A novel patient-reported outcome for paediatric localized scleroderma: A qualitative assessment of content validity

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What is already known about this topic?

No current health-related quality of life (HRQoL) measures have been creating using direct input from children and adolescents with localized scleroderma (LS). When compared to qualitative reports of HRQoL impact in youth with all LS subtypes, no existing patient-reported outcome (PRO) measures have appropriate content validity for individuals with paediatric localized scleroderma.

What does this study add?

This study proposes a novel LS-specific PRO and is the first qualitative assessment of content validity for any PRO measure in this population. Results from cognitive interviews with children and adolescents support the content validity of the newly developed item set and its ability to capture HRQoL impact in a clinical context.

What are the clinical implications of this work?

Incorporating a content-valid PRO of HRQoL impact into clinical practice would allow for the valid, on-going capture of patient experience in localized scleroderma. Although content validity is an important and necessary step in the process of evaluating validity, items within this novel measure will undergo additional psychometric evaluation before implementation in research and clinical settings.

Keywords: Patient-reported outcomes, survey development, content validity, localized scleroderma (morpha), paediatrics

Abstract

Background: According to current standards, no existing patient-reported outcome (PRO) measures have high quality validity evidence for use with individuals diagnosed with paediatric localized scleroderma. This lack severely hinders patient-centred LS-focused research, including much needed clinical trials.
Objectives: To develop a valid health-related quality of life measure for individuals with paediatric-localized scleroderma (LS) and to qualitatively evaluate its content validity using a patient-centred approach.

Methods: Previously collected qualitative data from youth with LS and their caregivers was used to develop items. The resulting item-set was administered in a clinical setting to participants aged 8-18 years old. Cognitive interviews were used to evaluate time to survey completion, readability/understanding of the items, appropriateness of the recall period, and construct representation.

Results: Seventeen children and adolescents with LS participated in the study. Interviews supported readability, understanding of the items, and appropriateness of the recall period in individuals >10 years old. Revisions were made to simplify the instructions and to be more inclusive of different subtypes of localized scleroderma. Three items were added to improve content representation.

Conclusions: Content validity was supported by the patient-centred development process of the outcome measure and via direct feedback from individuals with LS and their families. Although an important first step, the resulting PRO, termed the “Localized Scleroderma Quality of Life Instrument”, should be further evaluated in a larger sample before being implemented.

The lack of validated patient-reported outcome measures (PROMs) for paediatric localized scleroderma (LS) has limited our understanding of the experiences of these individuals, as well as restricted the design and implementation of LS-focused research. The goal of this project was to develop a disease-specific survey to accurately capture health related quality of life (HRQoL) in children and adolescents with LS.

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Background

LS, otherwise known as morphoea, is an autoimmune condition resulting in lesions of the skin and subcutaneous tissue.\(^1\) It is a rare condition, with the overall incidence estimated to be 2.7 per 100,000 people per year\(^2\) and typical onset around school age.\(^3\) LS has two disease phases: 1) an initial ‘active’ inflammatory phase, where lesions are erythematous and expanding, and 2) a later ‘damage’ fibrotic phase characterized by sclerosis and atrophy of the skin, connective tissues, and underlying fat and bone.\(^4\) The duration of the active phase can vary, but recurrence rates following remission are high for individuals with both paediatric- and adult-onset,\(^5\)-\(^7\) and children diagnosed with LS continue to be impacted well into adulthood.\(^8\)

Studies exploring outcomes in LS have been mixed;\(^8\)-\(^13\) with some concluding that individuals with LS have only minor impairment in HRQoL\(^14\) while others support more severe impacts.\(^15\) Along with methodological limitations (such as small sample sizes), these conflicting results could partly be attributed to problematic PROMs. An informal literature search of studies from 2000-2018 revealed ten different PROMs were used by LS researchers to evaluate HRQoL (Table 1), and to date, no \textit{qualitative} studies have been performed to evaluate content validity evidence in this population. Furthermore, poor results have been found in \textit{quantitative} psychometric studies.\(^16\) It is not surprising to clinicians that existing surveys, which focus primarily on skin symptoms, perform inadequately in this population, as patients with LS tend to experience musculoskeletal issues or extracutaneous manifestations in addition to skin involvement.\(^17\) These areas of impact make the experience of having paediatric localized scleroderma a unique one that has only recently been qualitatively described in the literature.\(^18,19\)
Building from formative qualitative work, we developed a set of items that captured important areas of HRQoL impact in children and adolescents with LS. Care was taken to ensure that the survey development process was highly patient-centered. Qualitative feedback from individuals and their families was used to evaluate content validity of the item set.

