Medical Device Session

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Humanitarian Use Designations and Humanitarian Device Exemptions
Provide incentive for development of devices intended for treatment or diagnosis, in small patient populations where otherwise a device manufacturer’s research and development costs could exceed market returns.
HUDs and HDEs

• Humanitarian Use Device (HUD)
• Humanitarian Device Exemption (HDE) application

- FDA Office of Orphan Products Development designates HUD
- Device intended to treat a disease or condition affecting fewer than 4,000 individuals in the US per year
- No comparable device available
- CDRH reviews HDE
HDE Approval Threshold

Device does not expose patients to unreasonable risk of illness or injury

AND

Probable benefit outweighs the risks of using the device, taking into account the probable risks and benefits of alternative therapies
Flexibility in HDE Review

- Probable Benefit Standard demonstrated by:
  - Clinical experience
  - Retrospective analysis
  - Clinical trial design: open-label, smaller trial size

- Post-approval studies
Medically Plausible Subset

If the disease or conditions occurs in > 4,000 patients/year, the device could be used in a subset of the disease or condition AS LONG AS:

• sponsor shows the subset is medically plausible (NOT just "readily identifiable")

• use of the device is limited to that subset because of some inherent property of the device and/or the disease

• sponsor must explain why the device couldn't also be used in all patients with the disease or condition
Second Sight Retinal Products, Inc.
Argus II Retinal Prosthesis System

Intended for patients aged 25 years and older with bare or no light perception vision caused by advanced retinitis pigmentosa
21st Century Cures Legislation

• Would change the threshold from fewer than 4,000 individuals to fewer than 8,000 individuals in the US per year

• House passed legislation in July 2015

• Senate bill under development
Pediatric Medical Devices
New Developments

• Leveraging Existing Clinical Data for Extrapolation to Pediatric Uses of Medical Devices Draft Guidance (May 2015)

• Hired Chief Medical Officer – Pediatrics and Special Populations
Flexible Regulatory Paradigms
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Continuum of Clinical Study Onset and Market Entry Points

CDRH Vision

Benefit-Risk Tradeoffs

Clinical Trials
- Early Feasibility Study Paradigm Guidance (2013)

Premarket-Postmarket Balance
- Expedited Access Pathway Program (2015)
- Balancing Premarket and Postmarket Data Collection (2015)

Science of Patient Input
- Medical Device Innovation Consortium (MDIC) Patient Centered Benefit-Risk Project

Should we have a progressive/conditional approval pathway?

Center for Devices and Radiological Health
Flexible Regulatory Paradigms

Smart Regulation When You Need It
No Regulation When You Don’t

Extensive deregulation of low-risk digital health technologies

- Mobile Medical Apps Guidance (2013)
- Medical Device Data Systems Guidance (2015)
- General Wellness Claims Draft Guidance (2015)

New framework for Software as a Medical Device under development intended to meet the needs of rapid innovation cycles, such as through greater reliance on quality system controls rather than premarket review
Breakthrough Devices
2014 - 2015 CDRH Strategic Priorities
Strike the Right Balance Between Premarket and Postmarket Data Collection

• Launched the Expedited Access Pathway Program in April 2015 for breakthrough devices
  ▪ Eligible devices are those subject to a PMA or de novo intended to treat or diagnose a life-threatening or irreversibly debilitating disease and address an unmet need
  ▪ Early, ongoing, and extensive interaction with review team, engagement by senior management, assignment of a case manager, and collaborative creation of a Data Development Plan
  ▪ Where appropriate, some premarket data collection shifted to the postmarket setting for PMA devices
Partnering with Patients
Where can patient perspectives inform the medical device TPLC?
Goal is to improve patient health by better matching needs and preferences

Patient Engagement + Science of Patient Input = Patient-Centric Healthcare
Partnering with Patients

• Investing in the Science of Patient Input
  ▪ Patient Preference Initiative
    • May 2015 Draft Guidance
    • May 2015 MDIC Framework
  ▪ Expanded use of Patient Reported Outcomes

• Establishment of a Patient Engagement Advisory Committee
Patient Engagement Advisory Committee (PEAC)

• **To help assure the needs and experiences of patients are incorporated into our work, the PEAC will:**

  1. Advise CDRH on ways to include and foster participation of patients where appropriate throughout the total product lifecycle
  2. Advise CDRH on patient perspectives about current and new approaches or policies for integrating patient input in regulatory decision-making
  3. Serve as a resource to CDRH as a body of experts in patient experience, needs, and the activities of the patient community

• **Inaugural Meeting in 2016**
Medical Device Development Tools
What is Regulatory Science?

