How Can We Enhance the Practical Application of Outcomes Research?

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KEY POINTS . . .

We need to change the conversation from "patient-reported outcomes" to "patient-important outcomes."

Early dialogue between manufacturers and the health technology assessment (HTA)/payer community is essential.

Evidence generation needs to reflect the 'real world.'

This is one of two articles in this issue on the topic of turning outcomes research theory into practice. Mr. O'Rourke's identifies three key concepts that enhance the practical application of outcomes research.

There are a number of ways to enhance the practical application of outcomes research, but for us at the Canadian Agency for Drugs and Technologies in Health (CADTH), an important step has been to include input from patients in our work.

CADTH is not a government agency; we are a nonprofit health technology assessment (HTA) organization funded by the Canadian federal, provincial, and territorial governments to provide independent assessments of pharmaceuticals, medical devices, diagnostics, procedures, and programs. We work with patients, clinicians, and policy makers who are faced with uncertainties on the clinical and economic value of health care technologies. Our work supports decision making by helping to close this uncertainty gap. We conduct health technology assessments across the lifespan of the technology, providing recommendations at adoption and advice on its appropriate use, as well as looking for opportunities to dis-invest from a technology where possible. Our work is based on four key principles: relevance, timeliness, impact, and quality. In brief, our role is to enhance the health of Canadians by ensuring that technologies improve patient outcomes and provide good value for the health care system.

Our Work in HTA

At CADTH, we carry out approximately 90 full reviews and about 400 rapid reviews each year on drugs and devices that can come to us from anywhere in the Canadian health care system. We provide our customers with evidence, advice, recommendations, and tools that inform decision making at the policy and practice levels. To do this, we conduct systematic reviews of the evidence, produce clinical and economic reports, incorporate patient input, and actively mobilize the knowledge that we generate. We also broker and contextualize evidence generated by other HTA producers and academic groups. CADTH also develops methodological guidelines and offers training in the methods utilized in the science of health technology assessment. Our newest offering, which was launched in January 2015, is a Scientific Advice program for pharmaceutical manufacturers. The Scientific Advice program is a voluntary, fee-for-service program that provides companies with advice on early drug development plans from an HTA/payer perspective.

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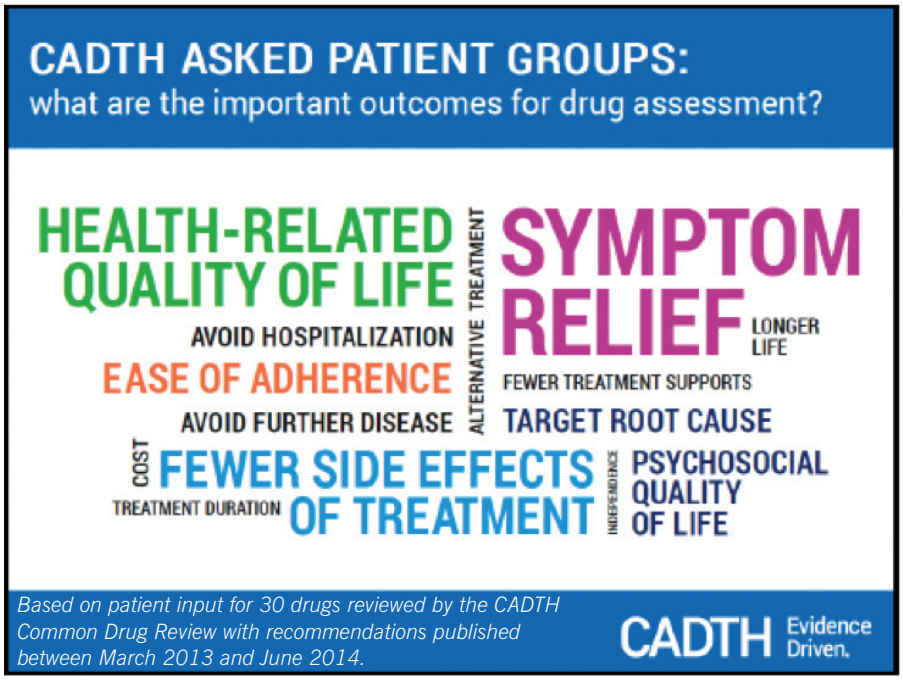
Patients Play Important Roles