METHODS

Methods for survey development were based on guidance from the Food and Drug Administration (FDA), the International Society for Quality of Life Research (ISOQOL), the International Rare Diseases Research Consortium, and The Standards for Educational and Psychological Testing. Methodology from the Patient Reported Outcomes Measurement Information System (PROMIS®) was also heavily referenced. Collectively, these provided a conceptual framework for the measurement development process, which incorporated a 3-phase mixed-methods design including a qualitative description study (Figure 1).

Phase 1: Development

1a. Development of conceptual framework. The conceptual framework for the items was developed over a period of four months. Four major theoretical domains of HRQoL impact were identified through published literature (via an unstructured literature review), content included in existing questionnaires (as listed in Table 1), and were confirmed via qualitative focus groups with paediatrics patients and their caregivers. A deeper exploration of the paediatric LS focus group data confirmed salience of the four domains and offered detail into important attributes within each domain.

1b. Item development. Items were generated based on the four theoretical domains of HRQoL impact. Wording and terminology for the items was heavily reliant on quotations from the
focus groups. Each item went through multiple rounds of review by the study team to ensure the wording was simple and appropriate for children (i.e. substituting complex words), slang was avoided (including medical jargon), and no items were double-barreled.

1c. Other survey characteristics. Care was taken to ensure appropriateness for children and adolescents in regard to the recall period, instructions, and response options. In developing the draft survey, the intended use of the item set, duration and stability of HRQoL concepts, and characteristics of LS were considered.

Phase 2: Administration of items and qualitative study

Sample. Participants were recruited from one specialized North American scleroderma clinic via the National Registry of Childhood Onset Scleroderma (NRCOS), one of the largest registries of its kind in the United States. The NRCOS includes standardized data and biological sample collection, and subjects can agree to be contacted for future research studies. For this study, participants who were actively being seen in the specialized clinic were purposely sampled from the NRCOS to ensure at least 5 participants in each age group (8-10, 11-14, and 15-18 year olds). All procedures performed in studies involving human participants were in accordance with the 1964 Helsinki declaration and its later amendments or comparable ethical standards. The University of Pittsburgh Institutional Review Board approved this study and informed consent (and assent for minors) was obtained from all individual participants.

Administration. Standardized administration of the item-set occurred within a private room during the participant’s regular clinic visit. Participants were encouraged to ask questions while completing the survey, and special care was taken to assure children that there were no right or wrong answers. During administration, the facilitator documented the time it took for each child to complete the survey and any specific questions that the child asked. The facilitator was extensively trained in good research practices, informed consent,
interviewing paediatric/high-risk populations, and building rapport. In addition, the facilitator was known to some participants through her previous position as clinical research coordinator of the NRCOS, during which she regularly met with families at their normal clinical visits to complete relevant forms.

*Cognitive Interviews.* After survey completion, the facilitator (CKZ) performed cognitive interviews using the probe technique by asking questions to elicit detailed information.26 Specific probes of interest included readability and understanding of the items, understanding of the response options, appropriateness of the recall period, construct under- and over-representation, and importance of specific domains/items.27 To record data, the facilitator captured the individual’s answers *in vivo* using standardized, formal notes that were expanded immediately after the clinic visit (the interviews were not recorded or videotaped). Parents were asked to remain silent during the interview to allow children to answer independently but were invited to express their own opinions afterward.

To limit the duration of the interview, only a select number of items were discussed with each child. Items were varied using a spiral technique (6 items per participant) to ensure coverage for every theoretical domain within each child and every item within each age group. The interview was further shortened for the youngest participants (8-10 years old). Prior to the self-report questions, all children were administered a set of anchoring vignettes that were part of a larger study (data not shown).

*Analysis.* The total time to complete the survey was calculated for each participant and the minimum and maximum times were reported. The numbers of skipped items and duplicate responses were tallied. For qualitative data, the facilitator aggregated major themes by probe using an applied thematic analysis approach.28 To keep responses anonymous, all pronouns used in the subsequent sections will be female, regardless of the participant’s self-identified gender.
Phase 3: Revisions

Items were candidates for revision if several participants asked for clarification while completing the survey, the item was reported to be irrelevant by several participants, or an item was frequently skipped (as recommended by current FDA guidance). All item revisions and modifications to the survey format were tracked and reported in the results.

