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Title: The financial and travel burden to access care for Canadians with scleroderma: results of a cross-sectional survey
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Reviewer 1: Mr. Frank Gavin
General comments (author response in bold)

1. Studies such as this are much needed. For several years, many people (including me) have noted that while the costs incurred by the healthcare system in treating patients with particular conditions can be broken down into a nickel a month for this and a dime a week for that, the costs to patients and families, while sometimes vaguely acknowledged, are neither well-understood nor taken sufficiently into account by clinicians, policymakers, and HTA bodies.
We appreciate the reviewer's comment. We agree that more rigorous research is needed to quantify the economic burden on patients and caregivers.

2. The purpose of the study is clearly described as are the 120 responders to the survey that yielded the results. Most of the limitations of the survey and the study—such as the fact that nearly all the responders live in just three provinces (BC, ON, and QC)—are clearly identified. That the "cross sectional nature of the survey" made it impossible draw any connection between travel burden and out-of-pocket costs on the one hand and health outcomes on the other is particularly unfortunate. We might assume there is some such connection, but not knowing for sure there is one and how strong and general it is means that the main "so what?" question remains unanswered.
We agree with the reviewer – one outstanding question from our analysis is whether disparities in access result in poorer outcomes from those living in smaller communities. We are actively discussing how we could go about exploring this question with existing or new data sources.

3. The survey of Canadians with scleroderma about the distance they had to travel to see their treating rheumatologist and the out-of-pocket costs they incurred to do so was attached to a wider survey of scleroderma patients about stem-cell treatments. The authors of the paper do not seem to have considered whether this might have attracted particular types of scleroderma patients to the survey more than it attracted others. Perhaps this is a non-issue—and the two scleroderma patients who were part of the research team may have been well able to identify it as a non-issue—but I found myself wondering if any scleroderma patients not interested in responding to a survey about stem cell treatments would bother making their way to the survey at the heart of this study after first making their way through the survey about stem cell treatments.
We agree that the representativeness of our survey respondents is an important consideration when interpreting the results. Our sampling approach would have missed those not affiliation with the Scleroderma patient groups that we used for recruitment, and the nature of the survey (stem cell treatments) might have resulted in selection bias with respect to who chose to respond (vs declined). We have listed this as the first potential limitation as follows:
Our analysis recruited patients using the mailing lists of Canadian scleroderma patient organizations, and used data from an international survey focused on the preferences of people with scleroderma for autologous stem-cell transplant.

Therefore, our sample may not be representative of the broader population of Canadians with scleroderma, such as those not affiliated with Scleroderma patient organizations

4. I do not think any reader would be at all surprised that people with scleroderma who live much further from their rheumatologist spend more money on travel (and sometimes accommodation) to access care than similar patients who live closer. Still, it is useful to see the actual figures, and some may be surprised by how large the difference is. More surprising, at least to me, was the difference in medical costs incurred by people who live in large communities and people who live in small communities—the latter group's costs being greater. There is no explanation offered for why this is or may be so.

We agree that it is important to understand the magnitude of the burden of out-of-pocket costs borne by patients. With respect to medical costs specifically we feel that the two-part models are particularly helpful in thinking through this issue. For example, the combined estimates from the two part models (Table 3) show that those from smaller urban centers or rural areas have higher mean costs ($2,024 vs $1,818) though this difference is not statistically significant (i.e., the confidence intervals from these estimates overlap). However, the individual two-part models estimates (Appendix B) demonstrate that those from smaller communities are much more likely to incur ANY medical costs aOR=3.56; however, those that incur costs do not incur higher cost expB = 0.83. It's not immediate clear why those in smaller communities may be more likely to report any costs – though this may reflect an access issue where they choose to pay out-of-pocket for access to providers not covered through public insurance.

5. The more serious problem is the lack of clarity about what is covered by the term "out-of-pocket medical costs" and how "additional medical [my emphasis] and non-medical expenses related to your scleroderma/crest such as alternative medicine, wellbeing, or childcare to attend medical appointments" (p. 5) can all be classified not as medical costs but as "other costs." In short, why are "additional medical … expenses" not included as medical expenses? I assume some of the out-of-pocket medical expenses are for drugs not covered, in full or in part, by public or private drug plans, but it would be good to know how large a portion of the medical costs are for drugs. It would also be useful to know what other "medical costs" are being paid for out-of-pocket and why patients from smaller communities are spending more in "medical costs" than are patients from larger communities.

The inclusion of the 'other expenses' category was to capture out-of-pocket costs related to scleroderma that were not captured in other categories. The wording of this question was developed through pilot testing, and we agree that the distinction between 'medical' and 'non-medical costs' is blurry, particularly given that we are interested in costs associated with scleroderma. We did allow respondents to specify what the 'other costs' however analysis of these open text comments is challenging. A cursory review found that these included expenses related to the categories specified in the question, including childcare, massage therapy, vitamins, clothing, and hand cream (not an exhaustive list). We agree that more granularity on medical costs would have been helpful, however we are not able to investigate this with our data.

