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# Accelerating Medical Device Innovation in the U.S.

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The National Academies  
Innovation Policy Forum  
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# It's About the Patients



# FDA & US Medical Device Innovation

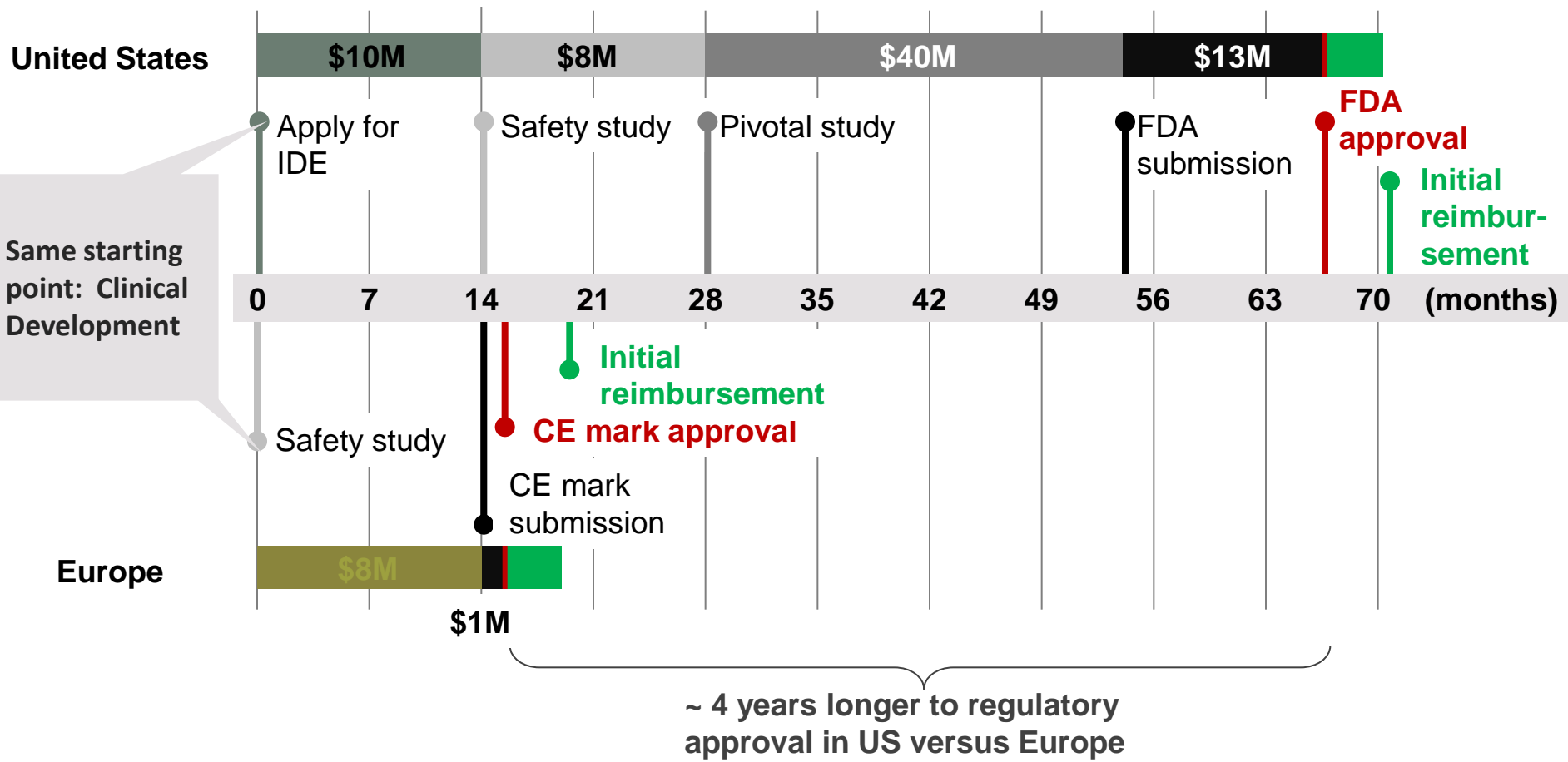
- **CDRH Mission**

- Facilitate medical device innovation by providing industry with predictable, consistent, transparent and efficient regulatory pathways
- Provide stakeholders with understandable and accessible science-based information about medical devices

