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Select Updates for the Breakthrough Devices Program Guidance: Reducing Disparities in Health and Health Care

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Draft Guidance for Industry and Food and Drug Administration Staff

DRAFT GUIDANCE

This draft guidance document is being distributed for comment purposes only.

Document issued October 21, 2022.

You should submit comments and suggestions regarding this draft document within 60 days (standard), days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff, Food and Drug Administration, 5630 Fishers Lane, Room 1061, (HFA-305), Rockville, MD 20852. Identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions about this document, contact CDRH Office of Clinical Evidence and Analysis (OCEA) at 301-796-5550 or BreakthroughDevicesProgram@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010

When final, this guidance will update the Introduction and Section III of "Breakthrough Devices Program," issued on December 18, 2018.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

Preface

Additional Copies

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Additional copies are available from the Internet. You may also send an email request to CDRH-Guidance@fda.hhs.gov to receive a copy of the guidance. Please include the document number 1833-R1 and complete title of the guidance in the request.

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This draft guidance, when finalized, will represent the current thinking of the Food and Drug Administration (FDA or Agency) on this topic. It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations. To discuss an alternative approach, contact the FDA staff or Office responsible for this guidance as listed on the title page.

I. Overview of Select Updates

The U.S. Food and Drug Administration (FDA or Agency) has developed this draft guidance to propose select updates to the FDA guidance document entitled, "Breakthrough Devices Program" ("Breakthrough guidance").[1] The existing guidance on the Breakthrough Devices Program is still in use, in its current form, until this draft guidance is finalized. FDA intends to incorporate the updates proposed in this draft guidance into one final guidance document after obtaining and considering public comment on these proposed select updates. The sections of the existing Breakthrough Devices Program guidance that are not affected by these proposed updates are not intended to be substantively changed.

Consistent with the goals of the Breakthrough Devices Program and the statutory designation criteria,[2] FDA is proposing updates to the Breakthrough Devices Program guidance to clarify how the program may be applicable to certain devices that benefit populations impacted by health and/or health care disparities, which would include adding clarifying language to the Introduction section of the Breakthrough guidance. Additionally, consistent with our obligations under the SUPPORT Act (FD&C Act section 515B (21 U.S.C. 360e-3)), FDA is proposing to update the guidance to state in the Introduction section that certain non-addictive medical

[1] Available at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/breakthrough-devices-program and subsequently referred to as the "Breakthrough guidance" throughout.

[2] As defined in section 515B(b) of the FD&C Act (21 U.S.C. 360e-3(b)).

products to treat pain or addiction may be eligible for the Breakthrough Devices Program.[3] FDA is also proposing updates to Section III of the Breakthrough guidance to clarify designation considerations for the first criterion of the statute, as well as add new information regarding FDA's intent to disclose the Breakthrough Device designation of designated devices once they obtain marketing authorization for an indication for use consistent with their Breakthrough Device designation.

The contents of this document do not have the force and effect of law and are not meant to bind the public in any way, unless specifically incorporated into a contract. This document is intended only to provide clarity to the public regarding existing requirements under the law. FDA guidance documents, including this guidance, should be viewed only as recommendations, unless specific regulatory or statutory requirements are cited. The use of the word *should* in Agency guidance means that something is suggested or recommended, but not required.

II. Updates to Section III Designation Request

FDA is proposing to update a subset of the recommendations included in Section III of the Breakthrough guidance. Existing language within each section will remain unchanged, and the language described below is being proposed for inclusion.

FDA is proposing to add the following language at the end of the first paragraph in Section III:

> When submitting a request for Breakthrough Device designation, sponsors should clearly indicate the proposed indications for use for which they are seeking designation, as illustrated in Appendix 1. The proposed indication(s) may target a subset of a broader disease population.