RESULTS

Phase 1: Development

1a. Conceptual Framework & Item Development. Four important areas of HRQoL impact were identified: (1) skin sensations, (2) physical functioning and musculoskeletal sequelae, (3) body image and social support, and (4) medication side effects (Figure 2). Transcripts from the focus groups were heavily relied upon to determine both salience, dimensionality, and importance of each domain (Table 2), by evaluating how often each topic was discussed, how much detail was included by participants, and the intensity of their feelings around each domain.

1b. Item development. The conceptual framework was used to develop corresponding items (Table 2). A total of eighteen items were developed for the first three theoretical domains (Skin sensations=3 items; Physical functioning and musculoskeletal sequelae=8 items; Body image and social support=7 items). The medication domain included another 11 items, but designed to be only administered if participants were currently taking systemic medications (i.e. methotrexate and/or corticosteroids).

1c. Other survey characteristics. When responding to items, participants were asked to reflect on how they felt during the past seven days. This period was chosen as a way to obtain a snapshot of the individual’s experiences at the general time of their clinical visit. We
hypothesized that increasing the recall period to a month would provide less reliable self-report, as it would be harder for children to reflect over 30 days compared with a week. In addition, LS disease features and treatment side effects often are long lasting and should not change significantly within the chosen timeframe, although the appropriateness of the timeframe was evaluated during Phase 2.

The interest was not only the amount of LS symptomatology present, but the extent to which the symptom bothered the individual. Thus, the response options were evaluative in nature and specified to be (0) does not bother me, (1) bothers me a little, (2) bothers me a medium amount, and (3) bothers me a lot. A four-point response scale was chosen, as its utility with children has been supported in prior research.29

Phase 2: Administration of items and qualitative study

Demographics. Demographic characteristics of the sample (Table 3) mirrored other published cohorts.30-32

Time to completion. The time to complete all items ranged from 1 to 10 minutes, with the majority of participants (13/17; 76%) completing all items (including the medication subscale if applicable) in <6 minutes.

Problems with administration. No items were skipped and there were no duplicate responses.

Readability. All participants >10 years old could read the questions aloud, but there were some observed issues with readability in the youngest children (<10 yo). Most issues were minor and related to clarification. One participant struggled with the word ‘scleroderma’ and another with the word ‘aches’. Once these individuals received an answer from the facilitator, they were able to successfully complete the survey.
Two of the youngest participants asked their parent to read the questions aloud, which meant that the parent also prompted a few of the answer choices. For at least one child, the facilitator felt that the request seemed to be related to fatigue but not ability, as the child had no trouble reading the items aloud once the parent declined assistance.

Understanding. All individuals in the oldest age group clearly described the item in their own words. Often their understanding of the item went beyond the listed examples, and individuals talked about their internal interpretation or other important examples from their life. For example, one participant talked about her skin pain as a “burning” feeling (item: painful skin where scleroderma is). Individuals 11-14 years old had very brief answers when compared to their older peers, reflecting low LS impact (responses=‘not bothered’). However, participants in this age group still indicated understanding of the items via talking about how their answers might have been different when first diagnosed.

Understanding was mixed in the youngest age group (8-10 yo). The two 10-year old participants had no issue describing the concepts behind each LoSQI item. For example, both were asked about ‘feeling different’ because of their LS, and had very detailed, personal responses (item: feeling different than other people because of my scleroderma). One said that she felt different but that she actually enjoyed that feeling. The other indicated she felt “a little different” and kept her lesions hidden to minimize that feeling. In contrast, the three youngest participants (<10 yo) were able to articulate understanding for only some items. When prompted, one individual was unsure if her skin was itchy although she responded ‘bothers me a little’ to the LoSQI item (item: itchy skin where my scleroderma is). Another child also indicated she could not understand longer questions, like item: problems using my hands when I write, text, or type for a long time. Another individual provided very short answers to the administrator during the interview, and it was unclear to the administrator if she was tired after her clinic visit, shy, or was unable to understand the items.
 Response process. Participants in all age groups who indicated understanding were also able to articulate the presence, and frequency, and how bothersome the symptom was. For example, one individual mentioned that she experienced itchy skin, but it did not bother her because she was “used to it” (item: *itchy skin where my scleroderma is*). Another child reported that she chose the response option ‘bothers me a little’ because her skin around her lesion “feels weird” (item: *uncomfortably tight skin where my scleroderma is*).

