

Courts, Challenges, and Cures: Legal Avenues for Patients with Rare Diseases to Challenge Health Care Coverage Decisions

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This paper examines the legal tools that may be available to patients with rare diseases seeking to compel Canadian governments to provide funding for required or desired treatments. In making health care coverage decisions, governments must decide whether to extend funding to cover potentially expensive treatments that benefit relatively few people, particularly when those treatments are experimental. If particular treatments are not covered by health insurance, patients with rare diseases may turn to the courts with claims based in constitutional, human rights, administrative, international or tort law, in an effort to compel the government to provide funding. Strategies that employ the courts in this way are unlikely to be successful, as courts tend to defer to government on these types of policy-driven decisions.

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I. Introduction

Canadian patients with rare diseases may face unanticipated barriers in pursuit of treatment. Given the plethora of diagnostic challenges associated with rare diseases, patients may go undiagnosed or misdiagnosed for years.¹ If and when they are correctly diagnosed, effective treatment may not exist, as characteristics of rare diseases may render the study of these illnesses and the development of medication difficult or unprofitable.² Even if treatment is available, it may not

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1. Erik Tambuyzer, "Rare Diseases, Orphan Drugs and their Regulation: Questions and Misconceptions" (2010) 9 *Nature Reviews Drug Discovery* 921 at 921; Paola Pierucci et al, "A Long Diagnostic Delay in Patients with Hereditary Haemorrhagic Telangiectasia: A Questionnaire-Based Retrospective Study" (2012) 7:33 *Orphanet Journal of Rare Diseases* (epub).
 2. Erika F Augustine, Heather R Adams & Jonathan W Mink, "Clinical Trials in Rare Disease: Challenges and Opportunities" (2013) 28:9 *Journal of Child Neurology* 1142; Tambuyzer, *ibid* at 922; John Forman et al, "The Need for Worldwide Policy and Action Plans for Rare Diseases" (2012) 101:8 *Acta Paediatrica* 805 at 805. Drug developers have tended to neglect rare diseases, opting to focus on developing medications for more common diseases, though many countries have introduced policies and legislation intended to stimulate research in this area: Samir Gupta, "Rare Diseases: Canada's 'Research Orphans'" (2012) 6:1 *Open Medicine* e23 at e23-e26; Abbas H Panju & Chaim M Bell, "Policy Alternatives for Treatments for Rare Diseases" (2010) 182:17 *Canadian Medical Association Journal* E787; Mae Thamer, Niall Brennan & Rafael Semansky, "A Cross-National Comparison of Orphan Drug Policies: Implications for the US Orphan Drug Act" (1998) 23:2 *J Health Pol* 265.

be covered by the public health care system and the cost may put the treatment out of reach for many patients.³

The recent controversy surrounding the drug eculizumab (brand name Soliris) provides a helpful example. The drug was approved by Health Canada as treatment for two rare diseases: paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS).⁴ The drug, with a price tag of about half a million dollars every year for each patient, is very expensive.⁵ The Canadian Drug Expert Committee, which makes suggestions to provinces regarding which drugs should be covered by their health insurance plans, recommended in both cases that the drug not be funded. In the case of PNH, the Committee believed the cost was too high, and in the case of aHUS, the Committee doubted the efficacy of the drug.⁶ In 2011, the provincial governments coordinated to cover Soliris for PNH.⁷ However, provincial health plans, for the most part, do not cover Soliris for aHUS.⁸ As this case demonstrates, patients with rare diseases may face agonizing uncertainty and disappointment about the scope of coverage. Coverage of a particular

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3. Media reports frequently feature personal stories of patients in need of medication not covered by the public system. See *e.g.* Sarah O'Donnell, "Family Wins Drug-Cost Coverage", *Edmonton Journal* (13 August 2013) A1; Joanne Laucius, "Rare Condition Could Leave 12-year-old a Drug Orphan", *Ottawa Citizen* (5 August 2013) online: Ottawa Citizen <<http://www.ottawacitizen.com>>; "North Vancouver Man Denied Life-Saving Drug", *CBC News* (10 April 2011) online: CBC News <<http://www.cbc.ca/news>>.
 4. Health Canada, "Product Monograph: Soliris (eculizumab)" (25 May 2009), online: Health Canada <<http://www.hc-sc.gc.ca>>; Laucius, *ibid.* An estimated 90 people in Canada have PNH: Sam Cooper, "Drug-Funding Agreement Gives Rare Disease Patients New Hope", *Edmonton Journal* (26 July 2011) A2.
 5. Laucius, *supra* note 3.
 6. Canadian Drug Expert Committee, "CDEC Final Recommendation: Eculizumab: (Soliris – Alexion Pharmaceuticals Inc.) New Indication: Atypical Hemolytic Uremic Syndrome" (18 July 2013), online: Canadian Agency for Drugs and Technologies in Health <<http://www.cadth.ca>>; Canadian Expert Drug Advisory Committee, "CEDAC Final Recommendation: Eculizumab: (Soliris – Alexion Pharmaceuticals Inc.) Indication: Paroxysmal Nocturnal Hemoglobinuria" (19 February 2010), online: Canadian Agency for Drugs and Technologies in Health <<http://www.cadth.ca>>.
 7. Cooper, *supra* note 4.
 8. Laucius, *supra* note 3 (it appears that Soliris may be covered for aHUS in Quebec).

medication may be extended only to certain groups of patients and denied to others. Furthermore, a “coverage patchwork” may develop across the country, leading to a situation where a particular medication is publicly funded in one province but not covered in adjacent provinces.⁹

This paper focuses on health care access challenges faced by patients with rare diseases. A disease is a “rare disease” if it affects only a small segment of the overall population, typically defined as 1 in 2,000 people.¹⁰ Although individual rare diseases affect only a small number of people, over 5 per cent of the total population has a rare disease.¹¹

As the above example of Soliris illustrates, in a publicly funded health care system like Canada’s, hard questions arise: Which treatments should be covered, and for whom? Who should pay for people with rare diseases to receive expensive drugs? How should governments choose to allocate scarce health resources? This paper will not provide a normative answer to these questions. Rather, it focuses on a different, yet related, problem that arises after these questions have been answered and funding decisions have been implemented: Do patients with rare diseases who are denied public health insurance coverage for desired treatments have recourse to the courts?

This paper reviews legal mechanisms available to patients with rare diseases who seek to establish entitlement to publicly funded medical treatment. It begins with an overview of how coverage decisions are made in Canada’s public health care system. This is followed by a consideration of different legal avenues – constitutional, administrative, human rights, international, and tort – and an assessment of their potential for success.

Throughout the following discussion, one issue that arises repeatedly is the efficacy of the medication in question. Sometimes the treatment sought by patients will be proven to be effective and, in the case of a drug, approved by Health Canada. At other times, patients may seek

9. Jamie R Daw & Steven G Morgan “Stitching the Gaps in the Canadian Public Drug Coverage Patchwork? A Review of Provincial Pharmacare Policy Changes from 2000 to 2010” (2012) 104:1 Health Policy 19; Wendy J Ungar & Maciej Witkos, “Public Drug Plan Coverage for Children Across Canada: A Portrait of Too Many Colours” (2005) 1:1 Healthcare Policy 100.

10. Gupta, *supra* note 2 at e23.

11. *Ibid* at e26.

unproven or unapproved¹² treatments or drugs.¹³ While many of the legal issues pertinent to these two circumstances overlap, the latter situation presents unique challenges. For example, if there is no scientific evidence establishing that a particular drug is an effective treatment for a particular disease, then the patient seeking access to that drug may be unable to satisfy his or her burden to prove the requisite elements of the claim. The issue of how medical efficacy may affect the outcome of a particular claim is addressed when germane to the discussion below.

