Breakthrough Devices Program:
Draft Guidance to Implement 21st Century Cures

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Breakthrough Devices Program: Draft Guidance to Implement 21st Century Cures

Written by Elizabeth Guo – 12/5/17

On October 25, 2017, FDA published a **draft guidance** that describes FDA's proposed approach to implement the Breakthrough Devices Program ("BDP" or the "Program"), a voluntary program to expedite access to medical devices intended for treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions. The BDP implements section 515B of the Federal Food, Drug, and Cosmetic Act (FDCA), as created by the 21st Century Cures Act. The Program focuses on reducing the time associated with the development, assessment, and review of eligible devices. Interested persons may submit comments to the draft guidance within 60 days of publication (December 24, 2017).

The BDP supersedes and builds upon FDA's experience with previous expedited access programs for medical devices. The Program supersedes the Expedited Access Pathway launched in 2015 and the Innovation Pathway launched in 2011, both of which intended to expedite the development and breakthrough of various devices. The BDP also supersedes the Priority Review Program, which implemented statutory criteria for granting priority review for medical devices requiring premarket approval applications.

Eligibility As a "Breakthrough Device"

To be eligible for the BDP, a device must "provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human disease or conditions." "More effective" means "a reasonable expectation

that a device *could*provide for more effective treatment or diagnosis" relative to current standard of care. A sponsor must demonstrate "more effective" treatment or diagnosis with evidence of "technical success" (that the device could function as intended) and "clinical success" (that a functioning device could more effectively treat or diagnose the identified disease or condition). "Life-threatening" is defined as a high likelihood of death unless disease course is interrupted. "Irreversibly debilitating" means a morbidity that has substantial impact on day-to-day functioning.

Eligible devices must also meet one of four conditions: (1) represent "breakthrough technologies;" (2) have no approved or cleared alternatives; (3) offer significant advantages over existing approved or cleared alternatives; or (4) its availability is "in the best interest of patients." These criteria are similar to the priority review criteria in former section 515(d)(5) of the FDCA. FDA may also designate "Breakthrough Device" status for multiple devices with the same proposed indication so long as each device meets statutorily required criteria.

Requesting a Breakthrough Device Designation

Sponsors must request a Breakthrough Device designation prior to submitting a PMA, a 510(k) notification or a *De Novo* request for classification. The Breakthrough Device Designation request should indicate whether sponsors intend to submit a PMA, 510(k), or *De Novo* request, with a rationale for their approach, and include the information specified in Appendix 2 of the draft guidance. A request for Breakthrough Device Designation should be submitted as a Q-Submission.

BDP General Principles and Considerations

The draft guidance provides a number of "principles" to guide FDA's approach toward the BDP. These principles include:

- Interactive and Timely Communication. FDA provides best-practice recommendations to facilitate collaborative communications between the sponsor and FDA.
- Premarket/Postmarket Balance of Data Collection. Breakthrough Devices requiring a PMA will need to meet the statutory standard of reasonable assurance of safety and effectiveness at time of approval. However, FDA may be willing to accept a greater degree of uncertainty in the benefit-risk profile of PMA devices if other factors, such as the device's probable benefits, offset the uncertainty associated with the device. FDA's guidance on balancing premarket and postmarket data collection describes how FDA will apply these factors.
- Efficient and Flexible Clinical Study Design. When scientifically appropriate, FDA will be flexible regarding clinical study design, including, for example, surrogate endpoints, composite endpoints, and phased study design.
- Review Team Support and Senior Management Engagement. FDA will conduct regular training for reviewers on Breakthrough Device teams to "ensure consistent and efficient application" of the principles and features outlined in the draft guidance document. Review staff will have subject matter expertise and experience assessing innovative therapies. Senior management (office director or designee) will work with the team and provide timely dispute resolution.
- Priority Review. All Breakthrough Devices will receive priority review status, which means that the submission will land at the top of the FDA review queue. Nevertheless, because of novel scientific issues, review times may take longer for Breakthrough Devices than for other devices.

- Expedited Manufacturing and Quality Systems Review. For submissions that typically require a preapproval inspection, FDA will expedite review of manufacturing and quality systems compliance. FDA may, on a case-by-case basis, allow a sponsor to provide less manufacturing information in a PMA "when the sponsor has a good track record for quality systems compliance." FDA may also forego inspection of certain manufacturing sites prior to approval of a Breakthrough Device.

BDP Benefits and Features

FDA will offer sponsors a "menu of options" for interacting with the agency as Breakthrough Device development unfolds, including the following:

- "Sprint" Discussions to Resolve Potentially Novel Issues. FDA will offer "sprint" discussions with sponsors to reach an agreement on specific topics within a set time period. "Sprint" discussions involve highly interactive communications on a specific topic (e.g., animal study protocol design, clinical protocol discussion) between a sponsor and FDA.
- Early Agreement on a Data Development Plan (DDP). The DDP is a high-level document that describes and provides a rationale for the data collected premarket and postmarket. Sponsors of Breakthrough Devices can work with FDA to ensure that the DDP reflects an appropriate, least-burdensome, and predictable approach to data collection.
- Binding Agreement on Clinical Protocols. A sponsor of a Breakthrough Device can obtain FDA agreement on clinical protocols that will be binding on both FDA and the sponsor. FDA and the sponsor would need to agree in writing to any changes to the previously agreed-upon protocol.

- Regular Status Updates. The sponsor and FDA can agree on regular status updates to discuss the general progress of the project, next steps, or plans for future discussions.

Applying these principles and features, FDA seeks to provide patients with more timely access to devices while preserving the statutory standard.