A. Updates to Section III.B Designation Considerations

(1) Update to Section III.B.1 First Criterion

a. Whether a Device Provides for "More Effective" Treatment or Diagnosis

In addition to the existing considerations described within subsection a, FDA is proposing to add the following language:

> The level and type of evidence needed to determine whether a device is reasonably expected to "provide for more effective treatment or diagnosis" may vary depending on the intended use of the device, its technology and features, and the available standard of care alternatives. When evaluating this part of criterion 1, FDA considers the totality of

[3] The Breakthrough Devices Program may be available for certain non-addictive medical products to treat pain or addiction. (Federal Food Drug & Cosmetic Act (FD&C Act) section 515B (21 U.S.C. 360e-3)). The considerations set forth in this guidance document apply to FDA's review of devices as non-addictive methods to treat pain or addiction.

information regarding the proposed device, its function, potential for technical success, potential for clinical success, potential for a clinically meaningful impact, and its potential benefits and risks. The determination of whether a device is reasonably expected to "provide for more effective treatment or diagnosis" is based upon all these factors.[4]

(2) Update to Section III.B.3 Additional Considerations

In addition to proposing clarifying language be added to the Introduction section of the Breakthrough guidance to state that the Breakthrough Devices Program may expedite the availability of certain devices that meet the statutory designation criteria and benefit populations impacted by health and/or health care disparities, thereby promoting and advancing health equity, FDA is proposing to add a new subsection d. to Section III.B.3 of the Breakthrough guidance, which includes the following considerations.

d. Reducing Disparities in Health and Health Care

Health and health care disparities exist and occur across many dimensions, including race, ethnicity, socioeconomic status, age, sex, disability status, sexual orientation, gender identity, language, and location, among others, as summarized in reports by the Agency for Healthcare Research and Quality and Department of Health and Human Services Office of Minority Health.[5,6] Addressing health and health care disparities is not only important for achieving health equity,[7] but also for improving the overall quality of life and health outcomes for all patients. FDA recognizes the urgent public health need for innovative technologies that help to reduce barriers to achieving health equity and help to improve health outcomes across diverse populations. When assessing eligibility for the Breakthrough Devices Program using the statutory designation criteria,[8] FDA intends to consider technologies and device features that may help to address health and/or health care disparities and promote health equity by providing for more effective treatment or diagnosis in populations that exhibit health and health care disparities.

One dimension contributing to health and health care disparities is the inability to recognize and address the ways in which treatment outcomes may differ by race, ethnicity, sex, and/or other factors. For some diseases, the pathophysiology, clinical features, and response to treatment may

[4] The considerations set forth for whether a device is reasonably expected to "provide for more effective treatment or diagnosis" are limited to evaluating the first Breakthrough criterion within the scope of this guidance.
[5] Refer to the 2021 National Healthcare Quality and Disparities Report available from: https://www.ahrq.gov/sites/default/files/wysiwyg/research/findings/nhqrdr/2021qdr.pdf
[6] As described in the 2015 Report to Congress on Minority Health Activities available from: https://www.minorityhealth.hhs.gov/omh/browse.aspx?lvl=2&lvlid=57
[7] For purposes of this guidance, the Agency defines health equity according to the World Health Organization definition as the absence of unfair, avoidable and remediable differences in health status among groups of people. See Health equity and its determinants; World Health Organization; 2021; available from: https://cdn.who.int/media/docs/default-source/world-health-day-2021/health-equity-and-its-determinants.pdf?sfvrsn=6c36f0a5_1&download=true
[8] As defined in section 515B(b) of the FD&C Act (21 U.S.C. 360e-3(b))

be impacted by these factors.[9] Health and health care disparities can be exacerbated due to a lack of recognition of these differences, including implicit biases, and/or the lack of devices designed to effectively diagnose or treat the condition in a manner that addresses these differences. The Breakthrough Devices Program can be used to provide more timely access to devices that address the unmet needs of populations that may experience health and/or health care disparities. FDA considers technologies and device features tailored to address characteristic differences, such as those arising from social factors, phenotypic variations, pathophysiology, and/or response to treatment, when evaluating if there is a reasonable expectation that the device may provide for more effective treatment or diagnosis as compared to the current standard of care, including the device's potential to be more effective in certain populations. For example, as part of FDA's assessment of whether a device is reasonably expected to be more effective, we consider if it is designed to address a pathophysiological or clinical characteristic associated with certain populations that could have a clinically meaningful impact for the treatment or diagnosis of the condition in those populations. Such a device may, therefore, be considered as reasonably expected to offer a more effective treatment or diagnosis as is consistent with criterion 1 of the designation criteria.

