement has democratizing claims of redistributing power between those who govern and those who are governed. Canada's response to the call for the engagement of citizens in problem solving and service improvements represents a step forward towards a shared system of healthcare governance with affected stakeholders. At the same time, to be truly open as a shared-decision model, governments would have to be prepared to formally divest power. In

the current health governance landscape, government officials appear to monopolize the authority as organizers and distributors of health services and goods.

4.2.4 International Landscape

This emphasis on the involvement of patients in drug coverage matters is not a Canadian phenomenon alone. There have been several patient input efforts in international Health Technology Assessment (HTA) settings that predate the Canadian experience. A decade earlier, European agencies started building engagement infrastructures to support the perspective of patients in HTAs. In the spirit of democracy, these initiatives provided patients with the means to exercise their right to participate in public institutions and demand recognition for their health needs. In spite of these advancements, patient input was still considered to be in its infancy in 2010 as per the European Patients' Forum (EPF)²⁰. Furthermore, patient input has not been consistently embedded into practice within and among nations. According to the EPF's survey of HTA agencies in Europe, the type and level of patient involvement varies widely, which is a reflection of the different rationales, motivations and approaches applied in each country. The survey was conducted between November 2010 and February 2011 and involved 40 out of 50 HTA agencies from as many as 23 European countries. The EPF report (2016) concludes that:

Very few HTA agencies currently involve and integrate patients' perspectives in their reports and conduct formal evaluation of the impact of patient involvement in HTA. Apart from financial resource constraints, the main challenges are perceived to be the lack of capacity, time and good methodologies to involve patients. Above all, the question of the exact stage of HTA where patient engagement is needed or is most useful is still being debated. The last phases of HTA (external review, and diffusion and dissemination) accommodate some patient involvement, but there is none or low

²⁰ European Patients' Forum , Patient involvement in Health Technology Assessment in Europe - An interim report on EPF survey with HTA Agencies, <http://www.eu-patient.eu/News/News-Archive/Patient-involvement-in-health-technology-assessment-in-Europe---An-interim-report-on-EPF-survey-with-HTA-Agencies/>, retrieved on February 23, 2016.

involvement in the first few phases of HTA (identification, filtration, prioritization).

In addition, the EPF survey respondents noted that “lack of legal or policy framework for patient involvement in HTA decision-making” was among the main challenges to the meaningful involvement of patient organizations in decision-making around health technologies (European Patients Forum 2016: 17). Because patient input is not ingrained in legal principles or policy principles in many jurisdictions, HTA practices take on many shapes. For these reasons, Health Technology Assessment international (HTAi) was formed in 2003 to guide and standardize the work of HTA organizations. HTAi is the global scientific and professional society for all those who produce, use, or encounter HTAs and has members from all stakeholder types (i.e., researchers, agencies, policy-makers, industry, academia, health service providers, and patients/consumers) from over 65 countries including Canada. HTAi is described as the leading global discussion platform for all stakeholders engaged in the efficient production and use of health technology assessments in decision-making. The vision of HTAi is that patient and citizen perspectives improve HTAs. It provides resources for HTA Agencies and policy-makers such as Frequently Asked Questions, Values and Quality Standards for Patient and Citizen Involvement, Patient Group Submission Templates, among others. Here, the first practical though variable application of patient involvement is seen in the world of HTAs.

Even with the inconsistencies in patient involvement practices, Europe is clearly a leader in patient involvement initiatives. Europeans promote a culture of openness in HTAs. The European Patients’ Academy on Therapeutic Innovation (EUPATI) developed four distinct patient involvement frameworks to guide the pharmaceutical industry, HTA bodies, regulatory bodies and ethics committees. As per EUPATI, these frameworks were developed to address an increasing need for the experience and specific knowledge of patients, and their day-to-day use

of medicines, to improve drug development and drug review. The frameworks propose a uniform way of organizing, structuring, managing and interacting with patients in order to “facilitate exchange of information and constructive dialogue at the national and European level where the views from users of medicines can and should be considered”²¹.

The HTA frameworks advance the following values²²:

Relevance	Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.
Fairness	Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.
Equity	Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with particular health issues, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.
Legitimacy	Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.
Capacity building	Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

During interviews, participants identified two platforms of patient input that were at the forefront of patient involvement initiatives, said to be exemplary applications of patient involvement values. The UK’s National Institute for Health and Clinical Excellence (NICE) and the Scottish Medicines Consortium (SMC) have received international recognition and were well established before the introduction of patient input in the Canadian setting.

²¹ <http://www.htai.org/interest-groups/patient-and-citizen-involvement/resources/the-european-patients-academy-on-therapeutic-innovation-eupati.html>, accessed on January 31, 2017.

²² Draft_HTA_framework_deliverable-7.2-external-consultation, https://webcache.googleusercontent.com/search?q=cache:nC90cyY-HP0J:https://3qksc436bu713cqimwcfglyj-wpengine.netdna-ssl.com/wp-content/uploads/2016/07/Draft_HTA_framework_deliverable-7.2_external_consultation.docx+&cd=1&hl=en&ct=clnk&gl=ca, retrieved on January 31, 2017.

NICE has the longest history and broadest scope of public engagement in HTAs (Littlejohns and Rawlings 2009 in Kreis and Schmidt 2012: 13). Since 1999, NICE has involved patients, service users, caregivers and the public, including voluntary, charitable and community organizations in its work. For instance, NICE has public representatives at the board level and on appraisal committees. In these settings, the public has full voting rights. In addition, NICE offers public input opportunities in each strand of its work on public health, health technologies, and clinical practice. In 2002, NICE established the Citizens Council to solicit the public's value judgement of HTAs (includes 30 members reflecting various socio-demographic profiles). NICE also published Social Value Judgements (SVJs), a periodically updated guidance document that sets out NICE's 2008 Value Framework. NICE expects that all appraisal committees consider SVJs in their decisions and "justify explicitly where they depart from its norms" (Kreis and Schmidt 2012: 13). SVJs incorporate principles of bioethics (i.e., moral principles, distributive justice and procedural justice) and promote equality while avoiding discrimination based on race, disability, gender, sexual orientation, age and socioeconomic status, among others. One of the core principles of all NICE guidance is patient and caregiver involvement. NICE published *The National Institute For Health And Care Excellence Patient And Public Involvement Policy*²³, which focusses on the humanity of patient experiences. The Institute created official channels for citizens and patients. The Public Involvement Programme (PIP) is a team that develops and supports patient, service user, carer and public involvement whereas Patients Involved in NICE (PIN) (comprised of 80 patient organizations) provides patient organizations who engage with NICE a system of mutual support and information sharing to enable patient groups to engage productively with the Institute. NICE has also encouraged extensive stakeholder involvement in

²³ <https://www.nice.org.uk/media/default/About/NICE-Communities/Public-involvement/Patient-and-public-involvement-policy/Patient-and-public-involvement-policy-November-2013.pdf>

HTAs. In the case of technology appraisals, stakeholder involvement begins at the scoping stage, where key stakeholders are invited to a scoping workshop. Every NICE committee has a patient representative.

Scotland's SMC also has a national policy on patient and public involvement since 2001²⁴. Their 2014-2020 *Engaging People Strategy* affirms that:

Scotland has a key role in supporting healthcare providers to make sure that their services are safe, effective and 'person-centred' so that people are informed and involved in their care and treatment, and are treated with dignity and respect.

Engaging people in our work is a powerful force for positive change and improvement. By focusing on people in all we do, we will communicate how care (and the experience of care) can be improved²⁵.

SMC values the experiences of patients, their families and caregivers with diseases and ways new medicines affect the quality of life of patients and caregivers. These values are key elements in their decision-making process. SMC works in partnership with patient groups to gather information about what it's like to live with a particular condition. Patients, members of their families and caregivers can provide unique knowledge and can explain advantages and disadvantages of medicines that may not be available in the published literature or quality of life measures. The SMC committee has three public partners, who are volunteers and work as part of the SMC Public Involvement Team. Their role is to help ensure the views of patients, caregivers and members of the public are taken into account during SMC decision-making. One of the key responsibilities of the public partners is to prepare presentations of patient group submissions, to

²⁴ [https://www.cadth.ca/sites/default/files/events/lecture-series/Karen%20Facey%20Presentation%20-%20Evolution%20of%20HTA%20in%20Scotland%20\[Read-Only\].pdf](https://www.cadth.ca/sites/default/files/events/lecture-series/Karen%20Facey%20Presentation%20-%20Evolution%20of%20HTA%20in%20Scotland%20[Read-Only].pdf)

²⁵ https://www.scottishmedicines.org.uk/Public_Involvement/20140930_Engaging_People_Strategy_2014-20.pdf, accessed on January 31, 2017.

accurately highlight key issues for patients, and present them during monthly SMC committee meetings. The agency created a Patient and Public Involvement Group (PAPIG), developed a patient submission form and provided feedback on these submissions. In 2013, the Scottish Government conducted a review on access to new medicines and consequently, the agency was asked to implement a series of recommendations to increase transparency, give patients and their representatives a greater role, and increase drug access. For the past two years, all SMC meetings have been open to the public to ensure that members of the public and other stakeholders can understand how evidence is assessed and interpreted and how recommendations are made.

Through their value-based frameworks and person-centered approach, both NICE and SMC have demonstrated national leadership in advancing a patient involvement platform grounded in principles and practiced in institutional drug-related activities. In developing the Patient Input Program, its pioneer explained that the Canadian Agency for Drugs and Technologies in Health (CADTH) considered these international models of engagement. The international landscape is therefore an enabling factor in the unfolding of patient involvement in drug coverage decision-making in Canada.

4.2.5 The Canadian Agency for Drugs and Technologies in Health (CADTH)

The authorities and responsibilities around drug accessibility and affordability are shared between four main parties. These involve Health Canada, who makes a decision about a drug's safety and efficacy; the Patented Medicines Review Board (PMPRB), who examine a drug's price in comparison to other developed markets internationally; CADTH, who compares the drug to existing treatments; and finally, the drug plans in each province and territory, who decide whether they will cover the drug for patients. In the absence of a national standard for drug reimbursement, each of the provinces independently developed their own drug insurance

program over the 1970s and 1980s (Daw and Morgan 2012). According to Grootendorst (2002), early provincial drug insurance programs were designed as subsidy programs for vulnerable populations such as social assistance recipients and elders, and not as extensions of medicare (except for Saskatchewan) (in Daw and Morgan 2012). Throughout the years, public drug programs expanded the scope of recipients eligible for drug coverage, but these changes were not uniform across provinces. This absence of uniformity and national standard prompted the creation of a body of expertise, leadership and advice, known as CADTH. As an evidence-based decision-making authority, the Agency assesses whether the drug is better than existing therapies, whether it is relevant to patients and whether the cost justifies the proposed health benefits. All these questions lead to the ultimate dilemma – will the government fund the drug or will it not. To support this decision, CADTH conducts evaluations of the clinical, economic, and patient group input on drugs under review. The Agency uses the evaluations to provide drug coverage recommendations to Canada’s publically funded drug plans through the Common Drug Review (CDR) process or pan-Canadian Oncology Drug Review (pCODR) in the case of cancer drugs.

Over the years, CADTH has become an institutional gatekeeper between its stakeholders and governments. It has created a hub of expertise in evidence-based decision-making. CADTH was first created in 1989, and within a decade, the Agency was able to expand its role and services due to a strong policy direction from the Deputy Ministers of Health, funding priorities of the Canadian Parliament, as well as its autonomy with regard to the production of economic guidelines. Quite rapidly, CADTH morphed into a centre of authority thereby setting scientific requirements for HTAs in Canada’s healthcare system and providing expert policy advice to ensure the best value-for-money in an evidence-based framework.

CADTH was originally the Canadian Coordinating Office for Health Technology Assessment (CCOHTA). Canada's federal, provincial and territorial (F/P/T) Ministers of Health conceived it in 1989 as an independent, not-for-profit national body that provided healthcare decision-makers with credible, impartial advice and evidence-based information about the effectiveness and efficiency of drugs and other health technologies. CCOHTA opened its doors on a three-year trial basis in August of 1990. In 1993, the Conference of Deputy Ministers of Health added Pharmaceutical Reviews to its mandate, and CCOHTA's budget more than tripled. Within a year, CCOHTA published Canada's first-ever set of guidelines for the economic evaluation of pharmaceuticals. These guidelines have been used throughout the country to assist producers of economic evaluations in generating credible, standardized economic information that is relevant and useful to decision makers in Canada's publicly funded healthcare system.

In 1998, a court ruling confirmed CCOHTA's independence from affected stakeholders by overruling the request of Canada's Bristol-Myers-Squibb (BMS) to block the publication of CCOHTA's negative decision report on Pravastatin (Pravachol). The report concluded that all statin drugs could be expected to reduce coronary events, and that Pravastatin therefore did not offer any additional benefits. BMS challenged the assertion that Pravastatin did not present additional clinical benefit in comparison to other statins. The Ontario Superior Court of Justice found that CCOHTA had followed proper procedures and conducted a responsible evaluation, and affirmed CCOHTA's autonomy by allowing the report's publication.

The following year, CCOHTA's role was solidified when the Conference of Deputy Ministers of Health approved its first five-year business plan. Later, the Conference of Deputy Ministers in 2002 mandated the Agency to manage the Common Drug Review (CDR) process for reviewing new drugs and providing formulary listing recommendations to participating

publicly-funded F/P/T drug benefit plans in Canada. The goal of this acquired mandate was to standardize listing decisions across provinces and territories. Further to CCOHTA's expanding mandate, in 2003, the Agency's funding envelope increased significantly (\$45 million over five years) following the 2002 *Romanow Commission on the Future of Healthcare in Canada* and the 2002 *Kirby Senate Study on the State of the Healthcare System in Canada*. The increased funding was to address the growing need for reliable, evidence-based information. This funding led to new initiatives and ultimately a heightened role for CCOHTA in the healthcare system. In 2003, CCOHTA launched the Liaison Officer Program, which places Liaison Officers within the provinces and territories to actively engage with jurisdictional healthcare decision makers, and create strong links between the Agency and its stakeholders. CCOHTA continued to expand in 2004, with the creation of the Canadian Optimal Medication Prescribing and Utilization Service (COMPUS). This service, in partnership with the F/P/T health ministries, marked the beginning of CCOHTA's involvement in the identification and promotion of optimal drug prescribing and use among healthcare providers and consumers, and in the creation of optimal use products. An Optimal Use project consists of a systematic review of the clinical evidence; a cost effectiveness analysis; a review of the legal, social, and ethical issues; and, the development of recommendations, guidance, and tools. Within this period, CCOHTA gained political support.

For instance, the F/P/T Ministers of Health approved a new Canadian Health Technology Strategy that arose from the 2003 Accord on Healthcare Renewal. The strategy confirmed CCOHTA's role as Canada's health technology agency and represented a collaborative approach toward ensuring that Canadians have ongoing access to appropriate healthcare technology. As well, to ensure that Canadian healthcare decision makers had access to accessible health technology information based on the best available evidence in a quick and efficient manner, the

Agency established in 2005 the Health Technology Inquiry Service now known as the Rapid Response Service. The Rapid Response Service responds to inquiries about drugs, devices, and procedures from individuals employed with the Canadian health ministry, health authority, hospital, or federally or regionally administered healthcare programs. Depending on the urgency and/or complexity of the request, information is provided within anywhere from 24 hours to 30 business days. More broadly, the Agency conducts environmental scans of healthcare practices, processes, and protocols inside and outside of Canada and conducts horizon scans to identify new and emerging health technologies likely to have a significant impact on healthcare in Canada.

In 2006, CCOHTA was rebranded as CADTH and in 2009, the Agency was reorganized with the integration of three core programs: the Common Drug Review, Health Technology Assessment, and the Canadian Optimal Medication Prescribing and Utilization Service. More recently, the pan-Canadian Oncology Drug Review (pCODR) process was transferred to CADTH to align pCODR and CADTH's CDR processes while building on the best practices of both. pCODR has been an integral part of CADTH since April 2014. One interview participant viewed the merger of pCODR and CADTH as a power grab by CADTH since the Agency "was in a lot of trouble with the federal government for overspending and not managing their budget very well. The merger was a way for CADTH to grab more power and more money" (Participant # 8). Of note, this view was not explored and therefore is not substantiated by other sources.

Despite the recent controversy with respect to CADTH's spending, the chronology of CADTH's history depicts how the Agency expanded its mandate, leveraged its expertise and gained social and political power. A series of tactics enabled CADTH to become the gatekeeper in the allocation of public funds. For example, the Agency implemented and standardized

economic norms in the allocation of resources. It acquired autonomy from other societal agents involved in drug coverage matters and succeeded to officiate its role as the only legitimate institution of evidence-based policy. In addition, it secured funding for its programs, entered new spaces of practice and made efforts to influence physician's prescribing practices. The Agency developed expertise on science, legal, social and ethical issues in health technology assessments, and maintained its status as an evidence-based policy institution by being the point of contact for credible and up-to-date information on health technologies.

Surprisingly, it is within this evidence-based policy framework that patient involvement was brought into practice in a structured manner. This occurrence merits more attention from a sociological perspective. From CADTH's viewpoint, patients themselves put pressure on CADTH to adopt a patient input platform. Many agencies responded to this call for a patient-based Health Technology Assessment (HTA) system; however, the structured involvement of patients in HTAs has not been widely adopted by HTAs (Bridges and Jones 2007). On the reasons for the uptake within CADTH, the Executive Director of the Agency explained that Dr. Bob Peterson was instrumental in pushing forward patient input as a strategic program priority. Under his leadership as Chair of the Canadian Drug Expert Committee (CDEC), the committee reacted to the outside noise created by patients.

Patients were not being heard and patients were going to politicians knocking on their door telling them that they got a negative decision on this drug. If there was patient input, they thought the decision may be more favorable, even if the decision wasn't favorable, they would understand the decision better. Bob Peterson and committee members were hearing this discussion from the outside world that patients were not being heard. So a part of this (the Patient Input Program) came from Bob Peterson and the committee that he was leading. They thought it would be a good idea to incorporate patient input. (Participant #27)

According to the founder of CADTH's Patient Input Program, the initial question in the program's development was how best to implement a patient input platform with the information available at the time and based on international and national experiences (Participant #24). The program's pioneer explained that the intention behind the creation of the Patient Input Program was to build experience and to continuously improve the program where possible. Program evaluations were therefore made public, feedback was sought on suggested changes, and this feedback led to program enhancements. In other words, when it was introduced, patient involvement was supported by CADTH's executive management and working-level staff. There was recognition based on CADTH's connection to the international HTA community that other HTA bodies were moving in this direction and that CADTH should follow suit.

Despite this movement towards anchoring patient involvement into HTA practices, the implementation was not immediate and resulted in much policy thinking. One key informant commented on her role in planning and implementing the Patient Input Program at the time when the Common Drug Review was being set up through discussions with the participating drug plans. Due to the lack of knowledge and know-how and many other tasks in establishing the process, public involvement was placed on the back burner. These discussions continued though to evolve within the policy circle. In 2003, the Common Drug Review Steering Committee discussed ways in which the public could provide meaningful input to drug coverage decision-making. In 2004, a discussion paper presented options for the Committee, and in 2005, a process evaluation by EKOS led to the recommendation that the Common Drug Review explore how best to incorporate public input in the Common Drug Review process. The drug expert committee (i.e., the Canadian Drug Expert Committee (CDEC)) also recognized during this time that the public should be involved. CADTH was prompted to commission a report from an

outside consultant on the different options for seeking public input in the Common Drug Review process. The board of directors also requested that CADTH propose an approach for public involvement. In 2006, a business case was developed recommending a staged approach to public input, beginning with the appointment of two public members to CADTH's drug expert committee (which was endorsed by CADTH's Board of Directors). The appointment of the first public members to the committee was in October 2006. A working group was also created in 2009 to develop a Patient Input Program for the Common Drug Review comprised of drug expert committee members and two public members, drug plan managers and CADTH staff. Through a literature search and by contacting international groups and HTA bodies (as well as Ontario and B.C. who were establishing their own processes at this time), the working group collected views and experiences, which led to the mapping of a Patient Input Program. Public consultation on this mapping process resulted in the launch of the Patient Input Program (2010) which seeks written submissions from patient groups on drugs eligible for funding.

Initially, there was no distinction between the public, citizens and patients. As part of the policy thinking around the form and function of patient input, some of the Agency's early patient input discussions revolved around the definitions of public and patient and the need for relevant public and/or patient representation on the drug expert committee (Participant #24). CADTH's focus began with the public but evolved. Although the term "public involvement" was used in Canadian public policy circles to refer to the engagement of citizens in government health matters, at the program level, and from CADTH's viewpoint,

There was recognition that public input and patient input were different. In the case of the Common Drug Review, public input is provided by the public members on the drug expert committee. Patient input is provided by patient groups or in the situation where there is no patient group by individual patients. It is recognized that patients who have a disease/condition for which a drug under funding review is indicated are

most knowledgeable about what it's like to live with that disease and any issues associated with its treatment/management. Their contribution is different from the public member on the committee. (Participant #24)

Thus, CADTH created a patient involvement audience that differed from the public.

To summarize, the findings suggest that key individuals were involved in the inception, elaboration and adoption of a patient-focussed Program in drug coverage decision-making. It appears as though the Program's implementation was achieved by the collaboration of bureaucrats and technocrats, with little consideration to the views of patients, despite the outside noise from patient groups for a patient input process.

Regarding the push from patients for their inclusion in drug coverage matters, I discuss their role as part of a broader patient activism movement initiated by cancer and AIDS patients in the 1980s. As I have noted earlier, new forms of genetic or biological citizenship enabled individuals to become political actors and voice a collective experience of human suffering. In the next section, I explore the rise of evidence-based activism and argue that both the government and patient groups contributed to a new notion of patienthood (as opposed to personhood) in the healthcare system. This notion builds on Armstrong's (2014) conceptualization of patienthood, which refers to the health responsiveness of patients and their agency in health-related decisions.

4.2.6 Evidence-Based Activism

As explained in the previous section, the government had a role in the transformation of citizens as subjects of the state to individuals who take increasing responsibility for health-related decisions and exercise agency. The findings show how governments came to view patients as political entities whose membership bestows rights and participation in healthcare matters.

Equally important is the role of patients themselves in redefining knowledge boundaries and reconfiguring boundaries of health governance. Patient activism has been a driving force towards the inclusion of patients in biomedical decision-making and health policy. Through lobbying efforts, some patient groups were able to put pressure on influential actors and institutions to become important political actors. Not only do patient groups understand the political system, they also succeed in prompting institutional transformations by blurring the lines of expert and non-expert knowledge. Akrich and Rabearisoa (2012) refer to patients' politicized, hybrid knowledge form as evidence-based activism. As noted earlier, evidence-based activism refers to the agency of patient organizations in their ability to change the politics of disease knowledge and use it to make state demands and sustain their claims-making. By doing so, patient organizations work from within the boundaries of science and politics to assert their needs, as demonstrated by AIDS and cancer activists.

For their part, AIDS activists formed a grassroots organization in the U.S. commonly known as ACT UP (acronym for the AIDS Coalition to Unleash Power) in 1987. As demonstrated by their 25-year chronology²⁶, this international advocacy group demanded a policy platform to end the AIDS crisis. This included the release of experimental AIDS drugs; accessibility to affordable AIDS drugs; a change in the disease etiology to include HIV-positive women; the creation of a federal needle-exchange program; and, implementation of a sex education program in primary and secondary schools. Some have framed AIDS as a civil rights issue²⁷. In 1992, a sub-group of members divided and created the Treatment Action Group (TAG) whose goal was to develop a political strategy on the treatment of individuals with

²⁶ <http://www.actupny.com/ENDAIDS/index.php/the-community>

²⁷ <https://www.aidshealth.org/civil-rights>

HIV/AIDS and called for improved clinical trial designs and a more coordinated AIDS research effort at the National Institutes of Health Research (NIH) through an office of AIDS research.

In Canada, a similar group formed in 1988. AIDS Action Now (AAN) highlighted the emergence of a new “poz” identity bringing together all people living with AIDS, no matter what their sexual orientation. Unlike the U.S. group, AAN established a division of labour between AIDS service organizations, which left AAN free to challenge government policies and practices while remaining at arm’s length due to their no state or corporate funding policy. AAN focussed on access to unapproved treatments, access to HIV information and access to drug coverage. It also forced the NDP government to create the Trillium Drug Plan in 1995²⁸.

Cancer activists on the other hand, also demanded a policy platform for cancer survivors and recognition for the cancer experience. In 1986, the National Coalition for Cancer Survivorship (NCCS) was formed when 23 leaders in the cancer community met in Albuquerque, New Mexico. NCCS’s initial efforts were directed at legislative and policy issues for cancer survivors. The importance of the patient perspective was recognized on a national scale when the NCCS became one of the first two consumer representatives to be invited to join the National Cancer Policy Board (now renamed the National Cancer Policy Forum). NCCS was also responsible for the shift in terminology from “cancer victim” to “cancer survivor”. “The founders envisioned an organization that would deal with the full spectrum of survivorship issues related to living with, through and beyond a cancer diagnosis”²⁹ and developed a hub of expertise on employment and disability law, healthcare consumerism, as well as psychosocial and behavioral research. NCCS contributed to the literature and evidence base for quality cancer care

²⁸ <http://www.aidsactionnow.org/>

²⁹ <https://www.canceradvocacy.org/about-us/our-history/>

by publishing articles on doctor/patient communications, survivors' rights and becoming informed and knowledgeable healthcare consumers. In 1993, NCCS created the Cancer Leadership Council (CLC) as a patient-centered forum of national advocacy organizations addressing public policy issues in cancer. NCCS developed its *Imperatives for Quality Cancer Care: Access, Advocacy, Action and Accountability* in 1995. This was the first report to approach quality cancer care from the patient/survivor perspective and provided the impetus that led to the creation of the National Cancer Institute's Office of Cancer Survivorship in 1996. The NCCS led a nationwide grassroots campaign to make the cause, the care and the cure of cancer the nation's top health priority.

