PROVIVO: Patient Reported Outcomes in view of symptom experience of late effects and self-management of adult long-term survivors after allogeneic haematopoietic stem cell transplantation – A mixed methods study

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Background

Allogeneic haematopoietic stem cell transplantation (SCT) is associated with a number of late effects in long-term survivors. Symptom experience and self-management are important yet poorly investigated factors in this patient population.

- Symptom experience
  - SCT is associated with a life-long increased risk for developing various adverse side effects; also termed ‘late effects’ (1,2)
  - Health care workers (HCW) systematically underestimate patient’s symptom experience
  - Symptoms related to late effects are distressing and burdensome to patients (3)
  - So far, no such Patient reported outcome instrument was available to assess symptom experience in long-term survivors after SCT
  - Such a PRO instrument should be developed starting from the Patient-Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE) from the National Cancer Institute (4,5)

- Self-management
  - Patient self-management is a key concept for chronically ill patients and has been linked with clinical outcomes
  - Patient self-management after SCT consists of 3 main tasks (6)
    - Managing emotions (e.g. worry, uncertainty)
    - Managing new life roles (e.g. re-uptake of job)
    - Managing medical & health tasks (e.g. taking medications, smoking cessation, regularly exercising and monitoring for signs of infection)
  - Patient self-management after SCT has not yet been described in SCT

Aims:

- To develop a PRO instrument to assess symptom experience related with late effects in SCT.
- To validate the newly developed scale and assess its psychometric properties
- To examine self reported symptom experience and objective measured late effects in 300 adult survivors ≥ 1 year after SCT
- To describe self-management behaviours in 300 adult survivors ≥ 1 year after SCT
- To assess the relationship between symptom experience and objective burden of late effects, survivors’ perception of late effects and self-management in view of managing emotions, managing new life roles and managing medical and health tasks in adult survivors after allogeneic SCT for haematological disease

Methods:

Design

- Sequential exploratory mixed methods design consisting of three phases and involving patients from two Swiss SCT centres

Phase I: Instrument development

- Culturally-sensitive translation of the PRO-CTCAE items into German
- Pre-selection of items via (a) cognitive debriefing interviews in 15 patients
  - (b) by experts’ opinion and evidence on a literature review
- Refinement of questionnaire by a second round of cognitive interviews in 15 patients
- Content validity index (CVI) testing in experts: Excellent scale CVI (0.94) and item CVI’s (median = 1; range 0.75-1).

- Inclusion criteria
  - Age ≥ 18
  - ≥ 1 year of follow-up after SCT
  - Ability to understand and read German

- Exclusion criteria
  - Psychiatric illness, visual and/or hearing impairment, being illiterate
  - Hospitalisation or terminal illness

Sample

- Convenience sample of patients attending follow-up visits in the long-term after allogeneic SCT for haematological disease

Phase II and III: Cross-sectional multicenter study

- 374 Patients (45% female, mean age 50.3 (SD 12.8), on median 7 years (3.5-12.2)) participated
- Variables and measurement
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  - Symptom experience of late effects (newly developed Scale)
  - Perception of late effects (Brief Illness Perception Questionnaire)
  - Self-management in view of
    - Emotional tasks (Hospital Anxiety & Depression Scale)
    - Medical & health tasks (Basel Assessment of Adherence to Immunosuppressive Medications & single items to different health behaviours)
    - Role tasks (single symptoms to work status)
    - Objective Late effects
      - (graded with CTCAE criteria by physician)
    - Demographical and clinical variables retrieved from records

Table 1: Content of PROVIVO instrument

<table>
<thead>
<tr>
<th>Domains</th>
<th># Items</th>
</tr>
</thead>
<tbody>
<tr>
<td>General well-being</td>
<td>1</td>
</tr>
<tr>
<td>Physical symptoms, including pain</td>
<td>29</td>
</tr>
<tr>
<td>Cognitive &amp; emotional symptoms</td>
<td>4</td>
</tr>
<tr>
<td>Sexuality and sexual symptoms</td>
<td>5</td>
</tr>
<tr>
<td>Impact on areas of everyday life</td>
<td>5</td>
</tr>
<tr>
<td>Infections</td>
<td>1</td>
</tr>
<tr>
<td>Issues to be discussed with care team</td>
<td>1</td>
</tr>
</tbody>
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Relevance of the study

- Development of a PRO instrument to assess symptom experience related to late effects in SCT
- Description of SCT survivors’ symptom experience and self-management
- Knowledge basis for the development of novel intervention programs

References