- **CDRH Vision**

- **Patients** in the US have access to high-quality, safe, and effective medical devices of public health importance first in the world
- US is the world's leader in medical device innovation

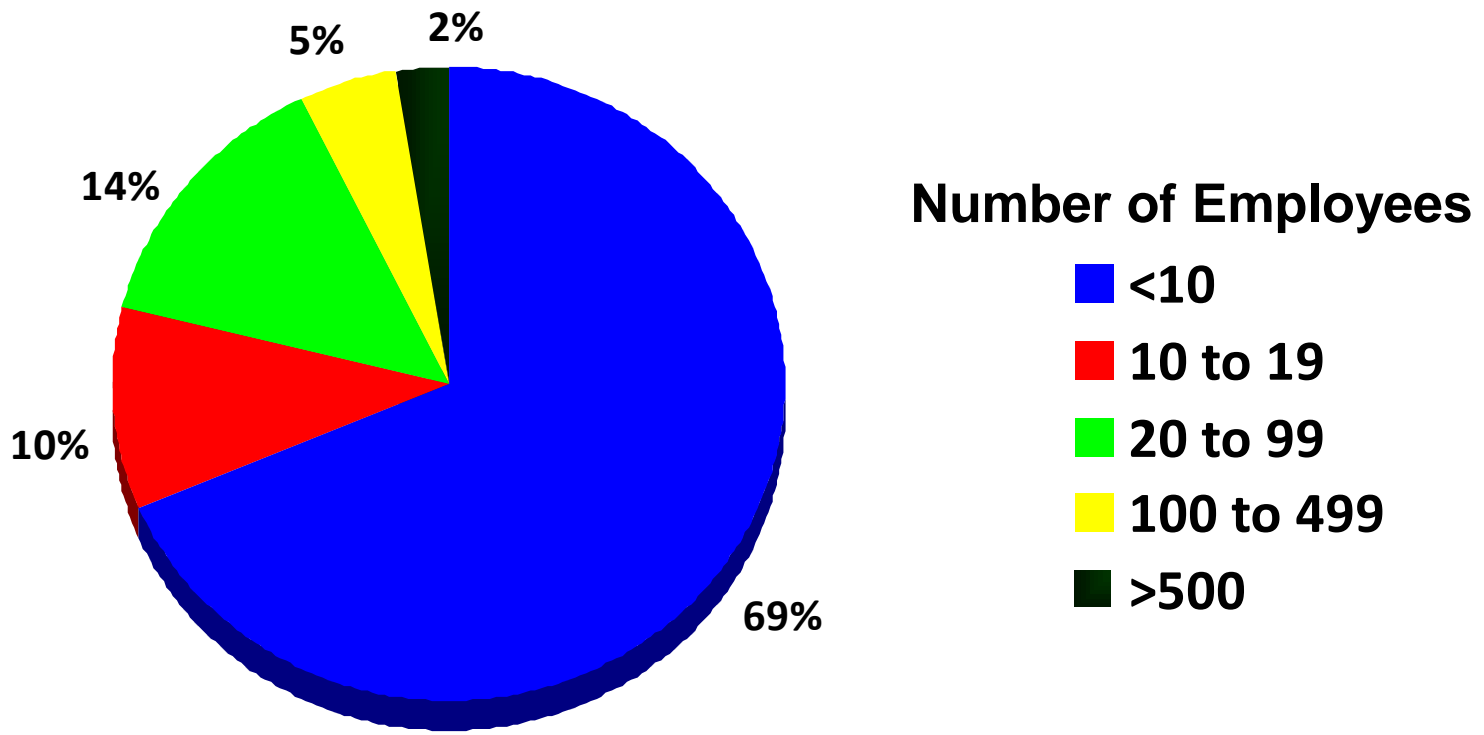
# Makower Report (2010): FDA Impact on US Medical Technology Innovation