Breast cancer activists also paved the road for patient advocacy worldwide with their legislative agenda. Since 1991, the National Breast Cancer Coalition (NBCC) has been a leader in Breast Cancer in the U.S. In response to NBCC's 2.6 million signature campaign in 1993, President Clinton met NBCC's demands and committed to a *National Action Plan on Breast Cancer*, an innovative collaboration of government, science, private industry and consumers. It established an annual Advocacy Training Conference to train women and men from across the country to become informed, active and effective advocates. In 1997, Brussels' *World Conference on Breast Cancer Advocacy-Influencing Change* was the first international conference on breast cancer advocacy (led by NBCC). People attended it from 44 countries and 6 continents. This meeting is thought to have helped shift the balance towards consumer participation, which was becoming a reality at that time.

Within the Canadian context, Breast Cancer Action Montreal was formed in 1991 to raise awareness of breast cancer issues; to advocate for better diagnosis, treatment and care; and to give women a voice in decisions about treatment, services, health policy and research. Again by

building awareness; facilitating a dialogue with government officials³⁰, researchers and scientists; attracting media coverage; providing advocacy training to its members; and network building within the cancer community, BCAM was able to drive a public policy platform for breast cancer. The organization promoted the enhancement of ethics standards in breast cancer research while remaining at arms' length from the pharmaceutical industry. BCAM implemented a policy that prevents the coalition from accepting funding from industry. In this regard, breast cancer patients paved the way for patient's role in the management of healthcare. One research participant, a veteran in medical oncology, recalled a patient-driven conference he attended in Montreal with breast cancer advocates. He was astonished by the fact that patients demanded an active role in all aspects of healthcare decisions, trial design and grant approval.

I was one of the few men and physicians there. I was quite taken aback by the conversations. They wanted a role in decision-making, in the trials and at the table approving grants. There were lots of demands and it was a shocking experience at the time. You are going back 25 years. (Participant #30)

Not only did patient groups build clout in science domains but also in health policy domains, including research funding, health services financing and health services delivery.

In addition to AIDS and cancer groups, several rare disease organizations also gained visibility in domains of science and health policy. For instance, the Cystic Fibrosis Foundation (CFF) was established in 1955 in the U.S. at a time when people born with the disease weren't expected to live to attend elementary school. Today, people with CF are living into their 30s, 40s and beyond (compared to 10 years of age in 1962) because CFF contributed to the search for a cure by funding research efforts and encouraging pharmaceutical companies to invest in rare-

³⁰ BCAM's co-founders testified at parliamentary hearings and helped initiate a cross-Canada letter campaign to raise awareness of the federal report, *Breast Cancer: Unanswered Questions*

disease research³¹. As a result, a group of CFF funded scientists discovered the defective CF gene and its protein product before the human genome was mapped. In 2000, the foundation established the Cystic Fibrosis Foundation Therapeutics (CFFT), a non-profit research affiliate of the Foundation, to govern drug discovery and development domestically and internationally and led to the approval of several therapies, such as Pulmozyme, TOBI, Kalydeco and Orkambi.

Another example of influential groups is the National Tay-Sachs and Allied Diseases Association (NTSAD)³², which was formed in 1957 in the U.S. and focussed on funding research, supporting over 500 families and individuals worldwide, and raising awareness to prevent disease. Their services include family support, funding and promoting research to developing treatments and cures by working with its Scientific Advisory Committee. NTSAD also identifies promising therapeutic approaches for funding through the Research Initiative grant program, offers educational and awareness programs and engages in advocacy activities on an individual, state and national level, on issues such as health insurance coverage and government (supplemental) funding. NTSAD was an early pioneer in the development of community education about Tay-Sachs disease, carrier screening programs and laboratory quality control programs, thereby ensuring that those being tested receive accurate and reliable information and test results. It created the Tay-Sachs Gene Therapy Consortium, which in 2009 received \$3.5 million in funding from NIH. Today, more than two million people have been tested for the Tay-Sachs gene, thousands of Tay-Sachs carriers have been identified and hundreds of healthy children have been born to high-risk couples. For nearly six decades, the organization has grown in size, scope and stature. As per the Association, NTSAD is committed to a leadership role in the application of its knowledge to the betterment of the lives of children,

³¹ <https://www.cff.org/About-Us/About-the-Cystic-Fibrosis-Foundation/Our-History/>

³² <https://www.ntsad.org/>

adults and families (e.g., in 2011, NTSAD hosted the conference on *Mechanisms and Interventions in Childhood Neurodegenerative Diseases*). In 2008, September was declared National Tay-Sachs Awareness Month.

For its part, the Hereditary Disease Foundation³³ was instrumental in advancing the Human Genome Project. It was created in 1968 by Dr. Milton Wexler to find treatments and cures for Huntington's disease and other brain disorders. To date, the Foundation has awarded more than \$60 million dollars to support cutting-edge scientific research by funding innovative research projects and organizing innovative workshops, which encourage scientists to collaborate and share their research findings and ideas. The Foundation focuses on curing Huntington's disease, not only because of its devastating consequences to individuals and families with the disease, but because it is a model for other brain disorders. According to the Foundation, HD is caused by a mistake in one gene. That mistake is the same in all families worldwide with HD, so curing and treating HD could help everyone worldwide with the disease. Also, as a single-gene disorder, HD is viewed as an ideal model for studying other brain disorders. For over three decades, HD has served as a model for the study of human diseases using genetic approaches. Supported by the Hereditary Disease Foundation, the Huntington's disease gene was the first genetic alteration (not found on a sex chromosome) to be mapped using only DNA markers. This feat, accomplished in 1983, helped launch similar studies in many other diseases and played an important part in the development of the Human Genome Project. The search for the HD genetic alteration itself, also led by the Hereditary Disease Foundation, pioneered many technologies for mapping and finding genes, and culminated in the identification of the HD gene, its defect, and the protein it encodes in 1993.

³³ <http://hdfoundation.org/>

These exemplary cases demonstrate a changing relationship among players in the healthcare system. Originally, disease-specific advocacy organizations, formerly known as support groups, supported and educated affected individuals and their families, but now they are engaged in various healthcare areas and drug-related activities. Some patient groups have succeeded in building their knowledge bases and legitimacy in expert health domains. They contributed to evidence-based medicine, and to the legitimacy of government policy-making. As well, they validated the relevance of scientific research and drug development and contributed to disease knowledge on national and international levels. This blurring of knowledge forms supports earlier statements that knowledge is an endeavour of collaboration and co-construction.

One group, PXE International, advanced this model of collaboration and gained international recognition. PXE International was founded in 1995 to promote research and support individuals affected by pseudoxanthoma elasticum (PXE). The organization works on behalf of individuals and their families to improve quality of life through advancing research and educating clinicians. As stated by the founder, “We learned that helping loved ones through a health crisis was not like taking a number at the deli counter. If research on PXE wasn’t being done, we couldn’t just wait until they called our number—they might never get to it”³⁴. PXE International met with other members of the advocacy community who challenged the status quo regarding translational research, including the National Psoriasis Foundation, the National Alopecia Areata Foundation and the AT Children’s Project (for ataxia telangiectasia). For PXE, a shift in viewpoint catalyzed a new model and established the essence of their research advocacy foundation, which involved new players, the advocates. Until this point, “most organizations that the PXE International founders had met with... were focused primarily on

³⁴ <http://content.healthaffairs.org/content/22/5/166.full>

building a strong organization as an end point, rather than as a means to an end". In this way, PXE International adopted aspects of academic models (rigorous science), commercial enterprises (commodification and accountability) and advocacy organizations (trust and agility), and used them to create a hybrid model for advancing research. It can be argued that PXE was able to tip the power balance between lay individuals and experts by using various knowledge forms pertaining to biomedical research, business administration and grassroots action. Other examples of this collaboration model include the UK Community Health and Information Network (CHAIN), the UK Haemophilia Alliance, the European Liver Patients Association (ELPA) and the UK LSD Patient Organization Collaboration.

Based on these examples, we can see the formation of a new era of citizenship and partnership in which disease organizations, tagged as biosocial groupings (Rabinow 1996; Wehling 2011), successfully achieved to change the locus of control over their own health through their actions and knowledge perspectives. The groups of engaged patients discussed above exemplify the production and use of knowledge to advance the needs of their membership. Engaged patients understand the scientific enterprise and are able to work within its boundaries. That said, patient groups socialize patients and socialize the disease they represent, thereby increasing their legitimacy in scientific, medical and social spaces (Rabeharisoa 2006).

The combination of patients' unique disease experience, their knowledge base, and claims to social justice led patient groups to distinguish themselves from ordinary citizens. They contributed knowledge of their lived experiences as patients (e.g., experience with drugs, the disease and the impact of these on their lives) as well as knowledge of a scientific nature. This hybrid knowledge form affirms patients' ability to conduct a critical reading of various sources of information, synthesise the information and formulate a well-founded opinion to inform the

evidence base in their disease area. The cases discussed above demonstrate how patients can become partners in the healthcare endeavour.

4.3 Concluding Remarks

This chapter elaborated on how the concept of patient involvement manifested itself both in the healthcare setting and the domain of health policy, and looked at the conditions that enabled a new model of patient input to emerge. In Canada, the patient involvement phenomenon appears in different forms and in several pockets of society. It carries certain messages about patients, their positions within the healthcare system, their roles and their rights. This fragmented story involving civil society, science and policy is now part of Canada's health policy history. There are different models of involvement with distinct underlying principles and social relationships therein (see Table 4.3). Citizen engagement is regarded as a democratic model that carries notions of citizenship and representativeness whereas patient input in the world of Health Technology Assessments (HTA) is regarded as a technocratic process with the goal of achieving evidence-based decision-making. Both models portray a relationship between governments and their stakeholders. One of the distinguishing features between the models is the recognition that patients and citizens have different wants and needs. On this matter, patient input promotes patients' ability as healthcare stakeholders to provide disease insights and exercise greater control over their own health. This distinguishing feature prompted the classification of patient input as a separate track in the spectrum of involvement.

Table 4.3 presents four quadrants of patient involvement as it emerged in different spheres of social life; each quadrant having unique elements of involvement.

Table 4.3 Forming of a Patient Input Model

<p><u>PATIENT ADVOCACY</u></p> <p>Human dignity Rights to justice Empowerment/Self-Care Vulnerable patients Patient autonomy Helping attributes Guardianship</p>	<p><u>PATIENT INVOLVEMENT</u></p> <p>Engagement in medical care Active role in the patient-doctor interaction Personal performance Health responsiveness Patients’ needs and preferences Transfer of power Shared ownership Self-advocacy Moral claims</p>
<p><u>CITIZEN ENGAGEMENT</u></p> <p>Democracy Systems of governance Power dynamics Interpretations of needs Elitism Marginalisation</p>	<p><u>PATIENT INPUT</u></p> <p>Policy instrument Patients as stakeholders in the HTA network Alternate understandings of science and disease Health claims Contested resource Value laden Involvement structures</p>

To this end, by examining patient involvement through the lens of provincial and federal bodies, international agencies, and patient activism, the empirical findings strengthen the aforementioned assumption that in health policy circles, patients are generally invited to participate in engagement activities only on an adhoc basis and in a non-structured way. Yet the findings also show that Health Technology Assessment (HTA) bodies prefer including patients in involvement structures, despite the absence of legal frameworks to guide the elaboration of involvement programs. On this point, public policy scholars argue that involvement structures may have the effect of forging relationships with involvement audiences. “Institutionalized approaches to public involvement may be a preferred route for organizations keen to foster more sustained relationships with the public or specific patient organizations” (Abelson et al. 2007: 42). If this assumption is correct, the question arises whether these structures leverage a stronger

relationship between HTA bodies, citizens and patients. I now discuss some of the social drivers and pressures linked to the structured inclusion of patients in drug coverage decision-making.

To date, the chapter conveyed that the inclusion of patients in evidence-based policy emerged from various social transformations and pressures that pertain to both the local and global spheres. The enabling factors that contributed to the advancement of public engagement as a key feature of healthcare include, healthcare reforms, political willingness regarding issues of public interest, the rise of evidence-based policy, and the emergence of a new patienthood. I argue that the notion of patienthood refers to two occurrences in the Canadian society. On the one hand, structural changes in healthcare and overarching commitments by governments to public engagement promoted the engagement of patients in matters of the state. On the other hand, certain well-organized and informed groups succeeded to politicize their claims and enter new spaces of expert knowledge as demonstrated by AIDS and cancer activists.

I also stipulate that the Canadian Agency for Drugs and Technologies in Health (CADTH) intended to foster a relationship with the public and patient organizations by creating involvement structures. However, by starting from the assumption that citizens and patients contribute non-technical knowledge to drug coverage decisions, the Agency did not open its decision-making framework to outsiders, and engaged in boundary work to prevent the encroachment of lay actors in their decision-making process.

When the goal is monopolization of professional authority and resources, boundary-work excludes rivals from within by defining them as outsiders with labels such as "pseudo," "deviant," or "amateur" (Gieryn 1983: 792-93).

By excluding patients from the decision-making apparatus, CADTH does not seem to bridge the existing gap between citizens and state institutions, as noted by public policy scholars.

Although CADTH made institutional changes to hear the voices of patients, the absence of change in the structure of decision-making leads to a “voice without influence” (term used by Cornwall and Gaventa 2001).

Further, CADTH emphasizes that the Patient Input Process “captures patients’ experiences and perspectives of living with a medical condition for which a drug under review is indicated”³⁵. During CADTH’s 2017 symposium, the rationale for including patients in Health Technology Assessments (HTA) was framed on their lived experience, and not on their ability to be equal players in the HTA network. As well, patients are assumed to be responsible for their treatment success, which suggests continuity of certain features of patient involvement models, notably the second model founded on the idea that patients are accountable for their health and can manage their health needs.

Patients (including family and informal caregivers) are directly affected by a health condition. They can bring a unique perspective on experiences, attitudes, beliefs, values, and expectations regarding health technologies. Taking into account the patient perspective could contribute to more relevant technologies and improve adherence”.³⁶

As demonstrated by Gieryn (1983), scientists can work to differentiate alternative repertoires of science to discredit the legitimacy of other claims. They can succeed in putting the boundary between their science and non-science. In the case of CADTH, the Agency was able to monopolize drug coverage decision-making through evidence-based policy-making, but still create an opening in its process for citizen and patient involvement. In line with public policy theories, the system’s opening indicates efforts by the Government of Canada to democratize the Common Drug Review and seek support from HTA stakeholders for allocation decisions, until

³⁵ <https://cadth.ca/about-cadth/what-we-do/products-services/cdr>

³⁶ <https://www.cadth.ca/sites/default/files/symp-2017/presentations/april24-2017/Concurrent-Session-B1-Marie-Pierre-Gagnon.pdf>

the concept of boundary work is introduced. This concept allowed me to emphasise CADTH's subtle rhetoric choices in characterizing patient input as anecdotal and describing the Agency's work as "rigorous and objective examinations of new drugs"³⁷. After all, the Agency was mandated to produce credible, standardized information that is relevant and useful to healthcare decision-makers. Although the Agency recognizes the value of patient repertoires in drug coverage decision-making, by advancing rhetoric on their lived experiences, CADTH strengthens the assumption in health policy that citizens and patients contribute non-technical knowledge. CADTH was therefore able to place a social boundary between expert and non-expert knowledge in the HTA decision-making network.

Another rhetoric choice is to advance that the public members are "tasked with bringing a lay perspective to the Committee and ensuring that patient concerns are accorded important consideration in CDEC discussions and deliberations"³⁸. By adopting a direct democratic mechanism to enable the public to play a more active part in HTA decisions, CADTH achieves the goals of democracy in terms of promoting citizens' agency (Cornwall and Gaventa 2001) and representativeness (Contandopoulos 2004; Martin 2008).

Placing an emphasis on inclusive participation as the very foundation of democratic practice...suggests a more active notion of citizenship. This recognises the agency of citizens as 'makers and shapers' rather than as 'users and choosers' of interventions or services designed by others (Cornwall and Gaventa 2001: 33).

It can be argued that adopting an indirect feedback mechanism for patients does not emphasise the agency of patients as makers and shapers of healthcare planning and resource allocation. Also, the majority of involvement examples discussed in this chapter required that patients be

³⁷ <https://cadth.ca/about-cadth/what-we-do/products-services/cdr/patient-input>

³⁸ https://www.cadth.ca/sites/default/files/cdr/consult/Revised_CDR_recs_options_CDEC_delib_process_e.pdf

organized in groups and their best interest communicated by representatives. This approach seems to accentuate the role of patient advocacy organizations in the determination of patients needs. As public policy theories have taught us, this representativeness can lead to an elite citizenry and misrepresentation of communities (Abelson et al. 2002; Tritter and McCallum 2006; Martin 2008). To this end, CADTH's use of certain democratic principles seems to conflate the role of public members and patients. One public member on CADTH's drug expert committee explained:

It is now well recognised that there is a need for the patient perspective to be considered during the development and regulatory and HTA review of new medicines and the role of patient representatives in these activities has been largely accepted by society. However, the role of public members within these activities has yet to be established and there is no consensus about what societal values they should represent or how to measure them and there is no established relationship between public and patient perspectives and values. Although they are not patient representatives, public members may be able to shed light on some common, yet incorrect assumptions regarding patient perspectives...³⁹

In this regard, the boundary work concept is useful to grasp how CADTH's lack of defined role for public members blurs the lines between social values and patients' values, thus creating competing demands between the public's best interest and patients' best interest. Though citizens and patients seem to be encouraged to work within the boundaries of health policy, their participation may be hampered by their ambiguous role in the HTA network.

Attending to the ideologies and discursive practices of CADTH furthers our understanding of the patient input model and the place of patients within the HTA network.

³⁹ Centre for Innovation and Regulatory Science, Workshop Report 2-3 December 2014, Vancouver, BC, Canada, http://www.cirsci.org/wp-content/uploads/2016/02/CIRS_-2-3_-December_2014_Workshop_report_final-1.pdf

Boundary work reveals some of the nuances and complexities of the institutionalisation of patient involvement. The findings suggest that despite being structurally involved in drug coverage decision-making through a patient input submission process, patients do not have a direct structural influence on the committees' decision-making process because they are not voting members of the drug expert committee. Further, patients' alternative understanding of science and knowledge does not appear to rank in the same manner as other evidence forms in the HTA decision-making network. I advance that this epistemic stance regarding lay expertise may have a negative impact on the relationship that CADTH is trying to forge with its stakeholders.

The following chapter explores the nature of patients' knowledge base in relation to the claims of evidence-based decision-making in the HTA network, and ways claims are articulated and interpreted by those who govern and those who are claimants of health-related services.

CHAPTER 5

THE POLITICS OF NEEDS IN THE WORLD OF RARE DISEASES

One of the main findings of this study thus far is that patients are outsiders in drug coverage decision-making and as a result, have a “voice without influence”. Even with the transformation of personhood to patienthood, which reflects a new relationship between those who govern and those who are governed in the healthcare system, patients do not have the same leverage as other actors in the HTA network. I subsequently argue that this lack of power is also reflected in the determination of their needs, which is the foundation of their claims-making in drug coverage matters. The central question in this chapter is how are claims crafted in the HTA network? This chapter therefore focuses on the ways claims are discursively interpreted, negotiated and politicized from below by patients and from above by elite groups. In other words, the chapter looks at how the needs of patients are manipulated to fit an evidence-based policy-making framework in resource allocation. The chapter contends that attending to claims-making points to those who determine needs and those who are claimants of needs. Moreover, this chapter focusses on the politicization process that underlies claims-making through the lens of the Canadian Organization for Rare Disorders (CORD). As has been articulated in the introduction, the dissertation considers whether this politicization of patients’ needs enhances their role in the Patient Input Program.

The empirical findings were collected from interview data, key documents employed by CADTH and information contained in the grey literature. This chapter elaborates on CORD’s political strategies to enter the realm of government and politics, and the way it translated the needs of rare disease patients into a rights-based discourse. I then explore the discursive practices

of main actors in the HTA network around patients' needs using the concept of needs talk. Finally, I describe drug coverage decision-making practices as a site of competing interpretations of patients' needs and discuss some of the hidden discourses at play.

5.1 The Discursive Construction of Needs Claims

In the introduction of the dissertation, rare disease patients were characterized as a vulnerable population. The rare disease community has high stakes in drug coverage matters. Rare diseases are defined as life threatening, seriously debilitating or chronic and only affect a very small number of patients (typically less than 5 in 10,000 persons)⁴⁰. Although rare diseases affect a small percentage of the population, the total number of people with a rare disease is large; there are approximately 7,000 rare disorders affecting 1 in 12 Canadians⁴¹. This represents nearly three millions of Canadians⁴². Taken together, rare disease patients form a broad patient community who share commonalities but also can differ significantly in their health needs. Rare disease patients continuously struggle to access timely, effective and affordable drugs because the cost conundrum is always at the forefront of resource allocation decisions and is founded on the idea that healthcare costs must benefit the aggregate. Hence a rare disease can be viewed as a social and political category that emphasizes patients' socio-political differences in terms of health and financial vulnerabilities. The rarity of their disease is a commonality among patients and is what distinguishes them from patients with common diseases.

Overall, the experiential basis of the experience of 'being rare' for the patients is a key feature of the category of rare diseases. The local use of this category is a political one, as a tool for voicing patients' unmet needs and transforming the statistical weakness of each individual disease into a larger group with political visibility. Thus, in the patients' view, this category has nothing to do with the refinement of

⁴⁰ Health Canada 2014, <http://news.gc.ca/web/article-en.do?nid=873619>

⁴¹ https://w.raredisorders.ca/content/uploads/CORD_Canada_RD_Strategy_22May15.pdf

⁴² <https://www.rarediseasefoundation.org/about>

diagnostic techniques or of medical descriptions, but rather with a political weakness (Huyard 2009: 468).

This political weakness of rare disease patients is explained in part by the challenges associated with the rarity of their disease and the nature of their needs, which turns patients affected by a rare disease into medical outliers. As science and technology studies scholars have demonstrated, patient groups have succeeded to mobilize around disease identities, socialize their disease through membership and make demands on public institutions by political action. In the world of rare diseases, some patient groups have created alliances under the common leadership of the Canadian Organization for Rare Disorders⁴³ (CORD). Through the work of CORD and its numerous strategies, the vulnerabilities and struggles of rare disease patients became publicized and politicized as problems of treatment access.

I highlight the role of CORD in the politicization of these struggles and demonstrate how the needs of rare disease patients were successfully formulated into political claims, thus shifting their private struggles onto the public agenda. To this end, CORD was able to establish political status for rare diseases by making demands on the state for special provisions and considerations with respect to rare disease drugs. Health Canada's Orphan Drug Framework and CORD's Rare Disease Strategy are both concept papers that aim to develop coherent policies and processes for quicker and more equitable access to rare disease drugs. As explained by an advocate for mucopolysaccharide (MPS):

Treatments are expensive, if you see the listing for the 10 most expensive therapies, the MPS drugs tend to be generally in the top 5. Then there are weekly or biweekly injections. They are significant therapies that are indicated for a lifetime and dosed by weight. So when you agree to list the drug to treat a child, that child's therapy is only going to become more expensive as they grow, especially if the therapy is doing its job. (Participant #7)

⁴³ CORD currently has 105 group memberships.

CORD emphasized the significant challenges of rare disease patients in terms of drug availability and drug affordability. As per Yaneva–Deliverska (2011), “A good medication for rare disease patients is a medication that is both available in the country where they live and affordable. If one of these two factors is missing, the drug is of little use” (Yaneva–Deliverska 2011: 116). By doing so, CORD defined how the needs of rare disease patients could be satisfied in a rare disease discourse based on health inequities and injustice. To this day however, CORD has not fully succeeded to secure the satisfaction of these needs since an Orphan Drug framework⁴⁴ has not been established and a separate track for the review of orphan drugs within the Common Drug Review process has not been implemented. Work in these areas is still underway.

Regardless of these outstanding goals, the advocacy efforts of CORD are not insignificant. These efforts led to securing direct benefits from the government of Canada in terms of launching a comprehensive portal on rare diseases in 2012 (the Orphanet database) and increasing research funds from the federal funding agency (i.e., the Canadian Institutes of Health Research) towards transnational rare disease projects. CORD was active in the promotion of newborn screening in Canada and the advancement of a Canadian clinical trial registry tailored for those with rare disorders. The organization is also committed to increasing access to genetic screening and genetic counselling for all rare disorders. Nationally, CORD continuously lobbies governments and health agencies to advance a political agenda for rare diseases. CORD’s 2015 Rare Disease Strategy is evidence of this politicization. It provides hope for Canadians suffering with a rare disease. The Report highlights the extraordinary burden faced by Canadian families with rare illnesses, such as misdiagnosis, unnecessary surgeries, social isolation, financial hardship, lack of treatment options and early death. This is in line with my interview

⁴⁴ The Framework proposes an official definition of rare disease and encourages drug development in small patient population.

findings. Often rare diseases are complicated multi-organ disorders, and consequently rare disease patients become medical experiments. Patients have said in interviews that they undergo various invasive procedures; they experience unnecessary delays in obtaining diagnostic tests; and, they frequently receive incorrect diagnoses leading to a treacherous path of unnecessary interventions. Children suffering from a rare disease usually live with physical and mental developmental challenges or die prematurely. Moreover, there is a common lack of information in the scientific community pertaining to the course of rare diseases on patients and to the therapies that could be beneficial to patients. Stemming from these challenges, CORD called for improvements to early disease detection, disease prognosis and drug access; enhancements to community support and patient care; and, the promotion of innovative rare disease research. CORD was able to gain presence and influence in the public sphere by advocating for better treatment of patients living with a rare disease and providing a strong common voice for rare disease patients.