The science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of FDA-regulated products

- Benefits patients by speeding the rate of important technologies reaching market
- Reduces time and resources needed for device development, assessment, and review. For example:
  - Can lead to quicker, more efficient device approvals
  - Can decrease the size and duration of pre-market clinical trials

Faster, Safer, More Cost-effective
Medical Device Development Tools

- MDDT Qualification
- Clinical Outcome Assessments*
- Biomarker Tests
- Nonclinical Assessment Models

*Includes Patient Reported Outcomes (PROs)
Medical Device Development Tools (MDDT) Program

Draft Guidance for Industry, Tool Developers, and Food and Drug Administration Staff

http://www.fda.gov/medicaldevices/deviceregulationandguidance/guidance - 11/14/13

• When finalized the draft guidance will represent FDA’s current thinking on qualification of MDDTs for use in device development and evaluation

Pilot Program for Qualification of Medical Device Development Tools – 8/15/14

• This document outlines:
  ▪ The guidelines underlying the MDDT Pilot Program;
  ▪ Appropriate candidates for the MDDT Pilot Program; and
  ▪ The procedures FDA intends to follow in the MDDT Pilot Program
Bridging Advances in Regulatory Science into Regulatory Application

TODAY: Tools considered and evaluated on a case-by-case basis

TOMORROW: Qualified for regulatory purposes within a defined context of use
Medical Device Innovation Consortium

A Public-Private Partnership collaborating on Regulatory Science to make patient access to new medical device technologies faster, safer, and more cost-effective
Precision Medicine
Precision Medicine Initiative: Modernizing FDA Regulation of Genomic Laboratory Tests

• New regulatory strategies for next generation sequencing
  ▪ Finalize regulation of laboratory-developed tests
  ▪ Develop and implement standards to assure quality
  ▪ Develop open-source tools to help test developers meet standards, facilitated by precisionFDA
  ▪ Develop a flexible standards-based regulatory alternative to premarket review

• Goals
  ▪ Protect patient safety by assuring genomic test quality
  ▪ Promote translation and innovation to advance precision medicine by adopting a flexible regulatory system
  ▪ Support the success of the PMI by ensuring the quality of the data used for future research
National Evaluation System for Medical Devices
Strengthening Our National System
Taking the Next Steps

Strengthening Our National System for Medical Device Postmarket Surveillance

Center for Devices and Radiological Health
U.S. Food and Drug Administration
September 2012

Strengthening Our National System for Medical Device Postmarket Surveillance
Update and Next Steps

Center for Devices and Radiological Health
U.S. Food and Drug Administration
April 2013
FDA’s Vision for a National System
For the Ecosystem, Governed by the Ecosystem

• Develops and communicates an evolving understanding of device benefits and risks throughout their marketed life using high-quality, linked electronic health information

• Identifies potential safety signals in near real-time from variety of privacy-protected data sources serving as a safety net

• Reduces burdens and costs of medical device postmarket surveillance

• Facilitates clearance and approval of new devices or new uses of existing devices
Proof of Concept
Use of Real World Evidence to Expand Minimally Invasive Heart Value Replacement Indications

Society of Thoracic Surgeons (STS) and American College of Cardiology (ACC) Transcatheter Valve Therapy (TVT) Registry

• Used for regulatory decision making: expansion of use, label change
• Linked to claims data for longitudinal study of transcatheter aortic value replacement
• Increased speed and efficiency of studies
Learning Medical Device Ecosystem

INFORMATION FLOW

TIME TO MARKET

Premarket Review

Expedited Access Pathway

Benefit Risk

Premarket Decision

Evidence from Clinical Experience

“Safety Net”

Center for Devices and Radiological Health
Thank You