Same starting point: Clinical Development



# U.S. Medical Device Manufacturing Companies by Number of Employees

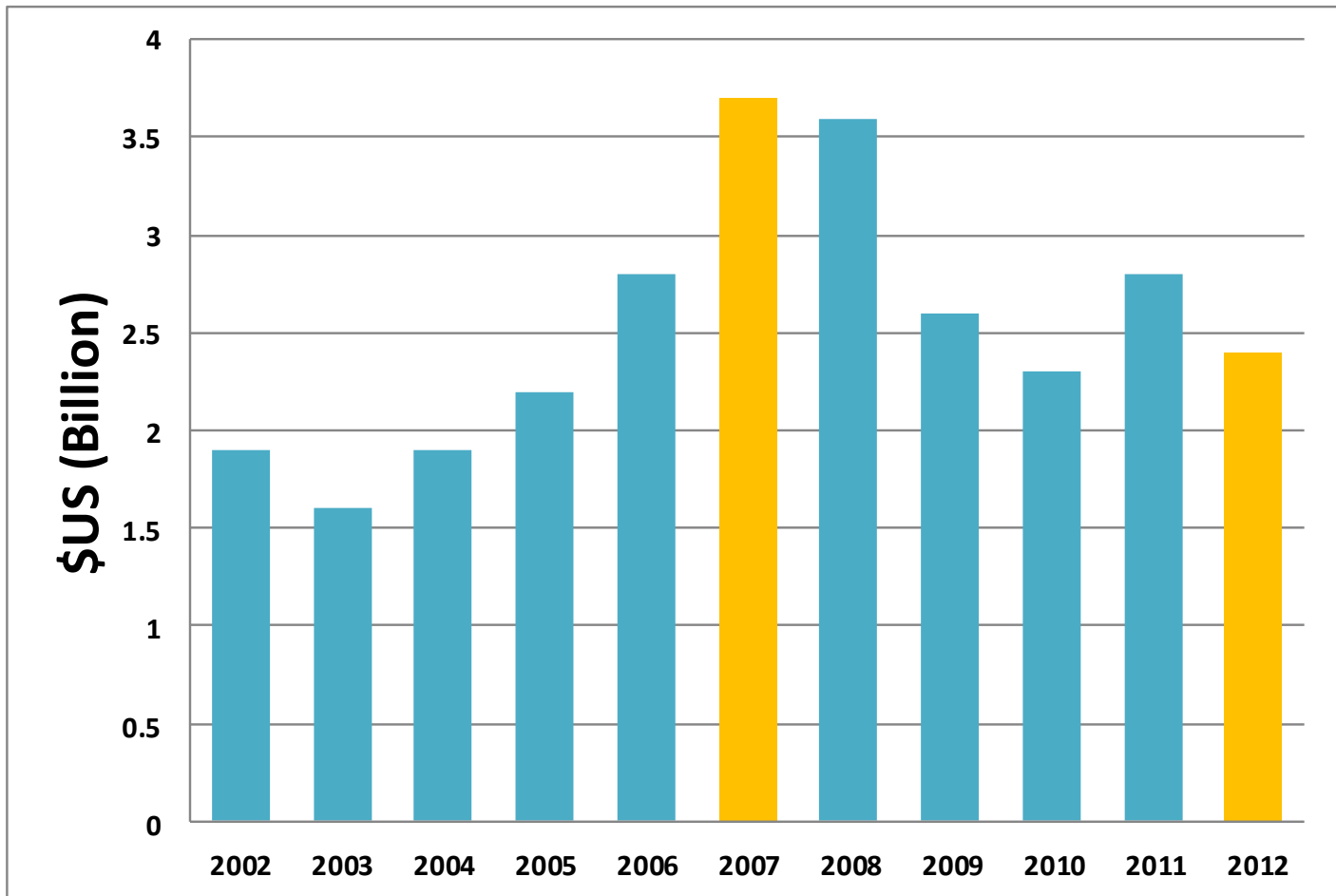


Source: Dun and Bradstreet, Inc.



