Optimising patient-reported outcome (PRO) capture in research

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Objectives

• Outline of the value of PRO/PROM data – linking to LWBC priorities

• Challenges - poor availability of high-quality PRO trials data

• What we can do now - resources which aid PRO research
What is a patient-reported outcome (PRO)?
What is a PROM?

‘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.’

The value of PROs data

“As an oncologist, when I sit with patients to discuss starting a new chemotherapy regimen, their first questions are often “How will it make me feel?” and “How did patients like me feel with this treatment?”

Ethan Basch, M.D.
The value of PROs data

Question 1
What are the best models for delivering long-term cancer care including screening, diagnosing and managing long-term side effects and late effects of cancer and its treatment (e.g. primary and secondary care, voluntary organisations, self-management, care treatment, use of digital technologies, etc.)?

Question 2
How can patients and carers be appropriately informed of cancer diagnoses, treatment, prognosis, long-term side-effects and late effects of treatments, and how does the affect their treatment choices?

Question 3
How can care be better co-ordinated for people living with and beyond cancer who have complex needs (with more than one health problem or receiving care from more than one specialty)?

Question 4
What causes fatigue in people living with and beyond cancer and what are the best ways to manage it?

Question 5
What are the short-term and long-term psychological impacts of cancer and its treatment and what are the most effective ways of supporting the psychological wellbeing of all people living with and beyond cancer, their carers and families?

Question 6
How can the short-term, long-term and late effects of cancer treatments be (a) prevented, and/or (b) best treated managed?

Question 7
What are the biological bases of side-effects of cancer treatment and how can a better understanding lead to improved ways to manage side-effects?

Question 8
What are the best ways to manage persistent pain caused by cancer or cancer treatments?

Question 9
What specific lifestyle changes (e.g. diet, exercise and stress reduction) help with recovery from treatment, restore health and improve quality of life?

Question 10
How can we predict which people living with and beyond cancer will experience long-term side-effects (side-effects which last for years after treatment) and which people will experience late effects (side-effects which do not appear until years after treatment)?

MACMILLAN CANCER SUPPORT
NCRI National Cancer Research Institute
UNIVERSITY OF BIRMINGHAM CPROR CENTRE FOR PATIENT REPORTED OUTCOMES RESEARCH
FUND BY NIHR National Institute for Health Research
Challenges…
Challenges

- Cancer patients have called for **greater availability of PRO trial data** to help them gain an insight into what their life will actually be like during and after a certain therapy, as well as how long they may survive.²

- ASCO³, EMA⁴, NICE⁵ - highlight need to **improve quality of PROM trial results**.
Systematic evaluation of Patient-Reported Outcome protocol content and reporting in cancer trials - EPiC


Objective: review the rigour with which PROs are incorporated into cancer clinical trials
Systematic evaluation of 228 completed cancer trials on the NIHR Portfolio collecting PROs 2000-2014.

We reviewed:

1. PRO protocol content
2. Whether the PRO results were published?
3. Quality of PRO reporting
Trial Protocols

<table>
<thead>
<tr>
<th>Recommended Protocol Item</th>
<th>Protocol Coverage</th>
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<tbody>
<tr>
<td>Detail regarding the rationale for PRO collection</td>
<td>missing in 68%</td>
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<tr>
<td>Description of PRO-specific objectives</td>
<td>missing in 83%</td>
</tr>
<tr>
<td>Justification of the choice of PRO instrument</td>
<td>missing in 66%</td>
</tr>
<tr>
<td>Methods to reduce avoidable missing PRO data</td>
<td>missing in 61%</td>
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CLINICAL TRIALS: IMPORTANT FEEDBACK ON NEW CANCER TREATMENTS FROM THOUSANDS OF PATIENTS REMAINS UNPUBLISHED

Macmillan Cancer Support and the University of Birmingham urge researchers to use newly developed international guidelines so that all key data is published and patients are fully informed about how cancer drugs affect quality of life.
Trial Publications

CONSORT: 63%
CONSORT-PRO: 22%

EXPECT DELAYS
key (free) resources...
PRO Roadmap

**Trial Phase**

- **Trial Inception & Design**
  - Comprehensive PRO protocol components
  - Involvement of PRO methodological and PPI expertise during trial design and throughout subsequent trial phases
  - Early formulation of PRO rationale, aims and objectives - which include the patient perspective
  - Use of SPIRIT PRO Extension guidelines during protocol development
  - Journal and funder endorsement of SPIRIT PRO

- **Data Collection & Analysis**
  - Trialist ‘buy-in’
  - High quality data
  - Appropriate analysis
  - Communication of PRO importance to trial staff and participants
  - Standardisation of PRO administration, including plans to minimise avoidable missing data
  - Pre-specification of PRO analyses, including statistical approach to missing PRO data
  - Use of SISAQOL guidelines to inform analysis plans

- **Dissemination**
  - High quality PRO findings to guide optimal patient care
  - Inclusion of PROs in the trial dissemination/impact plan
  - Use of CONSORT PRO Extension guidelines during preparation of the publication
  - Publication of PRO data alongside primary results, or as soon as possible thereafter
  - Journal and funder endorsement of CONSORT PRO
3 key (free) resources

Protocol Development

Trial implementation

Reporting

SPIRIT-PRO

PROlearn

CONSORT-PRO
and 1 in development…

The Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data (SISAQOL) Consortium has been convened by the EORTC with the aim to develop recommendations for standardizing the analysis and interpretation of patient reported outcomes and quality of life data in cancer randomized trials. The SISAQOL consortium is comprised of leading HRQOL researchers and statisticians, key individuals from various international oncologic and medical societies,
The Future?
Thank you

Protocol Development

Trial implementation

Reporting

SPIRIT-PRO

PROlearn

CONSORT-PRO

Don’t forget SISAQOL!

MACMILLAN CANCER SUPPORT

National Cancer Research Institute

UNIVERSITY OF BIRMINGHAM

CPROR

Funded by NIHR National Institute for Health Research
References


4. EMA. Appendix 2 to the guideline on the evaluation of anticancer medicinal products in man. The use of patient-reported outcome (PRO) measures in oncology studies.