II. Overview of Health Care Coverage Decision-Making

As a condition of federal subsidy under the *Canada Health Act*,¹⁴ provincial health insurance plans must cover “medically required” “physician services”¹⁵ and “medically necessary” “hospital services.”¹⁶ The contours of “medically necessary” – and thus what services must be covered – are not fleshed out in the federal act or in the provincial health acts which establish and operationalize provincial health care

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12. Under schemes set up by the provinces and the federal government, patients are able to obtain unapproved drugs in certain circumstances. A detailed description of these processes and the decision-making that occurs under them is outside the scope of this paper. For more information, see Timothy KS Christie, Marianne Harris & Julio SG Montaner, “Special Access Denied: A Case Study of Health Canada’s Special Access Program” (2006) 2:2 Health Policy 27; Health Canada, “Special Access Programme – Drugs”, online: Health Canada <http://www.hc-sc.gc.ca/dhp-mpps/acces/drugs-droguessapfs_pasfd_2002-eng.php>.
 13. See Simon Day, “Evidence-Based Medicine and Rare Diseases” in Manuel Posada de la Paz & Stephen C Groft, eds, *Rare Diseases Epidemiology: Advances in Experimental Medicine and Biology*, vol 686 (New York: Springer, 2010) 41 (“most new experimental treatments sadly do not work – or, even if they do work, their overall benefit-risk balance is not positive” at 44).
 14. RSC 1985, c C-6 [CHA].
 15. *Ibid*, s 2.
 16. *Ibid*.

themes.¹⁷ Despite the fertile academic literature on this issue,¹⁸ case law exploring and developing the term is sparse, leading to the rather unhelpful conclusion that “medically necessary” hospital and physician services are those that governments ultimately decide to cover under their public insurance plans.¹⁹

Given that these decisions are not based on legislative guidelines, it is important that patients who intend to challenge the scope of public health care coverage understand how these decisions are made. The manner of decision-making varies greatly depending on the sort of service in issue.²⁰ Coverage for physician services, for example, is determined through “negotiation between a provincial government and

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17. Colleen M Flood, Mark Stabile & Carolyn Tuohy, “What Is In and Out of Medicare? Who Decides?” in Colleen M Flood, ed, *Just Medicare: What’s In, What’s Out, How We Decide* (Toronto: University of Toronto Press, 2006) 15 at 17; Timothy Caulfield, “Wishful Thinking: Defining Medically Necessary in Canada” (1996) 4 Health LJ 63 at 67 [Caulfield, “Wishful Thinking”].
 18. See e.g. Caulfield, “Wishful Thinking”, *ibid*; Louise R Sweatman & Diane Woollard, “Resource Allocation Decisions in Canada’s Health Care System: Can These Decisions Be Challenged in a Court of Law” (2002) 62 Health Policy 275; Cathy Charles et al, “Medical Necessity in Canadian Health Policy: Four Meanings and...a Funeral?” (1997) 75:3 The Milbank Quarterly 365 at 370; Flood, Stabile & Tuohy, *ibid*; Commission on the Future of Health Care in Canada, *Medically Necessary: What is it, and Who Decides?* (Ottawa: 2002), online: Canadian Foundation for Healthcare Improvement <<http://www.cfhi-fcass.ca/Home.aspx>>; Donna Greschner, “Charter Challenges and Evidence-Based Decision-Making in the Health Care System: Towards a Symbiotic Relationship” in Colleen M Flood, ed, *Just Medicare: What’s In, What’s Out, How We Decide* (Toronto: University of Toronto Press, 2006) 42; Sara Rosenbaum et al, “Who Should Determine When Health Care is Medically Necessary?” (1999) 340 The New England Journal of Medicine 229. See also the Honourable Michael JL Kirby, *The Health of Canadians – The Federal Role: Final Report* (Ottawa: The Standing Senate Committee on Social Affairs, Science and Technology, 2002), online: Parliament of Canada <<http://www.parl.gc.ca>>.
 19. Flood, Stabile & Tuohy, *supra* note 17 at 16-18; Colleen M Flood & Michelle Zimmerman, “Judicious Choices: Health Care Resource Decisions and the Supreme Court of Canada” in Jocelyn Downie & Elaine Gibson, eds, *Health Law at the Supreme Court of Canada* (Toronto: Irwin Law, 2007) 25 at 29-30; Caulfield, “Wishful Thinking”, *supra* note 17 at 63-67, 74-77; Kirby, *ibid* at Vol 6, Part VIII, Chapter 17; Mel Cousins, “Health Care and Human Rights after Auton and Chaoulli” (2009) 54:4 McGill LJ 717 at 719.
 20. Flood, Stabile & Tuohy, *supra* note 17 at 17.

its respective medical association.”²¹ By way of contrast, the process of decision-making for drug coverage tends to be more formalized.²² Under the terms of the *Canada Health Act*, provinces must fund “drugs ... administered in the hospital”²³ and may (partially or fully) cover other prescription drugs as well.²⁴ Provincial drug coverage decisions are steered by the recommendations of the Common Drug Review, which bases its advice on reviews of efficacy and a cost-benefit analysis.²⁵

Once a coverage decision is made, the ability of patients to challenge the decision is limited. Some provinces provide internal review mechanisms, whereby health department employees can review coverage decisions in response to patient requests.²⁶ In certain provinces, administrative tribunals may be authorized to decide matters that touch on public health care funding.²⁷ For example, administrative boards play

21. *Ibid.*

22. *Ibid.*

23. *CHA*, *supra* note 14, s 2.

24. Kirby, *supra* note 18 at Vol 6, Part IV, Chapter 7.

25. Canadian Agency for Drugs and Technologies in Health, “About the Common Drug Review” (2015), online: CADTH <<http://www.cadth.ca>> (“[t]he CADTH Common Drug Review ... is a pan-Canadian process for conducting objective, rigorous reviews of the clinical effectiveness and cost-effectiveness, as well as reviews of patient input for drugs”). See also Pan-Canadian Oncology Drug Review, “About the pan-Canadian Oncology Drug Review” (2011), online: pCODR <<http://www.pcodr.ca>>. For information on the process of drug approval and price setting, see Amir Attaran, “Take Your Medicine?: The Risk of Patient-Led Legislation in Canada’s Medicine Access System” (2009) 3:1 McGill JL & Health 3 at 5; Steven Morgan et al, “Breadth, Depth and Agreement among Provincial Formularies in Canada” (2009) 4:4 Healthcare Policy e162; Natalie de Paulsen, “The Regulatory Gap: Off-Label Drug Use in Canada” (2005) 63:2 UT Fac L Rev 183 at 189. See also *Food and Drug Regulations*, CRC c 870, s C.08.004; *Patented Medicines Regulations*, SOR/94-688; *Patent Act*, RSC 1985, c P-4, ss 79-103; Ontario Ministry of Health and Long-Term Care, “How Drugs Are Approved: Funding Decisions” (27 June 2012), online: Ontario Ministry of Health and Long-Term Care <<http://www.health.gov.on.ca>>.

26. Flood, Stabile & Tuohy, *supra* note 17 at 22-29; Nola M Ries & Timothy Caulfield, “Accountability in Health Care and Legal Approaches” (3 May 2004), Health Care Accountability Paper prepared for the Canadian Policy Research Networks (Ottawa, Ontario) at 12, online: Canadian Policy Research Networks <http://www.cprn.org/documents/28717_en.pdf>.

27. Flood & Zimmerman, *supra* note 19 at 34-35; Flood, Stabile & Tuohy, *supra* note 17 at 23-25.

a key role in determining whether residents will be reimbursed for health care costs incurred out-of-province.²⁸ If these internal mechanisms are not successful, patients may seek the intervention of the courts through a variety of avenues, a review of which forms the remaining body of this paper.

III. Legal Mechanisms to Challenge Health Care Coverage Decisions

A. The *Charter*

In the years immediately following the adoption of the *Charter*,²⁹ some scholars suggested that it should be interpreted so as to provide protection for socio-economic rights, including the right to health care.³⁰ Such an interpretation would provide fertile ground to argue that the right to access particular therapies fell within its ambit. However, *Charter* claims seeking health care entitlements have been mostly unsuccessful to date.³¹

Patients with rare diseases seeking to use the *Charter* to challenge health care coverage decisions have two potential avenues of argument. First, patients could argue that their section 7 “right[s] to life, liberty and security of the person” are unjustifiably infringed by legislation that limits access to medical services.³² Second, patients could argue that the government failed to live up to obligations created by either section 7 or section 15 of the *Charter*.³³

Using the first avenue, patients may argue that legislation which removes or narrows medical options available to them contravenes

28. Flood, Stabile & Tuohy, *supra* note 17 at 25.

29. *Canadian Charter of Rights and Freedoms*, Part I of the *Constitution Act, 1982*, being Schedule B to the *Canada Act 1982* (UK), 1982, c 11.

30. See *e.g.* Martha Jackman, “The Protection of Welfare Rights under the Charter” (1988) 20:2 *Ottawa L Rev* 257; Martha Jackman, “Charter Review as a Health Care Accountability Mechanism in Canada” (2010) 18 *Health LJ* 1 at 15, 20 [Jackman, “Charter Review”].

31. Cousins, *supra* note 19 at 721; Colleen M Flood, Lance Gable & Lawrence O Gostin, “Introduction: Legislating and Litigating Health Care Rights Around the World” (2005) 33:4 *JL Med & Ethics* 636 at 638.

32. Ries & Caulfield, *supra* note 26 at 1-2, 8-9.

33. *Ibid* at 3.

section 7 of the *Charter*.³⁴ This avenue contemplates legislation which expressly prohibits patients from obtaining certain treatments or medical services, such as the kind of legislation that was at issue in *Chaoulli*.³⁵ In that case, a Quebec statute provided that patients could not obtain private health insurance for medical services available within the public health care system.³⁶ A patient challenged the constitutionality of this legislation, arguing that the lengthy “delays resulting from waiting lists”³⁷ in the public health system combined with removal of the option to obtain private insurance negatively impacted his health and thus infringed his section 7 rights to life and security of the person. Three of the justices of the Supreme Court of Canada agreed, finding further that the legislation was arbitrary and thus inconsistent with the principles of fundamental justice.³⁸ One justice preferred to decide the case under the Quebec *Charter of Human Rights and Freedoms*³⁹ and the three remaining justices upheld the provision under the Canadian *Charter*.⁴⁰

Patients with rare diseases will find *Chaoulli* of limited help, however, as their access to health care is impeded not by legislation that prohibits them from accessing certain services, but rather, by government inaction (*e.g.* not paying for necessary medical treatments or procedures). What is needed, from the perspective of patients with rare diseases, is a hook on which government obligation to fund treatment can be hung. The approach discussed below provides a more promising means to fashion such a hook.