# The *Crisis* in Medical Device Innovation

Start-Up investment down >35% since 2007 peak





# US Medical Device Industry: Innovation Challenges

Factors cited as having the highest impact on decisions to move medical device investment outside of U.S.\*

**Regulatory Challenges - 38%**

**Reimbursement Concerns - 18%**

**Clinical Trial Issues - 14%**

\* from National Venture Capital Association/Medical Innovation & Competitiveness Coalition survey of 259 NVCA member firms investing in the healthcare sectors; 60% (156 firms responding) October, 2011

# Time is Money

## Estimated Cost of FDA Decisions on a 30 Employee Company

	Expense to Company
8 Week Delay in Scheduling a Meeting	\$1.8 M
Additional 20 Animal Study (6 months)	\$5.5 M
Extra Year in Negotiating an IDE	\$10.8 M
Additional 100 patient study with 1 year Follow-up (24 months)	\$24.1 M

Versant Ventures







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# US Medical Device Development: Current Landscape

- **FDA acknowledges real or perceived issues:**
  - Early clinical study of novel devices moved/moving OUS
  - Device innovation may follow OUS
  - Devices are being exclusively developed for non-US markets
  - FDA's requirements can be an impediment to timely clinical study of novel early-stage devices
- **Growing concern regarding the time lag in the availability of beneficial medical devices for US patients**



## Case Study: Trans-Aortic Valve Replacement (TAVR)

- Initial devices were developed by start-up companies outside of U.S.
- It was 9 years from first-in-man (in France) to US regulatory approval
- It took 4 years from CE Mark to FDA approval
- U.S. was the 43<sup>rd</sup> country in the world to receive approval (Brazil 42<sup>nd</sup>, Albania 44<sup>th</sup>)
- 20-30,000 patients received TAVR worldwide before US approval
- Edwards and Medtronic have spent ~ \$2 Billion combined bringing devices to US market



# Climate Change at FDA

How can the FDA innovate itself?

How can the FDA spur innovation in the regulated industry?

What is FDA's role in strengthening the U.S. Medical Device Ecosystem?



# Climate Change at FDA

FDA recognizes:

- A need for a cultural change to emphasize the role of promoting public health while maintaining our standards in protecting public health
- Human costs associated with delaying access to new technology, in the context of the limitations of current treatment alternatives
- Advantages for the device development process with US clinical studies (including FIH and early feasibility studies)
  - Opportunity for close contact between developers and investigators
  - Early access to promising novel technology

# Words into Deeds

The core principles in meeting these priorities are the application of specific guidelines throughout the regulatory decision-making process:

- **Benefit/Risk Principles**

- Identifies the factors to make benefit/risk determinations during premarket review

- **Early Feasibility Studies**

- Application of benefit/risk principles to justify study initiation

- **The Innovation Pathway**

- Pilot program for innovative devices with promising and potentially far reaching benefits

- **CDRH 2014-2015 Strategic Priorities**



# Benefit-Risk Principles



Allow regulators to:

- Consider the **totality** of the benefit/risk profile for the device, for example:
  - Disease condition (e.g., life-limiting, life-threatening)
  - Limitations of and risks associated with currently available therapies
- Consider **patient tolerance** for risk and **perspective** on benefits
  - Severity and chronicity of disease
  - Availability of alternative treatments/diagnostic modalities
- Take into account **risk mitigation** strategies when balancing risks and benefits



# Early Feasibility Studies

- Intended to facilitate the earliest clinical evaluation of medical devices in the US under the Investigational Device Exemptions (IDE) regulations
- Elements that define an early feasibility study:
  - Device intended for a specific indication that may be early in development, typically before the device design has been finalized
  - When exhaustive nonclinical testing would not likely provide the information needed to further device development
  - Small number of subjects mitigate risks
  - EFS IDE approval may be based on less non-clinical data



