Patient-Centric Perspective: Models for Patient Engagement

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Evolution of PDUFA

Initial patient group engagement

Shift to consumer engagement

Patient groups re-engage

1993
PDUFA I: Backlog reduction

1998
PDUFA II: FDAMA
Reduce review times

2003
PDUFA III: BTP

2008
PDUFA IV: FDAAA
• Unintended consequences
• Process for engagement

2013
PDUFA V: FDASIA
• Benefit/Risk framework
• Biomarkers/PROs
• PFDD
• Rare diseases

2018
PDUFA VI
Patient engagement throughout the development lifecycle
Dialogue / Advancing Meaningful Patient Engagement in Drug Research, Development & Approval

• Promote a culture shift
• Facilitate open communication
• Create regulatory guardrails
PDUFA VI

Patient Engagement Guidances

Fiscal Year

- **FY 2018**: Burden of Disease
- **FY 2019**: Holistic Sets of Impacts
- **FY 2020**: Measures for Identified Impacts
- **FY 2021**: Clinical Outcomes Assessments
Value Model Development Process

- Planning
- Drafting and Refinement
- Dissemination and Implementation
- Evaluation
- Update and Maintenance

Patient Partnership
- Transparency to Patients
- Inclusiveness of Patients
- Diversity of Patients/Populations
- Outcomes Patients Care About
- Patient-Centered Data Sources
Questions?
Partnering with Patients
To Advance Medical Device Innovation & Patient Safety

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Office of the Center Director
FDA Center for Devices and Radiological Health

FDLI Annual Meeting, May 4, 2017
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The $136B Medical Device Sector is Critical to Our Nation’s Health
Patients are at the Heart of All We Do

CDRH Vision:
Patients in the U.S. have access to high-quality, safe, and effective medical devices of public health importance, first in the world.

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The Accelerating Pace of Change: Information Age and Digital Revolution

1. The accelerating pace of change...

   - Agricultural Revolution: 6,000 years
   - Industrial Revolution: 120 years
   - Light-bulb: 90 years
   - Moon landing: 22 years
   - World Wide Web: 9 years
   - Human genome sequenced

   2045: Surpasses brainpower equivalent to that of all human brains combined

2. ... and exponential growth in computing power...

   Computer technology, shown here climbing dramatically by powers of 10, is now progressing more each hour than it did in its entire first 90 years

3. ... will lead to the Singularity

   - Apple II: At a price of $1,298, the compact machine was one of the first massively popular personal computers
   - UNIVAC I: The first commercially marketed computer, used to tabulate the U.S. Census, occupied 943 cu. ft.
   - ENIAC: The electronic computer, with 1,500 vacuum tubes, helped the British crack German codes during WW II

   Source: Time Magazine, 2011
Evolution of the Patient’s Role

Traditional Medicine: Provider-led treatment decision-making

Emerging Diseases: Patient advocacy for availability of and access to new treatments

The Internet: Patient empowerment through information

The Future Today: Patient-Provider partnership in treatment decision-making
“You have to learn about thousands of diseases, but I only have to focus on fixing what’s wrong with ME! Now which one of us do you think is the expert?”
“...the FDA is working to give patients a greater voice in medical product development and evaluation. This kind of active involvement is an essential component of the President’s Precision Medicine Initiative. [...] 

Success in these efforts could lead to tremendous advances in the understanding of health, disease, diagnosis, treatment, and recovery, ultimately transforming patients' experience of health care by enabling physicians to tailor care to an individual's specific needs and preferences.”

Hunter NL, O’Callaghan KM, Califf RM. JAMA 2015
Partner with Patients

We interact with patients as partners and work together to advance the development and evaluation of innovative devices, and monitor the performance of marketed devices.

Goal 1 – Promote a culture of meaningful patient engagement by facilitating CDRH interaction with patients.

Goal 2 – Increase use and transparency of patient input as evidence in our decision-making.
Investing in Culture: Patient Focused

MDUFA IV Agreement in Principle

- Patient input in clinical trials
- Patient preference information (PPI)
- Patient reported outcomes (PRO)

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Promote a culture of meaningful patient engagement by facilitating CDRH interaction with patients

PATIENT ENGAGEMENT
How can we better utilize Patient Input in Regulatory Decision Making?
Where can patient input inform Medical Device development and evaluation?

- **Patient-Informed Needs**: Discovery + Ideation
- **Invention + Prototyping**: INVENTION + PROTOTYPING
- **Pre-Clinical**: PRE-CLINICAL
- **Clinical**: CLINICAL
- **Regulatory Decision**: REGULATORY DECISION
- **Product Launch**: PRODUCT LAUNCH
- **Post-Market Monitoring**: POST-MARKET MONITORING
- **Patient Preference Information in Benefit-Risk**: Patient Preference Information in Benefit-Risk
- **Patient-Centered Outcomes**: Patient-Centered Outcomes
- **Communicating Benefit-Risk Information to Patients**: Communicating Benefit-Risk Information to Patients
- **Patient-Informed Clinical Trial Design, Patient Reported Outcomes**: Patient-Informed Clinical Trial Design, Patient Reported Outcomes

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Increase use and transparency of patient input as evidence in CDRH decision-making

SCIENCE OF PATIENT INPUT
What can PROs and PPI tell us?

**Patient Reported Outcomes (PRO)**
- Endpoints in regulatory studies
- Outcomes to monitor postmarket
- Interest to payers, providers, patients

**Patient Preference Information (PPI)**
- Inform endpoints or effect size for regulatory studies
- Inform subgroup considerations
- Labeling changes / expanded indications
Significant Increase in Device Studies with PROs
Submitted to CDRH as of FY2015

- >500% increase in device studies w/ PROs
- 50% of PMAs contain PROs (FY15 cohort)
- 600+ premarket submissions contain PROs (CY2000-2014)

Use of PROM in Device Submissions*

*Based on search for PROs in CDRH’s historical submission archives

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Obesity PPI Case Study

- Broad array of potential devices with diverse benefit-risk profiles
- Treatments involve difficult benefit-risks tradeoffs
- Explore how to elicit and incorporate patient preferences into regulatory decision making
- “Minimum acceptable benefit” and “maximum acceptable risk” for various levels of risk tolerance, which inform the “minimum clinically meaningful benefit” used by CDRH to size, design and evaluate clinical trials for weight-loss devices
- Used in approval of Maestro system

Organization Adoption of Patient Science: 2016 Accomplishments

Staff Training:
- PPI Guidance
- PRO and PPI science

Research:
- Collaborative PPI research in obesity, neurology, oncology, pediatrics, women’s health
- PRO research in traumatic brain injury, urology, and women’s health
- Novel complementary PPI/PRO research in ophthalmics and prosthetics
Shared Goal
Improve patient health by better understanding patient needs, experiences and preferences

Art of Patient Engagement + Science of Patient Input = Patient-Centric Health Care
Summary

• Patients are at the heart of what we do. Patients have unique perspectives about the value and impact of medical device benefits and risks.

• FDA’s sharpened focus on patient-centered technology development, evaluation, and use has already begun to positively affect the development and availability of innovative therapies and clinical solutions.

• We invite the patient, industry, healthcare professional and academic communities to join in to advance this important work.
Thank You

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Resources

• CDRH Patient Preference Obesity Study

• Guidance for Industry and Food and Drug Administration Staff: Factors to Consider for Benefit–Risk Determinations in Medical Device Premarket Approval and De Novo Classifications

• Guidance on Medical Device Patient Labeling

• MDIC Patient-Centered Benefit Risk Project: A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology (http://mdic.org/pcbr)