INCORPORATING PATIENTS’ PERSPECTIVES

The FDA launched a collaborative effort with academic centers to develop a patient-reported outcome measure and a patient preference study specifically for individuals with mild to moderate glaucoma.

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Open-angle glaucoma is a leading cause of blindness and affects more than 2 million US citizens.1 Minimally invasive glaucoma surgical devices have expanded the treatment options available to patients with mild to moderate glaucoma beyond medication and laser therapy. With broader options, the choice of which treatment to initiate evolved from being led primarily by the provider to a decision shared by the patient and provider. This shift was largely cultivated by the proliferation of information on the Internet that has empowered patients. In today’s complex health care environment where patients have many different treatment choices, their perspective should be incorporated into the evaluation of medical devices used to treat their conditions.

PATIENT-REPORTED OUTCOMES VERSUS PATIENT PREFERENCE INFORMATION

Leading the charge on evaluating patients’ perspectives, the Division of Ophthalmic and Ear, Nose, and Throat Devices in the Center for Devices and Radiological Health (CDRH) at the Food and Drug Administration (FDA) undertook many efforts to ensure that the patient’s experience is reflected in the evaluation of premarket ophthalmic device submissions. In 2016, CDRH launched an initiative to increase the use and transparency of patients’ input as evidence in regulatory decision making for medical devices as one of the organization’s strategic priorities.2

Although patients’ input can take many forms and can inform medical device development and evaluation, their perspective can be quantified using patient-reported outcome (PRO) measures and patient preference information (PPI) studies. PROs are any report of the status of a patient’s health condition that comes directly from the patient, without interpretation by anyone else.3 In the PRO measure guidance document issued in December 2009, the FDA acknowledged the importance of appropriately and effectively incorporating the patient’s voice into the evaluation of all medical products.3 The incorporation of PROs in device clinical trials has increased over time (Figure), and it became a recommendation in many ophthalmic standards, including the American National Standards Institute standard for implantable glaucoma devices.4-6 PROs are often incorporated into clinical trials as an outcome for minimally invasive glaucoma surgical devices.

PPI is the qualitative or quantitative assessment of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.7 Put simply, PPI studies evaluate how patients weigh the benefits and risks of available treatment options when making decisions. PPI is useful in situations when one or more of the following is true:

1. There are many treatment options available, with none superior for all patients.
2. Patients’ views about the most important benefits and acceptable risks of a technology vary considerably within a population or differ from those of health care professionals.

Figure. Increase in patient-reported outcome collection in CDRH device submissions.
3. The evidence supporting one option over others is considerably uncertain or variable. Using these criteria, mild to moderate glaucoma is a preference-sensitive condition. The evidence collected in PPI studies may tilt the scales of regulatory decision making toward benefit, or it may highlight a particular group who would accept the risks in exchange for the benefits.

THE FDA’S EFFORTS TO FACILITATE GLAUCOMA DEVICE EVALUATION

To facilitate the incorporation of patients’ input on glaucoma devices, CDRH formed a collaboration with The Johns Hopkins University and the University of California, San Francisco (UCSF)/Stanford Centers of Excellence in Regulatory Science and Innovation (CERSIs). These academic institutions are funded by the FDA to conduct a patient preference study (Johns Hopkins) and develop a PRO measure (UCSF/Stanford) sensitive to patients with mild to moderate glaucoma who are eligible for minimally invasive glaucoma surgery. To frame these efforts, some of the American Glaucoma Society participants in this collaborative project drafted an editorial highlighting the need to evaluate the patient’s perspective in the assessment of minimally invasive glaucoma surgical devices.

With the assistance of the Johns Hopkins CERSI, the American Glaucoma Society is performing a systematic review of the literature to appraise the scientific rigor and relevance of current PRO measures for FDA-targeted minimally invasive glaucoma surgical trials. The inferences from this review will guide the PRO measure development process conducted by the UCSF/Stanford CERSI. In addition, the UCSF/Stanford CERSI convened an expert panel to better elucidate the symptoms reported by these patients and completed three focus groups across the nation of patients with mild to moderate glaucoma. The anticipated PRO measure derived from these discussions will be web-based and sensitive to the surgical experience among individuals with this stage of disease.

For the patient preference study being conducted by the Johns Hopkins CERSI, one-on-one interviews with patients who have mild to moderate glaucoma have led to the construction of a survey tool that instructs patients to weigh the risks and the benefits of treatment options. By quantifying their preferences, the study may provide scientific evidence to aid in the evaluation of investigational minimally invasive glaucoma surgical devices.

Both projects are actively enrolling patients to complete their objectives.

CONCLUSION

It is well known that, when making treatment decisions, patients with glaucoma consider trade-offs among the benefits and risks. The FDA’s collaborative effort with the Johns Hopkins CERSI is an effort to quantitatively evaluate these trade-offs, which may inform FDA review of minimally invasive glaucoma surgical device applications. In addition, a PRO measure sensitive to patients who have mild to moderate glaucoma and their experiences with surgical interventions will be a useful tool that could be incorporated into future minimally invasive glaucoma surgical clinical trials. In summary, FDA-funded efforts to incorporate patients’ perspectives into the device development and evaluation process will expedite innovation and the availability of this technology.


**AT A GLANCE**

- In today’s complex health care environment where patients have many different treatment choices, their perspective should be incorporated into the evaluation of medical devices used to treat their conditions.
- The FDA is funding academic institutions to conduct a patient preference information study and to develop a patient-reported outcome measure sensitive to individuals with mild to moderate glaucoma who are eligible for minimally invasive glaucoma surgery. These efforts to incorporate patients’ perspectives into the device development and evaluation process will expedite innovation and the availability of minimally invasive glaucoma surgical technology.

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