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Letter to the Editor: Emerging Guidelines for Patient Engagement in Research



We welcome the publication by Kirwan et al. [1] which highlights the increasing awareness that patients must have a role in defining which health outcomes are most meaningful to and desired by them. There is now widespread consensus among health stakeholders that patient involvement is a necessity and there is also a growing willingness to engage with patients as evidenced by the growing number of publications describing these efforts. The guidelines and principles described by Kirwan et al. echo and concur with those recently described, for example, by the Patient-Centered Outcomes Research Institute in their Engagement Rubric [2], by the collaborative definition of patient centrality by Yeoman et al. [3], and by the patient voice in value discussions [4].

It is encouraging to see how the dialogue is growing in richness, specificity, and urgency—indeed we believe that patient engagement has never been more requested across stakeholder groups and nor more timely. Two years ago, we joined with colleagues in a call to action for more globally consistent and systematic “patient involvement in industry-led medicines research and development, regulatory review, or market access decisions” [5]. In the same year, Patient-Focused Medicines Development (PFMD; www.patientfocusedmedicine.org) was established as a global, open, and equal coalition of patient groups, patients, the pharmaceutical industry, and regulators, welcoming also all other health care stakeholders. PFMD selected four priorities for patient engagement including 1) culture and process change, 2) development of a global meta-framework for patient engagement, 3) information exchange, and 4) training [6]. PFMD activities are ongoing or planned within each priority area and are regularly reported.

Importantly, the PFMD focus was to identify and address key factors impeding effective and meaningful patient engagement, namely, that there is/are currently 1) no platform for routinely sharing experience, 2) no platform for leveraging and integrating good practices, 3) no co-ordination of efforts but instead isolated and fragmented approaches, and 4) no globally accepted guiding principles around patient engagement. PFMD was established with a common aim of not duplicating or re-inventing the wheel but instead to learn from and build on the many valuable yet disparate patient engagement efforts underway and planned.

The first three key factors demonstrate that an essential first step is to bring together and share these efforts, connecting and mapping the patient engagement landscape so that we minimize duplication and optimize experience and learnings exchange. To this end, an open access global mapping and networking tool

has been developed and launched. It is a dynamic user-populated platform that categorizes and “maps” existing initiatives, organizations, and patient engagement frameworks to provide a global overview of the patient engagement landscape and a “who’s who” of patient engagement. It also provides a unique and searchable repository of patient engagement activities (currently > 160) and participating individuals worldwide, allowing users to connect and build collaborations. Inspiring real-world examples such as those shared by Kirwan et al. in their *Value in Health* article would be welcome additions to the tool—ensuring that a wide audience has access to their valuable insights and experience. We urge all organizations and individuals to use the global mapping and networking tool (<https://involvement-mapping-patientfocusedmedicine.org/>) to share their own activities and build working connections across the patient engagement landscape.

The fourth factor—the lack of (and need for) globally accepted guiding principles around patient engagement—is the focus of the article by Kirwan et al. They, and others such as Yeoman et al. and the Patient-Centered Outcomes Research Institute, are bringing together and validating important guiding principles for patient engagement and patient centrality. These form a sound basis for further development and agreement of globally accepted guiding principles. Work by other organizations such as the National Health Council’s Patient-Centered Value Model Rubric that helps evaluate the patient-centeredness of value model frameworks (<http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf>) and the International Consortium for Health Outcomes Measurement to codify standard sets of outcomes across medical conditions that are meaningful to patients add tangible structure and direction toward effective and meaningful patient engagement.

What is still lacking is a meta-framework for patient engagement and this is a current focus of PFMD activities. PFMD has convened multistakeholder working groups including representation from patients/patient organizations, industry (including clinical research organizations and biotech), independent experts (with backgrounds from academia, research, and industry), health technology assessment, and regulatory organizations. Their task is to identify key criteria for effective patient engagement using the very many existing efforts and experiences as a basis and augmenting these with suggestions where there may be gaps. A qualitative survey to understand expectations for patient engagement from the perspective of different stakeholders is also underway and will inform these efforts.

The meta-framework is a cocreation initiative that brings together and enhances existing good practice, learnings, and patient engagement tools and resources through a rational and systematic approach. The end goal is to have not only the meta-framework but also associated tools for its routine and practical implementation. This approach is complementary to the work being undertaken across the patient engagement community and resonates with the concluding remarks by Kirwan et al. that “Further work is needed to identify the skills, qualities, and approaches that best support effective patient-researcher partnerships.” This is true not only for patient-researcher partnerships but also for the essential collaborations that need to be forged across all stakeholder groups if we are to implement meaningful patient engagement. The article by Kirwan et al. is a welcome addition to the growing body of evidence for medicines to be made *with* patients and not just for patients.

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REFERENCES

- [1] Kirwan JR, de Wit M, Frank L, et al. Emerging guidelines for patient engagement in research. *Value Health* 2017;20:481–6.
- [2] Sheridan S, Schrandt S, Forsythe L, et al. The PCORI Engagement Rubric: promising practices for partnering in research. *Ann Fam Med* 2017;15:165–70.
- [3] Yeoman G, Furlong P, Seres M, et al. Defining patient centricity with patients for patients and caregivers: a collaborative endeavor. *BMJ Innov* (published online ahead of print March 24, 2017). <http://dx.doi.org/10.1136/bmjinnov-2016-000157>.
- [4] Perfetto E, Oehrlein E, Boutin M, et al. Value to Whom? The Patient Voice in the Value Discussion. *Value Health* 2017;20:286–91.
- [5] Hoos A, Anderson J, Boutin M, et al. Partnering with patients in the development and lifecycle of medicines: a call for action. *Ther Innov Regul Sci* 2015;49:929–39.
- [6] Boutin M, Dewulf L, Hoos A, et al. Culture and process change as a priority for patient engagement in medicines development. *Ther Innov Regul Sci* 2017;51:29–38.