# The Innovation Pathway

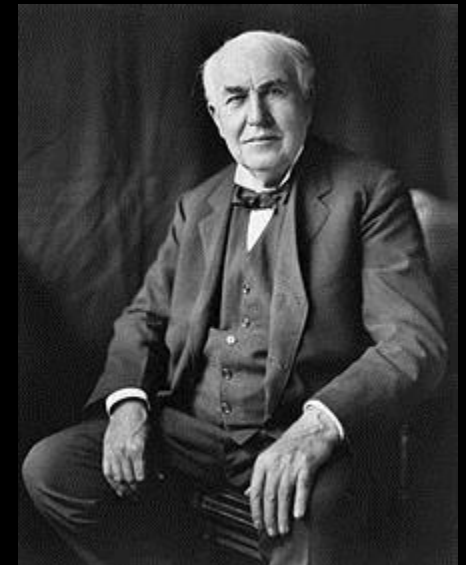
- Pilot program launched February 2011
- Targeted for pioneering technologies that address unmet clinical needs and have the potential to revolutionize patient care or health care delivery
  - Breakthrough technology that provides a clinically meaningful advantage over existing technology
  - No approved alternative treatment or means of diagnosis exists
  - Significant, clinically meaningful advantages over existing approved alternative treatments



# INNOVATION PATHWAY GUIDING PRINCIPLES

- 1) When the goals are understood, the methods to achieve them can be improvised.
- 2) We value improvisation, experimentation, risk-taking, and problem-solving over process-following and standardization (uniformity of approach).
- 3) Okay to fail.

*Edison: "Hell, we ain't got no rules around here. We're trying to accomplish something!"*



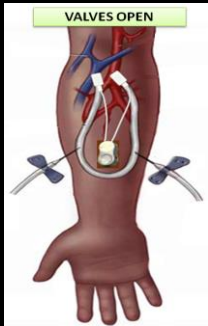


# Innovation Program Goals

- Shorten the time from concept to commercialization
  - Early and continuous interaction between FDA and sponsors to facilitate innovation; apply B/R principles
- Transform the user experience
  - Create shared “one team” view of success
  - Collaboration is the key
- Make decisions that create forward momentum
  - Understand costs (time) of different regulatory choices
  - Trade-offs; uncertainty; mitigations

# END-STAGE RENAL DISEASE INNOVATION PATHWAY CHALLENGE

- 32 Device applicants; 3 selected
- Collaboration Phase – Summer 2012



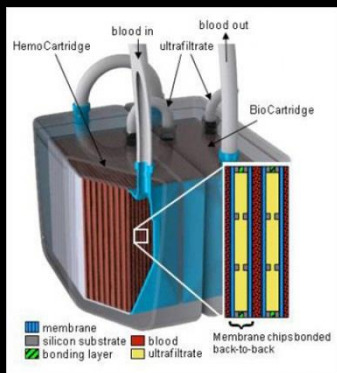
## HemoAccess Valve System

- A mechanical valve system allowing blood flow into AV graft only during dialysis



## Wearable Artificial Kidney

- A miniaturized wearable dialysis machine to provide hemodialysis



## Implantable Bio-Artificial Kidney

(Univ. California, San Francisco)

- Filters toxins from blood, and provide other biological functions giving patients 24/7 dialysis

# ENTRPRENEURS-IN-RESIDENCE (EIR) PROGRAM

- Goal: Transformational change by combining the internal and external talent applying lean engineering and Innovation Pathway principles
- Supported by the White House Office of Science and Technology Policy



## EIR 2011-2012



Time-limited recruitment of world-class innovators entrepreneurs and visionaries from a variety of medical-device related fields to join highly-qualified FDA staff in the development of solutions in areas that impact innovation



# Entrepreneurs in Residence 2012-2013

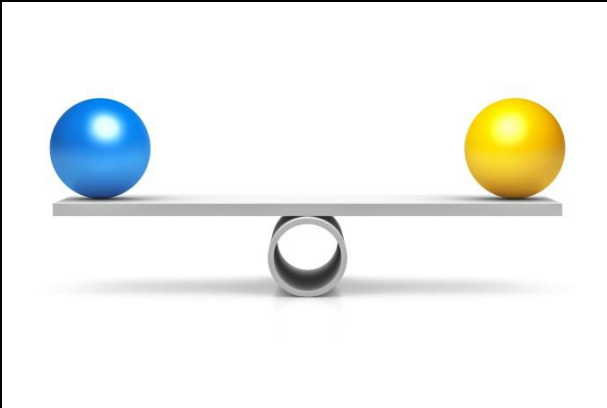
## Three New Projects (EIR groups):