34. *Ibid* at 9-12. See *e.g.* *R v Morgentaler*, [1988] 1 SCR 30; *Rodriguez v British Columbia (Attorney General)*, [1993] 3 SCR 519; *Jane Doe v Manitoba*, 2004 MBQB 285, *rev'd* by 2005 MBCA 109.

35. *Chaoulli v Quebec (Attorney General)*, 2005 SCC 35 [*Chaoulli*]; Ries & Caulfield, *supra* note 26 at 10-11.

36. *Ibid* at para 2.

37. *Ibid*.

38. *Ibid* at para 104.

39. RSQ c C-12.

40. As many scholars have noted, it is unclear exactly how *Chaoulli* will impact constitutional claims to health care, given the split decision. The decision has also received negative commentary. See *e.g.* Jennifer Llewellyn, “A Healthy Conception of Rights? Thinking Relationally About Rights in a Health Care Context” in Jocelyn Downie & Elaine Gibson, eds, *Health Law at the Supreme Court of Canada* (Toronto: Irwin Law, 2007) 57 at 77.

In theory, governmental obligation may be created by either sections 7 or 15 of the *Charter*. In practice, however, courts have shied away from recognizing positive obligations under section 7 of the *Charter*. The Supreme Court of Canada has stated that “[t]he *Charter* does not confer a freestanding constitutional right to health care.”⁴¹ And, lower courts have similarly dismissed suggestions that the government is in violation of section 7 when it declines to cover the costs of a particular medical treatment.⁴²

Patients with rare diseases must then look to section 15 and the right to equality to establish any funding obligation. A section 15 analysis requires a court to ask: “(1) Does the law create a distinction based on an enumerated or analogous ground? (2) Does the distinction create a disadvantage by perpetuating prejudice or stereotyping?”⁴³ As the Supreme Court has observed, “[t]he focus of the inquiry is on the actual impact of the impugned law, taking full account of social, political, economic and historical factors concerning the group” and “involves looking at the circumstances of members of the group and the negative impact of the law on them.”⁴⁴

Accordingly, a patient with a rare disease must establish that the refusal to pay for a particular medical service draws a “distinction [that] create[s] a disadvantage.”⁴⁵ The patient may point to the fact that the health care scheme created by federal and provincial legislation draws a distinction between insured and non-insured services, leading to differential treatment between patients whose medical care is covered and those whose medical care is not. The patient may further point to the fact that some of those in the latter group will include patients with rare diseases. It is not obvious on the face of the scheme that the distinction is drawn on the basis of an enumerated or analogous ground – in this

41. *Chaoulli*, *supra* note 35 at para 104. See also *Gosselin v Québec (Attorney General)*, 2002 SCC 84.

42. See *Flora v Ontario Health Insurance Plan*, 2008 ONCA 538 [*Flora*]; Cousins, *supra* note 19 at 721-26.

43. *Quebec (Attorney General) v A*, 2013 SCC 5 at para 324 [*Quebec v A*]; *R v Kapp*, 2008 SCC 41 at para 17 [*Kapp*]; *Withler v Canada (Attorney General)*, 2011 SCC 12 at para 30 [*Withler*].

44. *Withler*, *ibid* at paras 37, 39.

45. *Ibid* at para 30.

case, rare disease or disability – as both those with rare diseases and those without rare diseases are denied funding if the service in issue is not covered by the public system.

However, the law protects individuals from discrimination that, while not obvious on the face of the statute in question, is apparent following deeper and more probing scrutiny of the statute.⁴⁶ If, on closer examination, it becomes apparent that patients with rare diseases are disproportionately denied funding for medical services or disproportionately affected by denial of funding, then differential treatment is established. Such a conclusion may be reached by evidence illustrating how insufficient financing negatively impacts patients with rare diseases; patients may demonstrate that, without government funding, they cannot afford the treatment in issue, and, without that treatment, their pain and suffering is increased or their life expectancy shortened. On this point, if the scientific evidence is inconclusive on the matter of a therapy’s effectiveness, patients seeking these experimental treatments may be unable to tender compelling evidence that their physical pain is increased by the denial of coverage for that treatment. However, that is not the end of the matter, as these patients could underscore the emotional burden of being denied hope that experimental treatments offer. To establish that the law “create[s] a disadvantage by perpetuating prejudice or stereotyping,” patients with rare diseases may point to the economic disadvantage they shoulder by paying out-of-pocket for medical expenses. This burden increases the economic hardship on patients who may already be off work as a result of illness. Being denied access to treatment may also add to the sense of exclusion and stigmatization already experienced by patients with rare diseases.⁴⁷

If an infringement is established, the burden shifts to the government to prove that the breach can be justified under section 1 of the *Charter*.⁴⁸

46. *Egan v Canada*, [1995] 2 SCR 513 (“[a]dverse effect discrimination occurs when a law, rule or practice is facially neutral but has a disproportionate impact on a group because of a particular characteristic of that group” at 586-87).

47. See e.g. Fatma Ilknur Cinar et al, “Living with Scleroderma: Patients’ Perspectives, a Phenomenological Study” (2012) 32:11 *Rheumatology International* 3573 at 3576.

48. *R v Oakes*, [1986] 1 SCR 103.

An infringement is defensible if the legislative objective is “pressing and substantial,” the chosen course is rationally connected to that objective, the injury to the right is small, and the infringement is proportionate to the benefit and effect of the impugned law.⁴⁹ In the context of a section 15 challenge by a claimant with a rare disease, several factors will be relevant. At the section 1 stage, courts tend to show deference to government decisions that require balancing multiple and varied interests or allotting limited resources.⁵⁰ Health care coverage decisions appear to be the sort of decision that will generally attract deference. Because courts require that the government establish an evidential basis for its impugned action,⁵¹ a government will not simply be able to assert that its action achieves health care objectives and meets the other section 1 requirements without furnishing evidence.⁵² However, as von Tigerstrom points out, it may be challenging for courts to evaluate the evidence put forward by the government: Are purported financial worries genuine or is cost used to shield a discriminatory decision?⁵³ And when does cost justify a decision not to cover a certain treatment? Must it be too expensive for the public system to absorb, or is it enough that the government has decided to fund procedure x over treatment y, both being equally medically effective?⁵⁴

Two Supreme Court of Canada cases will be relevant to any claim brought under section 15.⁵⁵ In *Eldridge v British Columbia*,⁵⁶ the Court found that the failure of hospitals to provide sign-language services for hearing-impaired patients was a violation of section 15.⁵⁷ In finding that the government’s decision constituted discrimination, Justice La Forest

49. *Ibid.*

50. See e.g. *Irwin Toy Ltd v Quebec*, [1989] 1 SCR 927 at para 74.

51. See e.g. *Chaoulli*, *supra* note 35.

52. Ries & Caulfield, *supra* note 26 at 6.

53. Barbara von Tigerstrom, “Human Rights and Health Care Reform: A Canadian Perspective” in Timothy A Caulfield & Barbara von Tigerstrom, eds, *Health Care Reform and the Law in Canada: Meeting the Challenge* (Edmonton: University of Alberta Press, 2002) 157 at 171.

54. Ries & Caulfield, *supra* note 26 at 7-8.

55. See also *Shulman v College of Audiologists and Speech Language Pathologists of Ontario* (2001), 155 OAC 171 (SC) (holding that the decision to remove a particular service from the public insurance plan did not violate s 15).

56. [1997] 3 SCR 624.

57. *Ibid.*

for the Court noted:

In order to receive the same quality of care, deaf persons must bear the burden of paying for the means to communicate with their health care providers, despite the fact that the system is intended to make ability to pay irrelevant ... Once it is accepted that effective communication is an indispensable component of the delivery of medical services, it becomes much more difficult to assert that the failure to ensure that deaf persons communicate effectively with their health care providers is not discriminatory.⁵⁸

One could conceivably argue that patients with rare diseases who do not obtain necessary treatment are not receiving “the same quality of care” as those patients with or without rare diseases who have access to publicly funded treatments.

However, patients with rare diseases may find it difficult to establish an infringement of section 15 given the Supreme Court’s decision in *Auton v British Columbia*.⁵⁹ In that case, families unsuccessfully argued that the government’s failure to provide therapy for their autistic children was discriminatory under section 15. In reaching its decision, the Court reviewed the provincial set-up, which distinguished between “core services” and “non-core services,” the first being those made available by physicians and hospitals, and the second being those performed by other health care professionals and insured only if so stipulated in the regulations.⁶⁰ The Court held that the therapy, being a “non-core service” provided by professionals not designated under the regulations, was not “a benefit provided by law”⁶¹ as there was no legislative entitlement to it.⁶² The Court observed that “the legislative scheme does not promise that any Canadian will receive funding for all medically required treatment,”⁶³ apparently even if the treatment is “essential to the health and medical treatment of an individual.”⁶⁴ The Court also dismissed the suggestion that “the scheme itself [was] discriminatory,”⁶⁵ finding that it

58. *Ibid* at paras 71-72.

