Introduction to special section: measuring what matters

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In 2015, ISOQOL President, Claire Snyder, PhD, and the ISOQOL Board introduced two key themes for ISOQOL to focus on over the coming years: *Measuring What Matters* (e.g., what outcomes should we measure) and *Making Measurement Meaningful* (e.g., what do the scores mean) [1]. An additional topic, *Making Sense of Methods* (e.g., how do you interpret psychometric data), also subsequently emerged [2]. To advance the dialog on *Measuring What Matters*, ISOQOL held its inaugural *Measuring What Matters* educational symposium in July 2016. *Measuring What Matters* is more than just a catchy slogan or catch phrase. It is a strategic imperative and a call for action for researchers to develop patient-reported outcomes (PRO) and other instruments which assess meaningful health outcomes—but for whom and for what purpose? When selecting an instrument, we need to also consider the context in which the measure will be used.

The focus of the 2016 educational symposium was on measuring what matters in the context of the development of medical products. The application of PRO instruments in the regulatory process has developed over the past several years with the creation of the United States Food & Drug Administration (FDA) *PRO Guidance* and the European Medicines Agency (EMA) *Guideline on Evaluation of Anticancer Medicinal Products in Man* [3–5]. Numerous methodological and technical challenges remain, however. In particular, with the current emphasis on patient-centered

measurement strategy, measures must not only fulfill regulatory requirements, but also measure outcomes that are relevant to patients, clinicians, and payers to help facilitate evidence-based shared decision making. This is easier said than done, however, as the various stakeholders may not be aligned on what outcomes are most meaningful and which measures to use.

A key theme of the *Measuring What Matters* symposium was “Patient and Caregiver Engagement.” The theme of Patient Engagement has exploded over the last several years, as numerous global initiatives have sought to engage patients not just as research participants but as partners in the design and implementation of the research itself [6, 7]. ISOQOL has embraced the inclusion of patients as partners in research and has advanced this topic through the activities of the Patient Engagement Special Interest Group [8]. Patient Engagement is not a new concept, as it is at the foundation of patient-reported outcomes and community-based participatory research. The current emphasis on patient-centered outcomes research does, however, extend the context of patient involvement: whereas medical product developers have traditionally included patients only as subjects, it is increasingly desirable to include patients as partners in research.

With these questions in mind, the theme of the inaugural *Measuring What Matters* symposium was “Bridging industry regulatory needs with what matters to patients and clinicians.” Over 2 days, attendees were able to listen to presentations from a diverse group of experts who discussed ways to engage patients in developing and implementing a measurement strategy for clinical trials. Presentations also addressed methodological challenges including incorporating the disease model into the PRO measurement strategy, strategies for identifying and evaluating existing measures, and how to analyze and interpret the meaning of trial results [9]. In addition to the plenary sessions, the attendees participated in interactive breakout sessions, which illustrated many of the challenges in developing an effective engagement strategy and implementing a measurement strategy for clinical trial research.

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To complement the *Measuring What Matters* symposium, the Co-Editors-in-Chief (FJO and CES) have curated this special section of *Quality of Life Research*, selecting articles that complement the two main themes of the symposium—patient engagement and developing PRO measures which meet regulatory requirements for labeling. The articles included in this special section highlight the theory, methods, and application of patient engagement in the development and implementation of patient-centered outcomes for the evaluation of medical products.

Wilson et al. discuss the evolving role of patients and patient representatives in medical product development. They offer a conceptual framework for expanding the role that patients (and their representatives) play in the selection and development of clinical outcome assessments, including patient-reported outcomes. In addition, they offer recommended best practices and practical considerations for engaging patients in the development of a PRO measurement strategy [10]. Forsyth et al. provide an update from the Patient-Centered Outcomes Research Institute (PCORI) on how PCORI evaluates the impact of patient engagement in the design and conduct of PCORI-funded research. The PCORI evaluation framework and the WE-ENECT evaluation survey can be useful tools for others who are looking to assess the impact that patient engagement has had on their own research [11].

Coon and Cook provide an overview of the different methodological approaches for interpreting the meaningfulness of responder definitions. This article provides a useful overview and practical considerations for ensuring that measures are sensitive to change and that we know how to interpret the results [12]. Truitt et al. provide a comparison of two different approaches for seeking patient (and public) engagement in the prioritization of research topics: crowdsourcing using an existing online consumer platform (MTurk); and the use of an existing patient registry. They offer practical considerations for both approaches in seeking stakeholder input [13].

Wasson et al. discuss the development of the What Matters Index (WMI), a patient-centered tool which offers an alternative to utilization-based risk models in predicting quality-of-life outcomes and the need for care management in chronic conditions [14]. King et al. discuss the development and validation of the Measure of Ovarian Symptoms and Treatment (MOST), a patient-reported outcome measure intended to assess the symptom burden and the impact of chemotherapy in recurrent ovarian cancer [15].