- Streamlining clinical trials: Find ways to reduce the time and cost of clinical trials in support of FDA approval or clearance of medical devices
- Striking the right balance between pre- and post-market evidentiary requirements: Find ways to allow efficient use of pre-market studies to allow for timely approvals, with greater emphasis on capturing informative real-world data in post-market studies
- Streamlining approval to reimbursement: Find ways to reduce the inefficiencies and delays in data collection to support FDA approval and CMS coverage

# CDRH 2014-2015 STRATEGIC PRIORITIES



**Strengthen the Clinical Trial  
Enterprise in the US**



**Strike the Right Balance  
Between Premarket and  
Postmarket Data Collection**



**Provide Excellent  
Customer Service**

# Strengthen the Clinical Trial Enterprise



## IDE CYCLES

Reduce the number of IDEs requiring more than two cycles to an appropriate full approval decision

Sept. 30, 2014 – 25%\*

June 30, 2015 – 50%\*

## IDE APPROVAL TIME

Reduce the overall median time to appropriate full IDE approval by

Sept. 30, 2014— 25%\*

**Clinical  
Trials  
Program**

Increase the number of early feasibility/FIH IDE studies submitted to FDA and conducted in the U.S.

Implemented FDASIA Benefit-Risk Determinations

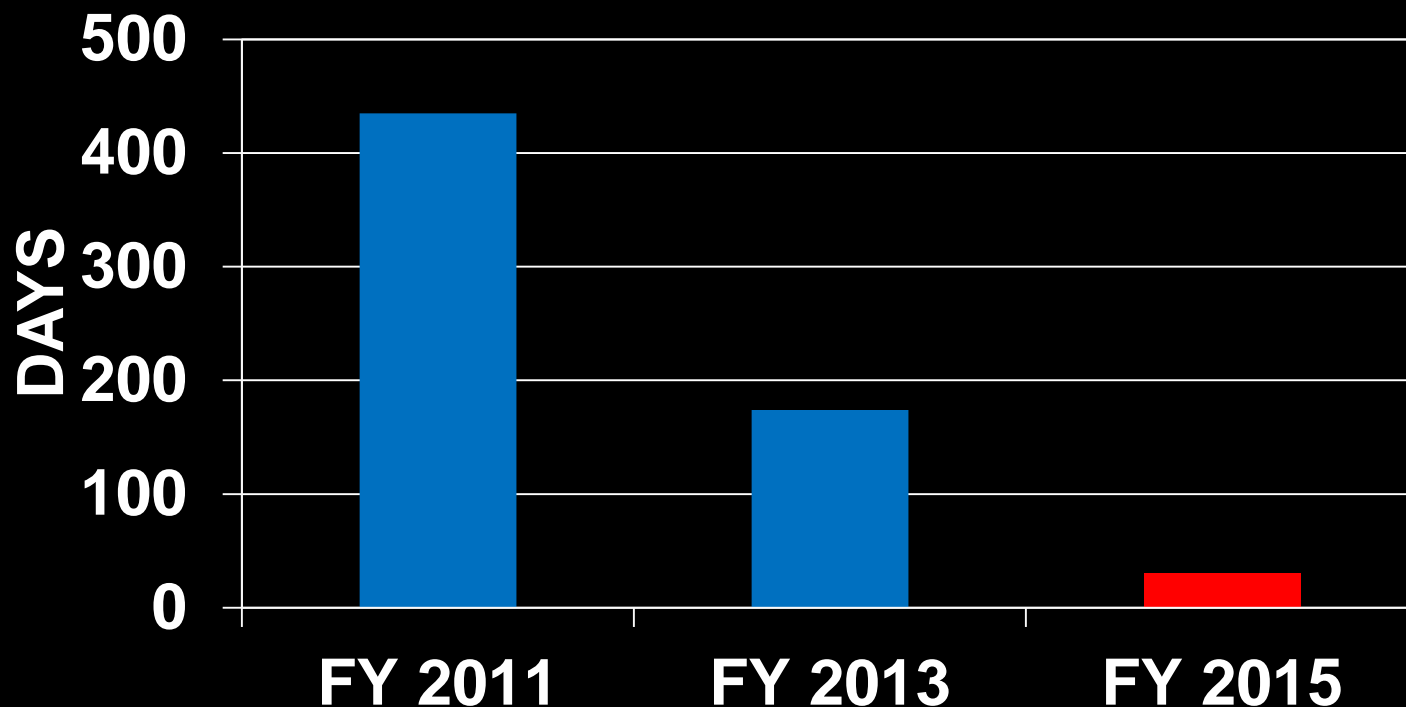
\*Compared to FY 2013 performance





# Strengthen the Clinical Trial Enterprise

**CDRH FY 2015 Target: Reduce overall median time to full appropriate IDE approval to 30 days**



**Median Time to Full Appropriate IDE Approval**



# Strike the Right Balance Between Premarket and Postmarket Data Collection

Assure the **appropriate balance** between pre- and postmarket data requirements to facilitate and expedite the development and review of medical devices

Device types subject to a PMA that have been on the market:

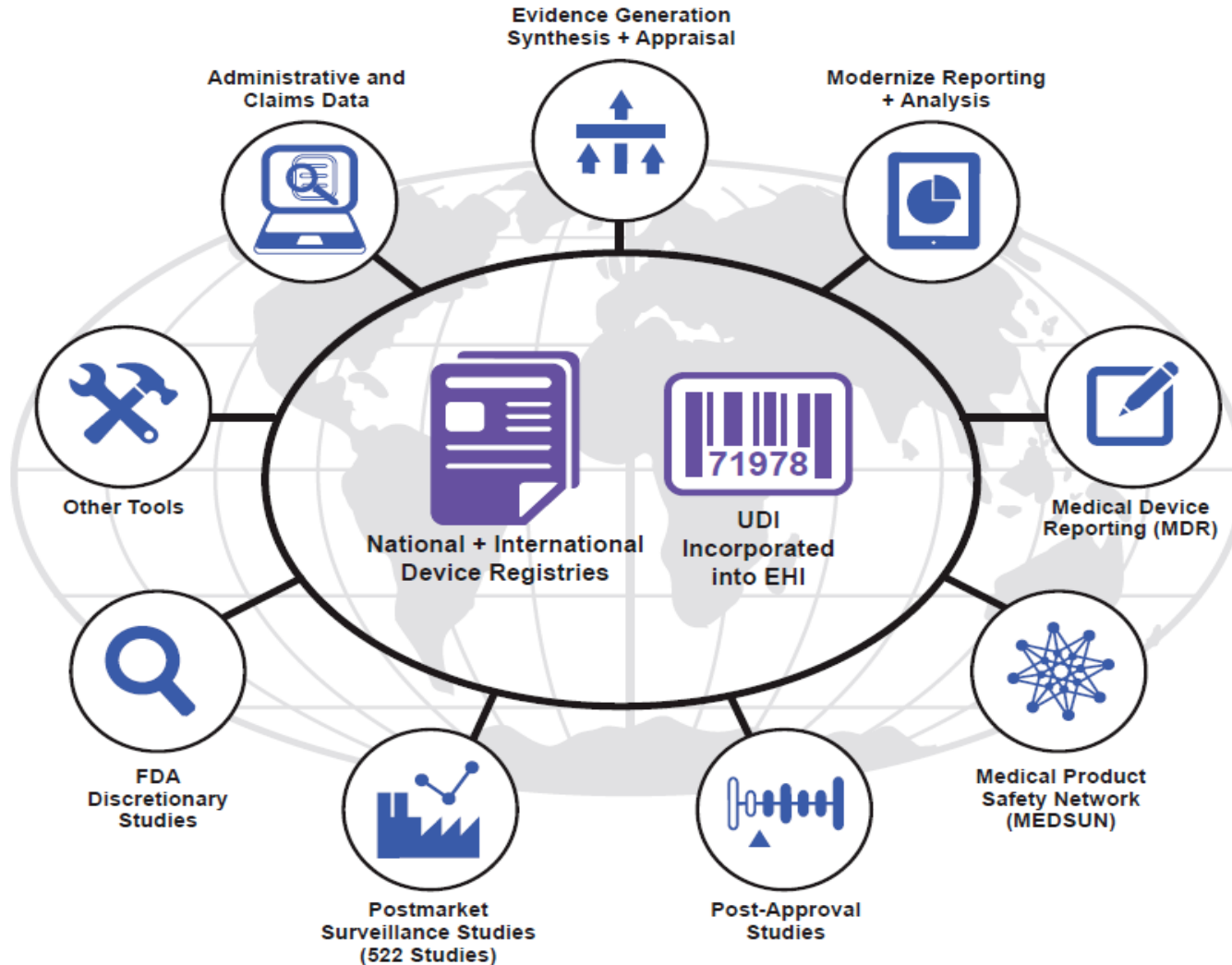
- By 12/31/14 review 50%
- By 6/30/15 review 75%
- By 12/31/15 review 100%

