Guidelines extended to improve the use of feedback from patients in clinical trials

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Researchers have recommended changes to international guidelines used in the development of clinical trials in an effort to gain information about the impact of the treatment on participating patients and their quality of life.

Protocols describe how a clinical trial will be conducted, including its objectives, design, methodology, statistical considerations and organisation, and ensures the safety of participating patients as well as the integrity of the data collected.

However, research* has found that sometimes there can be a lack of emphasis on patient-reported outcomes (PROs); feedback from patients about how the clinical trial has affected their overall health and quality of life.

Professor Melanie Calvert, Director of the Centre for Patient-Reported Outcomes Research (CPROR) at the University of Birmingham, said: "Patient-reported outcome data from clinical trials can provide valuable evidence to inform shared-decision making, pharmaceutical labelling claims, clinical guidelines, and health policy; however, clinical trial protocols often lack important information regarding the collection of quality of life and symptom data.

"Working in collaboration with international stakeholders we have developed consensus-based, PRO-specific protocol guidance to help ensure high-quality data to inform patient-centred care."

The recommended changes, published today in the *Journal of the American Medical Association*, follows research funded by charity Macmillan Cancer Support and was a collaboration by researchers across the globe including the universities of Birmingham, Toronto and Sydney.

Professor Jane Maher, Joint Chief Medical Officer at Macmillan Cancer Support, said: "As more new cancer treatments are tested as part of clinical trials, it is vital that we know about the impact they have on patients' lives. This will enable people to decide whether or not to have a particular treatment in the future.

"New cancer treatments can add months or even years to life, but they can also have side effects which can really have an impact on quality of life, in some cases long after treatment finishes. Such outcomes have often not been reported.

"These recommended changes to international guidelines will help ensure that people's experiences of new treatments are properly recorded and published, and I would urge researchers to adopt them."

Eleven extensions and five elaborations to the SPIRIT 2013 checklist are recommended for inclusion in clinical trial protocols where PROs are a primary or key secondary outcome. Extension items focused on PRO specific issues relating to the: trial rationale, objectives, eligibility criteria, concepts used to evaluate the intervention, time points for assessment, PRO instrument selection and measurement properties, data collection plan, translation to other languages, proxy completion, strategies to minimise missing data and whether PRO data will be monitored during the study to inform clinical care.

Professor Calvert adds: "While this guidance has been developed for trials where PROs are a primary or key secondary outcome, we are actively encouraging protocol writers to consider use of this guidance in

all trials or clinical research studies where PROs are collected.

"The guidance does not aim to be prescriptive, but instead to encourage and facilitate careful planning of PRO components of trials, and thereby improve PRO trial design, which we hope will help staff and patients understand the rationale for PRO assessment, improve PRO data completeness and quality, facilitate high quality analysis and reporting, and ultimately improve the quality of the global PRO evidence base."

Daniel O'Connor, Expert Medical Adviser at the Medical and Healthcare products Regulatory Authority (MRHA), said: "We welcome initiatives to facilitate the standardisation of PRO data in clinical trials, which can contribute to a more robust patient focused drug development strategy."

Patient partner Gary Price said: "From my own personal experience of a serious illness and reporting my own outcomes, I was able to see how clinicians could use my PRO's proactively, helping them to make the right decisions thus speeding up my treatment and recovery.

"This research by the University of Birmingham has given me a good overview of how PRO's were being used and how the need for more guidance and consistency was paramount, in order to help all patients and clinicians contributing towards PRO trials and day to day usage, benefit from the results.

"Working on SPIRIT-PRO Extension, I can now see how this gives more focused guidance for protocol content within PRO's. Moving forward, it should help ensure high-quality data that can meaningfully inform patient-centred care."

Prof Calvert adds: "Improved reporting of PRO data from trials could not only help cancer patients make informed choices about their care, but also patients with a range of chronic disease."

More information: *Journal of the American Medical Association* (2018). DOI: 10.1001/jama.2017.21903

*Davis et al (2017). 'Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13.' *BMJ*