59. 2004 SCC 78 [*Auton*]; Flood, Stabile & Tuohy, *supra* note 17 at 29.

60. *Auton, ibid* at paras 30-37.

61. *Ibid* at paras 37, 47.

62. *Ibid* at paras 34-38.

63. *Ibid* at para 35.

64. *Ibid* at para 13.

65. *Ibid* at para 39.

was designed and intended to cover only some, not all, medical services.⁶⁶ Thus, “exclusion of particular non-core services cannot, without more, be viewed as an adverse distinction based on an enumerated ground.”⁶⁷ As the Court’s conclusions in *Auton* make clear, the rigors of a section 15 analysis will not be lessened even in the case of patients who argue that access to treatment is necessary for their health and wellbeing.

Further, the Court held that once the position of the claimants in *Auton* was evaluated alongside the “appropriate comparator group” (*e.g.* persons without a “mental disability” desiring beneficial but novel medical services), “differential treatment either directly or by effect [was] not established”⁶⁸ as the evidence did not indicate that the government gave additional consideration to or was more likely to grant applications for unproven therapies made by persons in the comparator group.⁶⁹ Given the narrow characterization of the “comparator group” in *Auton*, the case would seem to preclude reliance on section 15 by persons seeking unproven treatments which are not included in the existing public insurance scheme, in the absence of evidence that other groups receive coverage for unproven therapies at greater rates. This reading of the case has significant implications for patients with rare diseases, given that many rare diseases have no treatment and thus a significant number of patients may desire to access unproven therapies.⁷⁰

66. *Ibid* at para 43.

67. *Ibid.*

68. *Ibid* at para 58.

69. *Ibid* at paras 58-62.

70. M Ian Phillips, “Gene, Stem Cell, and Future Therapies for Orphan Diseases” (2012) 92:2 *Clinical Pharmacology & Therapeutics* 182 (“currently only ~5% of the approximately 7,000 rare diseases have therapies” at 182). Anecdotal stories suggest that some patients with rare diseases place their hopes in unproven therapies. See *e.g.* Katie Nelson, “Aidan Seeger, 6, needs help to fight rare brain disorder that destroys nerve cells”, *New York Daily News* (27 June 2011) online: *New York Daily News* <<http://www.nydailynews.com/new-york/brooklyn/aidan-seeger-6-fight-rare-brain-disorder-destroys-nerve-cells-article-1.125738>>; Nicole Service, “Parents hope experimental treatments will conquer daughter’s rare illness”, *Daytona Beach News Journal* (14 November 2008) 1C. See also Matilda Anderson, Elizabeth J Elliott & Yvonne A Zurynski, “Australian Families Living with Rare Disease: Experiences of Diagnosis, Health Service Use and Needs for Psychosocial Support” (2013) 8:22 *Orphanet Journal of Rare Diseases* (epub) (some participants “perceiv[ed a] lack of access to drugs used overseas but not

Also relevant is the decision of the Nova Scotia Court of Appeal in *Cameron v Nova Scotia*.⁷¹ In that case, an infertile couple claimed that omission of ICSI from the provincial health plan discriminated on the basis of disability, as the plan covered IVF treatment for couples who suffered only from female infertility and thus did not require ICSI.⁷² The Court agreed that the policy was discriminatory, holding that denial of access to Medicare (“a cornerstone of social programs in Canada”⁷³) reinforced the “vulnerability”⁷⁴ and ostracism of infertile couples.⁷⁵ However, the Court found that the infringement was saved under section 1 of the *Charter*. As part of the section 1 analysis, the majority of the Court emphasized the importance of the purpose of the exclusion, namely ensuring “the best possible health care coverage to Nova Scotians in the context of limited financial resources.”⁷⁶

Based on *Auton* and *Cameron*, it seems that patients bringing a section 15 challenge to establish that a government has an obligation to fund a particular treatment will face an uphill battle, as courts generally show great deference to decisions of this nature. However, that hill may be mounted in a case with the right set of facts, a strong evidential foundation establishing discrimination, and weak government arguments at the justification stage. Additionally, equality jurisprudence appears to be undergoing transformation.⁷⁷ As the Supreme Court further develops section 15 doctrine and principles – by, for example, moving away from the “comparator group” analysis – the likelihood of success of this type of claim increases. After all, the “comparator group” was one of the stumbling blocks in *Auton*. Without the “comparator group” hurdle, it will be easier for a claimant with a rare disease seeking unproven medication to establish the existence of “differential treatment.”

Even if a *Charter* challenge is not successful, there may be merit in

approved in Australia” at 6).

71. (1999), 204 NSR (2d) 1 (CA), Chipman JA (Pugsley JA concurring); separate concurring judgment delivered by Bateman JA.

72. *Ibid* at paras 170-72.

73. *Ibid* at para 206.

74. *Ibid* at para 194.

75. *Ibid* at paras 177-208.

76. *Ibid* at paras 218, 225-45.

77. *Kapp*, *supra* note 43; *Withler*, *supra* note 43; *Quebec v A*, *supra* note 43.

bringing such a claim. For example, *Charter* challenges hold government actors accountable by compelling them to produce evidence justifying their actions and decisions in the health care realm.⁷⁸ Of course, these benefits need to be balanced against the potential costs (in terms of both money and time) of *Charter* litigation.⁷⁹

B. Administrative Law

Patients with rare diseases may also consider using administrative law processes and procedures to challenge coverage decisions. Like *Charter* litigation, administrative law obliges governments to account for their actions and ensures that institutional decision-making is done in a fair and impartial manner.⁸⁰ Administrative law may be preferable to *Charter* litigation, as it tends to be quicker and less expensive.

As noted above, some provincial administrative tribunals, like Ontario's Health Services Review and Appeal Board,⁸¹ have limited authority to weigh in on health coverage decisions. For example, the Appeal Board can determine whether persons who contest denial of insurance are indeed covered under the provincial Act.⁸² The board is also empowered to make decisions on reimbursement for health care expenses incurred outside of Canada.⁸³ However, the scope of the board's

78. Jackman, "Charter Review", *supra* note 30; Ries & Caulfield, *supra* note 26; von Tigerstrom, *supra* note 52 at 178.

79. Flood, Stabile & Tuohy, *supra* note 17 at 29.

80. Flood & Zimmerman, *supra* note 19 at 27.

81. *Health Insurance Act*, RSO 1990, c H.6, ss 20(1), 21; *Ministry of Health and Long-Term Care Appeal and Review Boards Act, 1998*, SO 1998, c 18, Sch H, ss 5, 6; Ries & Caulfield, *supra* note 26 at 12.

82. *Health Insurance Act*, *ibid*, s 20(1).

83. *Ibid*; RRO 1990, Reg 552, s 28.4(2) provides:

Services that are rendered outside Canada at a hospital or health facility are prescribed as insured services if,

(a) the service is generally accepted by the medical profession in Ontario as appropriate for a person in the same medical circumstances as the insured person;

(b) the service is medically necessary;

(c) either,

(i) the identical or equivalent service is not performed in Ontario, or

(ii) the identical or equivalent service is performed in

jurisdiction is limited, and it has no general authority to evaluate coverage decisions.⁸⁴

In addition to these administrative mechanisms, a patient may turn to the courts for judicial review of either the substantive decision (*i.e.* the decision to cover (or not) a particular medical service) or the process used to make that decision. Under the principles of administrative law, government decision-makers must act within the ambit of power bestowed upon them by statute and they must act in a way that is sufficiently fair and transparent.⁸⁵

As discussed above, coverage decisions are made by numerous government actors, acting pursuant to statutory authority. Generally, coverage decisions are made by the provincial cabinet and Minister of Health, regional health boards, and other officials within the provincial health department.⁸⁶ Judicial review is concerned with whether these

Ontario but it is necessary that the insured person travel out of Canada to avoid a delay that would result in death or medically significant irreversible tissue damage; (d) in the case of a hospital service or a service rendered in a health facility described in clause (a) of the definition of “health facility” in subsection (1), the service, if performed in Ontario, is one to which the insured person would be entitled without charge pursuant to section 7 in the case of an in-patient service or section 8 in the case of an out-patient service; and (e) in the case of an in-patient service, in Ontario, the insured person would ordinarily have been admitted as an in-patient of a public hospital to receive the service.

See *RS v Ontario (Health Insurance Plan)*, 2005 CanLII 77249 (ON HSARB), 05-HIA-0148 (finding that sought treatment was experimental and thus not covered); *Flora*, *supra* note 42 (upholding decision of the board to deny reimbursement for experimental, life-saving treatment received outside of the country; provincial health care scheme does not cover all medical treatment, even if life-saving); *Stein v Quebec (Regie de l'Assurance-maladie)*, [1999] RJQ 2416 (SC) (overturning board's decision to deny reimbursement for the cost of life-saving medical treatment received out of country; decision not consistent with the purposes of health care scheme).