Similarly, health and/or health care disparities may also arise in populations impacted by life-threatening or irreversibly debilitating diseases or conditions that are rare. In some cases, patients living with these rare diseases or conditions may have limited diagnostic and treatment options. FDA considers technologies and device features tailored to address unmet needs in these populations when evaluating if there is a reasonable expectation that the device may provide for more effective treatment or diagnosis.[10]Another major dimension of health and health care disparities is accessibility to quality health care. For the purposes of this guidance, we define accessibility as an individual or group's capacity to benefit from a medical device or procedure. This is distinct from use of the term "access" elsewhere in the guidance, which refers to commercial availability of a medical device following marketing authorization. Certain barriers, such as inequities in the availability of medical care, may prevent underserved populations from receiving medical treatment or diagnosis. Often the benefit of a device cannot be realized due to this lack of accessibility. New devices that have the potential to offer a clinically meaningful impact through improved accessibility may provide a significant benefit to patients by, for example, including user features that are adaptable or more easily used by diverse populations or allow for use in more diverse settings. As described in language proposed in this draft guidance for inclusion in Section III.B.1.a of the Breakthrough guidance, when determining whether a device is reasonably expected to "provide for more effective treatment or diagnosis," FDA considers the totality of available information regarding the device, including its potential for a clinically meaningful impact and its potential benefits and risks. Therefore, when evaluating the first Breakthrough criterion, FDA intends to consider technologies and device features that could

[9] As described in the 2003 report from the Institute of Medicine (US) Committee on Understanding and Eliminating Racial and Ethnic Disparities in Health Care titled Unequal Treatment: Confronting Racial and Ethnic Disparities in Health Care available from: https://pubmed.ncbi.nlm.nih.gov/25032386/

[10] Consistent with section 515B(c) of the FD&C Act, Breakthrough Device designation may be requestedany time prior to the submission of a PMA, 510(k), or De Novo request. Sponsors should only request Breakthrough Device designation if they intend to pursue one of these marketing pathways. Other marketing pathways (e.g., Humanitarian Device Exemption) are not eligible for consideration under the Breakthrough Devices Program.

allow for improved accessibility when evaluating if there is a reasonable expectation that the device may provide for more effective treatment or diagnosis as compared to the current standard of care. For example, improved accessibility of a device may be considered as reasonably expected to be more effective if there is information supporting its use in diverse settings such that a patient population with limited or no available options may have improved adherence to a prescribed medical regimen.[11]

B. Update to Section III.C Designation Review Process

FDA is proposing to add the following language to Section III.C of the Breakthrough guidance.

> Subject to the confidentiality provisions of the FD&C Act and implementing regulations, including FDA's Part 20 regulations covering information disclosure (21 CFR part 20)[12], and the Freedom of Information Act (FOIA) (5 U.S.C. 552)[13], FDA generally will not disclose the existence of requests for Breakthrough Device designation and our decisions on such designation requests. However, in certain cases, FDA may publicly disclose a Breakthrough Device designation that has been previously publicly disclosed or acknowledged by the sponsor of the Breakthrough Device designation request. Additionally, once a designated Breakthrough Device obtains marketing authorization for an indication consistent with its Breakthrough Device designation, FDA intends to publicly disclose its Breakthrough Device designation status for that indication for use.[14] Because Breakthrough Device designation is granted for a device and its indication for use, if a designated Breakthrough Device receives marketing authorization for an indication other than the indication covered by its designation, it is not considered a market-authorized Breakthrough Device and would not be disclosed as such.

[11] Consistent with the goals of promoting the development of impactful devices, FDA considers this potential for increased benefit to patients to satisfy the "more effective treatment or diagnosis" criterion when evaluating a Breakthrough Designation request. These considerations for determining eligibility to participate in the program are different from and do not change the statutory requirements for safety and effectiveness to support a marketing authorization.

[12] 21 CFR part 20 available at https://www.ecfr.gov/current/title-21/chapter-I/subchapter-A/part-20

[13] Additional information related to FDA's implementation of the Freedom of Information Act can be found at https://www.fda.gov/regulatory-information/freedom-information/foi-information

[14] FDA's Breakthrough Devices Program webpage is available at: https://www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program. This webpage includes a listing of Breakthrough Devices that have obtained marketing authorization.