5.1.1 Patients' Rights to Health Equity and Fair Treatment

This discussion raises important questions about patients' right to healthcare and the fact that this principle has not been established under the *Charter of Rights and Freedoms*. Federal legislation put in place conditions by which regional, provincial and territorial (P/T) governments in Canada receive funding for healthcare services, such as the condition that all citizens have reasonable access to healthcare facilities and that all citizens are entitled to the same level of healthcare. But patient testimonies suggest that the Canadian healthcare system is far from reducing health inequities among regions and among patients. In this regard, the findings suggest that high drug prices and inconsistent drug funding among P/T governments are creating health inequities among patients. Several participants perceived barriers to drug access

and commented on the inconsistencies in drug coverage recommendations. These inconsistencies create unevenness in drug accessibility. In cases where a drug has multiple indications, the drug can be covered for one condition but not another. As well, within Canada, a drug can be covered in one province but not another. Taking Soliris as an example, one patient advocate explained:

In Ontario, there is interim funding for a sub-set of patients with Atypical hemolytic-uremic syndrome (aHUS). In Quebec, the drug is funded on a case-by case basis under the Exceptional Access Program, while in the other provinces, there is no public funding at all. (Participant #20)

Another example of these health disparities was illustrated by CORD during the proceedings of the Standing Committee on Social Affairs, Science and Technology (March 21, 2013). CORD explained that one child in Ontario is receiving growth hormone therapy through private drug coverage, while another child in the same province has been denied the therapy by the Ontario public drug plan. Another child in Alberta obtained the therapy through an off-label drug program, while another in B.C. has been denied access by the public drug plan but receives it through the manufacturer's compassionate access program. Because P/T governments independently establish drug reimbursement plans,

the eligibility and reimbursement criteria of these plans for seniors, non seniors and social assistance recipients differ widely across the country. Thus the amount patients must pay for a given prescription burden is unequal across provinces...these inequities challenge one of the guiding principles of the Canadian healthcare system — that all Canadians should have similar levels of access to healthcare benefits (Demers et al. 2008: 408).

Fundamentally, patients want to have equal access to treatments that improve or save their lives. Here, what is morally right is it not necessarily achievable. Canada is said to be the only developed country with a universal healthcare system that does not provide universal

prescription drug coverage, even though the World Health Organization (WHO) has declared, “All nations are obligated to ensure equitable access to necessary medicines through pharmaceutical policies that work in conjunction with broader systems of universal health coverage” (Pharmacare 2020: The future of drug coverage in Canada 2015:1). The federal government responded to the demand for universal drug coverage with the development of a 2015 Report entitled, *Pharmacare 2020: The future of drug coverage in Canada*. The principles therein advance the ideas that all Canadians should have equitable access to medically necessary prescription drugs; no individual or group should be financially disadvantaged by their health needs; prescription drugs should be funded, prescribed, and used only in accordance with the best available evidence concerning risks and benefits; and the cost of medicines should be managed to achieve maximum value for Canadian society. We can appreciate how these principles are even more relevant to rare disease patients considering they do not have equal access to needed drugs and are financially disadvantaged by their health needs. Although the universal pharmacare program was a goal in the 2004-2014 Health Accord, it did not materialize as it was intended to do. That has left many Canadians struggling over the cost of their prescriptions drugs. As depicted by the following scenario, if a drug is not reimbursed by drug plans, it can have significant impacts on a patient’s life and put them in financial ruins:

In a family where a child has a rare disease, most often one of the parents either completely stops or significantly reduces work remunerated outside the home. As a consequence, while expenses increase dramatically, income is considerably reduced. In the case of an adult rare disease patient who is well enough to be able to work, the work hours must be adapted to allow for medical visits and appropriate care. In terms of logistics, much remains to be done to ensure real equality between a disabled and a healthy citizen. (Yaneva–Deliverska 2011: 118)

Some participants commented that their disease was not even recognized by certain insurance providers. In the case of Mitochondrial disorder, there are no cures and the only available treatment that has demonstrated effectiveness is a cocktail of supplements (“Mito cocktail” is a term recognized in the disease community). The cocktail includes Co-enzyme Q and Creatine. One patient has been financing the cocktail for 30 years with her Canada Disability Benefits and now with her Old Age Security Pension. Her monthly costs exceed \$300.00. In addition, she is not financially eligible for home care (assessed at \$1000 monthly) because co-enzymes and vitamins are not considered medications. Although she has Pacific Blue Cross coverage, the provider does not recognize Mitochondrial as a disease, which conflicts with the *B.C. Health Guide Handbook*.

A patient with Cushing's syndrome also commented on funding inconsistencies in terms of conflicting decisions among Health Canada and the Canadian Agency for Drugs and Technologies in Health (CADTH), both mandated by the Minister of Health. Cushing's syndrome is a rare hormonal disorder involving non-cancerous tumours of the pituitary gland, which cause the body to overproduce cortisol. Treatments include pituitary surgery, radiation, oral corticosteroid medication such as Ketoconazole, and often because of the radiation, hormone replacement therapy. Although approved by Health Canada in 2013, Signifor, a promising new drug for the treatment of adult patients with Cushing's disease, was not recommended for listing on the P/T drug plan formularies. Another participant explained that due to Canada's inadequate drug coverage program, “patients suffering from Vasculitis have to seek treatments outside of Canada”, including himself via the U.S. National Institutes of Health (NIH) (Participant #21). These narratives of patients are becoming common, and have been the subject of several media reports. Despite the negative press, CADTH has not publicly reacted to the claims of patients.

Thus, patients with rare diseases “live from day to day in a state of flux” (Participant #23). Statistics Canada published a 2016 report entitled, *Out-of-pocket spending on drugs and pharmaceutical products and cost related prescription non-adherence among Canadians with chronic disease*, which suggests that in 2011, Canadian households spent an average of \$476 out-of-pocket on prescription medications. This average of annual expenditures exceeded to \$600 for households with individuals older than 55. In 2010, 20% of households spent more than 1% of their after-tax income on prescription medications and 3% spent more than 5%. The 2016 Report also concludes, “The economic burden of out-of-pocket expenditures related to medications and healthcare in general has grown over time, particularly among the lowest income households” (Hennesey et al. 2016: 3). For rare disease sufferers, the burden of these costs can be significantly higher. The average cost of a rare disease drug ranges from \$1,750 to \$500,000 per patient, per year with an average cost of about \$44,000 per patient per year. Yet in Canada, less than 1 percent of our public drug budget is attributed for rare diseases⁴⁵. Most Canadians pay for prescriptions out-of-pocket or through private drug plans.

To this end, CORD denounced the shortcomings of the Common Drug Review process. The news wire reported that CORD’s President viewed the HTA system as inherently biased against rare disorders: “They are denying patients with rare diseases access to breakthrough, and potentially life-saving, new medical treatments”⁴⁶. CORD criticized the methods used in the Common Drug Review process, describing them as inappropriate, inadequate and unethical standards of drug evaluation, often resulting in negative coverage recommendations for rare disease drugs. It can be argued that CORD’s criticism of the process is an example of the

⁴⁵ <https://www.raredisorders.ca/content/uploads/Media-Planet-Rare-Disease-March-2014.pdf>

⁴⁶ <http://www.newswire.ca/news-releases/canadians-with-rare-diseases-de-nied-chance-for-life-by-common-drug-review-533677841.html>

politicization of patients needs. This perspective echoes what participants expressed during interviews in terms of inflexible evidence models to address the limitations of studying small patient populations. These challenges, which stem more broadly from the institutional norms of scientific knowledge and economic stakes in drug development, affect patients' ability to access treatments and participate in clinical trials for the purpose of advancing scientific knowledge in rare diseases whether by saving lives or improving quality of life. Quality of life is a key factor in patients' medical needs. Rare disease patients often have burdensome treatment regimens that interfere with daily life. The Chief Scientist Officer at Cystic Fibrosis Canada (also a parent of a child with cystic fibrosis) explained that in the case of cystic fibrosis, patients take several drugs daily, which amounts to 1 to 1.5 hours of therapy morning and night. It is difficult for patients to maintain therapy, manage their own care with hectic school, and work schedules (Participant #17). Consequently, a drug that has a negative impact on a patient's quality of life can alter a patient's adherence to the prescribed treatment regime. In fact, several participants share the sense that their medical needs are being neglected by the pharmaceutical industry. One mother discussed the difficulty of administering a life-saving therapy for her young son, who suffers from hypopituitarism. To give him a Solu-Cortef injection (an anti-inflammatory glucocorticoid containing hydrocortisone sodium succinate as the active ingredient), she has to combine the powder and the diluent, put it in a syringe, mix it and inject it into her son's muscle. The mother commented that intramuscular injections are very painful. Having access to a mechanism similar to the EpiPen would significantly improve her son's treatment regime.

During interviews, patients made claims for openness and transparency in the Common Drug Review process. Participants expressed significant concern in terms of not knowing who works on patient input summaries and not knowing how much weight is given to the patient

voice. In general, patients expressed desire for increased transparency in CADTH's decision-making process; they want the Agency to be honest about the ways coverage recommendations are made, and they want appropriate guidance in the case of negative funding decisions. One patient advocate explained that her society hoped that if their drug Vimizin, a promising treatment for mucopolysaccharidoses (MPS), was not approved for coverage, there would be a recommendation put forward on what should happen next: "If it is a cost issue, is there a recommendation to go through the Pan-Canadian Pharmaceutical Alliance (PcPA) for negotiation to bring down the cost? In the case of Vimizin, unfortunately, that did not happen" (Participant #7).

The President and Chief Executive Officer (CEO) of the Canadian PKU and Allied Disorders called for the democratizing of the Patient Input Program in terms of open meetings of the drug expert committee, webcasts, and public records of discussion. He felt that there should also be an opportunity for patient groups to make presentations to the committee. The Executive Director of the Canadian MPS Society echoed the idea of a more open process and welcomed a more open and inclusive process where patient groups and patients would have the opportunity to sit at the table and participate in decision-making. Notwithstanding these concerns and claims for openness, the President of CORD noted an evolution in the HTA process: "the HTA process has evolved into a more open process due to increased public scrutiny. Decisions are now more transparent" (Participant #14), which is consistent with that of my interview findings. CADTH staff explained that transparency is a priority for the Agency. Since 2007, CADTH has committed to increased transparency of its Common Drug Review process and made recommendations to respond to growing pressure from its stakeholders.

Since the CDR's inception, CADTH and the Canadian Expert Drug Advisory Committee (CEDAC) have been supportive of increased

transparency. This is evidenced by the posting of CEDAC recommendations and reasons for recommendation and the posting of the progress of submissions under review on the CADTH web site.

The goals of the transparency initiatives are (1) to increase understanding of recommendations by releasing key information related to each recommendation and (2) to increase accessibility of recommendations to the general public by providing information in plain language⁴⁷.

Some more recent transparency initiatives include posting summaries of patient group feedback and consultations that led to the merger of the pan-Canadian Oncology Drug Review (pCODR) and CADTH in April 2014. Several patient engagement opportunities were made available such as face-to-face meetings, in addition to written consultations. In this consultative exercise, CADTH explained that the broader questions were being discussed, including the possibility of allowing patient groups to attend open meetings⁴⁸, consider ways of selecting the right individuals to attend, and looking at the logistical implications of moving patients across the country. Overall, there was emphasis on the logistical issues associated with the implementation of transparency in the HTA process. For example, one of CDEC's public members discussed the potential downfalls of open meetings in terms of managing geographically dispersed groups of patients, as well as managing patients' expectations and frustrations, especially in cases where drugs receive negative recommendations. The participant added that: "Patients would invest a lot of time (travel and meeting preparation) and their involvement may not impact the decision in the end, especially if the drug offers no proven benefits over current therapies and costs a lot more" (Participant #29). He felt that direct involvement could slow down the process, which is already criticized for being lengthy.

⁴⁷ <https://cadth.ca/consultation-new-cdr-transparency-initiatives>

⁴⁸ To inform this discussion, CADTH commented that the Agency was seeking information from other HTA organizations on their experience with open meetings, the impacts on the committee dynamics, the voting processes, and issues of privacy.

In summary, based on principles of equity, accountability and transparency, CORD was able to articulate a strategic rare disease discourse founded on health inequities and injustice. As I will demonstrate in the next section, CORD also succeeded in redefining the problem of rare disease diagnosis to a problem of treatment access while placing blame on multiple players for Canada's lack of responsiveness to rare disease sufferers and presenting feasible solutions to improve their interests (Embrett 2014).

5.1.2 Lobbying for a Collective Good

Guided by the concept of needs talk, I consider the role of CORD in legitimizing the needs of rare disease patients in public policy. This involves examining implicit needs interpretations that circulate in society and the way patient needs are framed and by whom. First, CORD was able to validate their health needs as matters of legitimate political concern and displace the despair of individuals from the private sphere to the public sphere, in part under the radar of media. The organization also put pressure on the government with various tools, including the use of powerful numbers, facts and symbols, a petition⁴⁹, open letters to Canada's federal Minister of Health and meetings with health officials. It also contributed to parliamentary studies and organized rare disease rallies. CORD succeeded to mobilize the rare disease community and morph patients' suffering into a discourse about rights. As per Embrett (2014), "With CORD, a high level of collective benefits was symbolized in a dense network of people (rare disease patients and supporters) lobbying for a collective good, which engendered a sense of civic engagement and equity amongst these actors" (Embrett 2014: 7). It can also be argued that CORD contributed to the socialization and politicization of the rare disease category by emphasizing the socio-political differences of rare disease patients in terms of health and

⁴⁹ Petition: Approve the Orphan Drug Regulatory Framework, <http://lacf.org/en/petition-approve-the-orphan-drug-regulatory-framework/>, Retrieved on October 10, 2017.

financial vulnerabilities. Through CORD, patients acquired a rare disease identity and developed a specific language around disease prevalence, disease complexity and disease inheritance, which was used to advance their health needs in the public healthcare system. The organization socialized patients and socialized rare diseases. It was able to articulate the demands of rare disease patients in scientific, medical and social spaces, which resulted in building their legitimacy in the political arena (Rabeharisoa 2006).

Second, CORD was able to legitimize feasible solutions to improve the lives of rare disease patients by entering the realm of government and politics. The organization served as a witness on the Standing Committee of Health. To inform the Committee's study of technological innovation, CORD presented its Rare Disease Strategy and managed to place it on the political agenda. During Committee hearings, CORD discussed the need to encourage genetic research (as 80% of rare diseases have a genetic cause) and the development of accessible treatments, as well as bringing off-label use drugs into the drug regulatory system for on-label use, as many orphan drugs are used outside their authorized conditions of use. Furthermore, CORD downplayed the general assumption that rare disease drugs carry astonishing drug prices.

I will give you a bit of a reality check. If we think about all the drugs that are now currently being funded for rare diseases, including some that you've heard about as being very expensive, the cost still amounts to only 0.7% of the public drug budget because the numbers are very small (Proceedings of the Standing Committee on Health, Thursday, May 2, 2013⁵⁰).

CORD advanced a rare disease discourse that influenced agenda setting in Parliament and led to a commitment by the federal government to an Orphan Drug Framework (announced in 2012). It can be argued that CORD forged a new relationship between policy-makers and rare

⁵⁰ <https://openparliament.ca/committees/health/41-1/85/david-lee-1/>

disease patients. The organization continuously strives to secure the implementation of this Framework in an effort to push the federal government to fulfil its commitment. In this way, CORD is a poster child for patient mobilisation. It can be characterized as a pluralistic organization (Huyard 2009). First, CORD works with governments, researchers, clinicians and industry. Recently in 2016, it joined the Massachusetts Institute of Technology (MIT)'s NEW Drug Development ParadIGmS (NEWDIGS) program, which "brings together pharmaceutical and biotech companies, regulators, insurers, patient advocacy groups, and other healthcare stakeholders to design modern systems connecting scientific discovery to patientcare"⁵¹. Second, CORD's board of directors comprises an expert in drug pricing, patient advocates (including patients and their relatives), an educator, communications professional, management consultant, academic and lawyer. Its membership base includes drug companies and approximately 100 affiliate/ group members. Not only did the organization exercise political pressure on the state, but due to its internal composition and the diversity of its stakeholders, CORD performed well on the political scene, as it entered the space of politics and brought visibility to the predicament of rare disease patients. Third, as a pluralistic organization⁵², CORD enables stakeholders to collaborate on an equal footing. Its actions cover short-term goals such as providing information to patients and linking them to other support groups, and long-term goals that include the promotion of research, diagnosis, treatment and services for patients. And finally, CORD has a broad portfolio of work that doesn't solely focus on the development of medical breakthroughs. Beyond its composition and actions, the analysis demonstrates that CORD was able to construct a discourse on the needs of patients, which led to the transformation of their needs into objects of state intervention. The following section explores how the needs of patients are crafted in the

⁵¹ <https://www.raredisorders.ca/content/uploads/NEWDIGS-CORD-PR-170228.pdf>

⁵² Huyard' typology builds on Rabeharisoa's 2003 typology of disease organization by adding the category of "pluralistic organizations"; these formations are absent from Rabeharisoa's study.

HTA decision-making process. Although the translation of needs into political demands can benefit patients and increase their material gains, this process is far from being straightforward in drug coverage matters. As I will demonstrate, drug coverage decision-making practices are a site of competing interpretations of patients' needs.

5.2 The Political Process of Needs Determination

To reiterate, under the leadership of COD, rare disease groups fought to legitimize and establish their needs interpretation in public policy. This section focuses on other needs interpretations put forward by patients themselves during interviews and by dominant groups in society. In this regard, not only do discourses on the needs of rare disease patients occupy several spaces in society, they are also framed differently in institutional and discursive practices. The notion of needs emerged frequently in the interviews. To complement these findings, a search of the grey literature was performed to determine if other needs interpretations circulating in society were consistent with those of patients. Based on this search, I was able to produce a table of needs interpretations from the perspectives of interviewed patients, the pharmaceutical industry, CADTH, the Government of Canada and the public.

Table 5.2 presents how different groups and institutions part of the HTA network interpret the needs of patients. While some needs interpretations align, others conflict and hide unspoken agendas.

Table 5.2 Overview of Patients' Needs Interpretations

Patient Groups	
Drug	Cure
	Quality of Life
	Treatment Choices
	Coverage
	Research and Clinical Trial Participation
	Flexible Evidence Models
Engagement	Resources
	Transparency in Decision-making
	Patient-focussed Decisions
Pharmaceutical Industry	
Drug	Effective Treatment
	Adherence to Medications
	Quality of Life
Disease	Self-Management
	Positive Patient Experience
Clinical Development	Knowledge
CADTH	
Drug	Drug Access
	Quality of Life
	Relevant Health Outcomes
	Main Concerns and Expectations
Engagement	Partners
Government of Canada	
Services	Patient-centred Care
	Access to Quality Care
	Clinical Needs (as opposed to patient demand)
Engagement	Involved in a meaningful way
Public	
Services	Patient-centred Care
	Access to Quality Care [respect individual choice and are delivered in a manner that is timely, safe, effective and according to the most currently available scientific knowledge]
Holistic Experience	Treated as individuals and not just as a body or a condition
	Changing and vary among individual patients, settings, cultures, and other circumstances.
	Physical comfort and spiritual and psychosocial support
Drug	Access to drugs for rare diseases

The majority of patients and patient advocates interviewed referred to the personal and social consequences of living with a rare disease. Patients in the rare disease community desire improved disease experiences and share commonalities; they share similar feelings of aloneness in their diagnosis, feelings of empathy towards others in similar circumstances, and feelings of frustration with the level of care for their condition⁵³. Not only does the nature of their condition bring feelings of unfairness, living with a rare disease has implications in all areas of their lives and may lead to stigmatisation, isolation, exclusion from society and discrimination for health insurance. It can also reduce professional opportunities (Yaneva–Deliverska, 2011:116). In a similar vein, Huyard (2009), as discussed in a previous chapter, contends that while medical needs are diverse among patients with a rare disease, rare disease patients share common aspirations of fair treatment, which she places under the rubric of moral claims. These moral claims form a discourse about patients' rights to information about diagnosis that is realistic but humane; their rights to appropriate medical insights regarding daily life with the disease; and their rights to benefit from joining disease-related associations for combatting feelings of isolation. In general, participants expressed common aspirations for fair treatment because as a vulnerable population, rare disease patients struggle to establish recognition for their needs in the Common Drug Review process. I describe their needs as claims for health equity, openness and inclusiveness in the Common Drug Review process. Through these commonalities, rare disease patients have connected together and jointly developed a sense of social membership and shared identity. The inception of CORD was the expression of the collectivizing process of rare disease patients under a disease identity to advance their health needs in the public healthcare system. Because drug coverage is a major issue for rare disease organizations and patients alike as most

⁵³ <https://globalgenes.org/raredaily/rare-meetups-a-place-to-share-ideas-learn-and-connect-with-others-experiencing-rare-disease/> , retrieved on May 26, 2017.

drugs carry high costs, it can be argued that governments should have a stronger role in the fair and equal distribution of health resources, while giving special attention to rare disease patients. Citizens perceive the federal government as having the responsibility to distribute health services and drug therapies equally (Bombard et al. 2011). This perception may be one of the reasons why CORD put pressure on Health Canada to develop the Orphan Drug Framework and why CORD advanced the Rare Disease Strategy.

The issues of rare diseases and health inequities were also debated in the academic literature (Hughes et al. 2005; Clarke 2006; Panju and Bell 2010). Although the cost conundrum is always at the forefront of these discussions, the ethical considerations around drug access are also argued. Clarke (2006) suggests that CADTH issues more negative funding recommendations to drugs for rare diseases than for common conditions based on his assessment of CADTH's Common Drug Review (CDR) Reports as published on the website. Clarke (2006) states that: "In each of the cases involving new drugs for rare diseases, such as Fabry disease, Gaucher disease and mucopolysaccharidosis type I, the reviews commented on the high cost of treatment" (Clarke 2006: 189). In general, the literature attempts to qualify the needs of patients and justify government intervention. Again, the general discussion is whether rare disease patients should or should not be treated equally. Different arguments for and against the special treatment of rare disease drugs are put to light (Hughes et al. 2005). These arguments include the equity principle, a rights-based approach and the rule of rescue. According to Hughes et. al (2005), the equity principle promotes a utilitarian approach to distributive justice for the overall good to the greatest number, rather than give special consideration to persons with rare diseases (Hughes et. al 2005: 833). The rights-based approach promotes that all individuals have equal rights to quality of treatment whereas the rule of rescue allows public funding of expensive drugs

for a very small number of Canadians (as advocated by Panju and Bell, 2010). In short, there is an attempt in the scientific literature to define the needs of rare disease patients on principles of equity and “what is considered to be socially just” (Hughes 2005: 315). Social justice is at the heart of the debates.

On this point, the struggles of rare disease patients were brought to light in printed media. The role of media was paramount in building awareness about the challenges faced by rare disease patients in the broader public. For example, in 2014 Saskatchewan announced it would be the third province to fund the drug Kaledico, an expensive drug for the treatment of cystic fibrosis (approximately \$300,000 per person, per year). The Global News published a story about Liam, a little boy from Saskatchewan who despite having difficulty digesting has to swallow twelve enzyme pills a day to help with vitamin deficiencies. The article states that Liam has trouble gaining weight and he is at risk of lung infection. The article illustrates the hardships of caring for a child with a rare disease. The article refers to the drug Kaledico as a miracle drug and uses powerful quotes from Liam’s parents: “Keeping him healthy and safe are different for us than for most parents. This is a pretty big deal to help him breathe better and be more of a normal kid”⁵⁴. The article also points to the cost conundrum of rare diseases since the drug is only beneficial for a small subset of patients with cystic fibrosis, likely only a handful of patients in Saskatchewan. Liam’s parents are described as hopeful that their son will be eligible to receive coverage for the drug when he turns six. Yet the article states that the growth rate for orphan drugs is expected to double the growth in the overall prescription drug market in the next four years. It highlights that Saskatchewan funds 10 orphan drugs that benefit less than 20 people in the province. Health Minister Dustin Duncan explains these economic complexities: “We

⁵⁴ <http://globalnews.ca/news/1643793/orphan-drugs-the-high-cost-of-rare-diseases/>

recognize that not everything can be funded, within the system. There will always be more demands than we have the ability to fund. Most of the drugs are in the six-figure price range, representing one per cent of Saskatchewan's drug budget, or about \$3-million, per year"⁴⁰. Here, the cost conundrum is confronted to the fate of a child. Another powerful example of the use of media outlets to stir government action and public compassion is the story of two young Ontarian women suffering from Ehlers-Danlos Syndrome (EDS). In 2015, these women held a news conference and spoke of "financial ruin from maxing out their credit cards and re-mortgaging their houses to pay bills into the hundreds of thousands of dollars for treatment and surgeries"⁵⁵. These treatments were sought in the U.S. because they were not accessible in Canada. The underlying discourse is that Canada's healthcare system is failing to meet the needs of its citizens; interpreted as treatment access issues.

Besides academia and the press, the pharmaceutical companies also contribute to discourses about patients' needs. Based on my search of the grey literature, companies state publicly that they are concerned with meeting patients' needs in terms of developing effective and relevant treatments to improve quality of life, helping patients self-manage their disease, ensuring patients have a positive patient experience, and providing information to allow them to become more knowledgeable about clinical development and increase the value of their input in clinical trial design. Despite that, pharmaceutical companies are for-profit entities that are concerned with their bottom line. They have little interest in investing in rare disease research due to high investment costs of drug development and low-returns from small patient populations (Huyard 2009). This lack of interest from the pharmaceutical industry leads to knowledge gaps in several rare disease areas because the pool of clinical data is limited to

⁵⁵ <https://www.thestar.com/news/queenspark/2015/10/28/ontario-pledges-help-for-women-with-rare-disease-eds.html>

support rare disease drugs, as explained in *CORD's 2015 Strategy for Rare Diseases*. Yet patient centeredness has become a buzzword used by the pharmaceutical industry to make patients feel as though industry is working towards the well-being and empowerment of patients. The U.S. Institutes of Medicine defines patient centeredness as a notion that “encompasses qualities of compassion, empathy, and responsiveness to the needs, values, and expressed preferences of the individual patient”⁵⁶. In this regard, patient centeredness has become an element of their business model. For example, Pfizer’s Vera Rulon speaks to the company’s value equation and patient centeredness in terms of looking at the whole patient including how all the different aspects of life impact their health (i.e., dietary aspects, exercise, and sleep). She affirms that: “All of those things contribute to their quality of life, and therefore value, especially if the patient understands what they’re doing is best for them. It’s a holistic approach”⁵⁷. Other pharmaceutical companies such as AstraZeneca, GSK and Roche, have prioritized the patient’s ability to self-manage their disease. This vision reflects what we have described earlier as the new tenet of healthcare, which promotes health responsiveness or patients’ self-determination in the maintenance and management of their healthcare experience. To facilitate health responsiveness, the pharmaceutical industry introduced mobile health (mHealth) services and innovative technological tools, such as biosensors and smartphone applications to enable patients to monitor their disease symptoms.