 Appropriateness of recall period: All participants who articulated understanding of the items indicated that their answers would not have changed if asked to reflect over the past 30 days. 

 Under-representation of the construct. There were five concepts that participants felt were important but were not included in the draft survey (Table 4). 

 Over-representation of the construct. There was disagreement among participants about potential duplicate items. Two individuals considered bullying and teasing to be the same, while another specifically stated that she was teased but not bullied about her LS. The interviewer’s impression was that ‘bullying’ had stronger negative connotations to her.

 Two participants thought the skin sensation items were repetitive. In contrast, other individuals clearly distinguished between pain, itch, and tightness, and some discussed more specific sensations like “burning”, “soreness”, or “stabbing”.

 One participant reported that the item: *problems using my hands when I write, text, or type because of my scleroderma* was the same as the item: *problems using my hands when I write, text, or type because of my scleroderma for a long time*. This was directly contrasted to another individual who felt the two questions were different, stating she typically uses her hands without issue but has significant problems after prolonged movements.

 Feedback related to medication side effects. There was evidence that participants were not linking items on the medication side effects scale with their medications. This seemed most salient for items asking about general symptomatology that could be related to multiple
causes (i.e. stomach pain, not feeling like eating, feeling in a fog, fatigue, and headaches). For example, a participant chose the response option ‘bothers me a lot’ regarding item: feeling hungry all the time. When the administrator asked if she thought this was related to her medications, she said, “I’m just growing.”

Other Miscellaneous Results.

Inclusiveness of survey language. During interviews, the facilitator noted variation in how participants and their families talked about LS and which terms they used. Although not specifically probed, children, adolescents, and their parents reported using terms like ‘lesion’ and ‘skin’, as well as medical terms like ‘morphea’, ‘Parry Romberg syndrome’, ‘en coup de sabre’, and ‘scleroderma’. Parents of some of the youngest participants said that they do not typically discuss LS with their children, and thus, their children lack a vocabulary for some impacts of their disease and treatment.

Potential social desirability bias. One participant in the 11-14 age group indicated minimal impact of LS through her item responses and interview. However, her parent mentioned that LS bothered her much more than she discussed with the facilitator; itch bothered her at night and she recently requested a new bathing suit that covered more of her affected skin.

Respondent fatigue. The youngest participants (8-10 yo) were most at risk of respondent fatigue. One of the youngest participants was seen 3 hours after her appointment began, and the facilitator indicated that her interview was the most disjointed and hardest to perform.

Summary of Results. A summary of results across the age groups can be found in (Table 5). There was evidence for readability and understanding in the older participants (>10 yo) but more variability in younger individuals.
Phase 3: Revisions

Based on participant and parent suggestions, 3 items were added to the survey (Table 4). Instructions were simplified for clarity and to encourage participants to read them in their entirety. Minor formatting changes removed lines between items, added white space, and synchronized formatting. To ensure inclusion, other names and descriptions for localized scleroderma were added to the instructions. A qualifying statement was added to all medication subscale items reminding individuals to only consider symptoms specifically related to their LS medications. Although debated, no items identified as repetitive were removed based on contrasting feedback from participants.

DISCUSSION

This study describes initial evaluation of the first patient-reported outcome measure designed specifically for individuals with paediatric LS. In addition, we report the first conceptual mapping of important areas of quality of life impact in paediatric for all subtypes created specifically for development of a PROM. Termed the ‘Localized Scleroderma Quality of Life Instrument’ (LoSQI), support for the PROM measure was strengthened by the mixed-methods study design utilized in development, the strong reliance on a conceptual model developed from focus groups with patients and caregivers, as well as through enrolment of individuals with diverse LS subtypes. Taken together, the results and subsequent revisions generally support content validity of the items to measure HRQoL impact in a clinical context.

Cognitive interview findings upheld the theoretical conceptual framework, though there were minor indications of construct under-representation. Three items were subsequently added based on participant feedback. The conceptual framework was also supported by independent qualitative work from a separate institution.19 Although only
individuals with facial involvement participated (compared to a mix of subtypes in the focus groups that were used to inform this PROM development), themes were nearly identical. Specifically relevant, its participants also discussed negative questioning from peers, bullying, and adverse side effects from systemic medications.¹⁹

The time to completion of the items was faster than anticipated and there were no administrative problems, such as skipped items or duplicate responses. This could be due to familiarity with completing PROMs or the close facilitator supervision. Close supervision, although generally helpful for evaluating new surveys, may not be needed in future contexts, at least with older children. However, we recommend that time to completion be explored at other sites with individuals who might not be as familiar with self-report.

