PROVIVO: <u>P</u>atient <u>R</u>eported <u>O</u>utcomes 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 chronicall 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**

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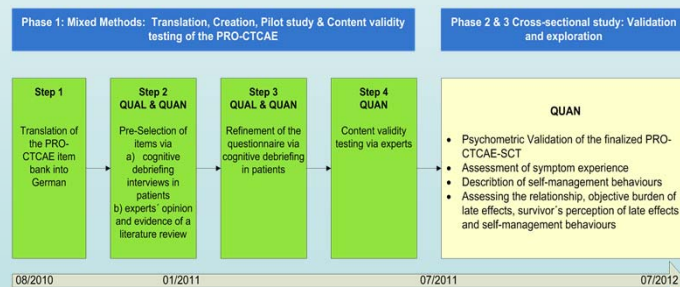

Figure 1: Study course

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

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).

Domains	# Items
General well-being	1
Physical symptoms, including pain	29
Cognitive & emotional symptoms	4
Sexuality and sexual symptoms	♀ 5 ♂ 5
Impact on areas of everyday life	5
Infections	1
Issues to be discussed with care team	1

Table 1: Content of PROVIVO instrument

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
  - ✓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

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

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