Despite its discourse that “patients matter”, it is widely recognized that the pharmaceutical industry employs a range of persuasive mechanisms to increase its profit margins, such as the use of drug narratives (Matheson 2008). These function as powerful “morality tales, in which the scientist, clinician or pharma company is allotted a role analogous

⁵⁶ <https://archive.ahrq.gov/research/findings/nhqrd/nhdr10/Chap5.html#ref1>

⁵⁷ <https://www.lifescienceleader.com/doc/how-can-you-provide-healthcare-value-in-a-patient-centric-era-0001>

to that of protector, sleuth or explorer, and truth and goodness are aligned such that to believe feels like a virtue” (Matheson 2008: 367). In this way, the pharmaceutical industry’s rhetoric of patient centeredness can be critiqued as a feel-good story of corporate social responsibility and a series of empty promises. It also points to the pharmaceutical industry’s role within truth discourses (Rabinow and Rose 2006; Matheson 2008). Although industry recognizes the unique challenges of rare disease patients, it prioritizes its business objectives. According to a witness at the Standing Committee on Health, “estimates suggest that over 50% of individuals with rare diseases do not have a correct diagnosis of their condition.... beyond the challenges of diagnosis, only about 200 therapies have been developed for thousands of rare diseases” (Standing Senate Committee on Health 2013: 40). P/T governments and federal government, including Parliament, have recognized these challenges. The needs of rare disease patients therefore circulate within these spheres, which emphasises the increasing political weight of rare disease issues. These needs interpretations do not however challenge the unequal status of rare disease patients within the healthcare system.

For instance in 2016, the Ontario Government launched a Working Group aimed to benefit people living with rare diseases thereby supporting improved diagnosis, treatment, and access to care⁵⁸. The Working Group determined that rare disease patients need service delivery improvements. These include diagnostic capacity and early detection, timely access to equitable and evidence-based care, supports to primary care providers in identifying and managing rare diseases, access to complex care and rehabilitation, access to clinical trials and potential promising therapies, as well as innovative research on the diagnosis and treatment of rare diseases. The Minister of Health and Long-Term Care publically stated:

⁵⁸ http://www.health.gov.on.ca/en/news/bulletin/2016/hb_20160902.aspx

We are proud of the work that Ontario has done to improve access to care for patients with rare diseases, but we know that more work needs to be done. The Rare Disease Working Group, comprised of clinical experts and patient advocates, is an important step in exploring how services for people with rare diseases in Ontario can be improved. I look forward to meeting with the working group to hear their ideas for how we can improve our healthcare system and improve the way we treat rare diseases for patients and their families.
— Dr. Eric Hoskins, Minister of Health and Long-Term Care⁵⁹

Due in part to the lobbying activities of CORD, the needs of rare disease patients were also debated in Parliament, as previously mentioned. The Standing Committee on Health looked at the proposed Orphan Drug Framework, the state of rare diseases in Canada and formulated recommendations directed towards the health agencies in terms of fostering international collaboration, creating hubs of domestic expertise and recognizing the need for alternative treatments in rare diseases. Parliament appears to be responsive to the needs of rare disease communities in terms of enhancing the role of technological experts in the field of rare diseases and placing Canada on the international research stage.

Health Canada, in addition to its support of Orphanet, participate in and contribute to international rare disease registries to facilitate international cooperation on the treatment of rare diseases.

Canadian Institutes for Health Research and the Public Health Agency of Canada, in collaboration with the Networks of Centres of Excellence, consider identifying clusters of rare disease research in Canada, and consider formalizing some of them as Centres of Excellence within the Network.

Health Canada consider whether it is necessary to establish a framework for non-pharmaceutical treatments such as medical devices used to treat rare diseases in the orphan drug framework or whether it is necessary to create a parallel framework for non-pharmaceutical treatments. (Standing Senate Committee on Health 2013:78)

⁵⁹ http://www.health.gov.on.ca/en/news/bulletin/2016/hb_20160902.aspx

As we have seen, in addition to CORD's rare disease discourse and the claims made by patients themselves, other discourses about patients' needs circulate in society. These discourses are tied to notions of social justice, public compassion, patient centeredness, healthcare delivery and technological innovation. The fundamental premise of these discourses is to increase treatment access. The consequence of generalizing these needs solely on the basis of treatment access is that it deflects attention from important political questions and policy options. In order to facilitate drug access, can governments place additional controls on excessive drug pricing by drug companies? Can patients be involved in setting the clinical criteria for drug reimbursement with the P/T governments? Can evidence models be revisited to adapt to the particularities of rare disease drugs? These questions are relatively undebated but are more likely to raise controversies and disagreements. In the following section, using CADTH's Patient Input Program, I further explore how patients' needs are staged and contested among experts and non-experts in the Common Drug Review process. Patients' needs are subject to interpretations and can be influenced by groups and institutions. This statement supports the argument that the determination of needs is a highly controversial and politicized process (Aronson 1993; Tronto 2010).

5.2.1 Disease Rarity and the Creation of Medical Outliers

As remarked in the previous chapter, the Canadian Agency for Drugs and Technologies in Health (CADTH) has become a powerful evidence-based policy-making institution. Within the Common Drug Review process, patients' needs are considered in conjunction with existing evidence models of biomedical science and pharmacoeconomics. They are judged against criteria of evidence-based medicine (EBM) and cost-effectiveness research (CER) and measured in terms of patients' unmet needs. Again, in this social space the unmet needs of rare disease

patients are interpreted as treatment access issues. The Common Drug Review process is driven by EBM which is employed as a tool for rationing decisions in resource allocation alongside CER. These domains of expertise are intertwined and have the power to exercise influence on health spending practices. As demonstrated in the social sciences literature, the HTA standardization system is founded on principles of EBM and CER and generates a privileged perspective of scientific knowledge that has the effect of discriminating against rare diseases. Discourses on patients' needs circulating in the HTA system are therefore closely connected to institutional norms of scientific knowledge production in the field of medical sciences and pharmacoeconomics. These fields of experts or communities of practice work collaboratively towards funding recommendations, though using different languages and tools specific to their domain of expertise. They are characterized by ontological, epistemological and methodological stances, and fundamentally drive CADTH's drug coverage decision-making. These communities of practice are comprised of clinical reviewers who develop Clinical Review Reports and economic experts who develop Pharmacoeconomic Reports. Based on principles of EBM and CER, their reports inform CADTH's final Common Drug Review Reports which are posted on their website as final funding recommendations. As per CADTH, EBM and CER guide fair healthcare funding recommendations⁶⁰. In contrast, one patient advocate for Life Raft Group Canada described evidence-based policy-making as "simply a cover story for politicians not to pay for stuff. They want to avoid debate. Their response is that it [funding] is a good idea but where is the evidence?" (Participant #4). Another participant noted that CADTH was still very concerned about the overall cost implications and stated that every recommendation comes out with the idea that the price should be lower. She explained that in the past, CADTH would say that they had concerns with the evidence. They did not believe it demonstrated that the drug was

⁶⁰ https://www.cadth.ca/sites/default/files/pcodr/Communications/pcodr_drug_funding_tutorial.pdf

safe. Then the provinces would deliberate and would say that there was not enough evidence that the drug actually worked (referring to the drug's efficacy). But at the end of the line, the manufacturers would lower their price and the provinces would list it. As per the President of CORD , “You don't need a lot of insight to really understand that it's a smokescreen. That's all it is” (Participant #14).

Here, the findings suggest that the cost conundrum is at the forefront of resource allocation decisions at the expense of patients' needs. It leads to a key question: are government officials hiding behind the cloak of EBM and CER to justify constraints in health spending. In this regard, the Clinical Review Report presents a portrait of a drug's benefit and risk profile based on the cumulative evidence of its use and on a comparison of existing therapies. One important focal point of the analysis is on the statistical significance of the drugs' effectiveness and the statistical significance of patients' treatment response. A search of CADTH's funding recommendations with the term “not statistically significant” resulted in 96 reports out of 448 recommendations published on CADTH's website between March 15, 2007 and November 4, 2015. Because the Common Drug Review emphasizes the “needs and preferences of the majority of patients”⁶¹, several participants questioned the applicability and relevance of CADTH's evidence markers in rare diseases. Participants felt that the evidence markers were unrealistic because the rare disease community are small in numbers. As per a patient advocate,

If you rely on evidence, statistically significant, from double-blinded studies using placebo, with a small group of patients, you will never get the numbers. How are you going to do a clinical trial? You have enrolment criteria, but all these patients have been treated with different drugs and therapies and combinations. You will not be able to randomize patients. It's an impossible standard because for a clinical trial, you want patients to have the same history; you don't want patients to have been treated with other drugs; and, you want

⁶¹ <https://arthritis.ca/getmedia/79211395-1ad9-4d67-b350-69467a9d3a1b/CADTH-Xeljanz-August-2014.pdf>

patients to be at the same disease stage. There will never be evidence. (Participant #4)

Yet to support evidence-based policy-making, CADTH's Clinical Review Report contains clinical information on the benefits and harmful effects of the drug that was collected through a systematic review of the literature. The report details information on the disease prevalence, standards of therapy, and the drug under review. In addition to the systematic review of the literature, CADTH experts perform a statistical analysis of the data to assess the statistical significance of the drugs' efficacy in the studied populations, the sample size, and the proportion of patients to complete the treatment phase or to withdraw from the study due to adverse events. They also review data reported on the participant's exposure to study treatments. The Report then presents a critical appraisal of selected studies while stressing the importance of published and unpublished randomized clinical trials (RCTs) with minimum study duration of six months. Other factors in the appraisal include patient population, potential subgroups, interventions (e.g., active comparator, placebo comparator), comparators, harms outcomes (e.g., mortality, adverse events) and efficacy outcomes, notably the health-related quality of life measures⁶² (HRQL) or the six-minute walk distance test (6MWT)⁶³. CADTH experts then validate the results from the trials and may speak to the generalization of these results to the Canadian population (when trials are conducted on foreign grounds) and to the key efficacy outcomes. The final part of the CDR Review Report includes a discussion on the summary of the available evidence and provides an interpretation of the results. This discussion also refers to existing products that are similar in

⁶² HRQL is used to measure dimensions such as: physical function, social function, cognitive function, anxiety/distress, bodily pain, sleep/rest, energy/fatigue and general health perception. HRQL measures may be disease-specific (e.g., heart disease or arthritis) or general (covering overall health) (Goodman, S. C., 2004).

⁶³ As per Pollentier et al. (2010, 14) "The six-minute walk test (6MWT) is frequently used to determine functional capacity in patients. The 6MWT is performed on a flat surface, with a predetermined distance measured and marked off. Patients are asked to walk self-paced back and forth between the 2 marked points for 6 minutes...The laps are counted, and a distance walked in 6 minutes is determined."

nature and provides the similarities and differences among products in terms of manufacturing information, formulations, indications, and dosing.

By using standard evidence models and applying the principles of EBM, CADTH attempts to quantify and justify the needs of patients in an objective and neutral manner. That is, patients' needs are framed in pragmatic and probabilistic terms, thereby overlooking the diversity of needs and any human considerations of living with a disease. During data analysis, this diversity in patients' health needs was reflected in the interview findings. In CADTH's view, this diversity of patients' needs is a key component of patient input submissions since it serves to highlight the range of human experiences within a disease community. The logic models used in the assessment of drug coverage decision-making do not account for this diversity. This supports earlier statements that EBM, in its constitutive nature, takes for granted the diversity of needs within patient populations. In other words, one might argue that the suffering and needs of rare disease patients are discredited because standard evidence models are not suited to account for the rarity, uniqueness and diversity of needs within a rare disease community.

In some rare disease areas, statistical significance may be achievable, but not in others. The President and Chief Executive Officer (CEO) of the Canadian PKU and Allied Disorders explained the challenge of defining rare disorders and ultra-rare disorders.

There is a practical difference between a rare disorder and an ultra rare disorder. The practical difference is that for the rare disorder, you can get enough patients to get to statistical significance in the pivotal trials. In the case of Kuvan for PKU, the Phase 3 trial (the pivotal one) started with 80 patients and finished with 80 patients. They got statistical significance on the surrogate maker alone by the skin of their teeth. There are not enough patients to reach statistical significance for ultra-rare diseases using the conventional standard. (Participant #1)

As an illustration of this practical difference between a rare disorder and an ultra rare disorder, a patient advocate commented that “there are less than 100 patients in Canada with Atypical hemolytic-uremic syndrome (aHUS), therefore a successful treatment in 9 out of 10 patients is promising because the drug is seen as a life-changer. It saves lives and restores kidney function” (Participant #20).

In this regard, standard clinical trial methods (that necessitate large pools of randomized subjects) and the outcome measures studied in trials are not always applicable in the field of rare diseases. In contrast to drugs intended for common diseases, rare disease drugs treat life-threatening diseases. As per a pituitary patient, “It is unethical to give a placebo to a patient and deprive them of life-saving therapies” (Participant #5). To reiterate, rare diseases affect a small number of people compared to the general population. The rarity of the disease creates logistical and ethical challenges in the conduct of clinical trials, especially for pediatric trials. For example, in the case of cystic fibrosis, it is difficult to get a clinical trial to demonstrate a benefit in children (i.e., six years of age) for two reasons:

The way you measure lung capacity is for expiration of volume and little children don't do it well. You don't get a good measure. You have to blow into a machine consistently. You don't get good, consistent results with children. Second, their lung capacity is very high when they are young. They have an average of 120% lung capacity. Their lungs start out perfectly fine. They haven't had that deterioration. It is then difficult for those patients to show that the drug is beneficial. (Participant #17)

Hence, the quality of life indicators that are measured by the pharmaceutical industry are often not relevant to patients' needs, which is evidence of a misalignment between the interest of industry and those of patients. Quality of life is subjective and depends on the patient's health

status and priorities. In the case of mucopolysaccharidoses (MPS), one patient advocate voiced her concern about the limitations of the 6MWT to measure clinical effectiveness in MPS patients: “There is no measure for quality of life in terms of increased independence of patients or relief of stress on caregivers or the therapy’s growing expense as children transition to adulthood” (Participant #7). Another patient advocate expressed this disjuncture in health outcomes: “As a result of the 6MWT, if there is a 50 % improvement, you can now walk 50 feet in 6 minutes as opposed to 30 feet, what a fucking difference! Patients say that is negligible, while some clinicians are saying this is clinically significant” (Participant #1). These realities in the area of rare diseases often produce uncertainty in terms of the evidence available to assess a drug’s benefit and risk profile. In general, rare disease groups recognize the shortfalls of CADTH’s decision-making framework. Participants interviewed questioned and challenged these evidence models. Thus, patients’ ability to critique evidence-based decision-making supports the idea that the 21st century patient can contest dominant paradigms in science. But in the world of HTAs, the critiques of rare disease patients have not successfully brought resistance to the established system of evidence-based decision-making because the HTA system is built on a privileged perspective of expert knowledge. Although the dissertation has demonstrated that patient input in the Common Drug Review can be conceived as patients’ alternative understandings of science and disease, patient input cannot be understood as a resource used by rare disease patients to influence drug coverage decision-making. We have discussed previously the focus of science and technology scholars on the ability of patient groups to reshape the social configuration among lay individuals and experts in various health domains and change the locus of control over health. In addition, in the case of CADTH’s Patient Input Program, the patient input platform does not allow for patients’ structured influence in decision-making processes. As

demonstrated in the previous chapter, it does not allow for a breakdown in knowledge boundaries between experts and non-experts.

Although there is effort by CADTH to shift its thinking about science and rare diseases, progress is slow in this area. For example, CADTH's 2016 *Recommendation Framework for CADTH Common Drug Review and pan-Canadian Oncology Drug Review Programs: Guidance for CADTH's Drug Expert Committees* recognizes the uniqueness of rare diseases. The guideline defines patients' needs as significant unmet needs and discusses instances where there is uncertain clinical evidence due to practical challenges of conducting robust clinical trials. It explains that in exceptional cases, where there is uncertain clinical evidence, the committees may issue a recommendation to reimburse a drug with clinical criteria and/or conditions. In these situations, although there is uncertainty with the clinical evidence, the available evidence must reasonably suggest that the drug under review could substantially reduce morbidity and/or mortality associated with the disease. This philosophical change within CADTH has not however triggered procedural changes according to patient groups. Based on 2010-15 data, the Common Drug Review (CDR) positive recommendation rate for rare disease drugs remains poor compared for common diseases drugs (Rawson 2016). Undoubtedly, the rarity of the disease turns patients affected by a rare disease into medical outliers.

5.2.2 Is it Good Value-for-money?

Within the Common Drug Review process, CADTH's pharmacoeconomic experts who conduct cost evaluations also consider patients' needs. Their task is to analyze, interpret and evaluate the cost analysis provided by the drug manufacturer. Cost-effectiveness research

(CER)⁶⁴ is employed to support funding recommendations. That is to say, CER analyses cost considerations by looking at key methodological designs and limitations of clinical inputs such as, efficacy, harms, mortality and quality of life, as well as the anticipated costs of the drug treatment cost. Pharmacoeconomic experts summarize this synthesis of evidence in a Pharmacoeconomic Review Report. As part of CER methodologies, economic evaluations are performed following rigorous analytical methods, starting with a relevant and well-defined study question based on the overarching question: is it good value-for-money? The main purpose of an economic evaluation is to “identify, measure, value and compare the costs and consequences of alternatives being considered to inform value-for-money judgments about an intervention or program” (Contandriopoulos, Champagne and Avargues, 2000 in CADTH’s Guidelines for the economic evaluation of health technologies, 2006: 1). Health outcomes and more particularly quality-of-life outcomes are usually measured as life-years gained (LYGs) or quality-adjusted life-years (QALYs)⁶⁵. In general, manufacturers must specify the target population(s) for the intervention and its expected use, including alternatives that point to different treatment strategies. They must consider alternative drugs that are listed on a formulary, those that are not listed as well as standards of care drugs, which are treatments accepted by medical experts as a proper treatments for a certain type of disease that is widely used by healthcare professionals. CADTH refers to two Canadian guidelines for the economic evaluation of drugs: the *2006 Guidelines for the Economic Evaluation of Health Technologies: Canada* and the *2016 Guidance*

⁶⁴ CER is “the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care” (Dartmouth Medical School 2010).

⁶⁵ An economic analysis in which LYGs are used is often referred to as cost-effectiveness analysis (CEA) with its parameter of interest being called incremental cost-effectiveness ratio (ICER), whereas an analysis in which QALYs are used is often called cost-utility analysis (CUA) and the resulting parameter is called incremental cost-utility ratio (ICUR)” (Jakubiak-Lasocka and Jakubczyk, 2014).

Document for the Costing of Healthcare Resources in the Canadian Setting. These documents set the tone for the conduct of cost evaluations. These evaluations usually include the cost of the drug and any drug administration costs from the payer's perspective (e.g., direct costs of drugs, costs of physician visits and hospitalizations) and from the societal perspective (e.g., indirect costs of loss of work, leisure, and transportation). According to these guidelines, to form the evidence base about the efficacy and the effectiveness of the drug, experts should perform a systematic review of the available literature. Further, there should be attempts to incorporate "real world" factors such as patients' adherence to treatment, screening and diagnostic accuracy, and healthcare providers' compliance and skill to modify the effect of the intervention. Where feasible, data should be included on the impact of adverse events associated with the intervention if they are clinically or economically important. Adverse events are to be analyzed appropriately and linked to patients' adherence, mortality, morbidity and HRQL, among others. The changes in costs and benefits associated with a particular drug over time need to be considered in the analysis through discounting methods. In the Canadian context, the costs and health outcomes that occur beyond one year are discounted to present values at the (real) rate of 5% per year.

Costing evaluations are based on the natural course of the condition and the likely impacts that the intervention will have on it (referred to as time horizon). Some of the costing considerations include the characteristics of public drug plans, the variability of pharmacy markups, and the dispensing fees across jurisdictions. Consideration is also given to the assumed costs for comparators that are not currently marketed in Canada (based on foreign country prices) and the weighted average price of the drug in comparison to other drugs within the same disease area based on prescription utilization data. Co-payments charged by public and private drug

plans to the consumer, private formulary prices and hospital drug formulary lists figure in the analysis. The cost calculations are conducted in the context of a set health budget.

Finally, the results, which include the probability of disease progression and the impact of health interventions, must be validated statistically by good modelling practices. Decision trees and Markov models are frequently used in pharmacoeconomic evaluations (Ademi et al. 2013). Markov modelling refers to a Markov chain of sequenced events in which the probability of an event occurring is dependent on the preceding event. The chain is comprised of exclusive health states, time cycles and transition probabilities. A search of CADTH's website identified 96 funding recommendations that refer to manufacturers' use of a Markov model to estimate long term healthcare costs and QALYs; to present the progression of the disease severity over a patient's life-time; and, to trace the natural history of a disease. This model incorporates estimates of small, moderate, and large effects of a drug therapy. Generally, Markov models replicate what is known about the disease history. It shows where treatments might affect disease progression and identifies potential life expectancy gains based on assumptions concerning the drug's effectiveness. The model generates a hypothesis about the drug, the disease and patients' health outcomes (Coyle, University of Ottawa, 2014). Markov modelling has been essential to the formulation of funding recommendations and has been used in Ontario's Drugs for Rare Diseases Program since 2010 "to set criteria for access and benchmarks for evaluating outcomes" (Wong-Reiger 2015). Winqvist et al. (2014) argues that "such modeling requires credible information about the natural history of the candidate disease, and for "ultra-ultra rare" diseases, this may not be feasible and limits the scope of evaluation" (Winqvist et al. 2014: 779). Such modelling techniques may also discriminate children who frequently require long-term drug therapies and who require higher drug dosages as they transition to adulthood. As well, we

have seen through the social sciences literature (Avorn 1984; Neumann and Johannesson 1994; Garber and Phelps 1997) that the use of CER and QALYs has discriminating effects on elderly patients because of their shortened life span. In addition, these methods can misrepresent a patient community because treatment effectiveness varies among patients and because patients value health outcomes differently. Again, the standard methods of costing are not adapted to the complexities of pricing for rare disease drugs. Given the fact that drugs are ranked on cost effectiveness, CER evaluation methods measures “favor common drugs due to the lower average cost to the former” (Fellows and Hollis 2013: 2). These measures also appear to favour drugs for typical adult patients. An exploration of new costing models for rare disease drugs would be beneficial but it is not within the scope of this dissertation.

5.3 Concluding Remarks

The chapter explains the way the needs of rare disease patients are formulated into demands in the HTA system. The dissertation first delineated rare disease patients by highlighting the intricate nature of their disease and the struggles it produces for patients, described as medical outliers. By the very nature of their nomenclature, rare disease patients represent a unique disease constituency. This demarcation stems from the nature of their needs, which have been grounded in health inequities. I demonstrated that the personal struggles of rare disease patients seeped into public policy because of CORD’s efforts to politicize rare diseases. In this regard, this politicization of rare diseases is far from that of breast cancer, which led to the transformation of women’s identity from tragic victim to heroic survivor (Klawiter 2004), or AIDS, which led to changes in HIV research and policy. However, the politicization of rare diseases stimulated debate in Parliament, attracted media coverage, and gained the attention of

policy-makers and regulators. Issues pertaining to rare diseases have also become a topic of academic research (Rose and Novas 2004; Novas 2006; Huyard 2009; Rabeharisoa et al. 2014).

As the findings suggest, CORD united rare disease patients, and succeeded in messaging a patient voice focused on health and financial vulnerabilities. The politicization of their needs could not have been achieved without the infiltration of CORD in the realm of government and politics, coupled with the organization's ability to craft a rare disease discourse using the tools of technocracy. Furthermore, CORD was able to formulate patients' needs into political demands against elite needs interpretations, by making visible its membership and proposing tangible solutions to enhance the government's role and accountability towards a vulnerable population. By doing so and continuously working towards the satisfaction of patients' needs, CORD forged a new relationship among policy-makers and rare disease patients. The dissertation also contends that the reframing of patients' needs into an issue of treatment access can have the effect of deflecting attention from important political questions and policy options that pertain to the bigger problems of excessive drug pricing, over-restrictive clinical criteria to for drug coverage eligibility, significant out-of-pocket expenses, and treatments being sought outside Canada. As we have seen, even CORD advances a partial perspective of patients' needs. The organization redefined the needs of rare diseases as a problem of treatment access where patients are in need of drugs in order to survive. This way of defining the needs of patients neglects the claims of patients to an open and inclusive Common Drug Review process, issues that surfaced during interviews with patients and patient advocates. Patients want to be engaged in decision-making and want information to be made publicly available. This articulation of patients' needs further complicates the notions of access and equality that dominate current debates about the needs of rare disease patients.

I argue that drug coverage decision-making practices can be understood as a site of competing interpretations of patients' needs. Within this framework of analysis, patients' needs are measured in terms of patients' unmet needs and translated in pragmatic and probabilistic terms on notions of statistical significance, value-for-money and treatment access. The HTA network adopts a definition of unmet needs on a medical model.

Clinical 'need' can take many forms, but in a healthcare setting generally relates to some aspect of human functionality that is lacking or compromised (e.g. hand function, kidney function, mobility) and which requires assistance or a bodily function that is functioning normally but for choice or social reasons requires controlling (e.g. conception). An unmet clinical need is where the aid or control mechanism is unavailable or unsuitable for an individual or group of people.

