Patient Engagement in 2017

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Efforts to incorporate the patient voice in healthcare decision-making has seen substantial advances, particularly in the US and Europe. Some of the credit for the advances made in 2017 goes to the increased involvement of regulatory agencies and their close collaborations with patient experts and advocacy groups. In Asia, these developments have started but taken place more slowly.

Americas

In response to safety and efficacy concerns raised around CART-T therapy, BioCanRx (a Canadian network of cancer researches, clinicians, patients, caregivers, industry partners, and other stakeholders) launched GO-CART, the first project of its kind in Canadian academia focused on immune-oncology product development. GO-CART (Getting better Outcomes with Chimeric Antigen Receptor T-cell therapy) takes a "team-oriented" approach to formulating an early-phase clinical trial protocol that integrates existing pre-clinical and clinical evidence with the values and preferences of cancer patients and other stakeholders. If successful, BioCanRx plans to expand the GO-CART approach to other cancer therapies.

In the US, passage of the *21st Century Cures Act* has been the primary catalyst for advances in patient engagement in 2017. The Act aims at bringing sought-after products to patients more rapidly while maintaining high safety and efficacy standards. To that end the new law warrants new FDA-issued guidances on how to reliably measure patient experiences and perspectives (Title III Section 3002).

In line with the requirements of the Act, the FDA has committed to the creation and maintenance of a website repository of publicly available tools for patient-focused drug development and to include the views of patients via PEAC (Patient Engagement Advisory Committee), the FDA's first advisory committee focused wholly on patients. It is also offering public workshops with multiple stakeholders, including patients, caregivers, and patient advocacy organizations.

Initiatives from other organizations include:

- PCORnet: The National Patient-Centered Clinical Research Network, an innovative PCORI initiative that directly involves patients in the development and execution of research.
- The *All of Us Research Program*, a key element of the National Institutes of Health (NIH) Precision Medicine Initiative that seeks to extend precision medicine to all diseases by building a national research cohort of one million or more US participants.

Asia

Although involving patients in health decision-making is still in its infancy in Asia, some countries have shown an increasing awareness of the need for patient engagement.

While members of the Chu-i-kyo in Japan (the Central Social Insurance Medical Council that includes payers, healthcare experts, and public interest stakeholders) largely see disadvantages and challenges to incorporating patient perspectives into decision-making, they believe that patient involvement is necessary to maintain good HTA practice. For example, more effective patient engagement throughout the healthcare development spectrum was an essential topic at the *14th DIA Japan Annual Meeting 2017*. The meeting spotlighted a report from the Consumer Organization for Medicine & Law's project training patient-citizens in how to best contribute to clinical study meetings, training courses, and various government healthcare council and committee meetings.

Europe

EUPATI (the European Patients' Academy on Therapeutic Innovation) and IMI PREFER (Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle) stood at the forefront of patient engagement in the EU in 2017.

EUPATI successfully transitioned to a sustainable ongoing program under the European Patients' Forum (EPF). At the core of this initiative are:

- EUPATI Toolbox of scientifically reliable, user-friendly educational materials.
- EUPATI Patient Expert Training Course.
- Development of local trainings through the EUPATI National Platforms.

Building on the work of EUPATI, the Medical Device Innovation Consortium (MDIC), as well as US and European regulatory agencies (FDA and EMA), the goal of the IMI PREFER project has been to assess when and how patient preferences on benefits and risks should be incorporated into decisions on medicinal products.

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