84. Flood, Stabile & Tuohy, *supra* note 17 at 24-25.
85. *Crevier v AG (Québec)*, [1981] 2 SCR 220; *Baker v Canada (Minister of Citizenship and Immigration)*, [1999] 2 SCR 817; *Dunsmuir v New Brunswick*, 2008 SCC 9 [*Dunsmuir*].
86. Katherine Cherniawsky, “Enforcement of Health Care Rights and Administrative Law” (1996) 4 Health LJ 35 at 45-46.

bodies have properly acted within their jurisdiction and thus each case will require a detailed analysis of the governing statute and the action purportedly taken under it.⁸⁷ General principles are canvassed below, but, of course, the old caveat rings particularly true in the context of administrative law: the outcome depends on the particular facts of the case.⁸⁸

In *Lexogest Inc v Manitoba*,⁸⁹ the Manitoba Court of Appeal found that the Manitoba Health Services Commission acted outside its jurisdiction by setting up a funding policy which covered abortion services if they were provided in hospitals, but not if they occurred in other health centres.⁹⁰ While the Commission had legislative sanction to determine which services would be covered, it could not exercise this power arbitrarily.⁹¹

The Ontario High Court of Justice in *Re Koonar*,⁹² dismissed an application brought by physiotherapists who sought review of the decision of the provincial health department to deny insurance coverage for their services.⁹³ The Court found that “[t]he extent of that insurance was a policy decision, a legislative decision which is not subject to review.”⁹⁴

In another case, following a decision by a government official to withdraw a particular drug from availability through the Special Access Programme (which provides an exceptional means for patients to access unapproved drugs), patients sought review on the grounds that the official acted outside the scope of his statutory authority.⁹⁵ The Federal Court agreed, holding that the official had, by limiting his decision in advance and in a manner not consistent with the legislative purpose, unlawfully

87. Sweatman & Woollard, *supra* note 18 at 283; Cherniawsky, *ibid* at 47.

88. Sweatman & Woollard, *ibid*; Cherniawsky, *ibid*.

89. *Lexogest Inc v Manitoba (Attorney General)* (1993), 85 Man R (2d) 8 (CA).

90. *Ibid*.

91. *Ibid*, per Helper JA (Philp JA concurring). See also *British Columbia Civil Liberties Association v British Columbia (Attorney General)* (1988), 49 DLR (4th) 493 (BCSC); *contra PEI (Minister of Health and Social Services) v Morgentaler* (1996), 144 Nfld & PEIR 263 (PEISC(AD)).

92. *Re Koonar and Minister of Health* (1982), 133 DLR (3d) 396 (Ont HCJ).

93. *Ibid*.

94. *Ibid* at para 14.

95. *Delisle v Canada (Attorney General)*, 2006 FC 933.

fettered the broad discretion granted to him under the statute.⁹⁶

With that background set out, it is apposite to turn to the particular situation at hand, namely, an administrative-based challenge to a government decision to exclude from its insurance plan a treatment desired by a patient with a rare disease. First, a patient may consider challenging the procedure used to make the impugned decision. At common law, administrative actors owe a duty of procedural fairness to individuals whose rights or interests are affected by specific, individualized decisions.⁹⁷ No duty is owed for general, policy decisions.⁹⁸ Most often, decisions about which medical services should be funded will be policy decisions, involving the apportionment of resources among competing groups, and thus no duty of procedural fairness will attach. In some cases, a duty of procedural fairness may be owed to an individual if the decision is sufficiently particular to that individual. This would likely be the case for a patient who requests reimbursement for out-of-province treatment. While the content necessary to satisfy the duty of procedural fairness varies from case to case, it may require that the affected individual be given an opportunity to respond or to submit evidence for the decision-maker's consideration. However, as noted, for the most part, patients with rare diseases who as a group seek coverage for medications are likely not owed administrative procedural fairness protections. The government may, on its own initiative and without legal compulsion, seek input from patients with rare diseases in making health care coverage decisions, and indeed, certain processes have been recently put in place to facilitate public participation in these types of decisions.⁹⁹

Second, a patient may challenge the substantive decision to include or exclude medical services from public insurance plans, by, for example,

96. *Ibid* at paras 125-28, 167-69.

97. *Martineau v Matsqui Disciplinary Board*, [1980] 1 SCR 602; *Cardinal v Director of Kent Institution*, [1985] 2 SCR 643; *Knight v Indian Head School Division No 19*, [1990] 1 SCR 653.

98. *Canada v Inuit Tapirisat*, [1980] 2 SCR 735.

99. For a comment on how procedural fairness fared under the Common Drug Review, see Attaran, *supra* note 25 at 13-14. Since publication of Attaran's article, the Common Drug Review has created a mechanism to enable patient involvement: Canadian Agency for Drugs and Technologies in Health (CADTH), "Patient Input" (2013), online: CADTH <<http://www.cadth.ca>>.

arguing that the decision-maker erred in giving weight to irrelevant considerations or misconstrued relevant evidence. A court must determine the governing standard of review, being either “correctness” (whereby the court embarks on a fresh assessment of the matter) or “reasonableness” (the more deferential standard, which asks “whether the decision falls within a range of possible, acceptable outcomes which are defensible in respect of the facts and law”).¹⁰⁰ If a board is granted considerable discretion under its enabling statute and it is deciding a question within its domain of expertise, its judgment will often be shown deference by the reviewing court.¹⁰¹ This will frequently be the case for health coverage decisions, as many statutes grant power to administrative actors in general terms which impute significant discretion to the body in question.¹⁰² However, as noted above, outcomes in administrative law depend heavily on the context and the statute in issue, and thus the matter will turn on the particulars of the statute.

C. Human Rights Legislation

Canadian human rights legislation guarantees protection from discrimination in the provision of public services.¹⁰³ Patients with rare diseases may seek to use human rights legislation to contest decisions that have the effect of denying them access to health care services. While it appears that no recorded cases have considered human rights legislation

100. *Dunsmuir*, *supra* note 85 at para 47.

101. *Sweatman & Woollard*, *supra* note 18 at 283; *Maple Lodge Farms v Government of Canada*, [1982] 2 SCR 2; Cherniawsky, *supra* note 86 at 60-61.

102. *Sweatman & Woollard*, *ibid*; Cherniawsky, *ibid*.

103. See *e.g.* *Human Rights Code*, RSBC 1996, c 210, s 8(1):

- A person must not, without a bona fide and reasonable justification,
- (a) deny to a person or class of persons any accommodation, service or facility customarily available to the public, or
 - (b) discriminate against a person or class of persons regarding any accommodation, service or facility customarily available to the public because of the race, colour, ancestry, place of origin, religion, marital status, family status, physical or mental disability, sex, sexual orientation or age of that person or class of persons.

See also Claire Mummé, “At the Crossroads in Discrimination Law: How the Human Rights Codes Overtook the *Charter* in Canadian Government Services Cases” (2012) 9 *JL & Equality* 103 at 104.

in the context of patients with rare diseases seeking treatment, this avenue has been successful in analogous situations, and thus a review of cases sheds light on how courts may decide claims brought by patients with rare diseases.

Human rights legislation has been successfully relied upon by transpersons seeking public insurance coverage for sex reassignment surgery. In *Waters v British Columbia*,¹⁰⁴ the BC Human Rights Tribunal found that the provincial health plan, which paid for vaginoplasty for transwomen but did not cover the whole cost of phalloplasty for transmen, was discriminatory.¹⁰⁵ Similarly, in *Hogan v Ontario*,¹⁰⁶ a majority of the Ontario Human Rights Tribunal held that the government's decisions to remove sex reassignment surgery from the list of funded services and not to extend stop-gap funding to cover the claimants who were in the midst of the procedure was a violation of the *Human Rights Code*,¹⁰⁷ as the government had not established it was incapable of accommodating this group of claimants.¹⁰⁸ The majority carefully noted that the government retained the power to make coverage decisions and to remove therapies from coverage.¹⁰⁹ But, in this case, it was unacceptable for the government to "pull the plug" on claimants who were well into the process.¹¹⁰

In two recent cases, male patients argued that provincial insurance plans, by covering screening for breast and uterine cancer but not covering screening for prostate cancer, discriminated on the basis of sex. The complaint was dismissed in both cases. In *Armstrong v British Columbia*,¹¹¹ the BC Court of Appeal upheld the conclusion of the adjudicator who found that the coverage decision was not related to sex,

104. *Waters v BC (Ministry of Health Services)*, 2003 BCHRT 13.

105. *Ibid* at paras 180-85.

106. *Hogan v Ontario (Minister of Health and Long-Term Care)*, 2006 HRTO 32 at paras 383, 389-465 [*Hogan*]. See also *May v Ontario (Minister of Health and Long Term Care)*, 2011 HRTO 2179; *C v BC (Ministry of Health)*, 2012 BCHRT 47.

107. *Ontario Human Rights Code*, RSO 1990, c H.19.