It is our hope that these articles will not only advance the science of patient engagement, but will also encourage others to engage patients as research partners in order to *Measure What Matters*.

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References

1. Snyder, C. (2015). President's message. *Quality of Life Quarterly*, 21(4), 1. Accessed at <http://www.isoqol.org/UserFiles/FALL2015NEWSLETTER-FINAL.pdf>.
2. Snyder, C. (2016). President's message. *Quality of Life Quarterly*, 22(2), 1. Accessed at <http://www.isoqol.org/UserFiles/QOL%20Quarterly/2016SpringNewsletter.pdf>.
3. FDA: Guidance for Industry. (2009). Patient-reported outcome measures: Use in medical product development to support labeling claims. <https://www.fda.gov/downloads/drugs/guidances/ucm193282.pdf>. Accessed 1 June 2017.
4. EMA: Evaluation of anticancer medicinal products in man. http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_001122.jsp&mid=WC0b01ac0580034cf3. Accessed 1 June 2017.
5. EMA: Appendix 2 to the guideline on the evaluation of anticancer medicinal products in man—the use of patient-reported outcome (PRO) measures in oncology studies. http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_001127.jsp&mid=WC0b01ac0580034cf3. Accessed 1 June 2017.
6. Fleurence, R., Selby, J. V., Odom-Walker, K., Hunt, G., Meltzer, D., Slutsky, J. R., & Yancy, C. (2013). How the patient-centered outcomes research institute is engaging patients and others in shaping its research agenda. *Health Affairs*, 32(2), 393–400. <https://doi.org/10.1377/hlthaff.2012.1176>.
7. Pushparajah, D. S., Geissler, J., & Westergaard, N. (2015). EUPATI: Collaboration between patients, academia and industry to champion the informed patient in the research and development of medicines. *Journal of Medicines Development Sciences*, 1(1), 74–80.
8. Haywood, K., Brett, J., Salek, S., Marlett, N., Penman, C., Shklarov, S., Norris, C., Santana, M. J., & Staniszevska, S. (2015). Patient and public engagement in health-related quality of life and patient-reported outcomes research: What is important and why should we care? Findings from the first ISOQOL patient engagement symposium. *Quality of Life Research*, 24(5):1069–1076. <https://doi.org/10.1007/s11136-014-0796-3>. [Epub 2014 Sep 7].
9. Measuring What Matters Symposium. ISOQOL Website. <http://www.isoqol.org/measuring-what-matters-symposium>. Accessed 1 June 2017.
10. Wilson, H., Dashiell-Aje, E., Anatchkova, M., Coyne, K., Hareendran, A., Leidy, N. K., McHorney, C. A., & Wyrwich, K. (2017). Beyond study participants: A framework for engaging patients in the selection or development of clinical outcome assessments for evaluating the benefits of treatment in medical product

- development. *Quality of Life Research*. <https://doi.org/10.1007/s11136-017-1577-6>.
11. Forsyth, L., Heckert, A., Margolis, M. K., Schrandt, S., & Frank, L. (2017). Methods and impact of engagement in research, from theory to practice and back again: Early findings from the patient-centered outcomes research institute. *Quality of Life Research*. <https://doi.org/10.1007/s11136-017-1581-x>.
 12. Coon, C. D., & Cook, K. F. Moving from significance to real world meaning: Methods for interpreting change in clinical outcome assessment scores. *Quality of Life Research* (2017). <https://doi.org/10.1007/s11136-017-1616-3>.
 13. Truitt, A. R., Monsell, S. E., Avins, A. L., Nerenz, D. R., Lawrence, S. O., Bauer, Z., Comstock, B. A., Edwards, T. C., Patrick, D. L., Jarvik, J. G., & Lavalley, D. C. (2017). Prioritizing research topics: A comparison of crowdsourcing and patient registry. *Quality of Life Research*. <https://doi.org/10.1007/s11136-017-1566-9>.
 14. Wasson, J. H., Soloway, L., Moore, L. G., Labrec, P., & Ho, L. (2017). Development of a care guidance index based on what matters to patients. *Quality of Life Research*. <https://doi.org/10.1007/s11136-017-1573-x>.
 15. King, M. T., Stockler, M., O'Connell, R., Buizen, L., Joly, F., Lanceley, A., Hilpert, F., Okamoto, A., Aotani, E., Bryce, J., Donnellan, P., Oza, A., Avall-Lundqvist, E., Berek, J. S., Feeney, A., Berton-Rigaud, D., Sehouli, J., & Friedlander, M. (2017). Measuring what matter MOST: Validation of the measure of ovarian symptoms and treatment, a patient-reported outcome measure of symptom burden and impact of chemotherapy in recurrent ovarian cancer (ROC). *Quality of Life Research*.