6. Section 2.2 of the paper ("Statistical Analysis"), especially paragraphs two and three, is very hard to penetrate, but that is not a problem if there is no intention or

assumption that it will make sense to the lay reader, even one with a graduate degree in English and six years' experience as a public member of an expert committee that regularly reviewed reports of clinical trials.
We agree that the methods could benefit from simplification. We have simplified the reporting in this section -particularly the third paragraph.

7. The involvement of the two patient-partners is generally well-described, especially in the completed GRIPP2 checklist that is part of the larger package of materials. I wonder, though, what the authors mean when they write that the patients "supported the analysis and interpretation of the data." I take it that the patients did not actually participate in the analysis and interpretation. Does "supported" mean they did something other than agree with it?
The patient partners supported the analysis and interpretation through discussions with the analyst. For example, we discussed different options for modeling the cost data, and settled on using a 2-part model given that it allows us to explore two different questions: 1) are people from smaller communities more likely to report ANY cost? And 2) Among those who report costs, do people from smaller communities report similar costs? Partners were also included in discussions about which covariates to include in the models (among other things).

5. Section 4 of the paper ("Interpretation") touches on several important issues. One is "virtual care," which of course is especially topical because of the pandemic. If carefully organized and provided, it certainly seems—as the authors suggest—a means of addressing some of the problems experienced by those who live a considerable distance from their rheumatologist. I wonder, though, how feasible the option of "supporting specialists to travel and provide care in smaller communities" really is, especially if the communities are quite remote and small. Another issue is how the burden of out-of-pocket expenses can or should be alleviated. The authors' comment that "one might expect that [a] universal health insurance system would mitigate the impact of out-of-pocket costs" seems a rather oblique way of putting on the table a key—probably the key—component of the solution to the problem the survey responses identify and, to some degree, flesh out. Of course, the health system in Canada—or, probably better put, the health systems within Canada—has never focused much at all on such mitigation. Many would say that was never an intention or mandate to do so. Others might say such mitigation is not the responsibility of the health system(s) but of other ministries such as those concerned with taxes or social welfare. Finally, the authors suggest that while they were not able to consider "the value of the time spent by Canadians with scleroderma and their family, friends, and/or caregivers in accessing care" such work is badly needed. They are not referring here only or mainly to lost income due to having to take time off work. It is a topic that merits careful thinking and wide discussion, but we are a long way from figuring out and agreeing on what the value of such time is, certainly in terms of money.
We agree with the reviewer that it is important to consider the feasibility of interventions – such as supporting specialists to travel and provide care in smaller communities. In British Columbia, Rheumatologists routinely travel to communities and hold clinics with patients from the region. We agree with the reviewer that this is not feasible for all communities (some are simply too small) – however even having clinics in larger, regional, communities can reduce the travel burden from those in nearby areas. We also agree with the reviewer that more

work is needed to value patient's time when accessing care. We are actively exploring opportunities to conduct work in this area.

6. Overall, the paper is to be welcomed for covering ground too little trod upon. What it most needs in the short run is greater clarity about out-of-pocket medical expenses and out-of-pocket non-medical expenses. Down the road a bit the effect on key health outcomes of the need to travel considerable distances to access care and to pay substantial out-of-pocket expenses should be assessed. Once this effect is known, the solutions should be easier to identify and apply.
We appreciate the reviewers comment that this is an important area for research, and echo the sentiment that future research is needed to understand whether patient borne costs have a detrimental impact on quality of life.

Reviewer 2: Dr. Christopher Longo
Institution: McMaster University
General comments (author response in bold)

Comments to the Author

1. This is a well written paper and an important contribution to our understanding of the financial burden on patients with scleroderma, and the relation to community size. I found mostly minor issues which I will outline below, but felt that an earlier (intro) and clearer definition of community size would have been helpful. I also wonder if the small community in an urban setting is appropriate to lump with the rural settings, but perhaps this was related to sample sizes.
We appreciate the reviewers' comment. We have expanded our discussion of community size in the methods section, please see author response to item 31 below.

Minor items (note: There are two sets of numbers and page references. I am using the pdf page references, and numbers to the far left closest to the papers edge when two set of line numbering are present)

2. Pg 5 of 35 lines 19-23 I find this sentence reads oddly. In the middle of the sentence it reads "…had increased costs of reporting any (OR=2.72….." . As the sentence ends with "travel costs" I assume the reader is supposed to interpret this as "any costs" but think it would read better just to say "any costs". This repeats in the abstract and the result so all versions should be adjusted. Pg. 8 of 35 lines 48-51 As above (Pg 5 lines 19-23) reword the sentence
We have modified this language throughout the manuscript. We have clarified that the odds of reporting both medical costs and travel/accommodation costs were elevated for those in smaller communities, as follows:
"…on average, those in smaller communities had increased odds of reporting any medical costs (aOR = 3.56, 95% CI: 1.51-8.86) and any travel/accommodation costs (aOR = 2.17, 95% CI: 0.99-4.87) (Appendix B)."