108. *Hogan*, *supra* note 106 at paras 99, 106-09, 120.

109. *Ibid* at para 7.

110. *Ibid* at para 120.

111. *Armstrong v British Columbia (Ministry of Health and Long-Term Care)*, 2010 BCCA 56, leave to appeal refused [2010] SCCA No 128.

but rather based on medical efficacy, as prostate cancer screening, unlike breast and uterine cancer screening, was not proven to be effective.¹¹² Similarly, in *Cochrane v Ontario*,¹¹³ the Ontario Human Rights Tribunal found that the evidence did not prove that prostate cancer screening increased survival rates and thus, like in *Armstrong*, efficacy – not sex – was the motivation for the decision.¹¹⁴ In both cases, the adjudicative body tied coverage decisions to evidence of efficacy, perhaps cementing a requirement that coverage decisions be evidence-based. If the approach in *Armstrong* and *Cochrane* is followed in the future, it seems unlikely that courts will order that the government pay for experimental treatments that lack at least some evidence of medical efficacy.

The BC Human Rights Tribunal similarly focused on evidence of efficacy in *Turnbull v British Columbia*.¹¹⁵ Under the provincial health plan, venous angioplasty was not covered for multiple sclerosis (MS), but was covered for other conditions.¹¹⁶ Turnbull argued that this constituted discrimination on the basis of disability, because the treatment would be covered if he had a different disease instead of MS.¹¹⁷ The fact that the treatment was novel and untried for MS factored heavily into the tribunal's decision to dismiss the complaint.¹¹⁸

In the well-known case of *Canada v Buffett*,¹¹⁹ however, Buffett successfully argued that the failure of the Canadian Forces to pay for in *intra*-cytoplasmic sperm injection (ICSI) for male service members while paying for *in vitro* fertilization (IVF) for female service members constituted discrimination on the basis of sex and disability.¹²⁰

Also relevant are decisions related to the obligation of governments to fund special programming for children with disabilities. In *Moore v British*

112. *Ibid* at para 33.

113. *Cochrane v Ontario (Minister of Health and Long-Term Care)*, 2010 HRTO 1477.

114. *Ibid* at paras 22, 24.

115. *Turnbull v British Columbia (Ministry of Health Services)*, 2011 BCHRT 324.

116. *Ibid* at para 89.

117. *Ibid* at para 77.

118. *Ibid* at paras 80-88, 96-98.

119. *Canada (Attorney General) v Buffett*, 2007 FC 1061.

120. *Ibid* at paras 48-64. See also Susan Krashinsky, "Funding Fertility: The Fight to Have Treatments Covered", *The Globe & Mail* (19 August 2009) online: Globe & Mail <<http://www.theglobeandmail.com>>.

Columbia,¹²¹ the Supreme Court of Canada found that the province acted discriminatorily when it abolished special programming that benefited a child with a mental disability.¹²² The Court rejected the assertion by the province and the school district that their action was necessary to tackle a “budgetary crisis,”¹²³ as “the cuts were disproportionately made to special needs programs”¹²⁴ and no consideration was first given to other ways in which the financial crisis could be resolved.¹²⁵ Extrapolating principles from this decision to the health care context, it would seem that, while budgetary considerations can justify delisting or excluding medical services from coverage, patients with rare diseases must not bear the brunt of financial constraints. In other words, governments cannot justify coverage decisions based on economic arguments if those decisions disproportionately impact patients with rare diseases.

As this brief review highlights, individuals have had mixed success in using human rights legislation to challenge governmental resource allocation decisions. For patients with rare diseases to make use of this tool, they must “demonstrate *prima facie* discrimination ... [by] show[ing] that they have a characteristic protected from discrimination under the [relevant human rights] Code; that they experienced an adverse impact with respect to the service; and that the protected characteristic was a factor in the adverse impact.”¹²⁶ Their efforts in this regard will be furthered by the types of arguments and evidence recounted under the discussion of section 15 of the *Charter*. In order to establish that “the protected characteristic was a factor in the adverse impact,”¹²⁷ patients may need to present evidence that the sought treatment is likely to be effective. If they are unable to do so, they may fail to prove that the impugned decision was in fact made on the basis of disability, rather than on the grounds that the therapy is not scientifically proven.

121. *Moore v British Columbia (Education)*, 2012 SCC 61 [*Moore*]. See also *Newfoundland and Labrador v Sparkes et al*, 2004 NLSCTD 16.

122. *Moore*, *ibid* at para 48.

123. *Ibid* at para 53.

124. *Ibid* at para 51.

125. *Ibid* at paras 50-53.

126. *Ibid* at para 33.

127. *Ibid*.

The government then has the onus of proving that denial of funding is justified and may argue that budgetary constraints and the high cost of orphan drugs warrant the impugned action or decision. However, as the Supreme Court of Canada stated in *Moore*, “accommodation is not a question of ‘mere efficiency’, since ‘[i]t will always seem demonstrably cheaper to maintain the status quo and not eliminate a discriminatory barrier’.”¹²⁸ The budgetary circumstances must be such that funding cannot reasonably be extended to cover the requested therapy without causing serious difficulties for the government. Given the exceptionally high cost of some orphan drugs, the government may be able to establish that it could not reasonably afford to cover these treatments. It should be noted, however, that data from Europe suggests that fees paid for orphan drugs constitute only a small fraction of the overall health budgets of many countries.¹²⁹ Further, if the cost of the treatment is minor, the court may reject the government’s assertion that covering the cost of therapies for patients with rare diseases would be an unreasonable burden.

D. International Law

International covenants affirm that facilitating access to health care is a crucial component in attaining the overall wellbeing of all people. By way of example, the *Universal Declaration of Human Rights* states that “[e]veryone has the right to a standard of living adequate for the health and wellbeing of himself and of his family, including ... medical care.”¹³⁰ Similarly, Article 12 of the *International Covenant on Economic, Social and Political Rights* (ICESPR) recognizes “the right of everyone to the enjoyment of the highest attainable standard of physical and mental health.”¹³¹ Parties to the Covenant commit to “achieving progressively

128. *Ibid* at para 50, citing *Council of Canadians with Disabilities v VIA Rail Canada*, 2007 SCC 15 at para 225.

129. Carina Schey, Tsveta Milanova & Adam Hutchings, “Estimating the Budget Impact of Orphan Medicines in Europe: 2010-2020” (2011) 6:62 *Orphanet Journal of Rare Diseases* (epub) (“the cost [of orphan drugs], as a pro-portion of total pharmaceutical expenditure, is likely to plateau between 4%-5%” at 9). See also Tambuyzer, *supra* note 1.

130. *Universal Declaration of Human Rights*, GA Res 217(III), UNGAOR, 3d Sess, Supp No 13, UN Doc A/810, (1948), Art 25(1).

131. *International Covenant on Economic, Social and Cultural Rights*, 16 December

the full realization” of this right, including “[t]he creation of conditions which would assure to all medical service and medical attention in the event of sickness.”¹³²

Canada is a party to the ICESPR and thus has obligations under international law to fulfill the commitments made under that treaty. The ICESPR has not found its way into domestic implementing legislation, however. Thus, while it cannot ground a claim by a patient with a rare disease in Canadian courts, it does form part of the background in which judicial interpretation of domestic legislation in, say, an administrative or *Charter* case, occurs.¹³³ The impact of international covenants is succinctly explained in a report to Parliament:

[W]hile unincorporated treaties do not necessarily alter Canadian domestic law, they can and do influence its interpretation. A common law doctrine, which applies in Canada, holds that in interpreting legislation, courts should presume that Parliament intended to legislate in a manner consistent with its international treaty obligations ... [I]t is clear that the courts can make use of international human rights law in interpretation.¹³⁴

Accordingly, patients with rare diseases cannot directly rely on the guarantees contained in international treaties. However, patients would be well advised to emphasize Canada’s international obligations if bringing claims under domestic legislation or the *Charter*, particularly given the generous scope of some provisions in international documents. For example, if the benchmark set out in the ICESPR were adopted by Canadian courts, then entitlement to many medical services would likely follow. These services would include therapies that are necessary to extend life or ease pain, and also, arguably, experimental therapies that, while unproven, are reasonably anticipated by patients to have some positive effects. The latter category is suggested on the basis of the stress,

1966, 993 UNTS 3, Can TS 1976 No 46 (entered into force 3 January 1976, accession by Canada 19 May 1976), Art 12.

132. *Ibid*, Arts 2, 12(1), 12(2)(d).

133. von Tigerstrom, *supra* note 52 at 160.

134. Standing Senate Committee on Human Rights, chaired by the Honourable Raynell Andreychuk, *Promises to Keep: Implementing Canada’s Human Rights Obligations* (Ottawa: Parliament of Canada, 2001), online: Parliament of Canada <<http://www.parl.gc.ca/Content/SEN/Committee/371/huma/rep/rep02dec01-e.htm>>.

disappointment and feelings of hopelessness associated with denial of funding for treatment (and thus, in effect, denial of treatment if costs make it unattainable). These emotional responses should be taken into account when considering what services are necessary in order for patients to achieve the “highest attainable standard of physical and mental health” as provided for by the ICESPR.

