U.S. FOOD & DRUG ADMINISTRATION

Welcome To Today's Webinar

Thanks for joining us!
We'll get started in a few minutes

Today's Topic:
Breakthrough Devices Program
Updated Final Guidance

November 14, 2023

Breakthrough Devices Program Updated Final Guidance

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Final Guidance

- **Breakthrough Devices Program**
  - www.fda.gov/regulatory-information/search-fda-guidance-documents/breakthrough-devices-program

Learning Objectives

- Describe CDRH's strategic priority to advance health equity
- Provide an overview of Breakthrough Devices Program
- Describe updates to Breakthrough Devices Program Guidance
  - Including how program may apply to certain devices that benefit populations impacted by health and/or health care disparities

CDRH Strategic Priority: Advancing Health Equity

Health Equity and Health Disparities

- **Health equity:** state in which everyone has a fair and just opportunity to attain their highest level of health.
- **Health disparities:** preventable differences in burden of disease or opportunities to achieve optimal health. Metric often used to measure progress toward achieving health equity.
- Often impacted by race, ethnicity, sex, age, disability, sexual orientation, gender identity, socioeconomic status, geography, preferred language, or other factors that affect access to care and health outcomes.

Source: Centers for Disease Control and Prevention

Examples of Health Disparities

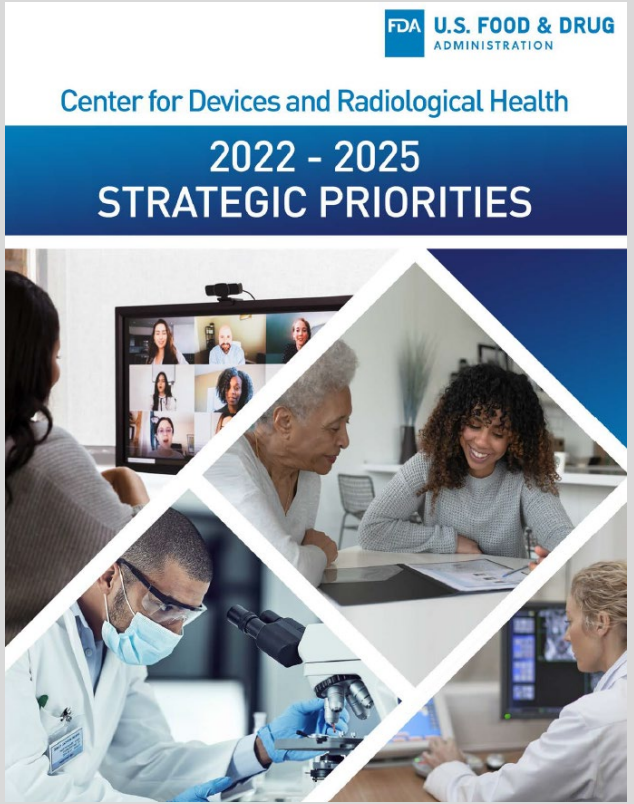
- **Life expectancies** vary by race, ethnicity, and sex
- **High blood pressure**, a major risk factor for heart disease, is more common and not as well controlled in African-American and Hispanic adults as in White adults
- In the United States, rural populations have higher rates of death due to **heart disease, chronic lower respiratory disease, and stroke** than urban populations
- Rate of **diabetes** is as high as 14.5% for Native Americans/Alaskan Natives compared to 7.4% for non-Hispanic whites
- 5-year survival of non-Hispanic Black and rural patients was consistently lower than urban patients for each **cancer** type, independent of sociodemographic or health care variables
- Non-Hispanic Black, Native Hawaiian and other Pacific Islander **babies** are twice as likely to die as White babies

Sources:

Health Disparities and Inequities: www.nhlbi.nih.gov/science/health-disparities-and-inequities
Statistics about Diabetes: diabetes.org/about-diabetes/statistics/about-diabetes
Infant Mortality: www.cdc.gov/reproductivehealth/maternalinfanthealth/infantmortality.htm
MW Lewis-Thames et al, jamanetwork.com/journals/jamanetworkopen/fullarticle/2792392

CDRH Strategic Priority: Advancing Health Equity

- CDRH can advance the development of knowledge and safe and effective technologies to meet needs of all patients and consumers
- Technology can help bridge divide while advancing better healthcare, quality of life, and wellness for all
- No person should be left behind in health care



Source: CDRH 2022-2025 Strategic Priorities: www.fda.gov/media/155888/download?attachment

CDRH 2022-2025 Strategic Priority: Advance Health Equity

Empower People
to make informed decisions regarding their healthcare

Facilitate Availability
of and access to medical technologies for all populations

