A sequential mixed methods study to develop a sarcoma-specific patient-reported outcome measure

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on behalf of the SAM Research Team

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Introduction

- Patient reported experience is central to evaluating quality of care in the NHS
- Incorporating a PROM into clinical practice improves processes, outcomes and satisfaction with care
- Current evidence inconclusive about the psychosocial impact of sarcoma (Storey et al. 2019 Sarcoma)
- Is this because current PROMs do not reflect the experiences of patients with sarcoma?
Background

- Sarcoma: cancer of connective tissue
  - Occur in any location in the body
- Over 130 different subtypes
  - Soft tissue sarcoma, bone and gastrointestinal
- Develops from birth onwards
- Treatment can includes CTx, RTx, surgery, PBTx
- 5 year survival – 55%
- No sarcoma-specific QOL measures
Aims

- Develop and validate a measure reflecting patient experience to guide clinical practice and consultation

- Explore if/how the Sarcoma Assessment Measure (SAM) can be used as an outcome measure
Phases of development

PHASE 1 → Stage 1: item generation

→ Stage 2: item reduction

→ Stage 3: pre-testing

PHASE 2 → Psychometric testing
## Target population

<table>
<thead>
<tr>
<th>Cancer Type</th>
<th>Stage</th>
<th>Metastases</th>
<th>Recurrence</th>
<th>Surgery</th>
<th>Duration of Illness</th>
<th>Type of Treatment</th>
<th>Clinical Trial</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bone</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Limb</td>
<td>1-4</td>
<td>Size</td>
<td>Yes/No</td>
<td>Amputation</td>
<td>&lt;2; 2-5; &gt;5</td>
<td>Chemo for relapse</td>
<td>Yes/No</td>
</tr>
<tr>
<td>Pelvis/spine</td>
<td></td>
<td></td>
<td></td>
<td>Limb preservation</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Craniofacial</td>
<td></td>
<td></td>
<td></td>
<td>other</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>GIST</td>
<td></td>
<td></td>
<td></td>
<td>None or RT</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Surgery or adjuvant Advanced</td>
<td>1-4</td>
<td>Size</td>
<td>Yes/No</td>
<td>Yes/No</td>
<td>&lt;2; 2-5; &gt;5</td>
<td></td>
<td>Yes/No</td>
</tr>
<tr>
<td>Soft Tissue Sarcoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Extremity</td>
<td>1-4</td>
<td>Size</td>
<td>Yes/No</td>
<td>Yes/No</td>
<td>&lt;2; 2-5; &gt;5</td>
<td>RT/ Chemo</td>
<td>Yes/No</td>
</tr>
<tr>
<td>Retroperitoneal</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Head and Neck</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

### Socio demographic variables:
- **Age**
  - (<25; 25-40; 41-65; >65)
- **Gender**
- **Race**
- **Income Level**
- **Education Level**
- **Place of care** (specialist centre)
- **Geography** (distance from follow up appointments)
Stage 1: item generation

- **Aim:** to understand experiences of living with and beyond a sarcoma diagnosis
- **Methods:** semi-structured interviews
- **Participants**
  - N = 121/175
  - 50% male
  - Aged 13 - 82 years
  - STS = 62%, BT = 28%, GIST = 10%
Conceptual basis of SAM

“...subjective, multidimensional and dynamic. It is unique to each individual and includes aspects of physical, psychological and social function. It is dependent upon not only the stage of development but also the illness trajectory. This involves the achievement of goals and aspirations and the constraints imposed through ill health and treatment” (Taylor et al. 2005 JCN)

- Physical wellbeing
- Emotional wellbeing
- Social wellbeing
- Financial wellbeing
- Sexuality
Stage 2: Item reduction

- Content analysis: 1,405 individual items reflecting experience after diagnosis
- Review by research team (researchers, clinicians, patient representatives)
  - Eliminate redundant items, group with similar meaning
- Item Reduction Questionnaire (n = 395 items)
  - Two scales: importance and frequency
Patient expert review

- Participants
  - n = 250
  - 51% Male
  - Aged 17 - 89 years
  - STS = 59%, BT = 36%, GIST <1%
- Calculated an impact score (importance + frequency)
- Top scoring impact items retained (≥5/≥6 for emotion)
Item reduction results

- $n = 160$ items
- Compared against existing PROMs
  - Removed 22 similar items
- Consensus review by research team
- $n = 66$ items to include in the Content Validity Questionnaire (CVQ)
Stage 3: pre-testing (content validity)

- CVQ administered online to patients and healthcare professionals
- Patients = 33
  - 42% male
  - Aged 19 – 82 years
  - STS = 58%, BT = 39%, GIST = 3%
- Healthcare professionals = 24
  - Nurse (11), Oncologist (9), Surgeon (2), Therapist (2)
Content validity results

- Items with a CVR score < .31 were discarded
- 22 ITEMS identified
  - Physical wellbeing: 7 items
  - Emotional wellbeing: 10 items
  - Social wellbeing: 3 items
  - Financial wellbeing: 1 item
  - Sexuality: 1 item

  - The wording of final set of items was reviewed and changed to fit the proposed response variable (strongly agree – strongly disagree)
Pre-test: Establish comprehension

- Interviews with patients
  - Can patients understand the items?
  - Can patients answer the items?
  - Are questions too sensitive?

- Participants:
  - n = 10
  - 40% male
  - Aged 27 - 56 years
  - STS = 2, BT = 7, GIST = 1

- Minor changes were made to 4/22 items
**SECTION 1: SARCOMA ASSESSMENT MEASURE (SAM)**

We are interested in your experience of being diagnosed and living with or beyond sarcoma. Thinking about your **current situation** please answer how much do you agree or disagree with the following statements by circling the number that best applies to you. If a statement does not apply to you, please circle **N/A** (Not Applicable).

<table>
<thead>
<tr>
<th>Strongly disagree</th>
<th>Disagree</th>
<th>Neither agree or disagree</th>
<th>Agree</th>
<th>Strongly agree</th>
<th>N/A</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. I do whatever I can to keep healthy</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>2. I am more conscious of what I eat since I was diagnosed with sarcoma</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>3. I can do everything without help</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>4. My amputee is not as strong as it was before diagnosis</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>5. My prosthesis is heavy and uncomfortable</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>6. I can still do the things I want</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>7. My painkillers don't take all the pain away</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>8. I worry about whether I will be able to have a family</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>
Phase 2: Testing SAM

- Reliability & validity
  - SAM, QLQ-C30, TESS, GRCQ
- Sample: 500 – 1,000 patients
  - Second SAM to 400 patients
- Recruitment: March – May 2019
  - 15 Trusts in the UK
  - CPES participants
SAM phase 2 participants

- N = 762
- Male/female = 349/407 (46%/54%)
- Ethnicity white: 714 (95%)
- Age: median 67 years (IQR 55,75)
- Time from diagnosis: 4 years (IQR 2,6)
- STS: 505 (66%); BT: 158 (21%); GIST: 78 (10%)
- Surgery alone: 274 (37%)
- On treatment: 169 (24%)
Is SAM valid?
What next?

- Is this a good measure of outcome?
  - Explore sub-populations

- Secondary data analysis
  - Route to diagnosis (*funded by Sarcoma UK*)
  - In-depth understanding of experience in sub-populations

- SAM-Paed (*funded by CCLG*)
  - Child self-report (8-16 years); parent report (0-16 years)
  - Rhabdomyosarcoma (FaR-RMS) study

- ICONIC (*funded by BCRT*)
  - Test ability to detect change longitudinally
SAM Research Team

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Research Associate
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