E. Tort Law

Tort law is another potential tool for patients seeking funding for desired medical services. This section addresses the issue of whether a patient who is unable to access treatment as a result of a government’s refusal to fund that treatment could claim against the government on grounds of negligence. This question considers the matter of resource allocation at the macro-level (*i.e.* the policy phase where “big picture” resource allocation decisions are made), as well as at the micro-level (*i.e.* everyday, individual decisions about how to make use of and expend resources). The discussion thus far has focused on legal claims brought to challenge the decision of governments or government actors, not individual physicians, though they too make a type of resource allocation decision at a patient’s bed-side when they decide whether to administer treatment.¹³⁵ This section of the paper considers whether individual physicians and other health care providers who refuse to provide treatment in order to conserve system resources could be liable if the patient then suffers harm.¹³⁶ In other words, can physicians rely on what Caulfield calls the “cost-containment defence”?¹³⁷

At the macro-level of resource allocation, what use can be made of tort law to contest allocation decisions? In a recent review of Canadian jurisprudence, Lorian Hardcastle identifies several types of claims that may be brought against the government: claims for mishandling pandemics; claims related to system management failures; and claims for

135. See *e.g.* Timothy A Caulfield, “Malpractice in the Age of Health Care Reform” in Timothy A Caulfield & Barbara von Tigerstrom, eds, *Health Care Reform and the Law in Canada: Meeting the Challenge* (Edmonton: University of Alberta Press, 2002) 11 at 13, 21-24 [Caulfield, “Malpractice”].

136. *Ibid* at 24-26. See also Sweatman & Woollard, *supra* note 18.

137. Caulfield, “Malpractice”, *supra* note 135 at 24.

deaths caused by long wait times.¹³⁸ A brief review of some of these cases is in order before a discussion of the probability that patients with rare diseases will succeed with system-level negligence claims.

The Ontario Court of Appeal has struck claims brought by patients and health care professionals who argued that the province failed to protect them and to manage the hospital system properly after they contracted communicable diseases.¹³⁹ The Court soundly rejected the plaintiffs' arguments in *Eliopoulos Estate v Ontario (Minister of Health and Long-Term Care)*, observing:

[T]o impose a private law duty of care on the facts that have been pleaded here would create an unreasonable and undesirable burden on Ontario that would interfere with sound decision-making in the realm of public health. Public health priorities should be based on the general public interest. Public health authorities should be left to decide where to focus their attention and resources without the fear or threat of lawsuits.¹⁴⁰

Similarly, the Quebec Court of Appeal dismissed an application for the certification of a class action brought by cancer patients who accused the government and hospitals of negligently delaying their treatment.¹⁴¹ The clear policy nature of the decision did not warrant allowing the claim to proceed.¹⁴²

In contrast, the Ontario Court of Appeal in *Heaslip Estate v Mansfield Ski Club*¹⁴³ declined to strike the claim of plaintiffs who argued that the government acted negligently by failing to send air-based medical support to transport an injured teenager, contrary to the government's own guidelines.¹⁴⁴ The Court believed that the situation fell within the

138. Lorian Hardcastle, "Government Tort Liability for Negligence in the Health Sector: A Critique of the Canadian Jurisprudence" (2012) 37:2 Queen's LJ 525 at 546.

139. *Williams v Ontario*, 2009 ONCA 378 at para 7; *Jamal Estate v Scarborough Hospital*, 2009 ONCA 376 at para 9; *Abarquez v Ontario*, 2009 ONCA 374 at para 23; *Laroza Estate v Ontario*, 2009 ONCA 373 at para 5; *Henry Estate v Scarborough Hospital*, 2009 ONCA 375; Hardcastle, *supra* note 138 at 546-47.

140. (2006), 82 OR (3d) 321 (CA) at para 33 [*Eliopoulos Estate*]; Hardcastle, *supra* note 138 at 546-47.

141. *Cilinger v Quebec*, [2004] RJQ 2943 (CA); Hardcastle, *supra* note 138 at 548.

142. *Cilinger*, *ibid* at para 16.

143. 2009 ONCA 594 [*Heaslip*].

144. *Ibid* at paras 1, 2, 17, 23-28, 31, 35; Hardcastle, *supra* note 138 at 548.

pre-existing class of duties which entailed liability if “a public authority ... negligent[ly] fail[ed] to act in accordance with an established policy where it is reasonably foreseeable that failure to do so will cause physical harm to the plaintiff.”¹⁴⁵ The Court added that a duty could be found on an *Anns* negligence analysis.¹⁴⁶ The Court distinguished the type of government decisions that are immune from liability due to policy considerations from the claim before it, as this claim was “based upon the negligent failure to respond to a specific request for a service that is being provided under an established policy” rather than a challenge to the development of general policy.¹⁴⁷

This review of cases suggests that tort law is not a promising prospect for patients with rare diseases who seek to challenge government health care allocation decisions. As Hardcastle notes, many of these cases are decided at the duty stage, and courts have been unwilling to find the requisite proximity between the plaintiffs and the defendant governments or hospitals which is necessary to base a duty of care.¹⁴⁸ Chief Justice McLachlin explained the proximity requirement in *Hill v Hamilton-Wentworth Regional Police Services Board* as follows:

The most basic factor upon which the proximity analysis fixes is whether there is a relationship between the alleged wrongdoer and the victim, usually described by the words “close and direct”. This factor is not concerned with how intimate the plaintiff and defendant were or with their physical proximity, so much as with whether the actions of the alleged wrongdoer have a close or direct effect on the victim, such that the wrongdoer ought to have had the victim in mind as a person potentially harmed. A sufficiently close and direct connection between the actions of the wrongdoer and the victim may exist where there is a personal relationship between alleged wrongdoer and victim. However, it may also exist where there is no personal relationship between the victim and wrongdoer.¹⁴⁹

Undoubtedly, government decisions about what medical services to fund or not fund will deeply and significantly impact some individuals. However, it seems unlikely that courts would expect the government to

145. *Heaslip*, *supra* note 143 at para 21.

146. *Ibid* at paras 23-31.

147. *Ibid* at para 29.

148. Hardcastle, *supra* note 138 at 554, 556-58.

149. 2007 SCC 41 at para 29 [*Hill*]. See also *Cooper v Hobart*, 2001 SCC 79 at paras 23-26, 32 [*Cooper*].

have individual patients “in mind” as “person[s] potentially harmed.” In a general sort of way, the government will expect that its decisions in the health care realm will affect people’s lives, but, generally, it will not anticipate the specific harms that may result or the particular persons (or groups of persons) that will be impacted. Unlike in *Hill*, where police were found to be adequately proximate to an individual suspect to ground a duty of care, no patient is “singled out”¹⁵⁰ or “particularized”¹⁵¹ when the government makes general health coverage decisions. The government is dealing with – to use the language from *Hill* – “the universe of all potential”¹⁵² patients.

Even if plaintiffs establish proximity, a court may find that the duty should be abrogated for policy reasons under the second branch of the *Anns* test.¹⁵³ In *Hill*, McLachlin CJC explained that “the final stage of *Anns* is concerned with ‘residual policy considerations’ which ‘are not concerned with the relationship between the parties, but with the effect of recognizing a duty of care on other legal obligations, the legal system and society more generally’.”¹⁵⁴ For example, under the “residual policy consideration” criterion, a court should ask whether there is “potential for conflict between a duty of care in negligence and other duties owed by”¹⁵⁵ the government, such as, “duties [owed] to the public at large.”¹⁵⁶ This concern motivated the Ontario Court of Appeal in *Eliopoulos Estate* (discussed above) to hold that “impos[ing] a private law duty ... would create an unreasonable and undesirable burden ... that would interfere with sound decision-making in the realm of public health.”¹⁵⁷ Courts are likely to find that duties to individual persons are inconsistent with the government’s overarching responsibility to provide a cost-effective, reliable and fair health care system. Courts will thus “negate” the duty on the basis of policy-related worries that the imposition of such an

150. *Hill, ibid* at para 33.

151. *Ibid.*

152. *Ibid.*

153. Hardcastle, *supra* note 138 at 555-570; *Cooper, supra* note 150 at para 38.

154. *Hill, supra* note 149 at para 31, citing *Cooper, ibid* at para 37.

155. *Hill, ibid* at para 48.

156. *Ibid* at para 130.

157. *Eliopoulos Estate, supra* note 140 at para 33.

obligation would unduly interfere with the government's discretion to allocate resources in a manner it believes best meets the needs and expectations of all Canadians.

Additionally, it must be noted that, pursuant to the Supreme Court's decision in *Just*,¹⁵⁸ governments are not liable for "true policy decisions"¹⁵⁹ that "involve or are dictated by financial, economic, social or political factors or constraints."¹⁶⁰ Health care coverage decisions, as they involve resource distribution and financial wrangling, are likely the type of decision for which a government will not be liable.