For medical technology innovation, identifying unmet need serves several roles:

It is frequently the initiator and driver of innovation;

It can start by identifying a problem with an existing technology or service or can highlight a completely new requirement;

It demonstrates at the outset that there is likely to be a demand for the new technology and, thus, future adoption of the technology by users is more likely, although by no means guaranteed; and

As an unmet need can be experienced by a single individual or a large group of people, it is important to determine the size and impact of meeting the need at the outset (McCarthy et al. 2015: 382).

This clinical interpretation of unmet needs, in combination with rationing and evidence-based models, may have the effect of disadvantaging rare disease patients, and rationalizing inequity on the limitations of clinical evidence and unjustified high drug costs. The findings therefore show that needs' discourses echo the interests of experts within their communities of practice. In fact, clinical experts prioritize the synthesis of evidence through evidence-based

medicine (EBM), the randomization of patients in clinical trials and the statistical significance of study results, whereas experts in pharmacoeconomics prioritize the use of cost-effective research (CER) as a value-for-money approach in funding recommendations and refer to effectiveness and economic evaluations. Both communities of practice dispute the legitimacy of patients' needs. In the case of rare diseases, the interpretations of patients' needs within the boundaries of EBM and CER only form a partial view of the healthcare landscape and have the effect of perpetuating health inequities and injustice, which are at the very heart of the rare disease discourse. In this way, through the lens of the Patient Input Program, the dissertation concludes that the needs of rare disease patients are framed implicitly in the CDR process as outlier cases and they are contested explicitly among experts as significant unmet needs.

As Fraser (1989) has pointed out, it is important to look at the consequences of rival needs interpretations. "In general, consequentialist considerations dictate that, all other things being equal, the best need interpretations are those that do not disadvantage some groups of people vis-à-vis others...and that most closely approximate ideals of democracy, equality, and fairness"⁶⁶. Beyond the general claims of medical needs, patients themselves want health equity, openness and inclusiveness in order to contribute to HTA evidence and HTA decision-making. It is only when we dig deeper into the struggles over needs interpretations that their controversial nature is rendered.

The struggles over the interpretations of needs, rights, demands, over existing normative forms, are in continual process in all societies, but especially in modern societies in which changes have been taking place over a long period...; however, it is true that, on occasions, there are moments of provisional closure of the discussion, in which a previously disputed interpretation may become more or less hegemonic. When this happens, the other interpretations which are also in play, though marginalised and unable to attract a large enough

⁶⁶ <https://www.marxists.org/reference/subject/philosophy/works/us/fraser-needs.htm>

audience, can provoke a new situation in which the hegemony of the dominant interpretation is destabilised; in this case the ‘subordinated’ interpretations erupt once more into the public sphere and manage to get themselves debated (Interview with Nancy Fraser - May 16, 2009)⁶⁷.

In this regard, I contend that the HTA network advances a hegemonic medical interpretation of patients’ unmet needs that effectively trivializes the personal and social aspects of living with a rare disease, such as quality of life issues like patients’ autonomy and capacity to work, and patients’ mental and physical health (i.e., depression, weight gain, fatigue). For instance, in 2013 CADTH’s drug expert committee recommended that Soliris⁶⁸ not be listed on drug formularies, despite the feedback provided by patient groups on the personal and social consequences of their disease as reflected in CADTH’s recommendation. Patient groups emphasized that there is an unmet medical need for aHUS patients that could be met by providing access to Soliris. For instance, “patients with aHUS report high amounts of emotional, financial, and responsibility-related stress, leading to feelings of isolation, fear, hopelessness, anxiety, and depression”⁶⁹. As well, parents of aHUS patients estimated that due to current treatment, their “children miss 30% to 40% of their school year, with the parents’ absenteeism from work at 20% to 40%.”⁶⁹.

Lastly, the research findings point to the shortcomings of CADTH in meeting these needs due to the primacy of scientific norms and economic considerations. Although the issue of rare diseases gained recognition in public policy through COR’s strategic discourse on treatment access, in drug coverage decision-making practices, dominant needs interpretations appear to

⁶⁷ <https://mronline.org/2009/05/16/interview-with-nancy-fraser-justice-as-redistribution-recognition-and-representation/>

⁶⁸ As presented in the introduction of the thesis, Soliris is the drug that Robert needs to receive a second kidney transplant.

⁶⁹ <https://cadth.ca/sites/default/files/cdr/advice/cdr-advice-Soliris-aHUS-June-2-2015.pdf>

depoliticize the needs of rare disease patients and inadvertently push them in the private domain and outside the realm of public policy. Thus, the personal and social consequences of living with a rare disease when treatments are not available or effective are not within the scope of what HTA experts define as clinical needs. This dominant interpretation of patients' needs goes uncontested in drug coverage decision-making. Subsequently, I argue that the politicization of patients' needs in the rare disease community did not enhance their role in the Patient Input Program. The following chapter builds on the study findings in identifying potential obstacles to patients' influence in drug coverage decision-making.

CHAPTER 6

THE PATIENT INPUT PROGRAM: FROM POLICY GOAL TO PAPER-BASED ARTEFACT

Up to this point in the dissertation, the main findings of the dissertation indicate that the Patient Input Program did not facilitate meaningful patient involvement since patients do not participate directly in decision-making. This unequal leverage power is also reflected in the determination of their needs, which is dictated by a hegemonic medical perspective of disease solely on physical impairments, rather than the personal and social aspects that accompany the impairment. These findings bring me to my last research question, if patients have different influential status within the HTA network, and the nature of their claims is controversial (i.e., they can be contested or influenced by groups and institutions), how is patient feedback considered in CADTH's decision-making? To address this question, the chapter will seek to examine the Common Drug Review (CDR) as a multiplayer process and determine whether the Patient Input Program enhances or hampers the involvement of patients in drug coverage decision-making. To do so, the chapter will examine the translation process at work that enabled the incorporation of the patient involvement concept into HTA decision-making practices, and the relationship between the logics embedded within these practices and the design of the Patient Input Program. As demonstrated previously, drug coverage decision-making draws on two distinct logics: evidence-based medicine and cost-effectiveness research.

I conceptualize the Patient Input Program as a boundary object because it is at the intersection of boundary interactions among those who participate in drug coverage decision-making; it has been effective in promoting a patient input feedback mechanism, and presupposes a dialogue and reconciliation of interests among HTA stakeholders. The premise for this analysis

is that the Patient Input Program is constructed as a set of expectations and demands, and reflects a particular social configuration in the HTA network. Along the lines of Allen (2009), I demonstrate how the patient involvement concept became a workable boundary object when it was integrated in CADTH's institutional practices as the Patient Input Program. The dissertation contends that because of institutional biases, the Patient Input Program fails to meet the needs of all actors in the HTA network. On this point, the Program has become a positive boundary object for policy-makers but may have become a negative boundary object for certain HTA experts and patients alike. The conclusions were drawn from interviews, key documents employed by CADTH and information contained in the grey literature.

6.1 Diversity of Interests and Stakes

This section describes the stakes of the most important groups of actors involved in the Common Drug Review process and presents their role in promoting the patient involvement agenda. It builds on the descriptions presented in the introduction of this dissertation. To reiterate, the patient involvement concept was put forward as a health covenant by the Romanow Commission and as a transparency and openness measure by the House of Commons through the publication of the Common Drug Review (CDR) Report. Patient involvement is interpreted in the policy realm as a democratic principle to maximise participation in decision-making. Through political commitments and its political discourse, the Government of Canada set the tone for a culture of public involvement in federal departments. In other words, the government of Canada was a strong socio-political force that influenced the course of patient involvement. In fact, participants interviewed discussed the role of political institutions in advancing the patient input platform in health policy, particularly in funding recommendations. In this way, the provincial and federal legislatures diffused a discourse of public involvement that promises a

state of democratic participation in health issues. These political institutions encouraged “HTA agencies to adopt a public involvement scheme by creating a favorable political climate adopting public involvement policies and allocating the necessary resources to support HTA agencies to involve the public” (Gauvin et al. 2011: 48). More specifically, the public involvement discourse promotes equal opportunity for the public to provide input into the Common Drug Review process. I discuss the trickling effect of these policy goals and CADTH’s technocratic response to public involvement as a key thematic element of healthcare.

The Technocratic Response

In CADTH’s bureaucratic structure, patient involvement is interpreted as a technocratic process that serves to incorporate patient input into drug coverage decision-making⁷⁰, which is founded on efficiency and economic rationality. As we have discussed in a previous chapter, the Agency built knowledge in clinical research, health economics, information services, knowledge exchange, information technology, finance, and project management. Quite rapidly, CADTH morphed into a centre of authority thereby setting scientific requirements for the assessments of therapies in Canada’s healthcare system and providing expert policy advice to ensure best value-for-money in an evidence-based framework. CADTH is a pan-Canadian body tasked by the federal Department of Health to make drug coverage recommendations through its Common Drug Review process, and is funded by Canada’s federal government and P/T public drug plans (with the exception of Quebec). In fact, Gauvin et al. (2011) affirm that governments steer HTA agendas “to some extent through accountability links, funding arrangements, and policies” (Gauvin et al. 2011: 48). In this way, CADTH is accountable to Health Canada, the federal Department of Health, for the use of its federal funding. In turn, Health Canada reports

⁷⁰ https://www.cadth.ca/media/pdf/2012_SECOR_Patient-Input-Review_e.pdf

its departments' activities to Parliament through its Cabinet Minister. CADTH's activities are monitored by Health Canada through committees. This federal involvement provides the government with the ability to influence strategic decisions and ensures that CADTH's activities align with its corporate activities. Under this reporting structure and due to the government's mandate in responding to parliamentary studies relating to the Common Drug Review process and prescription drugs, it is reasonable to assume that CADTH's Patient Input Program was imposed via a top-down implementation approach. CADTH had no discretionary power to refuse an involvement program. This may explain why there is a discrepancy between the broad goals of engagement and the practical applications of patient involvement (as noted earlier).

The findings suggest that the creation of the Patient Input Program in 2010 resulted in part by the publication of the Standing Committee on Health's 2007 Common Drug Review Report, which put forward a recommendation for greater transparency, openness and public involvement in CADTH's Common Drug Review process. However, during interviews, CADTH staff also explained that prior to the implementation of the Patient Input Program in 2010 a culture of patient involvement had been adopted within CADTH, starting in 2002. Clinicians who were contracted by CADTH and who had experience in specific disease areas would bring the patient perspective to the drug expert committee for deliberations during the Common Drug Review process. This was not direct patient involvement. It was not ideal nor was it provided in a consistent and formal way. According to the participant, "CDEC members felt it was important to understand patients' needs" even though there was no official or formal process (Participant #24). This culture change within CADTH coincides with the introduction of public input as a proposed health covenant for Canadians in the 2002 Romanow Report. Because CADTH recognized the value of patients' view and perspectives following the

publication of the 2002 Romanow Report, and formally secured patient input in the Common Drug Review after the publication of the 2007 Common Drug Review Report, the study findings suggest that two key documents steered the work of CADTH and triggered a culture of openness and involvement in CADTH's Common Drug Review process. This trickling effect was also felt by the drug industry.

Changing Business Models

For its part, the pharmaceutical industry is the central actor in the Common Drug Review Process as drug coverage decisions are based primarily on drug costs and scientific information produced by drug companies. On this point, companies are required to demonstrate the overall value of treatments in order to secure reimbursement, rather than just meet the safety and efficacy criteria. In this regard,

Although manufacturers are traditionally focused on regulators' requirements, being the ones necessary to get their technologies licensed, they must nevertheless equally be attentive to payers' needs if they want to maximize the chances of their technologies being adopted in clinical practice (Drummond et al. 2013: S10).

Drug development is a lengthy process that involves bringing a therapeutic discovery from the laboratory to a promising disease treatment to the clinical trial setting and clinical practice. Clinical trials are done in phases, each having a different purpose. Phase I trials test an experimental drug on a small group of people to evaluate the drug's safety, determine an adequate dosage and identify side effects. In Phase II trials, the drug is given to a larger group of people (usually 100 or more) to determine if the drug works for a particular disease or condition. Phase III trials are the largest and comprise groups of people (usually 1,000 or more) to confirm the drug's effectiveness and to collect information that will allow the drug to be used safely on the market in real-world conditions. Once sufficient information from Phase I-III trials are

collected to support the drug's safety (will not harm), efficacy (works in the intended population) and quality (good chemistry and manufacturing data), the drug sponsor may choose to file a new drug submission with Health Canada to market their drug. It is not uncommon for sponsors of industry-sponsored trials (i.e., pharmaceutical companies) to target most profitable markets to recoup the investments made in research and development. In the end, the drug is granted approval through issuance of a Notice of Compliance and Drug Identification Number, if Health Canada scientists conclude that the drug's benefits outweigh the risks.

For the pharmaceutical industry, drug approvals and drug reimbursement guarantees market sales and ideally, a margin of profit once the drug is available for use by prescribers. Drug sponsors are not required by regulations to assess an array of treatment options to determine what works best for specific patients in clinical situations. Their focus is on the benefit/risk profile of the drug proposed for marketing. Evidence-based medicine (EBM) and cost-effectiveness research (CER) are therefore not at the forefront of regulatory science. Because drugs have no exposure outside the clinical setting, it becomes difficult for the drug sponsors to quantify and anticipate the direct costs of the drug (e.g., cost of the drug, costs of physician visits and hospitalization) and indirect costs of the drug (e.g., loss of work, leisure and transportation) from the payer's perspective (Lothgren and Ratcliffe, 2004). As part of the Common Drug Review process, drug companies are expected to evaluate pharmacoeconomics throughout their product's life-cycle, which include real-world economic data and patient-reported outcomes (i.e., collecting data on a patient's account of the impact of a therapy on his/her quality of life or health state). Drug companies are required to factor the everyday use of a technology, meaning that clinical trials must be designed differently with a focus on a broader population base (Lothgren and Ratcliffe 2004). They must satisfy the evidence requirements set

by CADTH to complement their traditional clinical data. In turn, it can be argued that drug companies participate in the HTA process and are shaped by it. While using standards of scientific evidence to develop drugs and setting drug prices for consideration by the drug expert committee (CDEC), the pharmaceutical industry is forced to adapt to new trends in data gathering methods in terms of seeking patients' preferences and views. These expectations have been outside the boundaries of regulatory science but are ingrained in evidence-based policy circles in an effort to correct the misalignments between patients' needs and the drugs being developed. For the Executive Director of CADTH,

The Agency is trying to address a problem downstream when nothing has been done upstream in the regulatory world. When our reviewers look at what patients are asking in terms of medical benefits and outcomes, those outcomes are not even addressed in the pivotal clinical trials. There is a clear mismatch between how patients define clinical benefits and how regulators define clinical benefits or accept clinical benefits from industry. There is a clear misalignment and we (CADTH) are asked to solve that problem, but we cannot solve a problem downstream. We cannot change the information in front of us. (Participant #27)

The incorporation of patients' preferences and views has the effect of redefining drug development and the benchmarks for evidence in clinical trials. It also produces particular values, ideas, and beliefs about resource allocations. As a result of these new evidence markers in the world of HTAs, drug companies have no choice but to comply with CADTH's set expectations in order to gain end-users (i.e., prescribers and patients) and profit from drug sales. Within their business logics, it can be argued that drug companies interpret patient involvement as a mechanism to support drug use and drug coverage. On the flip side, because industry has a vested financial stake in decision outcomes, there is a general perception that industry may hijack any patient involvement mechanism to benefit their interests.

There is a risk that channels created for public input can be monopolized by the drug industry, which has the most concentrated economic interests in policy outcomes and strong incentives to wield what influence it can (Paris and Docteur 2006: 68).

The Big Data Revolution

Patient involvement is interpreted by patient communities as a means to achieve claims for health equity, openness and inclusiveness. The Patient Input Program can be understood as a new form of unconventional data, referred to as real-world evidence or big data. In other words, patient groups contribute to real-world evidence through the Patient Input Program because patient input submissions are gathered about the real-world impacts of drugs on patients' lives. The term "real-world data (RWD)" is now circulating in the drug world. It is defined as

an umbrella term for different types of data that are not collected in conventional randomised controlled trials. RWD comes from various sources and includes patient data, data from clinicians, hospital data, data from payers and social data. Through its use alongside traditional data sources such as clinical trials, RWD has the potential to provide new insights into medicines and their effects in the context of different patient groups (Miani et al. 2014, iii).

As a body of evidence, RWD encompasses patient input and is outside the boundaries of traditional scientific methods. As a result, real-world data struggles for legitimacy in science and policy circles. To illustrate this point, the Canadian Cancer Survivor Network commented that if it had to summarize the unofficial theme of CADTH's 2016 Symposium entitled, "Better Evidence for Everyone", it would be "Skepticism about the extent to which Patient Input counts as Evidence"⁷¹. This is consistent with results from the Program review conducted by SECOR in 2012:

Patient input is not given the same weighting as clinical and cost-effectiveness data due to perceived lack of objectivity of the data sensed by CEDAC members and CDR reviewers

⁷¹ <http://survivornet.ca/en/about>

Most CDR reviewers and drug expert committee members feel the information collected is relevant for CDR; however, not all are comfortable with using information for decision-making due to perceived conflicts/lack of objectivity/lack of representation⁷².

Indeed, real-world evidence has not been incorporated into the Canadian regulatory system. Other than patient input submissions, which are only a small sub-set of real-world evidence, the drug expert committee's systematic reviews do not consider unconventional data in their funding recommendations. One public member of the drug expert committee explained the practical challenges associated to real-world evidence. For instance, one drug (for a rare syndrome) had volumes of evidence on its safety and effectiveness. The evidence was tracked for a period of 10 years in an international database. For the committee, this situation raised questions about the variability of standards worldwide to enter information in the database and to report side effects; the quality of life indicators and measurements tools; and the lack of critical appraisal of the information. The participant felt strongly that the use of real-world data should be incorporated in systematic reviews even if it does not meet the scientific rigor of traditional raw data as this evidence is often lacking in the data generated by pharmaceutical companies. However, as per the participant, other than patient input submissions, CDEC is obliged to use publicly available information that is scientifically valid in its systematic review. "If the studies are not being done by pharmaceutical companies or if the study is not conducted in a proper form, it cannot be used in CDEC's systematic review" (Participant #28). Here, the public member stressed the methodological limitations of real-world evidence in the context of scientific work. A patient advocate explained the resistance from CADTH to accept unconventional data and questioned the weight of patient input in their decision-making:

⁷² https://www.cadth.ca/media/pdf/2012_SECOR_Patient-Input-Review_e.pdf

When you look at the decision that is on the CADTH website, it speaks to things like the data doesn't tell us certain things such as pain. Yet there is data on those things that came through the patient input submission that have clearly not been given significant weight. It speaks to the issues that we have around the integration of patient reported outcomes, not just into the Common Drug Review process and the way the drug expert committee (CDEC) reviews proposals, but integrating reported patient outcomes right into the design of trials. I think this is especially true in rare diseases cause we know the patients will never be there to provide the certainty the folks at CDEC seem to want. This is a good reason to ensure that they are collecting some kind of data on the things that matter to patients. (Participant #7)

The debate about the credibility of patients' perspective is apparent in CADTH's 2016 Symposium. First, definitions of patient input are bound to associated terms variously expressed as: anecdotal, other evidence, insights, patients' narratives, patient stories, experimental evidence and subjective input. Second, there is a push from academics to make the nature of patient input more scientific (Bridges and Jones 2007; Facey et al. 2010) by using rigorous methods to gather evidence about the social and psychological aspects of living with an illness or using a technology. Methods such as systematic reviews are perceived as tools that help ensure patient perspectives are reliable, verifiable and objective. Bridges and Jones (2007) suggest other measurement instruments such as discrete choice modelling, willingness-to-pay methods, budget allocation games and search protocols. As per Facey et al. (2010), "Exploring patient issues in an HTA requires the researcher to recognize that knowledge always arises from certain methodological, theoretical and analytical positions" (Facey et al. 2010: 337). Fundamentally, the general perception is that patient input is biased and of poor quality.

To summarize, patient groups contribute to the Patient Input Program by providing disease experiences, which constitute a new form of unconventional data, that the dissertation

suggest is a form of real-world evidence. Given CADTH's struggles with real-world evidence, patient input faces challenges within the Common Drug Review process since it is not recognized as valuable and legitimate empirical data. This brings us to the conclusion that patient input ranks differently in the evidence hierarchy. To remedy this deficiency of subjective evidence, CADTH hired a qualitative researcher in 2015 to develop a rigorous methodology for the synthesis of qualitative data. CADTH is now including a systematic review of patient preferences and experiences into assessments of medical devices, procedures and programs. This change has not yet occurred within the Common Drug Review process.

Fair Procedural practice

Public representatives interpret patient involvement as a fair procedural practice. Because health expenditures concern taxpayers, public representation is an important facet of the CDR process and most notably, CDEC's deliberations. Although CADTH seeks feedback from patient groups on their disease experience to understand gaps in disease knowledge and gaps in drug-related information, the Agency established a process in which two public members appointed to CDEC represent patients' perspectives. These members have a dual role: they represent both the public as taxpayers and patients as a collective. Participants interviewed discussed the role of public members in reimbursement recommendations. Each member is assigned different drugs and does a critical reading of the Pharmacoeconomic Report, the Clinical Review Report and patient input submissions. For example, members can question the clinical or economic models used or limitations of the models in question. There is an open dialogue between public members, technical experts and CADTH staff. Questions and clarifications can be discussed among them prior to CDEC's deliberations. More specifically, public members read the patient input submissions and compare it to the patient input summary,

which is drafted by CADTH staff and appended to the Clinical Review Report. Public members review in more detail the submissions and can make changes to the summary if it is not a true reflection of the input that was submitted by the group or groups. Recently, CADTH introduced process improvements to the Patient Input Program. CADTH staff now verifies the accuracy of patient summaries with patient groups. Once the totality of information is reviewed, public members create their own Report focussing on what is important to the patient, the unmet need as interpreted by patient groups, patients' expectations of the new therapy and their experience with the therapy under review (which may exist or not). This analysis involves comparing to the clinical evidence. According to one public member of the drug expert committee, what matters to patients is not always measured in the clinical studies conducted by drug manufacturers. Sometimes, there is no good evidence to support the patients' needs.

One of my roles is to make sure that the patients' needs remain in sight through the discussion of the drug. So I am presenting the patient group material, what is important to them, why it is important. I am also doing some analysis of the patient input submissions and linking and making comments, examining the relationship between their needs and the evidence that I can see. So for example, this is a difficulty we all face, patient groups will sometimes report universal or near universal enthusiasm about the people they have spoken with about a new therapy they have tried. (Participant #28)

In addition to representing the patient voice, processes were created to structure drug expert committee meetings (CDEC) around patient input submissions. To this end, one participant explained that during committee procedures, the public member is the first to speak. Patient input is the first discussion point, followed by the clinical trial results and pharmacoeconomics. Lastly, members vote to support (or not) formulary listing at the recommended price. At these meetings, public members ensure that patients' needs remain in

focus while examining the relationship between patients' needs and the evidence presented. The public member also represents the interests of taxpayers who want responsible allocation of public funds. Within this analysis, "the impact of the drug's price is assessed against the total health budget" (Participant #28). Health expenditures have a significant impact on drug plans and taxpayers. For this reason, the public's view on drug coverage is torn between patients' needs and responsible and equitable health spending.

The dissertation described the groups of actors at play within the HTA network; each group of individuals having its own set of conventions, language and practices, and each group of individuals having legitimacy and authority in a field of expertise (Star et al., 1998), except for patient groups whose legitimacy is contested with respect to the value of their input. In fact, the HTA network has within its realm diverging interests. The Government of Canada is promoting a sense of citizenship. CADTH and the P/T governments are trying to manage health budgets and be accountable to taxpayers, whereas pharmaceutical companies are striving to maximize profits on their research investments. Lastly, patient groups are making efforts to gain legitimacy for their input into the Common Drug Review process, while public representatives on CADTH's drug expert committee are both defending the public good and justifying patients' needs.

While each stakeholder has a specific task in the Patient Input Program, they also have very different ideas of what patient involvement entails, depending on their stakes in the process. In the next section, I explain how the patient involvement concept materialized as a social practice in spite of the diverging interests and stakes of the participants.

6.2 From Concept to Practice

From the outset, the Patient Input Program appears to be logical, seamless and socially just, but the research findings suggest otherwise. Furthermore, there is no common understanding of patient involvement (or patient input), what it serves and what it captures. For one patient advocate,

It seems to me there is broad support for the concept of patient input and it has certainly become a buzz word that you can find just about anywhere - governments, government-funded organizations and institutions (e.g. CADTH, CIHR, hospitals), industry, industry associations, professional organizations (e.g. CMA), health charities and patient advocacy groups, etc. These groups may have vastly different ideas about what patient input actually is, how to go about it, as well as different ideas about when successful and meaningful patient input has been achieved.

In terms of HTA specifically, I've noticed a change in recent years. The addition of patient input to the CDR review process is one of the most notable changes. (Participant #10)

As the above-noted citation explains, patient involvement has become acceptable in principle to a wide range of stakeholders. As per Star and Griesemer (1989), a boundary object has the “power to speak to different communities of practice” (Star and Griesemer 1989: 412-13). The appeal of patient involvement is its ability to respond to the interests of many stakeholders in the HTA network. On this point, the incorporation of patients’ preferences and views in biomedical sciences and health policy has become a rule of thumb and essential component of healthcare. Patient involvement however, remains vague to this day. The term “patient involvement” is obscure in the literature; the patient involvement narrative is blurred; and, little is known about the way patient involvement is practiced. In this way, using the boundary object concept is relevant to explore objects of analysis that are unstructured, abstract and ambiguous (Star and Griesemer 1989).

A boundary concept is a loose concept, which has a strong cohesive power. It is precisely because of their vagueness that they facilitate communication and cooperation between members of distinct groups without obliging members to give up the advantages of their respective social identities (Allen 2009: 355).