The FDA recommends use of narrow age bands to assure developmentally appropriate item wording; however, reading level for the items was targeted around grade 5 so items would be applicable across all age levels, similar to the approach used by PROMIS.³³ We observed some lack of understanding in the youngest participants (<10 yo), who were also most susceptible to respondent fatigue. To limit these issues, younger children require continued close supervision by a facilitator and administration of the tool early on in their clinic visit, and we recommend that alternative approaches be explored for children <10 years old.

Also of note, during the interview the facilitator discovered that some participants were responding to symptoms described in the medication side effects items that were not attributable to their medications. This issue is likely to endure, as most systemic medication side effects are also general symptoms related to other conditions, such as anxiety. During revisions, a qualifying statement was added to all items that reminded participants to only consider symptoms specifically related to their medications, but adequacy of this revision should be further evaluated.
Limitations and Future Directions

Participants were recruited from the NRCOS and were all established patients with relatively well-controlled LS. To completely discuss certain items, we had to rely on individuals’ memories of how their life was affected earlier on in their diagnosis. It is important to confirm these findings in a more symptomatic sample with more active disease.

Although establishing content validity is an important first step, we caution readers on utilizing the items in their own clinics or in research without further evaluation (and thus, we do not provide a copy of the administrative version of the LoSQI). More detailed psychometric properties of the item set are currently being examined in a larger sample from multiple sites across the USA and Canada. At minimum, test-retest reliability, internal consistency, internal structure evidence, and convergent and divergent validity evidence should be evaluated prior to the items being implemented clinically or for research.

Conclusion

The patient-derived PRO, termed the ‘Localized Scleroderma Quality of Life Instrument’ (LoSQI), was designed as a disease-specific PRO measure for individuals with paediatric LS. Based on the patient-centred development process, qualitative feedback from individuals with LS and their families provided convincing support for content validity. While these initial results are promising, additional psychometric evidence should be collected from a larger sample of individuals before the LoSQI can be implemented in clinical or research contexts.

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like to acknowledge the vital involvement of the participants and their families, whose experiences drive this work.

References


Table 1. Health-related quality of life PROMs used with localized scleroderma patients.

<table>
<thead>
<tr>
<th>Survey</th>
<th>Target Population</th>
<th>No. items</th>
<th>Domains</th>
<th>Author(s)</th>
<th>Citation</th>
</tr>
</thead>
<tbody>
<tr>
<td>CDLQI/DLQI</td>
<td>Dermatological conditions</td>
<td>10</td>
<td>Symptoms/ feelings, Leisure, School/ holidays, Personal relationships, Sleep, Treatment</td>
<td>Lewis-Jones, Finlay</td>
<td>13,34, 11,35, 9,36, 8,37, 14,38</td>
</tr>
<tr>
<td>CHAQ</td>
<td>Juvenile Idiopathic Arthritis</td>
<td>30</td>
<td>Disability, discomfort, pain</td>
<td>Athreya, Fries, Goldsmith</td>
<td>9,15</td>
</tr>
<tr>
<td>CHQ</td>
<td>Generic-Paediatric</td>
<td>87</td>
<td>Physical functioning, bodily pain, role/social-physical, general health perceptions, role/social-emotional behavior, mental health, general behavior, self-esteem, parental emotional impact, parental time impact, family impact.</td>
<td>HealthACT CHQ</td>
<td>9</td>
</tr>
<tr>
<td>Questionnaire</td>
<td>Domain/Medical Area</td>
<td>Items</td>
<td>Authors/Year</td>
<td>Notes</td>
<td></td>
</tr>
<tr>
<td>--------------</td>
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<td></td>
</tr>
<tr>
<td>CQOL</td>
<td>Generic-Paediatric</td>
<td>15</td>
<td>Activities, appearance, communication, continence, depression, discomfort, eating, family, friends, mobility, school, sight, self-care, sleep, worry</td>
<td>Graham, 1997</td>
<td></td>
</tr>
<tr>
<td>KINDL</td>
<td>Generic-Paediatric</td>
<td>24</td>
<td>Physical well-being, emotional well-being, self-esteem, family, friends school</td>
<td>Ravens-Sieberer &amp; Bullinger, 1998</td>
<td></td>
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<tr>
<td>SF-36</td>
<td>Generic-Adult</td>
<td>36</td>
<td>Physical functioning, Role limitations due to physical functioning, Bodily pain, General health perceptions, Vitality, Social functioning, Role-limitations due to emotional problems, Mental health</td>
<td>RAND, 1998</td>
<td></td>
</tr>
<tr>
<td>Skindex-29</td>
<td>Dermatological conditions</td>
<td>29</td>
<td>Emotions, symptoms, functioning</td>
<td>Chren, MM, 1997</td>
<td></td>
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<tr>
<td>ISDL</td>
<td>Dermatological conditions</td>
<td>42</td>
<td>Physical functioning, psychological functioning, stressors, illness cognitions, and social support</td>
<td>Evers, 2007</td>
<td></td>
</tr>
<tr>
<td>Visual Analogue Scale</td>
<td>-</td>
<td>1</td>
<td>Varies based on specific wording of VAS</td>
<td></td>
<td></td>
</tr>
<tr>
<td>“Questionnaire”</td>
<td>-</td>
<td>13</td>
<td>Appearance, social, emotional, symptoms</td>
<td>*</td>
<td></td>
</tr>
</tbody>
</table>