Thus, for patients with rare diseases, the policy hurdles to establishing a duty of care will be difficult to overcome. A patient would need to establish that the impugned decision is in fact an "operational decision" which executes established policy.¹⁶¹ One could attempt to characterize the decision to deliver accessible, high-quality health care a "policy decision" and choices about individual services "operational decisions" made in the course of realizing that policy. This approach may avoid running afoul of the Supreme Court's observation that, "[a]s a general rule, decisions concerning budgetary allotments for departments or government agencies will be classified as policy decisions,"¹⁶² because funding (or not funding) individual services may not implicate larger budgetary decisions if the "operational decisions" are made by "lower level"¹⁶³ actors within the parameters – including ultimate financial constraints – of the policy. That is, the decision regarding how much money to allocate to overall health spending may be a "policy decision" immune from liability, but, provided the budget is not exceeded, decisions about how to spend that money (on treatment x and not treatment y) may be "operational decisions." However, based on Canadian case law,¹⁶⁴ this approach, in

158. *Just v British Columbia*, [1989] 2 SCR 1228 [*Just*].

159. *Ibid* at 1239.

160. *Ibid* at 1242, citing *Sutherland Shire Council v Heyman* (1985), 60 ALR 1 (HCA). See also Sweatman & Woollard, *supra* note 18 at 287.

161. *Just*, *supra* note 159 at 1239-40.

162. *Ibid* at 1245.

163. *Ibid* at 1243 ("a true policy decision may be made at a lower level provided that the government agency establishes that it was a reasonable decision in light of the surrounding circumstances").

164. See *e.g.* *Gosselin v Moose Jaw* (1997), 155 DLR (4th) 374 paras 15-25 (SKCA);

so far as it seeks to challenge listing and delisting decisions, is unlikely to find favour before the courts unless and until courts perceive “money decisions” to be operational ones.

Turning to micro-level decision-making, physicians and other medical service providers are unlikely to escape liability for malpractice by arguing that fiscal restraints justified their decision not to provide a particular service.¹⁶⁵ In *Law Estate v Simice*,¹⁶⁶ the British Columbia Supreme Court rejected the physician’s defence that he did not send the patient for a CT scan because of monetary restrictions.¹⁶⁷ The Court noted: “if it comes to a choice between a physician’s responsibility to his or her individual patient and his or her responsibility to the medicare system overall, the former must take precedence in a case such as this.”¹⁶⁸ The Court observed that the physical harm to the patient is “far greater than the financial harm” to the system.¹⁶⁹

This line of reasoning will be useful if a patient with a rare disease is denied an expensive treatment by a physician on the basis of cost considerations. However, it is unclear whether this scenario is common¹⁷⁰ and thus *Simice* may not be particularly helpful for patients with rare diseases.

A lawsuit pursued against an individual physician will require consideration of whether the physician has met the requisite standard of care, which is assessed in comparison to “the conduct of a prudent

Gobin v British Columbia, 2002 BCCA 373 at para 44.

165. Caulfield, “Malpractice”, *supra* note 135 at 24.

166. (1994), 21 CCLT (2d) 228 (BCSC).

167. *Ibid* at para 28.

168. *Ibid*.

169. *Ibid*; Caulfield, “Malpractice”, *supra* note 135 at 25-26.

170. But see *e.g.* Ezekiel J Emanuel & Andrew Steinmetz, “Will Physicians Lead on Controlling Health Care Costs?” (2013) 310:4 *Journal of the American Medical Association* 374 (“85% [of participant doctors] strongly or moderately agreed that trying to contain costs is the responsibility of every physician” at 375). For discussion on whether costly therapies can justifiably be denied on the basis of resource shortages, see *e.g.* Dominic Wilkinson, “Which Newborn Infants are Too Expensive to Treat? Camosy and Rationing in Intensive Care” (2013) 39:8 *Journal of Medical Ethics* 502; Dyfrig Hughes, “Rationing of Drugs for Rare Diseases” (2006) 24:4 *Pharmacoeconomics* 315.

and diligent doctor in the same circumstances.”¹⁷¹ The efficacy of the sought drug may be relevant at this stage of the analysis. If, for example, the drug is experimental and unproven, a defendant physician can more easily establish that he or she acted “in accordance with the conduct of a prudent and diligent doctor in the same circumstances,”¹⁷² by arguing that his or her peers would be cautious about administering medication without the belief that the drug has at least some chance of benefiting the patient. The fact that the patient has a rare disease – and not a common illness – may also be relevant to the determination of whether the health care provider has fallen below the standard of care. Some lower courts have found that the rare nature of an illness militates in favour of a finding that the defendant met the applicable standard, as physicians in the same setting with the same experience would not recognize the unusual disease.¹⁷³

Even if claims against individual health care providers were likely to be successful, it should be added that tort law challenges to individual decision-makers may not be ideal from the perspective of patients with rare diseases as a group, as the individualized outcome in tort cases does not necessarily lead to the larger, policy change desired by many patients. As Caulfield notes:

[T]ort law is not the best tool for effectuating health care reform. Malpractice lawsuits are determined on a case-by-case basis. They focus on the rights and legal duties of individual physicians and patients. And while the principles of tort law obviously have social utility, such as the compensation of patients who are injured by negligence, the rights and duties of patients and physicians are rarely subordinated to the needs of the broader health care system.¹⁷⁴

While an individual patient may win his or her case, the larger group of patients is still left without access to treatment.

171. *Ter Neuzen v Korn*, [1995] 3 SCR 674 at para 33.

172. *Ibid.*

173. *Hancock Estate v Hanton* (2003), 344 AR 221 at paras 56, 84-93 (ABQB); *Grennan Estate v Reddoch*, 2002 YKCA 17 at paras 36, 48-49; *Shannahan v Johnson*, 2010 BCSC 700 at para 76.

174. Caulfield, “Malpractice”, *supra* note 134 at 12.

IV. Conclusion

This paper has discussed legal avenues that patients with rare diseases may pursue if they are dissatisfied with health care coverage decisions. Each of these avenues has distinct merits and obstacles. For example, *Charter* litigation is ideal for effecting policy change and may be embraced by plaintiffs who seek “big picture” change. However, it can be complicated, costly, and drag on for years. Tort law, administrative law, and human rights law offer individualized outcomes, and thus, while policy change is possible if the government is persuaded during or following litigation to amend its approach, these avenues will not necessarily result in increased funding or new approaches to decision-making.

A common theme running through this review of cases is the policy-heavy component of health care allocation decisions. One can expect that, in response to all types of claims, the government will put forward a defence which emphasizes the policy character of the decision. Policy aspects may be used to justify *Charter* infringements, militate against the finding of a duty of care, exempt decisions from procedural fairness requirements, and tilt the standard of judicial review to the deferential “reasonableness” standard. Courts frequently articulate the belief that policy making is best left to the government. Thus, health care coverage decisions, infused with policy and financial considerations, are seen to be in the government’s wheelhouse. Judicial deference means that for patients with rare diseases it will be difficult to use the above legal avenues to establish an entitlement to funding for a particular medical service.¹⁷⁵ Deference is not absolute however; courts will intervene to ensure compliance with the Constitution or to correct a discriminatory decision. Whether a case is likely to be successful ultimately comes down to the individual facts of the case.

Before choosing litigation, patients with rare diseases should be aware that these sorts of claims may impact decision-making in unintended ways. As Ries and Caulfield observe:

175. Flood & Zimmerman, *supra* note 19 at 27 (Flood and Zimmerman suggest that, because coverage decisions are often unsystematic and arbitrary, courts should not be quick to accept these decisions).

[S]ome of these mechanisms may undermine accountability by pushing complex policy decisions into courtrooms where attention will necessarily focus on the circumstances of individual litigants, perhaps to the exclusion of broader consideration of competing demands on public resources. In addition, successful claims may accord greater status to certain therapies by enshrining public funding for them as a fundamental human or constitutional right. As a result, governments may be compelled to reallocate funds to those specific services and reduce financial support for other programs or services that have not been the subject of litigation.¹⁷⁶

Patients with rare diseases are not one “group” of patients, but many groups of patients with different health care needs. While patients who share an illness may understandably seek to “constitutionalize” funding for a particular treatment, this may not be the optimal approach once the welfare of all patients with rare diseases is taken into account. Thus, in addition to the practical hurdles discussed in this paper, strategic choices may deter patients from using the court system.

On the other hand, litigation has benefits. If successful, patients may free themselves from financial hardship and associated stresses and worries. Further, even if a claim does not prevail in court, taking legal action may prompt governments to re-consider resource allocation decisions in the health sector, especially if lawsuits are combined with political pressure brought by patient groups.¹⁷⁷ Thus legal mechanisms, even if unlikely to be successful, may be useful tools for patients with rare diseases seeking funding for medical treatments. Ultimately, patients must weigh the advantages and disadvantages of litigation, determine their ideal outcomes, assess their probability of success, and consider the possible consequences of the suit before they decide to pursue a claim. If they decide to proceed, this paper has offered a brief summary of the legal mechanisms available to them.

176. Ries & Caulfield, *supra* note 26 at 33.

177. *Ibid* at 9.