The Challenge of Patient Reported Outcomes (PROs) in LWBC research

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1. Quick introduction

2. Points for discussion:
   - What is the value/strength of PROs in LWBC research?
   - How do we engage the oncology research community to embed LWBC PROs in research to address the Top 10?
   - How would you select PROMs for LWBC research?
   - How do we maximise the value/access to PRO data collection for clinical practice and those LWBC?
   - Challenges of PRO collection in clinical practice
What are Patient Reported Outcomes (PROs)?

‘A PRO is any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else’ (US-FDA)

- **Patient reported outcome measures (PROMs)** are the tools/questionnaires used to elicit information directly from patients

- Not to be confused with **Patient reported experience measures (PREMs)** which focus on aspects of the humanity or process of care (e.g. being treated with dignity or being kept waiting) – one example is the UK Cancer Patient Experience Survey (CPES)
Why consider patients’ views?

- Most healthcare aims to reduce symptoms, minimise disability, and improve quality of life – aspects which only patients themselves can assess.
- Patient assessments may be more accurate (avoiding observer bias which could happen if clinicians were assessing their own practice).
- Patients welcome being involved, and this may have health benefits in itself:
  - Assessment of PROs in cancer populations has been linked to increased survival.
- Considering patients’ views increases public accountability in health services.
Many of the issues identified in the Top 10 can be understood and investigated by PROs

- What are the ‘best’ models of care?
- What are the complex needs of those LWBC and how can these be managed?
- How to understand and manage fatigue/pain
- What are the psychological impacts of LWBC
- Information and treatment choices
- Predicting those who will have long term and late effects
What do PROs assess?

- PROs seek to understand:
  - Patients’ views of their symptoms/side effects
  - An individual's ability to perform normal daily activities (functional status)
  - Health related quality of life (HRQoL)

- They can go much further and assess the impact of cancer and its treatment on peoples everyday lives:
  - Return to work/usual activity, fear of cancer recurrence, lifestyle, wellbeing, body image, social networks and support, coping, confidence and ability to self-manage, etc.
Types of PROMs

- Broadly there are two types of PROM:

  **Generic**
  (e.g. EQ-5D, SF-12)
  - considers general aspects such as self care and mobility
  - enables comparisons across conditions

  **Disease-specific**
  (e.g. EORTC QoL Questionnaires)
  - tailored to symptoms and impact on function of a specific condition
  - greater validity and credibility
How are PROMs used?

• **Individual patient level:** Helps clinicians to provide better and more patient-centred care; facilitates shared decision making by patients and clinicians

• **Service level:** Enables comparisons of the quality of providers’ performance to stimulate improvements in services
  - PROMs can drive the changes in how healthcare is organised and delivered
  - National QoL Metric; Independent cancer taskforce

• **Evaluation of interventions:** Provides data for evaluating practices, treatments and policies.

• **Understanding the natural history of symptoms, problems and/or needs**
How are PROMs developed?

• Often a lengthy process
• Uses qualitative methodologies to identify issues that are important to patients
• Rigorously tested with patients (e.g. cognitive interviewing)
• Statistically analysed and refined; examines psychometric properties
• Additional development to translate and validate in other languages
Group Discussion (20 mins)

• What is the value/strength of PROs in LWBC research?

• Reflecting on the poll: Are PROs universally accepted? – what are the barriers to demonstrating their value?

• How do we engage the wider oncology research community to embed LWBC PROs in research to address the top 10?

• Did everyone agree and what was the reason why not?

+5 mins feedback to group

• Please nominate one person from the group to feedback
Group Discussion (15 mins)

How would you select PRO/PROMs for LWBC research?

+5 mins feedback to group

Please nominate one person from the group to feedback
Measuring what matters most to patients

- Evidence Review
- Qualitative Research
- Role of PPI
- Role of Theory
The role of theory


Life-course (expected and unexpected events through the life-course e.g. co-morbidities, recurrence, births, marriages, deaths, moving house, new job, retirement, increasing frailty)
Selecting the ‘right’ PROM for your study

- Appropriateness
- Acceptability
- Feasibility
- Interpretability
- Precision
- Reliability
- Responsiveness
- Validity

Macmillan Survivorship Research Group

Funded by Macmillan Cancer Support
Challenges of data collection and analysis

Collecting the data
- Participant burden
- Ensuring endpoints are covered in the aims of the study

Missing data
- Reduces statistical power
- Missing data may be due to the outcome measured

Analysing & Reporting the data
- Different analyses; interpretations
- Multiple statistical methods and research questions!
- Multidimensionality of measures
- Communicating complex analyses to your target audience(s)
Strategies available

**Training**
- PROLearn

**Missing data**
- Understand how missing data handled and reported – see literature; PROM documentation

**Analysing & Reporting**
- Clear research objectives and hypothesis apriori
- SPIRIT-PRO (in Protocols)
- CONSORT-PRO (for Reporting)
- Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data (SISAQOL) Consortium
PROs in clinical practice

- PRO surveys are commonplace in practice
  - Surveys/apps
  - National Quality of Life Metric – in pilot phase
- There is emerging evidence to show it does make a difference to patients
- How do we collect this data, avoid too much replication, report and share it?
  - How can we give people LWBC (and HCPs) access to PRO data to help prepare & support them to make decisions and to personalise cancer care and treatment.
  - How do we share datasets from research and practice.... and link to other outcomes such as survival?
- How do we use this data to convince commissioners/policy makes/NICE to reconfigure services?
Group Discussion (15 mins)

- What are the challenges of PRO collection in clinical practice?
- How do we maximise the value/access to PRO data for health care professionals, decision makers, and those LWBC?

+5 mins feedback to group

Please nominate one person from the group to feedback
Final thoughts....

- There are resources available to help
- The new cross-cutting Clinical Studies Group (CSG) will have a Methodology workstream which will aim to address some of these challenges
References & Resources


- Oxford PROMS website [http://phi.uhce.ox.ac.uk/home.php](http://phi.uhce.ox.ac.uk/home.php)


- SPIRIT PRO Extension (2017) [https://jamanetwork.com/journals/jama/fullarticle/2671472](https://jamanetwork.com/journals/jama/fullarticle/2671472)

- CONSORT PRO Extension (2013) [http://jamanetwork.com/journals/jama/fullarticle/1656259](http://jamanetwork.com/journals/jama/fullarticle/1656259)

- Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data (SISAQOL) Consortium [https://qol.eortc.org/projectqol/sisaqol/](https://qol.eortc.org/projectqol/sisaqol/)