LS = localized scleroderma; CDLQI = Children’s Dermatology Life Quality Index; CHAQ = Children’s Health Assessment Questionnaire, CHQ = Childhood Health Questionnaire; CQOL = Child Quality of Life Questionnaire; KINDL = German generic quality of life tool for children; SF-36 = Short Form Health Survey, ISDL = Impact of Chronic Skin Disease on Daily Life.

*Authors of publication created new survey without reporting their development process or psychometric evidence.
Table 2. Conceptual framework and draft items for the Localized Scleroderma Quality of Life Instrument (LoSQI).

<table>
<thead>
<tr>
<th>Theoretical Domain (Area of Impact)</th>
<th>Definition</th>
<th>Examples from Focus Group¹</th>
<th>Draft Items</th>
</tr>
</thead>
</table>
| Skin Sensations                    | Uncomfortable skin sensations in affected skin; pain, itch, tightness, or soreness | “Mine doesn’t usually hurt, but it sometimes itches.” Parent: “[Redacted] doesn’t have pain, but he does feel it.” | 1. Itchy skin where my scleroderma is  
2. Painful skin where my scleroderma is  
3. Uncomfortably tight skin where my scleroderma is |
| Physical Functioning and Musculoskeletal Sequelae | Limitations on fine motor functions during everyday activities, limitations in gross motor functions during everyday activities, worry or stress related to functioning, and joint or muscle pain. | “They ask…’You’ve been dancing for so long why can’t you do these basic things?’ And [I] tell them that I’m physically unable to.”  
“I’ve been taking standardized tests lately and my hand will cramp…I’m given 25 minutes to write an essay and…[I spend] 5 minutes just trying to wrestle my hand back to normal.”  
“I love playing soccer…when I first noticed it, I would always be careful. I would always be watching everywhere just to make sure nothing would hit me.” | 4. Problems doing active things like running, playing sports, or dancing because of my scleroderma.  
5. Problems using my hands when I write, text, or type because of my scleroderma.  
6. Problems using my hands when I write, text, or type for a long time because of my scleroderma.  
7. Worry about being able to do certain activities because of my scleroderma.  
8. Problems when I am doing fun things like painting or playing an instrument because of my scleroderma.  
9. My muscles hurting where my scleroderma is.  
10. Aches in my joints (like knees, hips, fingers, toes, ankles, elbows) where my scleroderma is.  
11. Stiff joints (like knees, hips, fingers, toes, ankles, elbows) where my scleroderma is.  
12. Feeling embarrassed because of how my body looks because of my scleroderma.  
13. Feeling nervous when I am around new people who don’t already know about my scleroderma. |
| Body Image and Social Support       | Feelings towards their own appearance and body image in relationship to others, negative peer interactions, worry/fear about new | “You wake up and you just look in the mirror and you’re just like what’s wrong? Why is that here?”  
“You just want to stay inside because you |
interactions or questions about their skin/appearance

Parent: “They laugh at her. They make fun of her and it’s terrible.”

“The staring makes me feel different and I used to wear a bunch of long-sleeved shirts and never show my arms.”