Reduce Barriers
and increase opportunities for participation by diverse populations in evidence generation

Support Innovation
of technologies that address health disparities

Breakthrough Devices Program Overview

Breakthrough Devices Program

- Voluntary program intended to provide patients and health care providers with timely access to devices
  - that provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions
- Expedites development, assessment, and review of certain devices that meet program eligibility criteria

Principles and Benefits

- Interactive and timely communication
- Prioritized review of marketing application
- Efficient and flexible clinical study design
- For devices subject to premarket approval:
  - Enhanced opportunity for pre/post-market balance
  - Expedited review of preapproval manufacturing and quality systems compliance
- Preserves the statutory standards for marketing authorization

Regulatory Context

- Voluntary program created under Section 515B of the Federal Food, Drug, & Cosmetic (FD&C) Act
  - Enacted as part of the 21st Century Cures Act in December 2016
  - Amended by the FDA Reauthorization Act of 2017
  - Amended by the SUPPORT for the Patients and Communities Act
- Guidance describing program's implementation was first issued in December 2018
  - Covers program principles, designation request process, and features available to sponsors
  - CDRH Learn Module Available (Section *How to Study and Market Your Device*, Subsection *Clinical Studies/Investigational Device Exemption (IDE)*)

Regulatory Context

- Select updates to existing Breakthrough Devices Program guidance were proposed in a draft guidance in October 2022
  - Open comment period for 60 days
  - Updates now incorporated into final guidance issued September 15, 2023

Eligibility Considerations

- Medical devices and device-led combination products
- Subject to future marketing authorization through Premarket Approval (PMA), De Novo, or 510(k)
- Meets Breakthrough criteria specified in Section 515B(b) of FD&C Act
  - Must fully meet Breakthrough Device Criterion 1 AND one of the sub-parts of Breakthrough Device Criterion 2

Breakthrough Device Criterion #1

Criterion 1: The device provides for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human disease or conditions;

Breakthrough Device Criterion #2

The device meets **at least one** of the following sub-parts in **Criterion 2**:

- 2A: Represents breakthrough technology
- 2B: No approved or cleared alternatives exist
- 2C: Offers significant advantages over existing approved or cleared alternatives
- 2D: Device availability is in the best interest of patients

Updates to Breakthrough Devices Program Guidance

Summary of Updates

- Guidance clarifies:
  - Criterion 1 designation considerations
  - Considerations for devices that benefit populations impacted by health and/or health care disparities
  - Availability of the program for certain non-addictive medical products intended to treat pain or addiction
  - Policy regarding disclosure of Breakthrough-designated devices
- Program designation criteria and goals remain unchanged

Background: Criterion 1

Criterion 1: *provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human disease or conditions*

- Sponsors should demonstrate a *reasonable expectation* that the device could provide for more effective treatment or diagnosis of the disease or condition identified in the proposed indications for use
  - Technical success: the device could function as intended
  - Clinical Success: a functioning device could more effectively treat or diagnose the identified disease or condition

Background: Criterion 1

Criterion 1: *provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human disease or conditions;*

- Mechanisms for demonstrating a reasonable expectation of technical and clinical success could include literature or preliminary data (bench, animal, or clinical)

Updates to Considerations for Criterion 1

Updates to Section III.B.1 of guidance clarifies that:

- 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:
  - Intended use of device
  - Device's technology and features
  - Available standard of care alternatives

Updates to Considerations for Criterion 1

- FDA considers totality of information when evaluating criterion 1, including device's:
  - Function
  - Potential for clinical and technical success
  - Potential for a clinically-meaningful impact
  - Potential benefits and risks
- Determination of whether a device is reasonably expected to "provide for more effective treatment or diagnosis" is based upon all these factors

Reducing Health/Health Care Disparities

- Health and health care disparities occur across diverse populations[1,2]
- Addressing disparities may help improve health outcomes for all patients
- FDA recognizes need for innovative technologies to help address disparities

1. 2021 National Healthcare Quality and Disparities Report: www.ahrq.gov/sites/default/files/wysiwyg/research/findings/nhqrdr/2021qdr.pdf
2. Described in 2015 Report to Congress on Minority Health Activities: www.minorityhealth.hhs.gov/omh/browse.aspx?lvl=2&lvlid=57

Reducing Health/Health Care Disparities

- When assessing eligibility for Breakthrough Devices Program, FDA will consider technologies that may help address disparities and promote health equity by **providing for more effective treatment or diagnosis in populations that exhibit health and health care disparities**