3. Pg. 5 of 35 lines 23-24 and 26-27 I realize that the abstract does not have room for explanations but I do find the term "small communities" somewhat problematic as a descriptor. You have three groups of small communities with arguably different characteristics….I am just left feeling the size of the community is not the defining characteristic across the three sub-groups in this cluster. Pg. 6 of 35 lines 39-40 This is

where a more fulsome discussion of smaller versus larger communities could be described, and if not here in the methods.
We agree that discussion about our exposure of interest is warranted. We have clarified that our framework for this variable comes from Statistics Canada, and is known as the Population Centre and Rural Area Classification. We have modified language throughout to emphasize that our binary variable is exploring the comparison between those living in large urban centres (>100k population) and those living in smaller urban centres and rural areas (<100k population).

4. Pg. 7 of 35 lines 21-26 I think more detail is needed here, I note the word "annual" for costs but not clear on travel distance and how it is incorporated. Are you capturing the number of trips in a year, and if so is this an extrapolation or are patients estimating the number of trips in the last year? Pg. 7 of 35 lines 32-34 This would be the second possible location for a more fulsome explanation of why you combined the way you did in defining small and large communities.
At the suggestion of the editorial team, we have removed the analysis that focused on travel distance and focused exclusively on costs. To answer the reviewer's question, the survey asked patients directly how far they travelled to their rheumatologist. The wording was: "How far to you have to travel to see your main specialist (e.g., rheumatologist) for the care of your scleroderma/CREST syndrome? Respondents were then able to specify the distance (in KM or miles).

5. Pg. 9 of 35 lines 10-12 Related to Pg 7 lines 21-26 if the total costs are a mixture of actual costs and extrapolations you should be clear about this. If both were collected prospectively or in the same manner no changes are required.
We have clarified that the 'total cost' estimates were generated by added up medical, travel/accommodation, and other non-medical costs. We have also added in the actual question used on the survey which asked: How much do you normally payout of pocket each year toward the cost of your scleroderma/CREST syndrome for any [medical expenses/travel and accommodation/other expenses]?"

6. Pg. 9 of 35 lines 44-45 reword "cost in access care" to either "cost in accessing care" or "cost in access to care"
We have updated this text.

Reviewer 3: Jennifer Barton
Institution: Veterans Affairs, Portland Oregon
General comments (author response in bold)

Trenaman et al present the results of a cross-sectional survey of financial and travel burden to access care among Canadians with scleroderma. Scleroderma is a rare condition and fully understanding the impacts of travel and cost on patient experience and outcomes is important and can inform future interventions to surmount these potential barriers/challenges to equitable care for all patients. The inclusion of patient partners in this study is a strength.

Abstract.

7. Background – it is not clear why scleroderma is focus of this study (though that becomes apparent later) from the abstract

We have modified the first sentence of the abstract to highlight why patient-borne costs are particularly relevant to those living with scleroderma.

8. Results – are these costs all Canadian dollars? Would clarify.
We have clarified in both the abstract and the manuscript that costs are in 2019 Canadian dollars.

9. Interpretation – is it possible to link this to financial/travel impact to health outcomes (or state if not)?
We agree that it would be interesting to explore the relationship between financial impact/travel burden and health outcomes. However, with our current data we are not above to answer this question. As per our response to reviewer 1, we are actively discussing opportunities to explore this question.

Introduction

10. Page 5, line 11-12: please clarify if this statement about cost-related non-adherence being reported by 10% of respondents was in a general population (this is not scleroderma, correct?) Page 5, line 14 – again please clarify what medical conditions (if any) in stated population
Both of these evidence sources come from the general population and are not specific to scleroderma. The first comes from respondents to the Canadian Community Health Survey which is designed to be representative of the general population. The latter comes from a survey of individuals living in rural/remote areas of BC who reported traveling for care in the past two-years. We have clarified this as follows:
"For example, a recent survey of 381 British Columbians living in rural and remote regions who travelled to access healthcare found that the average travel distance and costs for one episode of care were 1,966km and $777, respectively."

Methods

11. Self-reported scleroderma and ascertainment of participants for the study are both limitations (consider adding to limitations section). Patients who participate in patient organizations may not represent the actual population with scleroderma (in terms of demographics, socioeconomic status, etc.). Also, please clarify if the survey was also in French (or English language only). Were medications included in any of the analyses?
We have emphasized the limitations of our sampling strategy with respect to representativeness of the overall population with scleroderma. We have clarified in the methods that the survey was available in both English and French.

Results

12. How did the 120 participants included in the final analysis differ from those not included (158 were not included even though they completed the survey? This likely from incomplete data but would state if there were any demographic differences between the two groups
We excluded the 158 respondents because they did not meet our inclusion criteria – which required being Canadian. We included all Canadians, and overall had relatively low missingness. We used multiple imputation to impute missing data.

13. Page 7, line 23 – please clarify if these are adjusted odds ratios
We have indicated that these are adjusted odds ratios (aOR) – in addition the Table in Appendix B indicates that the ratios come from multivariate models

c. Was there any data related to medication use or clinic visits?
Unfortunately, we do not have detailed data on medication use or clinic visits in our sample.

5. Interpretation – thoughtful and well written discussion. Quote by Russell is indispensable.
We thank the reviewer for the comment. We agree that this analysis is just a first step, and future work should consider the value of patients' time in accessing care and treatment.