14. Feeling different than other people because of my scleroderma.
15. Feeling upset when people ask questions about my scleroderma.
16. Being bullied because of the way my skin, face, or body looks because of my scleroderma.
17. Getting teased about the way I look because of my scleroderma.
18. Covering up my scleroderma with things like long sleeves, long pants, makeup, or retainers.

<table>
<thead>
<tr>
<th>Medication Side Effects</th>
<th>Attitude/feelings towards systemic treatment and impact of possible side effects</th>
</tr>
</thead>
<tbody>
<tr>
<td>“With the methotrexate it would just feel weird, it’d make me throw up, I’d feel dizzy.”</td>
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<tr>
<td>Parent: “Having big puffy cheeks and gaining weight from the steroids. She hated it.”</td>
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<tr>
<td>“In the middle of the week I’d get sick and start throwing up and… then I’d just throw up and then go to school and try to fight through it.”</td>
<td></td>
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<tr>
<td>19. Worry about medication side effects.</td>
<td></td>
</tr>
<tr>
<td>20. Feeling embarrassed that I need to take medications.</td>
<td></td>
</tr>
<tr>
<td>22. Feeling hungry all the time.</td>
<td></td>
</tr>
<tr>
<td>23. My stomach hurting.</td>
<td></td>
</tr>
<tr>
<td>24. Not feeling like eating.</td>
<td></td>
</tr>
<tr>
<td>25. Feeling like I am in a fog or that it’s hard to think clearly.</td>
<td></td>
</tr>
<tr>
<td>27. Having headaches.</td>
<td></td>
</tr>
<tr>
<td>28. Gaining weight because of my medications.</td>
<td></td>
</tr>
<tr>
<td>29. Vomiting when I take my medications</td>
<td></td>
</tr>
</tbody>
</table>

1Focus group quotations were from a related study with children, adolescents, and their parents.  

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Table 3. Demographic characteristics for 17 individuals with paediatric localized scleroderma who were administered the item-set.

<table>
<thead>
<tr>
<th>Variable</th>
<th>n (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Female</td>
<td>13 (76)*</td>
</tr>
<tr>
<td>White</td>
<td>14 (82)</td>
</tr>
<tr>
<td>Age groups</td>
<td></td>
</tr>
<tr>
<td>8-10 years old</td>
<td>5 (30)</td>
</tr>
<tr>
<td>11-14 years old</td>
<td>6* (35)</td>
</tr>
<tr>
<td>15-18 years old</td>
<td>6 (35)</td>
</tr>
<tr>
<td>Subtype</td>
<td></td>
</tr>
<tr>
<td>Circumscribed morphea</td>
<td>3 (17.6)</td>
</tr>
<tr>
<td>Linear of the:</td>
<td></td>
</tr>
<tr>
<td>Trunk/limbs</td>
<td>2 (11.8)</td>
</tr>
<tr>
<td>Head</td>
<td>6 (35.3)</td>
</tr>
<tr>
<td>Generalized morphea</td>
<td>2 (11.8)</td>
</tr>
<tr>
<td>Pansclerotic morphea</td>
<td>1 (5.9)</td>
</tr>
<tr>
<td>Mixed morphea</td>
<td>3 (17.6)</td>
</tr>
<tr>
<td>On systemic medication</td>
<td>12 (71)</td>
</tr>
<tr>
<td>Disease duration (time from diagnosis to study enrollment)</td>
<td>4.6 (&lt;1 to 9.4 years)</td>
</tr>
</tbody>
</table>

*One participant completed the self-report questions but did not complete the cognitive interview.
*One male was included in each age group, with n=2 in the youngest group.
Table 4. Construct under representation as per 16 patients with paediatric localized scleroderma identified during cognitive interviews.