Even though the concept of patient involvement is abstract, “There are multiple players, groups/organizations as well as individuals who are involved in advocating for patient input and shared decision-making in healthcare as well as health research and policy-making” (Participant #10). As remarked in a previous chapter, the adoption of patient involvement was driven by a various social transformations and pressures that pertain to both the local and global spheres. These factors contributed to the broader changes that have occurred in societal expectations and values. But one thing remains, the meaning of involvement is obscure and subject to interpretation.

Although institutions and groups have different stakes in the HTA process, they all rally behind patient involvement and contribute to its achievement. I now turn to the collective process that is at work within the HTA decision-making process between patients, policy-makers and scientific experts. In this regard, patient input will be conceptualized as boundary object to show the weaving of different groups and stakes as it takes form and shape. This chapter builds on an earlier discussion that demonstrated ways CADTH was able to build itself as a centre of expertise in evidence-based decision-making by redrawing the boundaries of health policy-making on quantitative estimates of effectiveness and cost thresholds. The study shows how the Patient Input Program passes through these redefined boundaries, undergoes multiple translations, and becomes a product of social action.

Firstly, CADTH was able to apply the policy goal of public involvement from the political discourse into a Patient Input Program, which took the form of an institutional

mechanism. The government of Canada's public involvement agenda, which was based on democratic participation, was transposed into CADTH's processes as a way to hear the unmet needs of patients and respond to the call for openness and transparency in the Common Drug Review (CDR) process. The political climate fostered a change in CADTH's organizational culture. However, CADTH adopted this engagement platform while recognizing a different status for patients within the CDR process. The Patient Input Program was conceived on the notion that patients are a particular engagement audience, distinct from the public as taxpayers. This distinction between patient and citizen was not made within the Romanow Report and CDR Report. However, the idea of different publics was understood by CADTH. It could be argued that due to CADTH's proximity to clinicians, who are part of the CDR process and who have first-hand experience with patients, the Agency's understanding of public engagement was transformed. This understanding led to negotiating a place for the patient in the Common Drug Review process.

Secondly, to achieve the political mandate imposed by the government of Canada, CADTH developed a set of working language and processes to facilitate management of patient input. To this end, CADTH seeks patient's views and experiences on their unmet needs through its patient group submission template to understand the gap in disease knowledge and the gap in drug-related information. Patient groups are asked to make representative claims on behalf of their members. Patient groups must therefore comply with the standardized form of patient involvement and the set parameters. With the use of qualitative and quantitative methods for data collection such as polling members, interviewing patients, conducting focus groups, identifying unpublished data, patient groups must transpose the disease experiences of their members into a fillable form and submit within a strict timeframe. As per the template instructions,

In each of the following sections, guidance or examples are provided to help identify the type of information that CADTH, CDEC, and participating drug plans will find most helpful in understanding the needs and preferences of the majority of patients. Objective, experiential information that is representative of the majority of the patient group is preferred. There is no need for patient groups to submit published information, as CADTH's CDR review team and CDEC have access to current scientific literature through the manufacturer's submission and a rigorous, independent literature search. However, relevant unpublished studies may be submitted in addition to the completed template⁷³.

A total of 35 business days are provided for preparing and submitting patient input. Information is therefore collected from patient groups with the use of a questionnaire, which has a length limit of approximately 3, 500 words. The patient group submission must be in a ready-to-publish format and submitted electronically through CADTH's website. In this process of translation, CADTH transformed the policy goals of public involvement into an activity of data collection of patient stories. The dissertation has tagged this approach to public involvement as an information-gathering approach. Patient input is therefore transformed into a paper-based artefact.

A third translation process occurs when patient input submissions are collated together, analysed as a whole and summarized by CADTH staff. This paper-based artefact is transformed into a manipulable and quantifiable form. In this process, CADTH verifies information requirements and data collection methods. Once submissions are valid, summaries are prepared to inform CDEC's deliberations. The drug expert committee's public members review these summaries prior to the meetings. Public members compare the summary to the original patient group submission. One participant explained that if members require clarification from the patient groups, for example, on statistics or the number of patients interviewed or the reasons for

⁷³Template for Submitting Patient Group Input to CADTH's CDR, January 2014.

dissatisfaction with current therapies, their questions are filtered through CADTH. There is no direct contact between public members and patients. Again, once public members validate the summary of patient input submissions, they create their own report on the patient group input and focus on the unmet needs of patients in relation to the scientific evidence as presented in the Clinical Review Report, which is founded on evidence-based medicine (EBM). Based on a critical reading of patient group input, public members transpose their assessment into a balanced report of the submission's strengths and limitations for discussion with other committee drug experts. In this negotiation process, patient input is transformed, out of a sense of procedural fairness, whereby CADTH staff and public members corroborate the input that supports patients' views. To this end, the research findings suggest that CADTH employed its bureaucratic tools, language and loci of practice to implement a manipulable form of patient involvement that was familiar to bureaucrats. This manipulable and objectified way of presenting patient input may also have had the effect of easing the committee experts' perceived concerns that the information collected was not objective.

Finally, the fourth translation process involves patient groups who perceive the Patient Input Program as an opportunity to advocate for their health needs. They may optimistically participate in the process because they truly feel that their voice matters. As the following example illustrates, patient group communicate the humanity of the disease:

I talked a little bit about the strain on relationships and the neglect or perceived neglect of siblings. I have given much thought as to how to do justice to the constant pain, guilt, and impact of living with a child who has a catastrophic seizure disorder. I realized that I can't do it. No one outside of the people living this life 24 hours a day, 7 days a week, 365 days a year, could even begin to understand the complexities of their lives. So, I will stop trying to do the impossible. Instead, I will give a quote from the mother of a sixteen year old who I spoke about briefly.

My health has suffered because of the stress of watching my child almost die several times. There have been huge financial costs to the provincial health department because of emergency visits, etc. My workplace suffers because I have to take time away to care for my child. And after all of this, thank God that she is alive and able to smile and enjoy anything.

That's right, this Mother is grateful that her child can smile. She is grateful for every single seizure-free moment that she can have with this child. We know that Banzel, or no drug for that matter, will cure this child...this mother just wants the opportunity to have as many seizure-free moments as possible. (Participant #19)

As the findings demonstrate, multiple translations occur within the Patient Input Program. Each participating community of practice (or group of actors) seeks to satisfy the expectations set by CADTH, and follow the set work practices and organizational processes. By identifying the multiple translations within the Program (involving CADTH staff, CDEC members, patient groups and patient input processes) and outside the Program (under both the Liberal and Conservative governments), the findings show, from multiple viewpoints, how the patient involvement concept took form and was secured in the Common Drug Review process as the Patient Input Program. In this process of translations, each group of stakeholders has their task in the Common Drug Review, while maintaining their integrity within the HTA network. Drug expert committee members attend to science and economics, CADTH staff ensure that institutional processes are respected, and patient groups gather the needs and preferences of the majority of patients. In the next section, I discuss how the design of the Patient Input Program is marked with contradictions and tensions, which presents challenges for both those charged with inscribing this diversity of interests into the Patient Input Program and those who are targeted by involvement goals.

6.3 Work Management Through Methods of Standardization

The Health Technology Assessment (HTA) network comprises collaborating communities of practice in the field of health policy, medicine and economics, together forming a centre of scientific authority in drug coverage recommendations. The HTA reimbursement decision can be viewed as a record of reconciliation between the diverging interests of different communities of practice. As a boundary object, the Patient Input Program has multiple memberships: it involves the participation of diverse social groups with heterogeneous viewpoints, approaches and practices. In this context, the Patient Input Program only becomes possible through the participation and adherence of HTA stakeholders. By establishing information-gathering standards, document standards and processes that everyone supports and complies to, CADTH as the centre of scientific authority, has created a mesh through which drugs must pass to be considered for funding. Here, CADTH was able to establish a system in which diverse allies could participate concurrently in the work leading to drug coverage recommendations. The use of standardized methods of work (i.e., guidelines to manage data collection) and the use of standardized forms (i.e., submission templates) can be regarded as ways to ensure the integrity of the information collected and render the information analyzable and comparable. The Patient Input Program - its procedures, processes, guidelines, templates and tools - become “a common coin” in this new space of practice. In Star and Griesemer’s (1989) words:

By reaching agreements about methods, different participating worlds establish protocols, which go beyond mere trading across unjoined world boundaries. They begin to devise a common coin, which makes possible new kinds of joint endeavour. But the protocols are not simply the imposition of one world's vision on the rest; if they are, they are sure to fail. Rather, boundary objects act as anchors or bridges, however temporary (Star and Griesemer 1989: 414).

Here lies a contradiction. Although CADTH developed tools and methods through the Patient Input Program to develop a common working language among HTA stakeholders that makes it possible to incorporate patient input in drug coverage decision-making, CADTH also views patient input as a distinct form of knowledge that ranks differently in the evidence hierarchy. In spite of this epistemic difference, CADTH applies the same standardizing methods to patient input. By simplifying human suffering to a data collection model, it can be said that CADTH undermines the humanity of patients, as noted by one patient advocate: “I don't think we had an opportunity in the survey to relate the wealth or richness of the information of the families' experience because there isn't very much space to provide patient input. We couldn't do it justice”. (Participant #10)

Others echoed this loss of meaning:

I know the original person that worked on the first patient input submission had interviews with patients where they shared their stories and the impact of the disease on them but when you read the CADTH summary with the original input, I am thinking it's sounding way less dire than it really is. It's almost downplayed. (Participant #20)

Indeed, through the establishment of information-gathering standards (i.e., methods of data collection, types of information, presentation of data in a fillable form, and general summary template), CADTH “took away the humanity of it all. They lost the important parts and it lost its impact” (Participant #5). The Patient Input Program, for one patient advocate, feels like a “tick box approach” (Participant #19) (term employed by Tritter 2009). In Arnstein's (1969) words, this involvement approach can be regarded as an empty ritual of participation.

In order to manage a coordinated system of drug coverage recommendations that incorporates patient views, CADTH had to set up a process for the Patient Input Program so that diverse groups could contribute concurrently and fairly towards decision-making. Since 2010, the Patient Input Program has been allowing patients to provide their perspective on issues such as the severity of the disease and the nature of the unmet medical need. This input is collected in a structured and formal manner to inform the review of drugs for clinical and cost effectiveness. In practice, this means that patient group input is sought from patient groups via online questionnaires. The process requires that patient groups review the *Guide for Patient Group Input to the Common Drug Review*, which provides information about the type of input CADTH is requesting, how it will be incorporated into the Common Drug Review process, and how to process the patient input submission. Then patient groups must download the *Template for Submitting Patient Input to the CDR*, complete and submit the template, which cannot exceed 6 pages. Thus, patient input is standardized in fillable forms and patients' needs are simplified and analysed following requirements set by CADTH as the centre of authority. Patient groups must conform to these information-gathering standards to participate to the Common Drug Review process. For their part, clinical researchers and public representatives must conform to documentation standards to present their interpretation of patient input. These standards become obligatory passage points, which participants do not have the authority to displace.

As part of this process, each group engages in a negotiation process to facilitate a coherent and streamlined process. Every participant in the Common Drug Review process works toward a common goal – to ensure that resource allocation is socially just and follows a reasonable and democratic evidence-based approach. In this process, public representatives, policy-makers, drug expert committee members and patient groups contribute in direct and

indirect ways to evidence-based decision-making by negotiating, debating, and simplifying patients' needs in a standardized process in order to work towards a listing recommendation. From CADTH's perspective, the assumption is that standardizing the Patient Input Program ensures that the process and outputs are neutral. We have seen however with Bowker and Star (1996) the potential effects of standardization practices. That is, standardization can set conditions for work coordination, but it can also set the conditions for excluding certain readings of science and disease. Hence standardization practices are connected to power dynamics and effects of marginalisation. In the following section, I discuss some of these effects on patient groups.

6.4 Not All Are Made Equal

In this regard, CADTH's efforts to formalize, standardize and quantify patient input, have the effect of excluding certain evidence forms and leads to a specific social configuration in which patient groups face varying degrees of obstacles to their involvement. Moreover, the Patient Input Program accentuates the vulnerability of the rare disease community in terms of their social and material resources. The interview findings further provide insights on the struggles that emanate from the Patient Input Program.

6.4.1 The Numbers Game and the Art of Writing

In general, participants interviewed commented on the challenges associated with CADTH's Patient Input Program and the battle of living with a rare disease. Participants felt disadvantaged for various reasons. For instance, one patient advocate explained the difficulty of compiling patient experiences because of the rarity of the disease and the difficult or even unattainable standard of statistical significance in clinical trials. He also noted the logistical challenges of being a rare disease patient group because of the constant struggle for survival

given the low numbers in membership. Another participant provided an account of her foundation's experience with the Patient Input Program. She defined the experience as a challenging exercise due to their lack of experience with patient input submissions, the time requirements and time constraints associated with the submission process. She also commented on their limited human resources given the size of the foundation and its voluntary nature, and on the impossible task of reflecting the depth of the patient experience. Generally, she spoke of the general skepticism about patient groups' motivation in making patient input submissions, and the perceived conflicts of interests with industry (although this information is disclosed at the time of application). In short, the lack of social and material resources was a common theme in the interviews and was identified as major setbacks for groups in the Patient Input Program. In terms of gathering data and completing patient input submissions, the lack of resources of rare disease groups, expertise, manpower, experience, knowledge and training were key obstacles for patient groups in meeting CADTH's expectations with respect to patient input. The President of CORD explained that the Patient Input Program is complex, there is no clear process, no guidelines, the deadlines are unrealistic and the dialogue between patient groups and CADTH is poor.

Further, patient groups discussed the limitations of their organizational capacities. In the world of rare diseases, "we don't have the people power, don't have the expertise on the ground yet and we don't have the money to have institutional power" (Participant #7). These groups advocate on case-by-case basis and participate in various advocacy activities but the scope of their work is tied to their organizational capacities. The rarity of the disease is a crucial factor in a group's organizational capacities. One patient with Cushing's disease for which there is no

organized group, stated that she only knows two people around Canada who suffer from Adult Hormone Deficiency. To illustrate this challenge, one patient advocate commented that:

It's a numbers game. There is a huge difference between what we have, 107 members across Canada, and if you have 1,000 members. It is a different ball game. If you have 10, you can't even form a group or board. With 100, if we really work on it, we can keep 5 or 6 people committed enough to form a board. It's a struggle. People leave the board all the time for various reasons, one of which they die. They get sick, they get busy, and so it is a continuous struggle. If you have 1,000 patients, you would have no problem keeping a board. If you have 100,000 members, you are the Canadian Cancer Society. You have a huge office, a research arm, lobbyists and staff. For the patients, their interests are the same philosophically. For example, a woman with breast cancer is no different than one with GIST, but in one case there's an enormous organization to represent her, but on the other, you have a tiny organization. For other diseases, there is nothing at all. (Participant #4)

Another factor affecting the group's ability to provide their input is the health state of their members. Health can be a major barrier to participation. Yet this aspect of living with a disease is absent in the discourse on public involvement that promises equal opportunity for the public to provide input into the CDR process. The patient advocate explained:

There are plenty of cases where people are just too sick to make that input and there are cancers where the prognosis is so poor, they can't organize a group. I know someone with Lyomyosicarma, and she says there is no group for my disease, why? Nobody lives long enough to form a group. So you get this incredible bias effect. The patient input isn't really reflective of the problems, it's reflective of the ability of people to get together and do something about that. (Participant #4)

That said, in addition to patients' health state, the group's size is a determining factor in a group's ability to succeed in completing a patient input submission. One patient advocate explained that the bigger and smarter the group is, the more input they are providing to CADTH. The more grassroots and the closer the groups are to patients, the less input they are providing

because those people are out in the field and do not have the time or resources to complete a submission (Participant #19). Here, a select group of organizations are in positions of privilege (Martin 2008). That is, the more legitimate and representative a patient group is, the less grassroots participation is (Contandriopoulos 2004). On the other hand, based on CDEC's experience with patient input submissions, "large organizations can provide bland submissions because paid staff do the writing without actually talking to patients, whereas smaller groups, who are closer to patients, can give really good insights into the disease, unmet need, or experience with the drug (which are the three key issues of importance to CDEC)" (Participant #28). Although there is recognition that grassroots organizations are closer to the patient experience, there is no discussion about the nature of these groups. Often, these groups are not engaged in advocacy work but rather, they focus on the immediate needs of their membership in terms of their daily struggles. They assist patients and their families in the 'here and now' of their lives. The issue of drug coverage, although being a significant factor in a patient's ability to attain quality of life, competes with other organizational priorities.

Another factor that affects the group's ability to meet CADTH's expectations with respect to patient input is the level of maturity and sophistication of the group. Some groups explained the difficulty in meeting these expectations. "CADTH is really not getting what they want or what they need cause the expectations are too difficult to meet, they don't give us time, they don't give us the funding...of course the smaller the organization the more difficult it is to meet these expectations" (Participant #19). Others explained the process to gather information in terms of conducting a series of interviews with caregivers, circulating a survey among the membership and, in one case, posting a survey on their website to target members and non-members who may have come across the drug. According to a participant, the process is time

consuming. Another participant explained that with the drug Naglasim, the group decided to collaborate and submit one submission, together with a partner group out of Toronto. This approach was taken instead of doing all the leg work and placing burden on families with multiple requests for feedback, input and information. The group went “through the process of collecting data, reviewing data and putting one submission together as two organizations” (Participant #7).

A group’s level of maturity and sophistication can also hinder its ability to act as ambassador for their membership. For instance, one participant argued that the Patient Input Program creates a bias effect on individuals who are not as eloquent and compelling at expressing their needs. In this process,

The poster child will now be prioritized over people whose needs may be greater. Patient input is a little bit like the victims’ rights idea that before a criminal is sentenced, the victim should have a right to speak to the court and say how the crime affected them. I am very much of two minds about that process. On the one hand, it is important because people want to feel that they have a right to be heard. It is important for the victim, and the patient is in the position of the victim. They want to look at the drug makers and decision makers in the eye and say this is why it means a lot to me. On the other hand, the consequences of these practices both in the criminal case and the drug funding case, is that it comes down to how people can make the most eloquent presentation, write most beautifully, and speak most compellingly. (Participant #4)

For another patient advocate, patient groups “just need to master the art of writing submissions” (Participant #19). Another patient echoed this sentiment.

In fact, CADTH has a patient representative that attended the Toronto conference and that is when I learned that there is an art form to submitting information. I am thinking, why is there an art form to submitting information? There's a guideline to assist in submitting, but that is pretty complicated for the average Joe who knows squat. So now you're asking for an organized group (that is if there is one), to collect the data information from their patients, caregivers, than come up with something that has to fit with this guideline that CADTH

basically figures is acceptable. It's not the point of it. The point is to talk to or listen to a few people to understand their viewpoint. They seem to forget they are dealing with citizens out here. (Participant #5)

In addition to the onerous task of completing patient input submissions that follow standardized methods of data collection, patient groups mentioned that submission deadlines were problematic. The two-week deadline is difficult to meet when groups have no resources and little time to prepare the submissions. One patient advocate discussed the letter she received from CADTH in May 2015, thanking the association for their input and offering advice on how to improve future submissions. The letter suggested that the group: i) poll its members to confirm that no patients had experience with the drug in question and potentially, hold focus groups with patients; ii) name the therapies that patients are currently using and explore their use; and, iii) use direct quotations from individuals with uncontrolled seizures to powerfully communicate their experience. The participant felt that this feedback had very little use. Indeed, CADTH sets institutional expectations that create systemic barriers for patient groups. Besides, due to confidentiality issues and practical considerations, it is not reasonable for CADTH to expect patient groups to collect information on patients receiving the drug under review (Participant #19). The Patient Input Program requires groups to survey and collate patient input. However, “patients may be reluctant to divulge the details of their medical histories. They may be afraid to “rock the boat” since they are often in precarious positions with respect to access to treatment” (Participant #4). Another patient advocate commented that: “To access this information, a system would need to be in place to connect the group to known patients. This would be time-consuming and would entail relying on healthcare professionals and their staff” (Participant #19). Nevertheless, some groups have managed to pull resources quickly to meet CADTH’s expectations. A patient advocate with the Pulmonary Hypertension Association (PHA) explained

how she prepares patient input submissions with as much community feedback as possible. The process entails finding patients who have experience with the drug that will be reviewed by CDEC (either by taking the drug, participating in a clinical trial or accessing the drug through compassionate use), and by reaching out to patients via the group's website and social media. If these outreach methods fail, the participant contacts the 15 centres for PHA treatment across Canada and relies on the nurse coordinators to connect her to patients taking the drug, who might be interested in providing feedback.

On another note, one patient advocate stated that there were differences between the provincial and national processes in terms of timelines. B.C. has longer deadlines (2 months) and has a bi-weekly notification system via an electronic bulletin that provides advance notice of drugs being reviewed with their associated diseases (Participant #22).

One group in particular explained that it had to turn to the Canadian Organization for Rare Disorders (CORD) in putting their submission together.

It was the first submission I had ever done. There was initially a lot to understand. What is a patient input submission? What kind of information is required? CORD was very helpful because it had experience with many submissions and understood the CDR process extremely well. CORD was able to provide guidance and gave us some sample surveys that other groups had used for different drugs. It was helpful to know what types of questions to ask. Then we developed a detailed survey because there was lot of experience with this drug and because we wanted specific information around the types of impacts the drug was having. Even though it took probably at least 45 minutes, people did take the time to complete the survey and provide lots of information about their experience. (Participant #10)

Despite these challenges, organizations like the Canadian MPS Society are committed to the process in an effort to respond to patients' needs:

Even as imperfect as the system is right now, we will still do everything we can to ensure that that patient input is at the table even if it's not given the weight we wanted it to have and even if we have to scramble and overcome some capacity barriers to make it happen. That's what we are here for in terms of our responsibility to our members. Although it feels sometimes like you are spinning your wheels, you still go through the process. (Participant #7)

In summary, several participants provided evidence supporting the notion that patient groups have unequal status and resources, which inhibit their ability to satisfy CADTH's institutional expectations. Here, reflecting on Bowker and Star's (1996) theoretical framework, the dissertation argues that power reveals itself through the power dynamics within the HTA network: smaller-scale patient groups facing the most obstacles in their involvement in the Patient Input Program. Their voice is the least captured in the Patient Input Program. For instance, one patient expressed that CADTH made the process too complicated. "They made it on their level and seem to forget they are dealing with rare people and rare people with rare diseases. Looking on the outside, it looks like there is a great access to the system but I think they made it too complicated" (Participant #5). In an effort to follow through on its commitment to Patient Input Program and standardize the form in which patient input is submitted, CADTH widened the gap between the Agency and further marginalized vulnerable patients. Moreover, their use of technocratic language and complex processes served to delegitimize patients' disease experience.

6.4.2 The Patients' Right to be Heard: Fiction or Reality?

Another facet of the patient input problematic pertains to the representation of patients by patient groups. Due to the unique nature of rare diseases, the multiplication of genetic mutations, the diversity of medical conditions and patients' medical needs, it is becoming more difficult for patients to mobilize around a one-size-fits-all therapy. The advancement of personalized

medicine is evidence of this new reality in health. In this regard, one patient advocate stated: “Patient groups are not necessarily representative of their membership” (Participant #4). Another patient suffering from Mitochondrial disease⁷⁴ discussed the divergent interests of members within her patient group, which she has since left. She commented that the group’s board of directors shifted the group’s focus on children’s needs and this was problematic for adult patients. As per the participant, the needs of children differ from those of an adult patient, and the needs of a young adult differ from those of an elderly person who may be suffering from other conditions or who may be caring for a sick spouse. The patient spoke about the stress of dealing with her husband’s cancer, heart attacks and pulmonary embolism. “Tomorrow I will be 72 years old. My health is tinkering at the moment. My husband has been quite ill for the last couple of years. I have not followed my health like I should...just lots of stress and stress is the worst enemy for Mito” (Participant # 23). The participant’s commentary illustrates the potential conflict between the unique needs of patients and their group’s mission and objectives. In this regard, a few patient groups stated that they are now directing their efforts and resources to help children transition to adulthood. The child-to-adult transition was described as a new trend in clinical research that patient groups are now following. Because of this strategic outlook of patient groups, some participants felt that those who presumably represented their interests were not satisfying their health needs. As disclosed by other participants, patient groups prioritize the needs of their members, emphasising some while neglecting others. With this in mind, are patient groups best placed to represent diversity within their membership? This is an important question because CADTH seeks input primarily from patient groups rather than from individuals.

⁷⁴ Mitochondrial disease is genetic disease and broad catch-all term for related conditions such as cancer, heart disease, multiple sclerosis, Alzheimer and Parkinson’s disease, among others All these conditions are characterized by a failure of the mitochondria.

Since 2015, CADTH has begun accepting individual submissions only in cases where no patient group exists. However, diversity was presented as a key element in the Patient Input Program. During interviews, CADTH staff explained that the diversity of patients' needs should be reflected in the patient input submissions to provide an understanding of the different needs of patients within a disease community. According to the participant, CADTH recognizes that patients have unique disease experiences and needs. The Agency encourages patient groups to hear the range of experiences from their membership. For CADTH, the term "patients' perspectives", coined by a public member of the Canadian Drug Expert Committee (CDEC), accurately describes the multiple views in rare diseases. CADTH gave the example of a family living in a rural area as opposed to an urban city. "Their experience may be very different to that of a university educated family in the city. Both those experiences are important" (Participant # 25). CADTH therefore relies on the assumption that patient groups reflect the diversity of needs within their membership. By doing so, CADTH may be setting unrealistic expectations by which patient groups are doomed for failure. By only recognizing the representative claims made by patient groups who are assumed to act on behalf of patients' best interests, CADTH is contributing to marginalising certain individuals. The findings suggest that in fulfilling the goals of democracy and representativeness, the Patient Input Program cannot claim to convey or represent the diversity of voices and experiences.

Although CADTH accepts individual input submissions since 2015, participants interviewed were not aware of this change in process. However, CADTH explicitly states that only in specific cases individual input will be accepted:

Individual patient and caregiver input will not be accepted when one or more patient groups exist that represent the particular condition or tumour for which a drug under review is used. Where patient groups

do exist, individual patients and caregivers are encouraged to work directly with a patient group to have their input included in the group's submission⁷⁵.

