Listening to Patients’ Voices
by Cheryl L. Barton and Sara Sleigh

The role of patients in changing the pharmaceutical industry’s research agenda is evolving. Patients are greatly affected by drug R&D, with its potential to provide cures or treatments for a wide range of different medical conditions. But new product development does not always meet all patients’ needs. For example, drugs for Parkinson’s disease most often aim to treat movement disorders, whereas patients are also concerned about pain, sleep problems, lack of bowel and bladder control, and sexual dysfunction (1).

Some organizations have always included patient perspectives in their research. For example, the Cochrane collaboration (an international network of academics and clinicians who carry out systematic reviews of evidence about treatments) has always believed in equal partnerships among researchers, providers, practitioners, and patients. And the James Lind Alliance (a nonprofit initiative) was established in the United Kingdom in 2004 to bring together patients, care providers, and clinicians who share an interest in particular health problems. It aims to identify “unanswered questions” about treatment effects and prioritize the top 10 uncertainties.

Only in the past decade have patients truly begun to participate in discussions of how clinical research should be carried out to produce outcomes that will be more relevant to them. Groundwork for such involvement was laid in the 1980s and 1990s through the work of patient-advocacy groups. The AIDS community in the late 1980s is a notable example. Because of their activities, patients are now involved in FDA advisory committees and in defining research agendas. Similarly, the European Medicines Agency (EMA) established a Human Scientific Committees’ Working Party with Patients’ and Consumers’ Organizations (PCWP) to implement a framework for appropriate interactions (2).

Increasing collaboration between patients and industry continues through the work of various projects, including the EU Patient Partnership Project (PatientPartner), which set out to promote the role of patient organizations in clinical trials. It created the European Network of Patients Partnering in Clinical Research, a virtual network that enables European patient organizations to interact with other stakeholders. Significant efforts have been made within this project to define best practices for a sound platform on which to build relationships among stakeholders in the future.

The industry has focused on potentially collecting patient-reported outcome (PRO) measures, with a view to such inclusions in drug labeling. PROs require a great deal of upfront planning, particularly for cases in which an appropriate measuring instrument is unavailable and must be engineered and validated as part of drug development. Nevertheless, PROs have become an integral part of many programs. Most of the largest companies have integrated such strategies into their planning activities, often making use of external expertise in PRO instrument engineering and validation. Guidance from both the FDA and EMA provides support in this area.

The industry is also aware that PRO measures could provide relevant information to reimbursement authorities wanting to ensure real value to patients and healthcare systems from all new treatments. But it is becoming clear that capturing outcomes reported directly by patients involved in clinical trials is separate from ensuring their relevancy to patients. Regulatory authorities are working with some patients to define appropriate risk–benefit ratios for different disease areas. Industry and regulatory professionals working on such projects agree that gaining insight from patients is a challenging yet useful step. Reimbursement authorities are taking different approaches to determining value, from including patient representatives on decision-making committees to using formal statistical analyses to identify endpoints that will be of greatest value to patients.

The 2010 launch of the Patient-Centered Outcomes Research Institute sent a clear signal that the US government is beginning to emphasize outcomes and comparisons that matter to patients.

New opportunities are beginning to emerge for the industry to understand what matters to patients and engage with them for mutual benefit. Online communities of patients — e.g., PatientsLikeMe and CureTogether — enable companies to collect information on what matters to patients and learn about their products from real-world experiences. Such communities are beginning to demonstrate their power to stimulate research and to respond positively and quickly to pertinent questions.

Opportunities for engaging with patients are becoming easier and more well defined. The industry must make the most of them to help build research programs that answer relevant questions and generate new drugs for unmet medical needs.

REFERENCES

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