<table>
<thead>
<tr>
<th>Additional Concept Suggested</th>
<th>Specific Participant Feedback</th>
<th>Steps taken to revise survey</th>
</tr>
</thead>
<tbody>
<tr>
<td>Time spent away from school due to doctor’s appointments, occupational therapy, and/or feeling sick.</td>
<td>One patient was very distressed about the time LS took her out of school. All the doctor's appointments and occupational therapy after her initial diagnosis caused her grades to drop ‘a lot’ and she could not keep up with classwork. Her mom mentioned that she had to really advocate for her daughter at the school and let them know what was going on.</td>
<td>Drafted additional item: 30. Having to miss school for doctor’s appointments, therapy, or feeling sick because of my scleroderma.</td>
</tr>
<tr>
<td>Fears related to blood work, lab draws, and/or skin biopsies</td>
<td>One patient mentioned that the survey did not ask about blood work, lab draws, or skin biopsies, but these were very bothersome to her (caused anxiety/fear/discomfort).</td>
<td>Drafted additional item: 31. Getting my blood drawn because of my scleroderma.</td>
</tr>
<tr>
<td>Uncomfortable skin sensation not currently listed</td>
<td>Some patients reported that their skin did not necessarily “hurt” or “itch”, but felt “different” at the site of their lesions and this feeling was bothersome.</td>
<td>Drafted additional item: 32. My skin feeling different where my scleroderma is.</td>
</tr>
<tr>
<td>General questions about living with LS</td>
<td>Another patient wanted more open ended questions to be included in the survey. Particularly, she suggested that we ask about (1) how patients felt when they first got LS, (2) how life changes after symptoms start, (3) confidence and how that changes around other people, and (4) how people act “towards you about disease”.</td>
<td>None. These questions are important and most of the broad themes are touched upon in the survey items. However, the depth of her suggestions were considered outside the scope of the survey’s intended utilization and not appropriate to its closed-ended format.</td>
</tr>
<tr>
<td>Plastic surgery or consideration of plastic surgery.</td>
<td>A parent of another patient with an en coup de sabre lesion mentioned that there were no questions about plastic surgery or the consideration of plastic surgery. She has found that her daughter was originally uninterested in this option, but now that she was older, she had started to consider it.</td>
<td>None. Body image due to LS may or may not prompt someone to consider plastic surgery interventions. However, there are more direct ways to measure body image (as per study team), and thus, this was not specifically added to the survey.</td>
</tr>
</tbody>
</table>
Table 5. Overview of qualitative results from 16 cognitive interviews of paediatric localized scleroderma patients by age group.

<table>
<thead>
<tr>
<th></th>
<th>15-18 years old (n=6)</th>
<th>11-14 years old (n=5)</th>
<th>8-10 years old (n=5)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Readability of items</strong></td>
<td>No stumbling over any words.</td>
<td>Speed of reading varied</td>
<td>Large variability of reading ability.</td>
</tr>
<tr>
<td></td>
<td>None required help from facilitator/parent (indirectly or through direct questions).</td>
<td>All could read items aloud.</td>
<td>Majority asked questions about the survey directions, specific items, or had trouble with certain words.</td>
</tr>
<tr>
<td></td>
<td></td>
<td>A few had questions related to specific words.</td>
<td>Some asked their parents for assistance with reading.</td>
</tr>
<tr>
<td><strong>Understanding of items</strong></td>
<td>Could describe all items in their own words.</td>
<td>Could describe all items in their own words (even patients who were slower readers or asked for clarity on specific words).</td>
<td>Some had trouble indicating understanding of items</td>
</tr>
<tr>
<td></td>
<td>Nothing they would add to items to make them clearer.</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Response process</strong></td>
<td>Could clearly articulate the option they chose and why.</td>
<td>Could clearly articulate the option they chose and why.</td>
<td>Generally able to describe how they answered the question and why they answered the question.</td>
</tr>
<tr>
<td></td>
<td>Could differentiate between experiencing the symptom vs the symptom bothering them.</td>
<td>Overall low rate of impact in this group, but patients were able to relate back to past symptoms that had/had not bothered them, providing further support for understanding of response process.</td>
<td>One of the youngest patients was unable to explain her answers when probed by the facilitator, even though she had provided answers to all items.</td>
</tr>
<tr>
<td></td>
<td>Could link their chosen response to how much the symptom bothered them.</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Recall period</strong></td>
<td>Appropriate</td>
<td>Appropriate</td>
<td>Appropriate</td>
</tr>
<tr>
<td><strong>Construct under-representation</strong></td>
<td>About half had specific suggestions/areas they would add.</td>
<td>A few patients had additional areas to add to the survey.</td>
<td>None of the patients mentioned additional areas to add to the survey.</td>
</tr>
<tr>
<td><strong>Construct over-representation</strong></td>
<td>Two participants indicated potential repetitive items.</td>
<td>One indicated potential repetitive items.</td>
<td>One indicated potential repetitive items.</td>
</tr>
</tbody>
</table>
Fig 1
Overview of the three phased survey development process

Fig 2
Conceptual framework of important areas of quality of life impact for paediatric localized scleroderma identified through focus groups with patients, clinical experts, and the literature.