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Food, Drug And Device Law Alert - FDA Finalizes Guidance On Including Patient Preference Information In Certain Medical Device Premarket Submissions

October 11, 2016 | Atlanta | Chicago | Columbus | Dallas | Delaware | Elkhart | Fort Wayne | Grand Rapids | Indianapolis | Los Angeles | Minneapolis | South Bend

The Food and Drug Administration (FDA) recently issued a final guidance titled “Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling.” The guidance is a supplement to an earlier guidance titled “Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications.”

In the earlier guidance, the FDA stated that its reviewers may consider certain data measuring patient perspectives during the premarket review process for premarket approval applications (PMAs) and de novo classification requests. In other words, patient tolerance for risk and perspective on benefit may be considered in the FDA’s assessment of the benefit-risk profile of certain devices when the information meets the FDA’s standards for valid scientific evidence.

The guidance defines “patient preference information” as “qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.” The guidance states that it may be useful for sponsors to collect and submit patient preference information for PMAs, humanitarian device exemption (HDE) applications, and de novo requests, particularly where usage decisions by patients and health care professionals are “preference-sensitive.” Preference-sensitive decisions may be required when a patient has multiple treatment options and there is no option that is clearly superior for all preferences, when the evidence supporting one option over others is considerably uncertain or variable, and/or when patients’ views about the most important benefits and acceptable risks of a technology vary considerably within a population.

The guidance states that the following circumstances may present “preference-sensitive decisions:”
• Devices with a direct patient interface
• Devices intended to yield significant health and appearance benefits
• Devices intended to directly affect quality of life
• Certain life-saving but high-risk devices
• Devices developed to fill an unmet medical need or treat a rare disease or condition
• Devices with novel technology

RELATED PEOPLE

Lynn C. Tyler, M.S.
Partner
Indianapolis

P 317-231-7392
F 317-231-7433
lynn.tyler@btlaw.com

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Importantly, the guidance identifies several recommended qualities of patient preference studies. Excerpts from the guidance discussing these qualities are:

1. **Patient Centeredness:** Patient preference studies should ensure that the patient, not the healthcare professional, is the central part of the study.
2. **Representativeness of the Sample and Generalizability of Results:** A study should measure the preferences of a representative sample of adequate size to ensure that the study results can be generalized to the population of interest.
3. **Capturing Heterogeneity of Patients' Preferences:** It is important to account for variations in patient gender, age, race, socioeconomic and cultural background, condition, treatment, etc., when considering patient preference information. Patient preference information should reflect the preferences of patients from the entire spectrum of disease for which the device is intended to be used.
4. **Established Good Research Practices by Recognized Professional Organizations:** The quality of a study may be established if it follows guidelines for good research practices established by a recognized professional organization.
5. **Effective communication of benefit, harm, uncertainty, and risk:** It is important for patient preference studies to define the context of the benefit-risk tradeoffs, explain the level of effectiveness and the severity of treatment-related harms, and help patients conceptualize probabilities using appropriate numeric, verbal, and graphic representations of uncertainty.
6. **Minimal cognitive bias:** Study design should minimize potential cognitive biases such as framing (e.g., describing changes as gains or losses), anchoring (e.g., signaling a reference value), simplifying heuristics (e.g., recoding numerical values or percentages as low, medium, and high), or ordering effect (e.g., the response to a question depending on its relative position in the question sequence).
7. **Logical soundness:** The data should include internal validity tests of logic and consistency and should be verified for conformity with logic and consistency.
8. **Relevance:** Critical aspects of risk, benefit, and uncertainty should be included in the elicitation of preferences, and omission of any should be well justified.
9. **Robustness of Analysis of Results:** After measurements are made in a scientific study, an analysis of these results should ensure appropriate interpretation of the collected evidence.
10. **Study Conduct:** The validity and reliability of study results depends in large part on compliance of research staff and study participants with the study protocol.
11. **Comprehension by Study Participants:** Efforts should be made to ensure that study participants fully understand the risk and other medical information being communicated to them.

Patient Preference in Labeling

The guidance also addresses use of patient preference information in labeling. For a device for which the FDA considers patient preference information in its benefit-risk determination, the guidance states that in

addition to the standard elements of labeling (e.g., indications for use, contraindications, benefits, risks, warnings, and user instructions), the labeling should describe the patient preference study data, including the range of patient preferences and characteristics of patients who considered the device's probable benefits to outweigh its probable risks.

Generally, labeling should be written in plain language so that patients are able to understand the information presented and form realistic expectations of the treatment and its potential risks. The patient labeling should use terminology and numerical data in a way that is easily recognized and understood by the average layperson. When appropriate, pictorials, graphics, or tables, should be included as an adjunct to the written word. In addition, the labeling should include a clear indication of the population for whom the device is appropriate.

The patient labeling should contain information that may assist patients in understanding:
• if they might benefit from use of the device
• the potential benefits from use of the device
• the potential risks or complications from use of the device, and the likelihoods of each
• any relevant contraindications, warnings, and precautions
• if they share characteristics with the group of patients who view the benefits as outweighing the risks, and
• any additional information about what is known and not known about patient outcomes (e.g., long-term outcomes, rare complications)

Compared to the draft version of this guidance, the final version includes a new section describing when and how the FDA might consider patient preference information. According to the guidance, "patient preference studies can be informative for devices under PMA and de novo review by providing patient perspectives on benefits, including whether results are significant from a patient perspective, and risks, including whether patients would consider the risks to be unreasonable." Further, for Humanitarian Device Exemption (HDE) reviews, patient preference studies can provide patient perspectives on the statutory standard of whether "the probable benefit to health from the use of the device outweighs the risk of injury or illness from its use, taking into account the probable risks and benefits of currently available devices or alternative forms of treatment." The guidance includes several hypothetical examples of how FDA might consider patient preference information when making benefit-risk assessments.

A copy of the final guidance can be found here.

For more information, please contact the Barnes & Thornburg LLP attorney with whom you work or one of the following attorneys in the firm's Food, Drug & Device Group: Lynn Tyler at (317) 231-7392 or lynn.tyler@btlaw.com; Beth Davis at (404) 264-4025 or beth.davis@btlaw.com; or Alicia Raines Barrs at (317) 231-7398 or alicia.rainesbarrs@btlaw.com.

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