Inside this edition:

President’s Corner

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The ‘Right’ Way to Develop Core Outcome Sets

A few months ago, we were preparing a grant application for a research project that would have systematically looked at how core outcome sets differ when developed through the kind of multi-stakeholder consensus process we use. Our process includes not only clinical experts, and patients or advocates, as is fairly common, but also regulators, public and private payers, health technology assessors, and researchers or regulated industry participants who are working in the clinical area of interest as well. So the question we wanted to ask was: do core sets developed in this way tend to differ systematically from those developed with mainly clinical experts and some patients, and if so, how?

To vet the idea further, I scheduled calls with a series of potential participants and partners asking if the question was useful and the method sound. Most of the potential partners and participants with whom I spoke expressed interest, and some of them enthusiastically joined me in my delight of discussing methods and study designs. We were onto something! However, one colleague had a different and provocative point of view. My colleague, a senior director in a prominent health plan, said with apparent disapproval, “Why do you want to do this?” Blindsided after so many positive conversations, I stammered. I began to verbally review the peer-reviewed literature on the Delphi method in the context of core outcomes—questions methodologists are asking about the appropriate size and composition of the Delphi panel, and of what constitutes a “valid” core set. I thought I made
a convincing case.

My colleague was not convinced. She persisted, “Why do you need to prove anything to them?” I stammered again, weakly saying something about wanting to engage with the methods community on these questions. My interlocutor drove it home: “You include all key stakeholders in your consensus process because it’s the **right** thing to do. You and your organization are committed to giving all stakeholders a voice. You do it this way because it’s right. So just do it that way!”

There’s nothing like having colleagues who are willing to challenge our assumptions! As a self-proclaimed “methods wonk,” I revel in the literature and deliberating the various uses and permutations of the Delphi method—a technique for systematic opinion-gathering that has been endlessly modified and adapted since its original development by RAND in the 1940s. The Delphi method is difficult to do well, and the multi-stakeholder approach we use creates methodological challenges that could be simplified greatly by excluding stakeholders other than clinical experts and patients. These challenges need to be considered carefully and approached with an attitude of continual improvement. To the extent we can understand how the multi-stakeholder process (when done with careful design, planning, and preparation) affects the output, it could be enlightening.

Nevertheless, it would be easy to lose sight of the big picture. We use a multi-stakeholder approach because, by definition, a “stakeholder” is someone who has a germane interest and legitimate claim to be at the table when we discuss clinical trial outcomes. Ultimately, it is an ethical choice for which we are duty-bound to work out the best methods.

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**CMTP Launches Partnership with PPMD**

Green Park Collaborative (GPC) is thrilled to announce a new partnership with Parent Project Muscular Dystrophy (PPMD), which is fighting to end Duchenne Muscular Dystrophy (DMD). DMD is a fatal, rare, genetic disease characterized by progressive muscle loss. It primarily affects boys. The disease is caused by mutations in the dystrophin gene which result in an absence or decreased amount of functional dystrophin protein. Insufficient levels of functional dystrophin lead to muscle degeneration, orthopedic complications, and respiratory and cardiac failure, causing patients with DMD to experience developmental delays, functional losses, and greatly decreased life expectancy. DMD occurs at birth in about 1 in 3,500 males, and symptoms typically develop around age three to five years.

PPMD advocacy focuses on advancing research and providing tools and information needed to equip other advocates to push legislation and regulatory efforts forward. Together, CMTP and PPMD will be doing a scoping exercise to better understand stakeholder perspectives on outcomes in DMD and explore the possible development of a core outcome set (COS). A COS is a minimal set of outcomes agreed by consensus to be collected in all clinical studies of similar purpose for a specific condition. The project will engage patients, providers, payers, HTA organizations, guideline developers and other ‘post-regulatory decision-makers’ with regulators and drug developers. Together, we will work to develop a shared understanding of the most essential and stakeholder-relevant priorities for assessing new therapies in DMD.
The Role of HTA in Core Outcome Sets

We are excited to share that the abstract for our poster presentation, “HTA Role in coreHEM, A Multi-Stakeholder Core Outcome Set Project” (shown below) was published this month in the *International Journal of Technology Assessment in Healthcare*. The abstract uses coreHEM, our core outcome set project on gene therapy in hemophilia, as an example to show why it is beneficial for HTA groups to participate in core outcome set projects and how the core sets can increase the predictability and consistency of appraisals, coverage, and reimbursement decisions by payers and HTA agencies. The poster was presented at the Health Technology Assessment international (HTAi) 2018 Annual Meeting in Vancouver, BC in June. Check out the abstract here: http://dx.doi.org/10.1017/S0266462318002386.