to determine whether or not to shift some premarket data requirements to the postmarket setting or to pursue down classification, and communicate those decisions to the public

Develop and seek public comment on a **framework** for when it is appropriate to shift premarket data collection to the postmarket setting

Develop and seek public comment on a **new pathway** to market for devices subject to a PMA that address an unmet public health need by shifting appropriate premarket data needs to the postmarket setting and incorporating features of the Innovation Pathway pilots

# National Medical Device Postmarket Surveillance Plan



# Provide Excellent Customer Service

**Implement Customer Service Standards**

**By 12/31/14, achieve at least 70 percent customer satisfaction.**

- **By 6/30/15, achieve at least 80 percent customer satisfaction.**
- **By 6/31/15, achieve at least 90 percent customer satisfaction.**

**Assess customer satisfaction using a standardized survey tools**

**Implement the principles and practices outlined in the CDRH Quality Management Framework to improve the quality and performance of CDRH processes and services.**

# Keys to Patient Access

## Evidence



## Value



## Reimbursement



## Market





# Medical Device Reimbursement Task Force

“Winning coverage and payment has become a steeper challenge than gaining FDA approval for small device firms in recent years” Mike Carusi on behalf of NVCA, AdvaMed and MDMA

## Mission

Streamline the pathway from regulatory clearance or approval to reimbursement to support patient access to innovative medical devices

## Plan

Develop a voluntary process that facilitates earlier interactions with payers, including private 3<sup>rd</sup> party payers about evidence to support coverage and reimbursement

## Staff

OCD / ODE / OIR with support from CMS

# What are we proposing?



FDA Review Team



Manufacturer



Payer

Provide a process to enable manufacturers to include and engage payers during meetings with FDA using the Pre-Submission program.



## Looking to the Future

# EXPEDITED ACCESS PMA

- Based on Innovation Pathway pilots
- Focus is to expedite patient access for:
  - High risk medical devices
  - Treatment or diagnosis of a life-threatening or irreversibly debilitating disease or condition
  - Addresses an unmet medical need that
    - Has no alternative treatment or means of diagnosis
    - Represents breakthrough technology
    - Provides a clinically meaningful advantage over existing technology

Draft guidance issued April 23, 2014

<http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm393879.htm>



## Looking to the Future

# BALANCING PRE AND POSTMARKET DATA COLLECTION

- Based on 2014-15 Strategic Priority #2
  - Supports expedited patient access pathway for PMA devices
  - Determined by Benefit-Risk Profile
  - Supported by FD&C Act, section 513(a)(3)(c)
  - Allows for approval with greater degree of uncertainty for devices that serve an unmet need
  - Supported by the National Medical Device Postmarket Surveillance system



# THANK YOU



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