- Update includes creation of subsection III.B.3.d of Breakthrough guidance

Characteristic Differences

- Health and health care disparities exist and occur across many dimensions, which may influence treatment response/outcomes[1]
  - Treatment outcomes may differ by race, ethnicity, sex, age, disability, and/or other factors
  - For some diseases, pathophysiology, clinical features, and response to treatment may be impacted by these factors
  - Example: Influence of age-related physiologic changes on health outcomes in pediatric and geriatric patients

1. 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: pubmed.ncbi.nlm.nih.gov/25032386/

Characteristic Differences

- Disparities may occur when:
  - Differences go unrecognized
  - Lack of treatment options designed to effectively diagnose or treat conditions in a manner that addresses these differences

Characteristic Differences

- When evaluating if reasonable expectation that device may provide for more effective treatment or diagnosis as compared to current standard of care:
  - **FDA considers devices tailored to address characteristic differences, including device potential to be more effective in certain populations**
  - Examples: social factors, phenotypic variations, pathophysiology, and/or response to treatment

Rare Diseases and Conditions

- Disparities may occur if limited diagnostic and treatment options for rare conditions
- Updated guidance reflects FDA's consideration of devices tailored to address unmet needs in these populations when evaluating Criterion 1
- Marketing pathway eligibility requirements still apply

Accessibility

- Disparities may occur when lack of accessibility prevents patients from receiving medical treatment or diagnosis
- Often device benefit cannot be realized due to this lack of accessibility

Accessibility

- Devices that offer improved accessibility may provide significant benefit to patients
- When evaluating Criterion 1, FDA considers technologies that allow for improved accessibility as compared to standard of care
  - New devices that have 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.
  - Improved accessibility of a device may support that device is 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

Medical Products to Treat Pain or Addiction

- Guidance highlights availability of program for certain non-addictive medical products used to treat pain or addiction
  - Consistent with obligations under the SUPPORT Act
  - Updated Introduction section of guidance

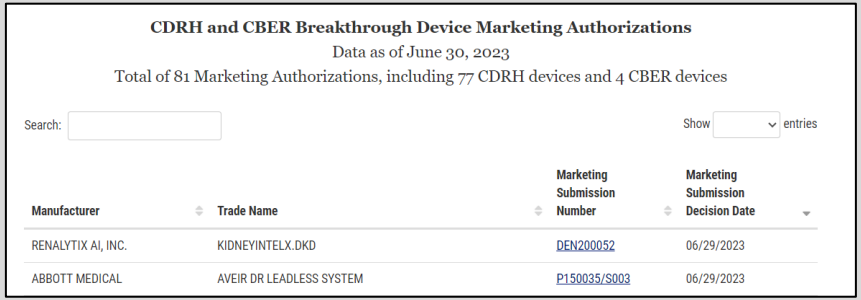
Disclosure of Designated Devices

- Update to Section III.C of guidance
- In general, FDA will not publicly disclose existence of Breakthrough designation requests
  - Consistent with provisions of FD&C Act (including 21 CFR 20 and Freedom of Information Act)
- Exception for designated devices that have been previously publicly disclosed or acknowledged by sponsor

CFR = Code of Federal Regulations

Disclosure of Designated Devices

- Breakthrough Devices Program Webpage: www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program
- FDA will disclose Breakthrough Devices that receive marketing authorization for an indication consistent with its designation
- Breakthrough Devices marketed for an indication not covered by its designation will not be considered a Breakthrough Device and would not be disclosed as such



Resources

Slide Number	Cited Resource	URL
7	Health Disparities and Inequities	www.nhlbi.nih.gov/science/health-disparities-and-inequities
7	Statistics about Diabetes	diabetes.org/about-diabetes/statistics/about-diabetes
7	Infant Mortality	www.cdc.gov/reproductivehealth/maternalinfanthealth/infantmortality.htm

Resources

Slide Number	Cited Resource	URL
7	MW Lewis-Thames et al: Racial and Ethnic Differences in Rural-Urban Trends in 5-Year Survival of Patients With Lung, Prostate, Breast, and Colorectal Cancers: 1975-2011 Surveillance, Epidemiology, and End Results (SEER)	jamanetwork.com/journals/jamanetworkopen/fullarticle/2792392
8	CDRH 2022-2025 Strategic Priorities	www.fda.gov/media/155888/download?attachment
13	CDRH Learn	www.fda.gov/training-and-continuing-education/cdrh-learn
15, 16	Section 515B (b)(2) of the Food, Drug, and Cosmetic Act	uscode.house.gov/browse.xhtml
23	2021 National Healthcare Quality and Disparities Report	www.ahrq.gov/sites/default/files/wysiwyg/research/findings/nhqrdr/2021qdr.pdf

