Patient-Centered Outcomes Composites? A Glimpse of the Future

Running title: Califf; Patient-Centered Outcome Composites

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Journal Subject Codes: Ethics and policy:[100] Health policy and outcome research

Key words: Editorial, outcome, major adverse cardiac event, patient-centered care, qualitative research, decision aids
The field of medicine is rushing headlong into a new, information-intensive era of practice and research. As medicine learns to wield the tools of “big data,” there is growing confidence that physicians and researchers will be able to obtain, measure, and analyze information from multiple sources—including the directly solicited views and preferences of patients.

In this week’s issue of Circulation, Stolker and colleagues provide insight into where this major trend may be headed. Using a simple questionnaire administered to cardiovascular clinic patients, they uncovered several intriguing findings. First, they noted a difference between clinical trialists and patients regarding the importance ascribed by each group to preventing different specific outcomes that comprise the classical major adverse cardiovascular event (MACE) endpoints used to assess the safety and efficacy of cardiovascular therapies. Second, they found that among the patient respondents to their survey, these weightings also varied as a function of demographic factors. On the basis of these findings, Stolker and colleagues propose that weighted composites could be used to personalize evidence assessment to inform healthcare delivery based on these differences.

The measurement of patient preferences has a long history that nevertheless seems to undergo perpetual rediscovery. Yet the key issues in this arena have received scant attention from funding agencies, and very little support has been directed toward research aimed at elucidating patient preferences and decision-making processes. As a result of this inattention, many decisions at the individual, population, and policy levels are made without a true understanding of the preferences of either the downstream decision-makers (doctors, other providers, and administrators) or the patients and caregivers who will be affected. By demonstrating that different people may have notably divergent interpretations regarding the import of clinical trial results, Stolker and colleagues have made a significant contribution to our
emerging understanding of patient-centered approaches to medicine.

The cognitive and social aspects of decision-making have enjoyed intensive scrutiny in the field of behavioral economics, and business schools have long focused on preferences and choices. The present work is limited by the methods that were available, but the direction of the findings points to major areas ripe for exploration. To Stolker and colleagues’ credit, they surveyed the relevant population—patients in a cardiovascular clinic. However, it is well understood that the framing of questions is of critical importance in survey-based research.

The very brief questionnaire the authors administered does not provide vignettes describing the range of disability that might be expected from nonfatal cardiovascular events; further, it frames the question in terms of “reducing the risk of” each outcome. One could assume that reducing the risk of stroke or myocardial infarction (MI) would also reduce the risk of death, and it is unclear what summative considerations went into the responses. The finding that patients rated reducing the risk of MI as equal to or more important than reducing the risk of death seems to run counter to previous reports; while the clinical trialists’ responses, which rated death as much more severe than nonfatal events, were consistent with prior data. Other literature indicates that after more framing, patients weight nonfatal MI as less important than stroke, while they tend to assign similar importance to stroke and death. Studies using questionnaires that provided more detailed descriptions of the physical manifestations of mild or severe MI or stroke have tended to elicit much more finely grained preferences. Clearly differentiating nonfatal MI and nonfatal stroke from fatal events is a critical issue for future research.

The subgroup findings from Stolker and colleagues support a narrative that many would find pleasingly consistent with conventional wisdom. Older patients valued reducing the risk of
death relative to stroke as much less important than did younger patients (possibly reflecting differences in the relative valuation of quality of life) and wealthier patients valued reducing the risk of death more than lower-income patients (possibly reflecting differential concerns about the costs of nonfatal events). Future work should aim at further development of techniques and systems for eliciting preferences. In particular, the Patient-Centered Outcomes Research Institute’s massive National Patient-Centered Clinical Research Network (PCORnet) program presents a significant opportunity to interact with patient groups on a national scale and evolve a better understanding of risk and outcomes for people with specific diseases. Furthermore, as patients learn more about the possible future states entailed by diseases and conditions (as well as by their treatments), it is likely that thoughtful, effective use of Internet-based tools and resources can help individuals and groups iterate toward realistic and accurate estimates of preferences.

Methods for depicting findings when weighting schemes are applied to composite outcomes also deserve further articulation. Distinguishing any effect from random variation is difficult enough, given the modest benefit of most effective therapies. But because these weighted comparisons will involve more stratification based on preferences for different weights for different outcomes, there exists the great risk of introducing considerable false-positive evidence into the decision-making matrix. However, a massive increase in sample sizes is achievable if we can successfully create a learning health system in which patients and families interact with providers and electronic records on a more continuous basis. PCORnet’s access to millions of interested research participants will make possible comparative effectiveness studies on a sufficiently large scale to produce a “consumer reports” outcome assessment that enables different patients and practitioners to make different choices based on the array of predicted
outcomes.

Stolker and colleagues have made a solid contribution to this novel but increasingly important field. As larger and more rigorous systems for assessing preferences are developed and become commonplace, the relevant outcomes for clinical and outcomes trials will be optimized by the ability to appropriately match the preferences of patients, their caregivers, providers, and health systems. The most fertile ground for detailed work awaits the finding of true differences among types of people and roles in the system (e.g., patients, caregivers, providers, administrators, etc.). Deeper dives will often reveal issues with the framing of preferences, but differences that persist after framing and cognitive biases are accounted for can lead to true personalization of health care.

Conflict of Interest Disclosures: Dr. Califf receives support from the National Institutes of Health and the Patient-Centered Outcomes Research Institute. Dr. Califf also receives grant support from Amylin, Novartis, Eli Lilly and Company, Schering-Plough Research Institute, Johnson & Johnson/Scios, Aterovax, and Bayer; grants and personal fees from Bristol-Myers Squibb, Janssen Research & Development, Merck, Roche; and personal fees from Genentech, GlaxoSmithKline, Heart.org/Daiichi Sankyo, Kowa, Servier, Medscape, The Medicines Company, Pfizer, Gambro, Gilead, DSI-Lilly, CV Sight, Heart.org/Bayer, Bayer Pharma AG, Bayer Healthcare, Parkview Health, Pozen, Orexigen, Nile Pharma, and WebMD. Dr. Califf holds equity in N30 Pharma, Portola, and Nitrox LLC. Disclosure information for Dr. Califf is also available at https://dcri.org/about-us/conflict-of-interest and at http://www.dukehealth.org/physicians/robert_m_califf.

References:


2. Tong BC, Huber JC, Ascheim DD, Puskas JD, Ferguson TB Jr, Blackstone EH, Smith PK.


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Circulation. published online September 8, 2014;
Circulation is published by the American Heart Association, 7272 Greenville Avenue, Dallas, TX 75231
Copyright © 2014 American Heart Association, Inc. All rights reserved.
Print ISSN: 0009-7322. Online ISSN: 1524-4539

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