Toward a Science of Research Prioritization? The Use of Value of Information by Multidisciplinary Stakeholder Groups

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In this issue of *Medical Decision Making*, Carlson and others[1] describe the process of convening an external group of diverse stakeholders to identify 3 high-priority cancer genomics tests for further research and to rank these in order of priority for conducting further research. The external multidisciplinary stakeholder advisory group involved 13 members, including patient advocates, payers, test developers, a regulator, policy makers, and practicing oncologists. This group committed to meet over 2 years to help the South West Oncology Group (SWOG) leadership and investigators identify and prioritize future research areas and to help assess where scarce research funds would be best spent. The stakeholder group began by identifying 3 topics for research from a larger number of possible options in the area of cancer genomic tests. They were provided with a landscape review, and they developed priority-setting criteria in collaboration with the SWOG investigators to undertake this process. They identified 3 genomic tests for which further research would be useful: 1) ERCC1 expression testing for platinum-based adjuvant therapy in fully resected early- stage non–small cell lung cancer (NSCLC); 2) epidermal growth factor receptor (EGFR) mutation testing for erlotinib maintenance therapy after first-line chemotherapy in advanced NSCLC, and 3) breast cancer tumor markers (BC markers) for detection of recurrence after primary breast cancer therapy.

The stakeholder group was subsequently provided with value of information (VOI) analyses to quantify the value of conducting further research on each of these genomic tests. More specifically, they were provided with expected value of perfect information (EVPI) analyses, which estimate the upper level dollar value of reducing the uncertainty about which strategy is the optimal intervention. Providing the stakeholder group with these analyses involved a number of complex steps: developing simulation models for each genomic test, calculating the EVPI, training the external group of stakeholders in understanding what these results mean and how to use them, and discussing the results and reranking their choices based on what they may have learned from the VOI analyses. Indeed, Carlson and colleagues describe how the stakeholder group ranking of the tests changed from 1) ERCC1, 2) EGFR, 3) BC markers to 1) ERCC1, 2) BC markers, 3) EGFR. The BC markers ranked higher relative to EGFR compared with the initial prioritization vote in part because the VOI analyses helped the stakeholders appreciate the benefits of studying a technology associated with high uncertainty. Of the stakeholders, 69% reported finding these analyses helpful and 53% reported that they changed their priority ranking because of the information provided by the VOI results.

The science of research prioritization has made important advances over the past 20 years. Research prioritization has moved from an approach largely based on convening groups of experts for unstructured discussions to a scientific process that studies who and how to engage in priority-setting decisions and provides measurements of the value provided

Received 11 March 2013 from Patient Centered Outcomes Research Institute (RLF); Department of Medicine, Department of Economics, and the Harris School of Public Policy Studies at the University of Chicago (DOM). Accepted for publication 20 March 2013.

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DOI: 10.1177/0272989X13486979

by additional research, as exemplified by VOI analyses. The process described by Carlson and colleagues nicely highlights these 2 important components of the science of research prioritization as well as the associated challenges.

Whom to engage in priority-setting exercises and how best to engage participants in this endeavor remain issues of high uncertainty, and there is a need for more study on how to do this optimally. Several questions require further investigation: How are the optimal group size and composition determined? How can the group be engaged to capitalize on the benefits of bringing a diverse group of stakeholders to bear on the issues, gaining from their diverse experiences and knowledge while avoiding having too many different perspectives that cannot be reconciled for the purpose at hand? How can bias be minimized while including many views? How does one test the validity and reliability of the process? Would randomizing different groups of stakeholders be an adequate approach to help explore these issues?

Other process issues involve when and how often to engage stakeholders in the prioritization process. In the example described by Carlson and colleagues, participants were engaged for 2 years through a number of moderated webinars. However, a number of questions remain as to how best to do this. Whether stakeholders convene face-to-face or virtually and how often they convene are issues likely to weigh on the outcomes of the decision making. The VOI estimates presented for 3 of the 4 options are reasonably close, and it would be interesting to explore how a wider spread between VOI results would have influenced stakeholders' rankings. More studies will clearly be necessary to evaluate the association between the absolute and relative values of VOI results and how these affect stakeholder decision making. The field has yet to develop valid metrics to evaluate the impact of involving multiple stakeholders in priority-setting processes and how to best involve them.[2]

Another aspect described in the paper by Carlson and colleagues is quantifying the value of generating further evidence through research as exemplified by the VOI techniques. Carlson and colleagues provide an important example of the use of VOI analyses to help support research prioritization processes and build upon several prior examples where stakeholder discussions have used VOI as one of the elements to help make decisions.[3] The use of VOI to support priority-setting exercises has made strides, although the technical aspects and time required to develop models remain somewhat daunting for agencies and research groups seeking to undertake these quantitative analyses. Indeed, the authors describe the real limitations associated with the time and resources needed to build and validate complex models that support VOI analyses. The authors point out that other types of VOI analyses may be useful although these are also resource heavy. For example, expected value of sample information (EVSI) and expected value of partial perfect information (EVPPI) allow investigators to delve in more detail into the potential study design (and this will be important when considering a randomized controlled trial or a retrospective data analysis as well as which outcomes may need more research).[4] The time required to conduct complex analyses and train stakeholders would also have to be accounted for. Developments to simplify some of the VOI modeling approaches are underway to help alleviate this burden.[5]

Funders and investigators alike seek to continue developing the emerging science of research prioritization in order to make optimal use of scarce research money and generate evidence that is responsive to patient and clinician needs to enable more well-informed health care decisions. Although confined to a specific research area in cancer genomics, the prioritization example described by Carlson and colleagues highlights important questions for further research that are generalizable to larger research entities engaged in making research prioritization decisions. The Patient Centered Outcomes Research Institute (PCORI) was authorized as part of the Affordable Care Act of 2010, and central to PCORI's mission is conducting research that helps people make informed health care decisions. A key strategy for ensuring that the research is truly helpful is the engagement of patients, caregivers, and other stakeholders in the generation of research topics and in research prioritization. To this end, PCORI has begun convening multistakeholder advisory panels to support research prioritization across a broad range of diseases and conditions using explicit criteria based on conceptual VOI to do so.[6] PCORI will face some of the challenges descried by Carlson and colleagues in convening multistakeholder groups to undertake research prioritization and continue to explore novel methodological approaches supporting VOI.[7] The science of research prioritization is a dynamic and growing field. It's encouraging to observe the research community move toward more acceptance of the use of explicit criteria to undertake research prioritization. The pilot study by Carlson and colleagues points to the opportunities to improve this process and to generalize it to broader areas.

Disclaimer: Dr Fleurence is the Acting Director, Accelerating PCOR Methods Program at the Patient-Centered Outcomes Research Institute (PCORI). The views expressed in this article do not necessarily represent those of PCORI.

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