Resources

Slide Number	Cited Resource	URL
23	2015 Report to Congress on Minority Health Activities	www.minorityhealth.hhs.gov/omh/browse.aspx?lvl=2&lvlid=57
25	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	pubmed.ncbi.nlm.nih.gov/25032386/
27	515B(c) of FD&C Act	uscode.house.gov/browse.xhtml
30	SUPPORT for Patients and Communities Act	uscode.house.gov/browse.xhtml
32	Breakthrough Devices Program Webpage	www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program

Summary

- Breakthrough Devices Program is intended to provide patients and health care providers with timely access to Breakthrough Devices
- Updates clarify designation considerations, including Agency's intention to consider technologies aimed at reducing health and health care disparities and devices intended to treat pain and addiction, and timing of Breakthrough designation disclosure
- Guidance updates promote innovation of technologies that address health and health care disparities as a part of CDRH's strategic priority to advance health equity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Let's Take Your Questions

- **To Ask a Question:**
  1. Raise your hand in Zoom 

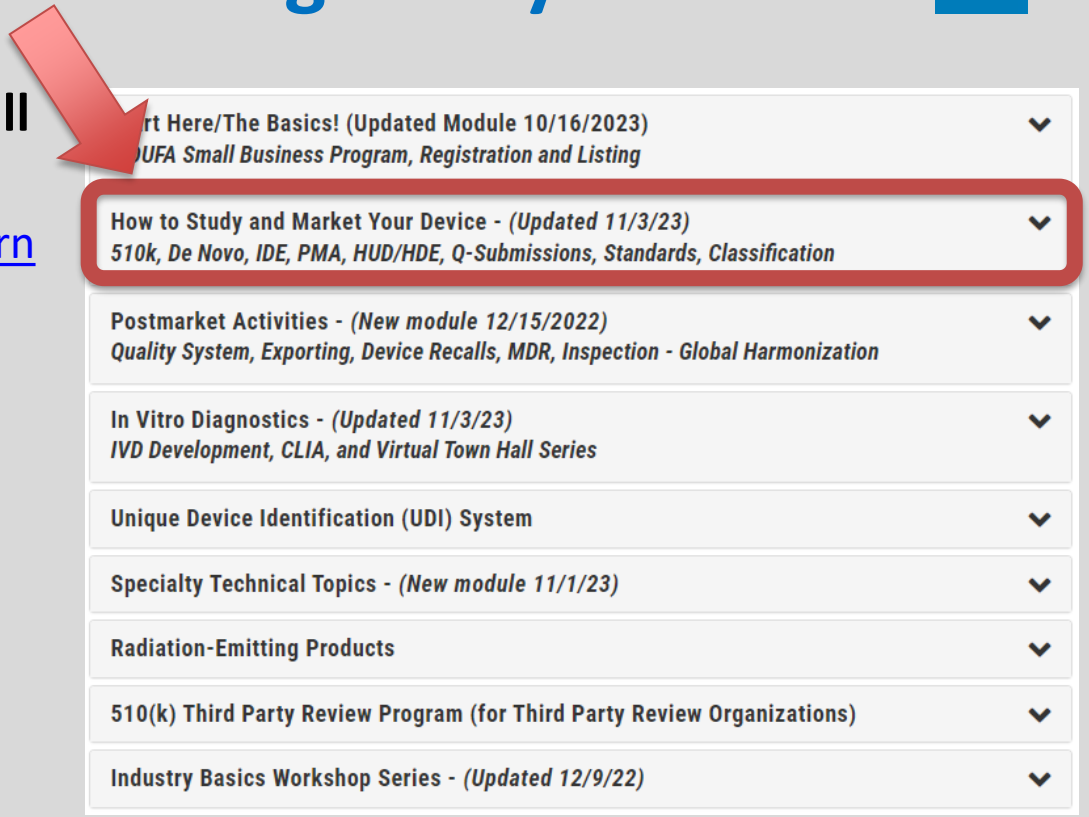
  2. Moderator will announce your name and invite you to ask your question
  3. Unmute yourself when prompted in Zoom to ask your question

- **When Asking a Question:**
  - Ask one question only
  - Keep question short
  - No questions about specific submissions

- **After Question is Answered:**
  - Mute yourself and lower your hand
  - If you have more questions - raise your hand again

Thanks for Joining Today!

- **Presentation and Transcript will be available at CDRH Learn**
  - www.fda.gov/Training/CDRHLearn
- **Additional questions about today's presentation**
  - Email: DICE@fda.hhs.gov
- **Upcoming Webinars**
  - www.fda.gov/CDRHWebinar

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