Perspectives of patients in clinical trials often not considered

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Many pragmatic trials, which test the effects of medical treatments in everyday conditions, fail to collect data reported directly by patients (such as patient experience, burden of disease, or quality of life), or involve patients as research partners (referred to as patient engagement). These findings come from a new study led by Shelley Vanderhout of the

University of Ottawa publishing February 8th in the journal *PLOS Medicine*.

Pragmatic trials aim to answer questions about medical treatments that are meaningful to patients and can be applied in real-world health care settings across diverse patient populations. Ideally, these studies should measure patient-reported outcomes such as pain levels, changes in quality of life and other patient experiences. Pragmatic trials should also have an aspect of patient engagement, where patients are involved in the research process. Inclusion of these two factors make pragmatic trials more patient-centered, but how often the studies include them is unknown.

To determine how often pragmatic trials report patient-reported outcomes or have patient engagement, the new paper's authors searched an online database for pragmatic trials published between January 2014 and April 2019. Of the 415 trials they identified, about half measured patient-reported outcomes as a primary or secondary focus of the study, and only about 9 percent included patient and public engagement. Further analysis showed that trials conducted in Europe more commonly included patient-reported outcomes, while trials involving young children or the elderly and those that recruited patients in low- and middle-income countries were less likely to collect these data.

Pragmatic trials are meant to take patient values and priorities into account, but the authors conclude that many studies have much room for improvement to accomplish these goals. They urge research funding agencies, research institutions and scientific journals to encourage researchers to include patient-reported outcomes and patient engagement in pragmatic trials in the future to promote a more patient-centered approach.

The authors add, "Patient-reported outcomes can provide a window into

patient experience and well-being, but we found that they are not routinely measured in clinical trials which aim to answer questions about patient care in everyday life. It's worth considering the potential benefit of partnering with patients and caregivers when designing this type of research, to ensure the outcomes that are important to them are captured."

More information: Vanderhout S, Fergusson DA, Cook JA, Taljaard M (2022) Patient-reported outcomes and target effect sizes in pragmatic randomized trials in ClinicalTrials.gov: A cross-sectional analysis. *PLoS Med* 19(2): e1003896. doi.org/10.1371/journal.pmed.1003896

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