Due to the diversity of patients' needs, the question becomes can patient groups legitimately represent their members? One patient advocate argued, "CADTH is not getting patient input. It is getting organization input" (Participant #19). In the current Patient Input Program, one patient advocate noted that patient input goes from the patient to the group and the group to CADTH in a nice, efficient bureaucratic line. "Patient, you talk to your group, group, you talk to CADTH. It is simple, organized and logical" (Participant #19). The participant felt that this process was completely illegitimate. He explained that patient groups are just a bunch of people who are incorporated. There is no law that says that if you are a Canadian with a disease, that the patient group is your MP, that they are responsible and accountable to the patient. He stated that:

Any patient has a right to say, "I don't like these people, they don't represent me." So what right do you have as a government via the Patient Input Program to say talk to these people? Even in the case of GIST, there are many forms of GIST. Some of those forms are not treatable with Gleevec. Currently, there are putting huge efforts to argue for Gleevec. One of our patients could say, that doesn't work for me. You should be working on something for me. You can't assume that everyone have the same interests. If you have to funnel everything via patients groups, you are building a kind of systemic discrimination. The more rare the disease, the more you are discriminated against. (Participant #4)

For instance, what percentage of patients does a patient group represent? The patient advocate explained that groups do not really know. "All we can do is guess based on

⁷⁵ <https://www.cadth.ca/news/expanding-the-cadth-drug-review-process>

epidemiologic studies in other countries how many patients there might be in Canada and compare to how many active members we have, but 95% of patients don't bother joining a support group for free" (Participant #4). According to the participant, patient groups cannot claim in any legitimate way that they are representative.

Furthermore, one patient advocate discussed the perceived role of CDEC's public members as representative of the patient view.

Although some public representatives on drug expert committee have had disease experiences or have had a family member with a condition, it is not the condition that is involved in the review. They may have experience being a patient but each condition can be so different. They will read the patient input in more depth probably because they are tasked with representing the patient's view to the committee and providing the highlights they feel are in the submission. They are the voice piece for the groups that make the submission and present what we include in the submissions, but don't exactly have direct experience with the disease. (Participant #10)

CORD's President explained that the challenge of representing the patient is in people's mindset.

When I speak to the public members who sit on the drug expert committee, they do see themselves representing the patient summary but they are responsible for the public's best interest. In Ontario, the patients that are sitting at the table are members of the committee and are here to make sure that the patient's interest is actually represented. This is something that should not be minimized. You got to have somebody there who is willing to be an advocate for the patient because you have someone who is an advocate for the economics and somebody there who is an advocate for the whole safety issue. We got to have someone there who can speak to the best interest of the patient. (Participant #14)

Even the role of patient representatives at the provincial level was debated. One respondent who was actively involved in the inception of Ontario's patient input program,

discussed Ontario's policy considerations in terms of defining the role of patient members on a technical committee such as Ontario's Committee to Evaluate Drugs (CED). She spoke of the challenge to address the diversity of patient disease experiences within CED's broad scope of work (i.e., review of all drug types), and the challenge to assess the ability of patient members to represent the perspective of patients with diseases that are unfamiliar to them. The participant gave the example of Hepatitis C and MS patients versus patients with an inherited metabolic disease or breast cancer. Notwithstanding this difficulty, CED ensures that the patient perspective is brought to the table, whereas CADTH ensures that the listing recommendations have an informed public view. This was seen as a fundamental and philosophical difference within the federal and provincial programs. Throughout my interview with one member of CADTH's pan-Canadian Oncology Drug Review (pCODR), it became apparent that patient input was much more defined in the context of oncology drugs. Patient input was seen as a continuum of patient engagement. In the words of the participant: "This continuum of engagement involves communicating, listening, consulting and engaging patients as partners" (Participant #26). This participant distinguished the engagement of patients from a feedback mechanism in which patients are simply providing data. In Ontario, patients are active participants in an interactive relationship with the HTA body. When patients are active participants, their input is not only sought on the funding review but also on the draft recommendations. The participant described this engagement approach as an approach based on symmetry of information, meaning that all stakeholders (i.e., manufacturers, provinces and patient group) receive the same information at the same time with one response timeframe.

In short, in lieu of having patients sit on CADTH's drug expert committee, the Agency designed the Patient Input Program around the objective eye of the public member and

community leaders. On the one hand, it is not clear if the public member is the patient's representative, mediator or spokesperson because public members represent both the interests of taxpayers and patients. On the other hand, it is not clear if community leaders (i.e., patient group representatives) are best placed to communicate the diversity of needs within their membership. As Martin (2008) reminds us, those who speak on behalf of the silent must "know and can make knowable their constituencies" (Martin 2008: 49). Given the material and human resources needed to successfully prepare a patient input submission, and the timelines that need to be satisfied, community leaders may not be able to effectively convey the unique needs of rare disease patients. CADTH's criteria concerning group input may have the consequence of leaving rare disease patients at the margins of social participation. The findings suggest that the Patient Input Program may actually be further marginalizing vulnerable patients, especially in the case of rare diseases.

6.5 Concluding Remarks

Scholarship on boundary objects has revealed the generative power of artefacts in the organization and performance of medical work (Berg 1997) and the mapping of professional boundaries between nurses and physicians (Mackintosh and Sandall 2010). This scholarship also demonstrated that boundary objects can become roadblocks to innovation when the stakes of the different communities of practice are not represented (Carlile 2002), or they can become inhibitors when the value of artefacts is not recognized by certain intended users (Allen 2014). Interestingly, most scholars explain how boundary objects are effective or not effective in connecting different social worlds in a collective undertaking, which can lead to new knowledge, new practices or new relationships among stakeholders. On this point, their studies demonstrate that boundary objects are not always easily transferred from one context to another: "it is not

sufficient to introduce a boundary object and then wait for it to do its magic. The local process of making, introducing and using” them is crucial for making them work as boundary objects, and continuous work is needed to sustain them (Håland et al. 2015: 21). As previously discussed, in her study of integrated care pathways (ICPs), Allen draws attention to the passage of boundary concepts to boundary objects-in-use and the challenges it presents for implementers and beneficiaries. In a similar fashion, the chapter explored the trajectory of the patient involvement concept to its adoption as the Patient Input Program. The findings demonstrated that in the absence of clear political direction with respect to the form patient involvement should take, the Patient Input Program was designed as a paper-based artifact, creating implementation and collaboration challenges for both HTA experts and patients. Though CADTH facilitated the shaping of patient group feedback into a working language that HTA experts could interpret against principles of evidence-based policy-making, there is no evidence to date suggesting that patient input is listened to and acted upon within drug coverage decision-making. As Marston and Watts (2003) caution, evidence-based policy can have the effect of devaluating certain knowledge forms. “There is a risk that ‘evidence-based policy’ will become a means for policy elites to increase their strategic control over what constitutes knowledge about social problems in a way that devalues tacit forms of knowledge, practice based wisdom, professional judgment, and the voices of ordinary citizens” (Marston and Watts 2003: 158).

As remarked earlier, despite the imprecise nature of the patient involvement concept, it can draw multiple stakeholders around one main goal: that is to ensure that resource allocation is socially just and follows a reasonable and democratic evidence-based approach. Regardless of their common goal, institutions and groups who are contributing to the Common Drug Review process perceive patient involvement differently on principles of democracy, technocracy,

economics, health equity and fairness. Yet the Patient Input Program appears to reconcile the interests of all stakeholders within the HTA network. These interests include promoting civic engagement (government), ensuring responsible health spending (CADTH and P/T governments), gaining market access while recouping profit margins (pharmaceutical companies), legitimizing health claims (patient groups), and defending public and patient interests (public representatives). For Allen (2014), boundary concepts can give the illusion that a consensus among divergent stakeholders is possible. She argues that: “this apparent consensus disguises conflicting agendas”, which must be satisfied if the boundary object is to meet the needs of all stakeholders (Allen 2014: 810). In this way, the chapter identified four sets of translations, from which the patient involvement concept was transformed from concept to practice, that is from policy goal to an institutional mechanism, from an institutional mechanism to a paper-based artefact, and finally, from a paper-based artefact to a manipulable and quantifiable form. These translation processes led to a discrepancy between the policy goals of involvement and the form of the Patient Input Program, which ultimately creates institutional biases for marginalized patients. In other words, the Patient Input Program is effective in promoting a patient input feedback mechanism; its design however does not meet the needs of certain stakeholders. “...when a single representation is used as the basis for visualising activity to quite different ends, this always introduces problems for one perspective or another” (Suchman, 1995 in Allen 2009: 360).

The findings have shown that some HTA experts are not all comfortable with incorporating patient input into their decision-making due to certain assumptions with respect to the legitimacy and credibility of the information. For their part, patients have discussed certain institutional barriers to their involvement in the Patient Input Program. The research findings

demonstrate that CADTH's use of standardization methods, processes and representativeness to capture patient input has the effect of delegitimizing patients' disease experiences and further marginalizing vulnerable individuals and groups. To this end, the Patient Input Program functions as a positive boundary object for governments and policy-makers in terms of accountability and social responsiveness, but may function as a negative boundary object for HTA experts and patient groups. As shown by Levina and Vaast (2005), to become an established practice in a real-time setting, "agents must use and make sense of them (artefacts) in the context of each field" (Levina and Vaast 2005: 354). It is conceivable that HTA experts value the role of conventional and scientific evidence and are not equipped with the right skill set to assess patient input. For rare disease patients, the Patient Input Program represents hope, but due to the rarity of their disease, their health state and the variable size and sophistication of their patient group, patients often fail to meet the agency's institutional expectations with respect to data collection. To this end, the dissertation argues that the Patient Input Program, by its constitutive nature, upholds the unequal status, power and resources of rare disease patients and is not effective in fostering a dialogue among HTA stakeholders and reconciling the interests of HTA experts and patients.

In line with Mackintosh and Sandall's (2010) study of standard communication protocols in hospitals, the study findings suggest that patient involvement practices may have unintended consequences, such that "the standardization and rationality underpinning this protocol reinforces hierarchies of knowledge, prioritising diagnosis and pathology at the expense of that which is characteristically nebulous, unformulisable and individualised" (Mackintosh and Sandall 2010: 1685). Similarly, the logics underpinning HTA decisions reinforce knowledge hierarchies, a reading of disease solely on physical impairments, and aggregated data, at the

expense of patients' raw experience and individual suffering. In fact, the epistemological bases of drug coverage decision-making are founded on the principles of objectivity, which contradict the diverse and experiential nature of patients' needs. In Jasanoff's (2011) words,

Objectivity...partakes of the neutrality and impartiality of science itself, and demonstrations of objectivity can insulate the claimant against charges of arbitrariness or self-interest. In the skeptical modern world, objectivity is therefore a priceless adjunct to government power (Jasanoff 2011: 2).

In this way, CADTH seeks to objectify patient input by requiring conflict of interest declarations, encouraging patient groups to utilize quantitative and qualitative data gathering methods, and requiring that input reflect the interests of the majority. Patient groups are asked to describe the methods of data collection and to generalize patients' disease experiences. Experiential knowledge is framed in terms of objective data.

The chapter has argued that the patient involvement concept has received broad support in various health domains and appears to foster a new relationship and compromise among HTA stakeholders. Yet at the local level, certain players contest the usefulness of the Patient Input Program since it creates challenges in terms of understanding the unconventional form of evidence provided by patients, and it reduces the expression of patienthood to an indirect paper exercise. Although the Patient Input Program is an appealing mechanism of social participation, and reflects the new imperative of evidence-based policy-making, it also hides certain inherent tensions and "disguises a fuzzy periphery characterized by conflicting agenda" (Allen 2009: 360). The dissertation argues that patient involvement marks differences in knowledge forms between experts and lay individuals and emphasises the legitimacy of scientific knowledge on principles of evidence-based medicine (EBM) and cost-effectiveness research (CER). EBM and CER go unquestioned and infer higher claims of methodological robustness and validity.

Although patient involvement, as an end product, does not encourage a collaborative relationship between the Agency and patients, its technocratic undertones and constraints did not stir debate or controversy in the public policy forum. Moreover, in the HTA decision-making network, the patient involvement audience is over-shadowed by those with strong interests in maintaining HTAs.

I suggest that the Patient Input Program could be considered a boundary object, structuring a specific social configuration among HTA stakeholders, particularly between HTA experts and patients. I contend that the Patient Input Program does not channel effectively the demands of marginalized patients because it was adopted within standardizing practices and evidence hierarchies. In this regard, the dissertation offers insights on the lower ranking of patient input in the broader scope of scientific knowledge and notably, against higher claims of EBM and CER that go unchallenged. As well, the dissertation suggests that standardization in the Patient Input Program ensures the stability of the HTA system and the marginalisation of patients' understanding of science and disease. By constructing the Patient Input Program as a data collection mode, patients have suggested that CADTH undermines the humanity of patients. The findings therefore highlight that the Patient Input Program evokes the struggles of rare disease groups as medical outliers in the HTA system; a healthcare system driven by a rationing agenda. To this end, CADTH sets institutional expectations through its bureaucratic language, loci of practice and organizational processes, which in turn create systemic barriers for patient groups in the field of rare diseases. Further, the Patient Input Program reflects more broadly the efforts of patient groups in gaining legitimacy for their claims and overcoming bureaucratic hurdles. CADTH's use of standardization methods, processes and representativeness to capture patient input has the effect of making the Patient Input Program less grassroots and more elitist.

In this regard, the power dynamics within the HTA network become known. The Patient Input Program design creates obstacles that hamper meaningful patient participation in evidenced-based policy decision-making, particularly for smaller-scale groups, patients that suffer from diseases with less political status, and patients with poor health states.

CHAPTER 7

CONCLUSION

The study aims to understand the social dimensions of the Patient Input Program and the set of interactions and expectations therein. The dissertation is interested in the local application of the patient involvement concept into the Patient Input Program, and its capacity to bind different knowledge forms and join different perspectives. The main research question addressed in this dissertation is: *What is the role of knowledge and disease experience in the Patient Input Program and how do they influence drug coverage decisions?* In order to answer this question, I formulated three sub-questions:

- I. How was the Patient Input Program brought into practice in Canada?*
- II. How are claims crafted in the context of the HTA network?*
- III. How is patient feedback considered in CADTH's decision-making?*

In the section below, I present the main findings and conclusions of the study and discuss their contribution to the objectives of the study. The last section provides suggestions for further research.

7.1 Study Findings

The first sub-question focusses on the emergence of patient involvement and the enabling conditions of its existence in evidence-based policy. Firstly, the findings presented in chapter 4 indicate that over the span of several decades, the concept of patient involvement crossed multiple disciplines, fields of work, policy and health domains. Thus, far in its short history, the patient involvement narrative took on a particular non-linear course shaping relationships,

producing controversial discourses about health needs, and creating new public involvement practices. Through a review of interdisciplinary literatures, the dissertation exposes the existence of different models of patient involvement tracing back to patient advocacy and a discourse on human rights. These models differ conceptually; they involve particular modalities of care and redefine relationships among those involved in the healthcare endeavour. The first model emerged as a safeguard for certain patient populations and is connected to notions of human dignity and the right to justice. The second model is founded on the idea that a partnership of continuous interactions and communications is needed between healthcare professionals and patients, who are responsible for their health and can manage their health needs. The third model is inspired by democratic notions of citizenship and representativeness that can lead to elitism and marginalisation. Finally, the fourth model is used as a policy instrument in evidence-based decision-making and recognizes patients as important stakeholders of in the HTA network.

The chapter suggests that the fourth model of involvement emerged from various local and global forces, including the emergence of a new patienthood, the occurrence of healthcare reforms, a political willingness regarding issues of public interest, and the rise of evidence-based policy-making. Within this HTA-specific model, patients are most often structurally involved in decision-making processes instead of on an ad hoc basis. The chapter also demonstrates that certain design and rhetoric choices by CADTH resulted in patients having a voice but no real influence in drug coverage decision-making. In this model of involvement, patients are not considered makers and shapers of healthcare services – they remain outsiders to elite, closed-door decision-making processes. Consequently, I contend that this lack of structural influence on the HTA decision-making network may have a negative impact on the relationship CADTH is trying to build with its stakeholders.

The second sub-question looks at claims-making from the patients' perspective and from evidence-based policy. Chapter 5 concludes that the lack of leverage power of patients within the HTA network is also reflected in the determination of their needs. Indeed, patients' needs are subject to various interpretations and are influenced by groups and institutions. These needs are sometimes tied to social justice, public compassion, patient centeredness, healthcare delivery and technological innovation, scientific uncertainty and value-for-money. These interpretations are closely connected to communities of practice and institutions of knowledge production; they echo the interests of various stakeholders. In the context of drug coverage, patients' needs are debated in a value-laden HTA system that defines their needs on medical interpretations pertaining to physical impairments, rather than the personal and social aspects that accompany the impairment. The dissertation concludes that HTA experts prioritize the synthesis of evidence, randomization of clinical trial patients, statistical significance of study results, value-for-money and clinical effectiveness. Due to the rarity of their disease, I suggest that patients are regarded as medical outliers in a healthcare system driven by a rationing agenda.

Chapter 5 also focusses on the role of the Canadian Organization for Rare Disorders (CORD), which successfully made rare diseases visible to the public, policy-makers and politicians. The composition and actions of CORD were instrumental in advancing a rare disease discourse on health inequities and injustice. CORD advanced the needs of rare disease patients and was able to influence agenda setting in Parliament. Through the work of CORD and its numerous strategies, the struggles of rare disease patients became publicized and politicized as problems of treatment access. However, the chapter contends that this politicization of the needs of rare disease patients by CORD did not enhance their role in the Patient Input Program because drug coverage decision-making is primarily focussed on scientific norms, economic

considerations and the clinical needs of patients. These values work to trivialize and depoliticize the personal and social aspects of living with a rare disease. At the broader systems level, CORD politicizes the predicament of rare disease patients, but at the micro-level, the dissertation demonstrates the consequences of dominant needs interpretations in terms of hiding the contested nature of patients' needs, privileging one reading of science and disease, and not accounting for the contribution of patients to HTA evidence and HTA decision-making. These consequences have the effect of perpetuating health inequities and injustice among patients and deflect from the bigger political issues including excessive drug pricing, over-restrictive eligibility criteria, out-of-pocket expenses, and treatments being sought outside Canada.

The third sub-question analyzes the materialization of the Patient Input Program – from concept to practice and demonstrates the trickling effect of the patient involvement concept on key stakeholders in the HTA network. Chapter 6 contends that the concept of patient involvement varies in meaning for different HTA stakeholders. For CADTH, patient involvement is interpreted as a technocratic response to broader government priorities. For the drug industry, it signifies a change in business model, including evidence benchmarks. For patients, involvement in decision-making processes can be understood as a means to achieve claims for health equity, openness and inclusiveness in the HTA network, as their input represents real-world data, a form of unconventional data that is becoming increasingly important for healthcare decision-making. For public representatives, the incorporation of patient input in the Common Drug Review is considered as a fair procedural practice. They are tasked as the voice box of patients, and ensure that the patients' perspective is accounted for in the deliberations of the drug expert committee. The chapter explains that despite the vagueness of

the patient involvement concept, and the social transformations it produces, it has been accepted in principle by a wide range of stakeholders.

In addition, chapter 6 shows the multiple translations that took place between the conceptual and practical level of patient involvement. What started as a broad policy goal was transformed into a paper-based artefact that was manipulable and quantifiable. On this point, the Patient Input Program adopted scientific data collection methods, document requirements and principles of representativeness through patient groups and public members to objectify to the extend possible patients' feedback. Consequently, the Patient Input Program functions as a positive boundary object for governments and policy-makers in terms of accountability and social responsiveness, but may function as a negative boundary object for HTA experts and patient groups. In this regard, the chapter highlights that the Patient Input Program does not satisfy the needs of all stakeholders. For HTA experts, the credibility of patient input is in question. This may be explained by the fact that they do not have the necessary skill set to assess patient input. For patients, the Patient Input Program evokes the struggles of rare disease groups, and reflects more broadly their efforts in fighting health inequities and overcome bureaucratic hurdles. While patient input is perceived as a necessary vehicle to voice patients' needs around treatment access, patient groups are often left to deal with unfulfilled promises and fail to meet institutional expectations. Through its standardizing practices and evidence hierarchies, the Patient Input Program was said to reinforce conventional science and undermine the humanity of patients.

7.2 Discussion

Based on all the findings summarized in this chapter, the emergence of patient involvement can be viewed as shaped through social interactions and relationships. It is a story

about social participation, patients' claims-making on the state, ways of knowing the world, and the power dynamics that underlie the reality of social participation for marginalized communities.

The dissertation demonstrates how patient groups in the field of rare diseases use patient involvement to make claims but meet resistance due to the rarity of their disease and skepticism about the validity of their input. By using technocratic language, complex processes and reducing patient involvement to an indirect paper-based exercise, the dissertation posits that CADTH widens the gap between the Agency and its stakeholders, the patients. As well, it is argued that CADTH's standardization methods of information-gathering and documentation requirements serve as obligatory passage points and lead to delegitimizing patients' disease experiences. Though CADTH came to recognize patients as a particular engagement audience with unique contributions to evidence-based decision-making, patients have little say and control over funding recommendations, thus limiting any risks to the Agency's legitimacy and stability. As a separate track in the patient involvement spectrum, the dissertation advances that the Patient Input Program is not yet a patient-oriented system of involvement because it has not shifted the locus of power to patients in a way that empowers them to take control of their health and financial vulnerabilities.

7.2.1 Research Limitations and Challenges

To further understand the controversial nature of patients' needs within the context of drug coverage decision-making, fieldwork would have been useful in terms of observations at the drug expert committee meetings. Participant observation may have served to understand the committee's internal workings, the power dynamics within, and the nature of committee deliberations. Longer-term fieldwork would have been necessary to immerse myself into

CADTH's work environment given the committee only meets on a monthly basis. On the other hand, considering that CADTH's drug expert committee for oncology drugs (i.e., pCODR) has patient representatives while CADTH's drug expert committee (CDEC) does not, it would also have been relevant to compare their meeting deliberations to see how the patient input discussions differ in scope and whether decision outcomes diverge. As well, only a small number of CADTH and pCODR staff were interviewed. Their views cannot be generalized. A fuller image of the Patient Input Program may have emanated if other staff or patient representatives with experience of provincial processes had also been interviewed.

Another limitation of the study is its sample size. By no means do I suggest or claim to have identified all views or generalized the views of patients. The study only reflects the views of those patient groups that were able and willing to participate in the study by self-identification. I also acknowledge the potential weaknesses associated with the use and selection of evidence sources to support the case study (Tellis, 1997). The documents and archives were selectively identified based on their relevance to the case study. I may have overlooked sources because they did not match the subjective measure of pre-determined criteria. Additionally, there is always potential for bias in interviews due to the nature of questions posed and respondents' recollection of experiences.

Finally, keeping in line with the boundary object approach, I attempted to present multiple views in the case study analysis. I was limited in my data collection methods as interviews were only conducted with CADTH, pCODR and patients groups, giving them a privileged point of view. Although the perspectives of government and parliamentary officials, and the pharmaceutical industry were only identified via document searches and content-analysis, I did try to interpret their views as accurately as possible. Having said that, I am not

claiming that these are universal representations considering I was limited by my document sources.

7.2.2 Research Contributions

The overarching objective of this dissertation is to understand the social dimensions of the Patient Input Program. The study explores the emergence of patient involvement and its local application, with a focus on rare disease patients. A case study approach was chosen to explain the intrinsic aspects of the Patient Input Program, showing the shaping of the Program and its shaping effect on societal agents and knowledge forms. Serving as a backdrop for the study, the dissertation situates patient involvement as it appeared in the literature and presents a spectrum of patient involvement practices as they evolved in different health domains. This holistic approach to patient involvement allows me to delineate models of involvement and relate involvement to notions of democracy, citizenship, knowledge construction, empowerment and patients' needs.

Although the findings suggest that the Patient Input Program was imposed top-down, the research suggests that CADTH had a degree of discretion in the way it designed the Patient Input Program: a program built on technocratic principles and bureaucratic processes. What the research exposes is far more concerning than a simple discrepancy between policy goals and program applications as noted by public policy theories. Within the HTA network exists an organizational culture and institutional norms of scientific knowledge production that create barriers to patient's meaningful involvement. In this regard, the dissertation uncovers the challenges, tensions and contradictions in the implementation and adoption of the Patient Input Program. The concepts of boundary work and boundary object serve to demonstrate how various HTA experts and non-experts with diverging interests engage in boundary interactions via the

Patient Input Program. In addition, they illustrate how the Program gives the illusion that the interests of all stakeholders within the HTA network are reconciled and that drug coverage decisions are socially just and follow a reasonable and democratic evidence-based approach. While bureaucrats with interests in government accountability and responsiveness embrace the Patient Input Program, the Program is contested by technocrats who struggle with the nature of patients' knowledge and disease experience, and by patients themselves who hit an institutional wall. As a construction of social participation, the Patient Input Program reflects choices and ideologies, embodies rationing and scientific frameworks of reference, assumptions about lay knowledge, biases against the most vulnerable citizens, and reproduces social relations. The conceptual tools offered by science and technology studies enable an analysis of standardizing practices and knowledge hierarchies, and the legitimacy and marginalisation effects of privileged knowledge forms. This discussion highlights the role of CADTH in privileging forms of evidence at the expense of others, and supports earlier assumptions that expert decision-making networks can be reductionist and specialized.