We engage closely with patients and patient groups [1]. For example, we have patients or public representatives as voting members of our expert committees. We have a structured process for obtaining patient group input to our drug and device reviews, and we include patient input into the scientific advice we provide to pharmaceutical manufacturers. Our patient input process involves asking patients and caregivers specific questions about the impact of the disease, their experience with current therapies and the therapy under review, and their expectations for the new therapy. We also regularly consult with patient groups regarding the outcome of our work as well as potential changes to our policies and processes.

What We Learn from Patients

Patients help us define value because real shared decision making involves finding out what matters to the patient and understanding what is at stake for them [1]. We had been doing this for about five

Figure 1. Questions and Response to Patients



years and thought we were doing well. Nevertheless, we decided to carry out a study that examined the patient input we had received for 30 drugs.

We identified 119 outcomes that matter to patients, and found that we had been interested in about 75% of them. In addition, we found that the clinical trials on these 30 drugs captured only 50% of the outcomes identified as important from the perspective of the patient. It turns out that many of the outcomes that are important to those who design and carry out clinical trials were not necessarily the outcomes that patients valued most. These results made us realize that we still have a lot of work to do to be able to capture the outcomes that are most important to patients.

Early Dialogue with HTA Bodies and Payers

My second message is about the importance of early dialogue between manufacturers and the HTA/payer community regarding market access. Historically, clinical trials are designed primarily to get regulatory approval to market a drug. The flaw in this model is that many aspects of the drug in question that are important to payers are not captured in the clinical trial—things such as real outcomes (not just biomarkers or surrogate outcomes), comparators, and quality-of-life measures. This leads to situations where regulators and payers make somewhat different decisions based on a similar evidence package (e.g., where the regulator provides approval under their benefit-risk model of assessment, and the payer denies funding because health technology assessment has determined that the technology does not provide good value). Therefore, a number of scientific advice initiatives have been introduced over the past few years to provide early dialogue opportunities with the manufacturer regarding the types of evidence required by HTA/payers. It is also crucial to have communication—early and often—between HTA bodies/payers and the regulators.

Evidence and the 'Real World'

There has been significant momentum towards the evolution of regulatory and HTA processes towards an adaptive pathway model that will involve the use of real-world data. I believe that 'adaptive pathways' are the way of the future. Originally called 'adaptive licensing,' the model has evolved to include other stakeholders beyond the manufacturer and the regulator; hence, it is now being referred to as Medicines Adaptive Pathways for Patients (or MAPPS). While there is still some trepidation to this approach from the payer community, almost everyone involved in drug reimbursement has recognized the need for a new approach, as the current model is not sustainable for drug budgets. So, my third message is all about using 'real-world evidence' to help assess safety and effectiveness in the post-marketing phase. There are many initiatives both in Europe and North America aimed at acquiring and analyzing real-world evidence.

In conclusion, and linking my second and third points to my first, patient inclusion is required if we want to truly optimize efforts to answer the original question posed at this plenary, which was "Outcomes Research: Are We Ready to Put Theory into Practice?" I'd like to revise that title to ask: "How Can We Enhance the Practical Application of Outcomes Research?" I submit that by including patients—their input and determination of what they value—into the HTA process, we will not only enhance the practical application of outcomes research, but bring outcomes research into the 'real world,' which will benefit patients, clinicians, payers, and manufacturers.

References

[1] From Greenhalgh and Evidence Based Medicine Renaissance Group. BMJ, June 2014. ■

Additional information:
The preceding article was based on the plenary session, "Outcomes Research: Are We Ready to Put Theory into Practice?"

To view Dr. O'Rourke's presentation, go to: http://www.ispor.org/Event/ReleasedPresentations/2015Milan