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Accounting for Patients' Preferences in Studying Treatment Effectiveness: A Call for More Person-centered Health Care Research

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Driven by people's longevity and cost-containment measures, healthcare practice and policy have changed radically during the past decade; the concepts of person-centered health care (i.e. a more holistic form of personalized health care dictated by patients' overall profile including their preferences and not by their genetic information only) have penetrated many areas in health care, at least in Western Europe. Prevailing paradigms in health care policy and practice demand, for instance, increased patient involvement and participation. This is reflected in more self-management, shared decision-making and empowered interactions with health professionals. Clearly, the patient voice counts and so do his preferences, needs and values, i.e. the building blocks to person-centered or personalized medicine (van Bruinessen et al, 2014).

Interestingly, such a democratic approach does not yet count for health care study designs; a Random Controlled Trial (RCT) is still considered the gold standard for a clinical trial. In a traditional RCT there is no place for patients' treatment preference. RCTs make an assumption of equipoise; the treatments under study are perceived as equally desirable. The patient's preference (if any) for one treatment intervention over another is taken for granted instead of taken into account. This is likely to explain disappointing recruitment rates, high drop-out and less clinically and externally valid results (KNAW, 2014). However, a specific preference for (or against) one of the treatments under study may moderate treatment efficacy, especially in case of highly preference-sensitive treatments. If preference is an effect modifier, then a certain treatment may be maximally effective for those who prefer it and minimally effective for those who do not. An intervention study in patients with depression indeed shows that allowing patients to have their preferred treatment promotes outcomes (Chilvers et al, 2001).

To account for a patient's treatment preference in traditional RCTs, patient preference trials (PPTs) have been proposed (Quang et al, 2014). A patient preference trial examines the causal effects of choosing treatment rather than being randomly assigned, and thus represents more closely the conditions of usual clinical care (Aikens et al, 2015; Quang et al, 2014). The main advantage of PPT designs is that eligible patients who normally would refuse to participate in trials because they have a strong preference for one treatment, will now participate in clinical research. An disadvantage of this design could be that the statistical power of the study may diminish if a high proportion of participants prefers the same treatment (KNAW, 2014). These and other arguments make clear that more research is needed to investigate the added value of PPTs. A complicating factor is, however, that various PPT designs are in use that differ in randomization procedure and in the moment of treatment choice. It is unclear which subtype is really the most person-centered. Moreover, insight is lacking into the added value of a patient's preference for investigating the effectiveness of innovative, (web-based) self-management enhancing interventions. Contrasting traditional RCTs with more personalized trials like a PPT, does seem to have relevance for the wider field of evidence-based medicine (EBM) especially within the context of promoting person-centered medicine. Getting more insight into 'what works in which

circumstances and for whom' rather than merely into 'does it work', is likely to be helpful for health professionals and policy makers. The use of a tool to check the level of person-centeredness of a research design, like the 5Cs scoring framework presented by Buetow (2011) in a previous issue of the IJPCM (International Journal for Person Centered Medicine), is another helpful step to reach this aim.

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