Furthermore, the dissertation brings a discursive approach to the study of patient involvement and links the content of patient input to needs interpretations. The findings demonstrate how dominant needs interpretations can divert attention from what really matters to patients. Using the concept of needs talk leads to the political dimension of needs determination in the healthcare setting, and brings attention to those who have power to frame and advocate patients' needs. Social policy studies provide the conceptual basis to understand how needs are crafted into political demands on the state, and are manipulated to fit an evidence-based policy-making model in resource allocation. The findings point to the controversial nature of patients' needs, which is concealed by the appointment of public members in the Common Drug Review,

and reveals aspects of involvement not accounted for by the literature on patient involvement, that is the competing discourses and interests circulating within the HTA network. These scholars do not expose the controversies within and the weight of other political issues in HTA decision-making process. Due to the absence of a Canadian pharmacare program, patients face excessive drug pricing by drug manufacturers, the setting of over-restrictive clinical criteria at the provincial and territorial level, significant out-of-pocket expenses, and sometimes seek treatments outside Canada.

Finally, the findings should not be perceived as the naming and shaming of CADTH for implementing a program that is less than perfect. Rather, the dissertation serves to uncover potential effects of patient involvement such as social marginalisation. Patient involvement practices can selectively empower some patients and further marginalize others. As mentioned earlier, to be truly a patient-oriented program, CADTH would need to formally divest power to patients within its decision-making structure. On this matter, the dissertation responds to the call of public policy scholars for more empirical research on patient involvement in HTAs that can also guide health policy-makers in terms of implementing involvement methods that are more humane, and more likely to be accepted by intended users. I suggest it is only by knowing the patient communities they serve that governments can interact with patients in a meaningful way.

7.3 Future Research

By mapping patient involvement in drug coverage recommendations, the study establishes the precarious nature of patient involvement and explains the enabling conditions that contribute to its prevalence in contemporary society as a social practice and site of contested needs. Despite demanding meaningful engagement in drug funding recommendations and recognition for their needs, vulnerable patients in the field of rare diseases face further

marginalisation. As the findings suggest, not all diseases have the same political status in the healthcare system and not all patient groups have the same legitimacy and representativeness. Furthermore, patients with worst health states appear to have the least degree of involvement in evidence-based decision-making. The question for policy-makers is how to reach individuals who are the most vulnerable and who are at the margins of society?

My empirical findings highlight a number of issues in relation to the practical implication of patient involvement that foster more thinking on appropriate ways to involve patients in the Common Drug Review process. Currently, the Patient Input Program establishes powerful constraints. Thus, patient involvement should not be viewed solely as a bureaucratic exercise but rather, it should encompass a continuum of engagement approach that seeks to empower patients. In other words, a comprehensive and coherent approach to healthcare is needed among players in the healthcare system that puts patients first. Patient involvement should reassure patients that governments are listening and intervening for their well-being. As demonstrated in the study findings, although patient involvement meets the goals of democracy, the means by which it seeks patient input (i.e., indirectly through a tick-box approach) is not an effective method to hear patient voices and to gain patient's trust. Digital engagement methods may be relevant in Public Opinion Surveys but are less relevant to small patient populations. CADTH should engage in a two-way discussion with patient groups and individual patients who are not organized in groups, no matter what the logistical challenges. The Agency should formalize a position on individual input submissions, as their current thinking is ambiguous. Patient involvement cannot truly be just or democracy-enhancing if patients are forced to align with patient groups that do not necessarily have their best interests in mind. In some cases, patients can be their best advocates. In this line of thought, patients must have a seat at the table to inform

the drug expert committee's decision-making. This entails narrowing the role of public members and enhancing the role of patients as makers and shapers of healthcare services.

In the current value laden HTA system, the authority of scientific methods is protected to the detriment of other knowledge forms. As sociology of science has demonstrated via boundary work, that elite professions insist on contrasts, divides, and drawing lines to assert professional dominance and/or protect their professional autonomy. In the case of evidence-based policy, experts within the Common Drug Review process who work within traditional paradigms of biomedicine and pharmacoeconomics contest the needs of patients because their working paradigms reflect particular values, ideas, and beliefs about the nature of evidence. In reflecting on the expanded role of HTAs, Syrett (2016) notes that in the case of the United Kingdom's National Institute for Health and Care Excellence (NICE),

Although there was an awareness from the date of the establishment of the Institute in 1999 that its activities would necessitate making judgements on matters beyond those which could be inferred from the available scientific evidence base,⁴ 'initially NICE found it difficult to address issues of equity with any degree of sophistication⁵ and thus, its recommendations were based primarily on evaluation of data on clinical efficacy and cost-effectiveness. This 'technocratic fix' proved inadequate because, as leading figures in the Institute noted, scientific evidence is neither perfect nor all-embracing (Syrett 2016: 566).

From the outset, by including patients in the allocation of healthcare resources, governments aspire to forge stronger ties between experts and non-experts, decision-makers and end-users, and to complement the unfulfilled space left by the limitation and sometimes irrelevance of scientific evidence. However, these new ties appear to address more accurately a legitimacy problem in Canada's distribution of scarce healthcare resources, allowing governments to reframe drug coverage decisions as fair and socially just, not only economically

and scientifically sound. By showing the true face of patient involvement and drawing attention to the power struggles within, the dissertation hopes to stimulate further critical analysis of patient involvement practices and the relation between science, policy and civil society.

Fundamentally, the research raises questions about the potential role of social scientists in multi-disciplinary committees. The inclusion of social scientists in involvement processes and within CADTH's drug expert committee may be beneficial to foster a closer relationship between CADTH and patient communities, and getting to know patient communities better. The research also suggests a greater role of social scientists in the design of involvement programs. Involvement processes may benefit from social science research on involvement. As noted earlier, Zevallos (2010) argues that sociology provides a useful skill set for students entering the workforce that can be applied to policy problem solving. I add that this skill set is relevant in evidence-based policy-making, given the new role of real-world evidence and patient communities. Further analysis on the topic is encouraged.

The strongest contribution of the research is on its empirical contribution. Again, the study demonstrates how patients negotiate their role in health policy. However, the Patient Input Program is only one mechanism from a range of engagement initiatives that patients can use to become visible constituents. It is not useful to interpret CADTH's Patient Input Program as a failed attempt to engagement. Though the Patient Input Program can be viewed as an inhibitor to meaningful and influential involvement of patients, patient involvement practices can also push patients to regroup and think of innovative ways to put pressure on the system. The Right to Try movement is an example of innovative social participation, where frustrated patients with Amyotrophic lateral sclerosis (ALS) who could not access therapies for their incurable condition, engaged with parliamentarians to curtail the drug regulatory agency. These patients mobilized

around a private members' Bill to legislate their right to try experimental therapies without government intervention. ALS patients want to exercise their agency in healthcare and control their health. As the Right to Try movement exemplifies, the dissertation can open avenues in social movement research in terms of the range of engagement possibilities that are available to patient groups to bring about social changes. Failed attempts at patient involvement can lead to innovative ways to mobilize.

Lastly, but not least, the study findings lead to investigations on ways that patient groups can contribute to policy-making without formal structures of engagement and outside formal solicited involvement processes. On this point, Wheling (2012) tells us “even invited participatory processes can give an important impulse for further independent and “uninvited” civil society engagement” (Wheling 2012: 57). Conversely, involvement processes “are not necessarily completed when they have come to their “official” end, but may offer opportunities for long-term engagement of citizens”. In this regard, the Patient Input Program should not be understood as an end in itself. Despite the limited role and influence of patients’ knowledge and disease experience in the Patient Input Program, through this process of involvement patients gain experience in government-citizen interactions; they gain knowledge about institutional processes; and, they gain insights about certain inadequacies of the system. The experience acquired through involvement processes can provide patients with hindsight and foresight to fuel transformative and sustainable changes for patients who feel dissatisfied in the current healthcare system.

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APPENDICES

Appendix A - Recruitment Text for CADTH

Subject: Case Study on CADTH's Patient Input Program

Given CADTH's expertise with patient involvement, I am seeking your organization's participation in a doctoral research project, which investigates the emergence of patient input as a platform for fulfilling a knowledge gap in orphan drug policy. Through a case study, I would like to conduct one-hour interviews (in English or French) with 10 CADTH officials to seek feedback on the importance of patient input as a public involvement mechanism; learn about your program from inception to implementation; and, inquire about the state of patient-government relations in the context of orphan drug policy.

To initiate the recruitment process for research informants, I would appreciate if you could distribute the recruitment text to your staff on my behalf and invite interested participants to contact me directly via email or telephone. Attached is the consent form that explains the research purpose, scope of participation and the relevant ethical considerations.

The research proposal has been reviewed/approved by the University of Ottawa (UoO) Thesis Committee (December 3, 2014) and the UoO Research Ethics Office (as of January 2014).

This research endeavour is being conducted independently from the organization from which participants are being recruited.

Do not hesitate to call if you have any questions.

Regards,
Alexandra, Principal Investigator

Appendix B – Recruitment Text for CORD

How is patient input important for you and rare disease patients?

The Patient Input Program allows patient groups to share information about their disease state and quality of life. Its purpose is to help drug decision makers understand your point of view and make decisions that are right for the patient.

Canada has been a leader in this area since the implementation of a patient input pilot within CADTH's Common Drug Review Process.

Given CADTH's particular focus on the needs of patients with rare diseases, I would like to hear your opinions on the patient input initiative as well as the benefits and challenges that this process brings to you.

If you would like to participate in a research study and share your feelings on this matter, I would like to conduct interviews with you. The interviews will last about one hour and will be recorded. However, the information will be kept confidential.

This project has met with the ethics requirement of the University of Ottawa.

If you are interested in contributing to this research, please contact:

Alexandra Bray, PhD Candidate, University of Ottawa

Appendix C - Interview Guide for CADTH

Patient Input Program - Main Questions
How long have you been employed with CADTH?
Can you explain CADTH's mandate with respect to patient input?
Can you describe the Patient Input Program within CADTH?
Can you tell me about your experience with the Patient Input Program?

Concept to Implementation - Main Questions
Can you explain how patient input became an integral part of CADTH's mandate?
What was the role of rare diseases in this program development?
What models were used in the development of patient input?
Was stakeholder involvement considered in the development of the Patient Input Program?
Can you speak to the successes and challenges of the Patient Input Program?
How have the different levels of government and various stakeholders influenced the conceptualisation and development of the Patient Input Program?

Government and Stakeholder Relations - Main Questions
Can you describe your relationship with the different levels of government?
Can you describe your relationship with disease communities and patient advocates?
How does CADTH maintain these relationships?
In your opinion, has CADTH's Patient Input Program changed your relationship with disease communities?

Appendix D - Interview Guide for CORD

Patient Involvement

Main Questions	Additional Questions	Clarification Questions
<p>Can you tell me about your experience with the Canadian Organization for Rare Disease (CORD)</p> <p>OR</p> <p>How long have you been involved with CORD?</p>	<p>-How did you learn about CORD?</p> <p>-Why did you join?</p> <p>-Why is CORD important for people suffering from rare diseases?</p> <p>-What are CORD's main priorities?</p>	<p>-Can you expand a little on this?</p> <p>-Can you tell me anything else?</p>
<p>Can you talk about CORD's patient involvement (PI) agenda?</p>	<p>-How long has CORD been an advocate for patient involvement in healthcare/drug policy?</p> <p>-How is CORD moving the PI agenda?</p> <p>-In your opinion, is there general support for PI by the various players in the orphan drug policy network, e.g. pharmaceutical industry, government of Canada and general public?</p> <p>-If so, why has PI become central to drug policy processes?</p> <p>-Have you noticed changes throughout the years in terms of support for patient involvement initiatives?</p> <p>-Has CORD made allies in its quest for a PI agenda?</p>	<p>-Can you give me some examples?</p> <p>-What do you attribute these changes to?</p> <p>-Were there any challenges?</p>
<p>Why is patient involvement important to CORD?</p>	<p>-How did PI become of primary importance in CORD's mandate?</p> <p>-What were the conditions that promoted this new focus?</p> <p>-How do you define patient input?</p> <p>-What is the primary goal of PI and the expected outcomes?</p> <p>- How does PI differ from other advocacy activities such as lobbying, use of media, creation of coalitions (among others)?</p>	<p>-Can you expand a little on this?</p>

Common Drug Review: Patient Input

Main Questions	Additional Questions	Clarification Questions
-Can you explain your ties to CADTH?	-How long have you been involved in CADTH initiatives? -How important is the work of CADTH for rare disease sufferers? -Why is CADTH an essential player in orphan drug policy?	Can you give me some examples?
-Was CORD involved in the development of CADTH's Patient Input Program?	-How did you learn about the initiative (e.g. newsletter, media, CADTH officials)? -What was CORD's role in CADTH's patient input program?	
-Can you tell me about CORD's experience with CADTH's Patient Input Program?	-What were your initial expectations? -Why is this initiative important for rare disease sufferers? -How does patient input contribute to your role in orphan drug policy?	-Can you expand a little on this? -Can you expand a little on this?

Third Sector - Government Relations

Main Questions	Additional Questions	Clarification Questions
Can you discuss CORD's priorities and how they align with the health priorities in Canada?	-Ideally, how do you envision the role of the Canadian government in the area of rare diseases? -In your opinion is the government committed to the rare disease cause? If so, how have rare diseases become a government priority? -What does that mean for rare disease sufferers? -How could the government further facilitate the empowerment of rare disease patients?	-Can you expand a little on this? -Can you give me some examples?

<p>Can you tell me about CORD's experience with the different levels of government policy-making, including CADTH?</p>	<p>-How has CORD engaged in the development of drug policies in rare diseases? -How has the government responded to CORD's plea for the advancement of rare diseases? -Were there any challenges?</p>	<p>-Why?</p>
<p>Can you describe your relationship with the Canadian government (and associated agencies)?</p>	<p>-What is the nature of your relationship (e.g. political ally, adversary, funder, partner)? -How would you describe your involvement (e.g. political engagement, social engagement)? -What is the importance of this relationship? -How has this relationship evolved over time? Is there a need to build on this relationship?</p>	<p>-Can you give me some examples?</p>

Conclusion of interview		
Main Questions	Additional Questions	Clarification Questions
<p>-In your opinion, has CADTH's Patient Input Program changed your relationship with Canadian policy-makers?</p>	<p>-In your opinion, are your views being heard? -Are rare disease patients influencing drug decisions? -Do you feel part of the drug policy network? - How would you describe your function in the Patient Input Program (e.g. key stakeholder, patient advocate, public)</p>	<p>-Can you expand a little on this? Why or why not?</p>



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Appendix E – Consent Form for CADTH

Title of the study: Mapping Patient Input in Orphan Drug Policy: Boundary Work in the Context of Canada's Health Technology Assessment Agency

Principal Investigator:

Alexandra Bray
PhD Student
School of Sociological and Anthropological Studies
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Ottawa, ON

Thesis Supervisors:

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Assistant Professor
School of Sociological and Anthropological Studies
University of Ottawa
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Michael Orsini
Chair, Institute of Women's Studies
Associate professor
Faculty of Social Sciences, Political Studies
University of Ottawa
Ottawa, ON

Invitation to Participate: I am invited to participate in the abovementioned research study conducted by Alexandra Bray as part of a doctoral thesis at the University of Ottawa under the supervision of Professors Kathleen Rodgers and Michael Orsini. The research is being conducted independently from your organization.

Purpose of the Study: The research investigates the emergence of patient input within Canada's HTA Agency as a platform for fulfilling a knowledge gap in orphan drug policy.

Participation: My participation will entail a semi- directed interview (taking approximately one hour) in winter 2015 to seek feedback on the Patient Input Program, from inception to implementation. I will also be given the opportunity to comment on interview transcripts, preliminary research results and draft research thesis. Materials will be sent to me by the researcher with a 2 week timeframe for feedback.

Risks: Given the fact that the identity of CADTH officials is in the public domain, my identity could be recognized. I have received assurance from the researcher that every effort will be made to minimize these risks.

Benefits: My participation in this study will contribute to shaping public involvement knowledge, and shed light on the conditions that enabled Patient Input Programmes in the HTA setting.

Confidentiality and anonymity: I have received assurance from the researcher that the information I will share will remain strictly confidential. I understand that the contents will be used only for the thesis, publication in scientific journals or meeting presentations, and that my confidentiality will be protected at all times (during and after the research endeavor). The only people who will have access to the research data are the thesis co-supervisors, Kathleen Rodgers and Michael Orsini. While my name will not be used in the published thesis or associated materials, my organization and position will be revealed (unless I do not grant permission to the researcher), which could indirectly identify me. Every effort will be made by the researcher to protect my identity.

- Yes.** My position/organization can be revealed in the published thesis or associated materials.
- No.** My position/organization cannot be revealed in the published thesis or associated materials.

The researcher would like to audiotape the interview to make sure that the conversation is recorded accurately. I may still participate in the research even if I decide not to be taped.

- Yes.** I agree to being audiotaped during the interview.
- No.** I do not agree to being audiotaped during the interview.

Privacy of information: Answers to open-ended questions may be paraphrased in presentations or thesis publications. I will be given the opportunity to correct information if inaccurate, at which time I may decide to retract information. In this case, the information will not be used in the thesis or any associated materials. Any use of quotations would require my approval.

Conservation of data: The data collected (i.e., interview transcripts and researcher's notes) will be kept in a secure manner in a locked filing cabinet in the office of the thesis supervisors at the University of Ottawa for a period of 5 years at which time they will be destroyed. Electronic data will be conserved on an encrypted memory stick, which will be deleted at the end of the conservation period.

If I agree to being audiotaped during the interview, the audiotape of the interview will be placed in a locked filing cabinet in the office of the thesis supervisors at the University of Ottawa until a written word-for-word copy of the discussion has been created. As soon as this process is complete, the tapes will be destroyed.

Voluntary Participation: I am under no obligation to participate and if I choose to participate, I can withdraw from the study at any time and/or refuse to answer any questions, without suffering any negative consequences. If I choose to withdraw, my data will be destroyed, unless permission to use it is granted.

Information about the Study Results: Throughout the research process, I will be given the opportunity to verify the accuracy of interview notes and provide comments on preliminary research results and draft research thesis.

The collection of research material (i.e., interview transcripts) will be used for the thesis.

The PhD thesis that will stem from this research will be made available on the University of Ottawa website. Access to the thesis would be possible through the University of Ottawa Library, which maintains a thesis database.

Acceptance: I, (*Name of participant*), agree to participate in the above research study conducted by Alexandra Bray of the Department of Sociology and Anthropology, University of Ottawa, under the supervision of Professors Kathleen Rodgers and Michael Orsini.

If I have any questions about the study, I may contact the researcher or her supervisor.

If I have any questions regarding the ethical conduct of this study, I may contact the Protocol Officer for Ethics in Research, University of Ottawa, Tabaret Hall, 550 Cumberland Street, Room 154, Ottawa, ON K1N 6N5

Tel.: (613) 562-5387

Email: ethics@uottawa.ca

There are two copies of the consent form, one of which is mine to keep.

Participant's signature: (*Signature*)

Date: (*Date*)

Researcher's signature: (*Signature*)

Date: (*Date*)



uOttawa

Faculté des sciences sociales
Faculty of Social Sciences

École d'études sociologiques et
anthropologiques / School of
Sociological and
Anthropological Studies

Appendix F – Consent Form for CORD

Title of the study: Mapping Patient Input in Orphan Drug Policy: Boundary Work in the Context of Canada's Health Technology Assessment Agency

Principal Investigator:

Alexandra Bray
PhD Student
School of Sociological and Anthropological Studies
University of Ottawa
Ottawa, ON

Thesis Supervisors:

Kathleen Rodgers
Assistant Professor
School of Sociological and Anthropological Studies
University of Ottawa
Ottawa, ON

Michael Orsini
Chair, Institute of Women's Studies
Associate professor
Faculty of Social Sciences, Political Studies
University of Ottawa
Ottawa, ON

Invitation to Participate: I am invited to participate in the abovementioned research study conducted by Alexandra Bray as part of a doctoral thesis at the University of Ottawa under the supervision of Professors Kathleen Rodgers and Michael Orsini. The research is being conducted independently from your organization.

Purpose of the Study: The research investigates the emergence of patient input within Canada's HTA Agency as a platform for fulfilling a knowledge gap in orphan drug policy.

Participation: My participation will entail a semi- directed interview (taking approximately one hour) in summer 2015 to seek feedback about the importance of patient input, the goals and expected outcomes of patient input initiatives and the state of patient-government relations pre and post 2010, date of CADTH's patient input implementation. I will also be given the opportunity to comment on interview notes, preliminary research results and draft research thesis. Materials will be sent to me by the researcher with a 2 week timeframe for feedback.

Risks: Although the identity of CORD members are not in the public domain, my identity could be recognized due to the fact that the rare disease community in Canada is small and tight-knit. I have received assurance from the researcher that every effort will be made to minimize these risks.



Pièce 10005 / Room 10005
10^e étage / 10th floor
120, rue Université / University Pvt.
Ottawa ON K1N 6N5

Benefits: My participation in this study will contribute to shaping public involvement knowledge, and shed light on the conditions that enabled Patient Input Programmes in the HTA setting.

Confidentiality and anonymity: I have received assurance from the researcher that the information I will share will remain strictly confidential. I understand that the contents will be used only for the thesis as well as for publication in scientific journals or meeting presentations, and that my confidentiality will be protected at all times (during and after the research endeavor). The only people who will have access to the research data are the thesis co-supervisors, Kathleen Rodgers and Michael Orsini. Every effort will be made by the researcher to protect my identity. Although CORD will be named in the thesis and associated materials, I will not be identified in the published thesis or associated materials, unless I grant permission to the researcher.

Yes. I want to remain anonymous (use of pseudonym).

No. I do not want to remain anonymous (use of real name).

The researcher would like to audiotape the interview to make sure that the conversation is recorded accurately. I may still participate in the research even if I decide not to be taped.

Yes. I agree to being audiotaped during the interview.

No. I do not agree to being audiotaped during the interview.

Privacy of information: Answers to open-ended questions may be paraphrased in presentations or thesis publications. I will be given the opportunity to correct information if inaccurate, at which time I may decide to retract information. In this case, the information will not be used in the thesis or any associated materials. Any use of quotations would require my approval.

Conservation of data: The data collected (i.e., interview transcripts and researcher's notes) will be kept in a secure manner in a locked filing cabinet in the office of the thesis supervisors at the University of Ottawa for a period of 5 years at which time they will be destroyed. Electronic data will be conserved on an encrypted memory stick which will be deleted at the end of the conservation period.

If I agree to being audiotaped during the interview, the audiotape of the interview will be placed in a locked filing cabinet in the office of the thesis supervisors at the University of Ottawa until a written word-for-word copy of the discussion has been created. As soon as this process is complete, the tapes will be destroyed.

Voluntary Participation: I am under no obligation to participate and if I choose to participate, I can withdraw from the study at any time and/or refuse to answer any questions, without suffering any negative consequences. If I choose to withdraw, my data will be destroyed, unless permission to use it is granted.

Information about the Study Results: Throughout the research process, I will be given the opportunity to verify the accuracy of interview notes and provide comments on preliminary research results and draft research thesis.

The collection of research material (i.e., interview transcripts) will be used for the thesis.

The PhD thesis that will stem from this research will be made available on the University of Ottawa website. Access to the thesis would be possible through the University of Ottawa Library which maintains a thesis database.

Acceptance: I, (*Name of participant*), agree to participate in the above research study conducted by Alexandra Bray of the Department of Sociology and Anthropology, University of Ottawa, under the supervision of Professors Kathleen Rodgers and Michael Orsini.

If I have any questions about the study, I may contact the researcher or her supervisor.

If I have any questions regarding the ethical conduct of this study, I may contact the Protocol Officer for Ethics in Research, University of Ottawa, Tabaret Hall, 550 Cumberland Street, Room 154, Ottawa, ON K1N 6N5

Tel.: (613) 562-5387

Email: ethics@uottawa.ca

There are two copies of the consent form, one of which is mine to keep.

Participant's signature: *(Signature)*

Date: *(Date)*

Researcher's signature: *(Signature)*

Date: *(Date)*

Appendix G - Individuals Interviewed

	Gender	Function
Participant 1	Male	President and CEO Canadian PKU and Allied Disorders
Participant 2	Female	Patient Advocate, Magic Foundation Canada
Participant 3	Male	Member of the Choroideremia Research Foundation of Canada
Participant 4	Male	Professor of Biochemistry at the University of Guelph in Ontario and President of Life Raft Group Canada
Participant 5	Female	Patient, Canadian Pituitary Network [Pituitary Network Association]
Participant 6	Female	Chair of the Canadian Chapter of the National Niemann-Pick Disease Foundation
Participant 7	Female	Executive Director, Canadian MPS Society
Participant 8	Female	Chair, Canadian Treatment Action Council (CTAC)
Participant 9	Female	Chair, Dravet Canada
Participant 10	Female	Board Member, Dravet Canada
Participant 11	Female	Executive Director, Mito Canada
Participant 12	Male	Vice-Chair, Chronic Lymphocytic Leukemia Patient Advocacy Group (CLL PAG)
Participant 13	Female	Patient Advocate, Mito Canada
Participant 14	Female	President of the Canadian Organization for Rare Disorders (CORD)
Participant 15	Female	Founder, Sickle Cell Awareness Group of Ontario and President and CEO of the Sickle Cell Disease Association of Canada
Participant 16	Female	Patient Advocate, Mito Canada
Participant 17	Male	Chief Scientific Officer, Canadian Cystic Fibrosis
Participant 18	Female	National Manager, Pulmonary Hypertension Association of Canada
Participant 19	Female	Executive Director, Epilepsy Newfoundland and Labrador Epilepsy Support
Participant 20	Female	Patient Advocate, aHUS Canada
Participant 21	Male	Board Member, Vasculitis Canada
Participant 22	Female	Program and Communications Coordinator, BC Epilepsy Society
Participant 23	Female	Patient Advocate, Mito Canada
Participant 24	Female	Founder of Patient Input Program, CADTH [Retired]
Participant 25	Female	Patient Engagement Officer, CADTH
Participant 26	Female	Executive Director, pan-Canadian Oncology Drug Review (pCODR)
Participant 27	Male	Executive Director, CADTH
Participant 28	Male	Public Member, Canadian Drug Expert Committee (CDEC)
Participant 29	Male	Public Member, Canadian Drug Expert Committee (CDEC)
Participant 30	Male	Medical Oncologist, Member pCODR