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CDRH Patient Preference Initiative & Beyond
Patients are at the Heart of What 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.
Assure that patients and providers have timely and continued access to safe, effective, and high-quality medical devices and safe radiation emitting products.....

Facilitate medical device innovation by advancing regulatory science, providing industry with predictable, consistent, transparent, and efficient regulatory pathways, and assuring consumer confidence in devices marketed in the U.S.

Provide consumers, patients, their caregivers, and providers with understandable and accessible science-based information about the products we oversee.....
Evolution of the Role of the Patient

- **Old School:** Paternalistic provider-patient relationships
- **Emerging Diseases:** Patient advocacy for availability of and access to new treatments
- **The Internet:** Patient empowerment through information
- **The Future Today:**
  - Patient preferences informing development and assessment of medical products
  - Participant engagement is a focus for the President’s Precision Medicine Initiative
  - Patient Data Donation movement is gaining momentum
Patients are:
- using devices themselves especially in a home healthcare settings
- more involved in shared decision-making and disease management with their healthcare professionals
- communicating and connecting with each other through social media and other forums, sharing symptoms, side effects, advice, and providing support

Patient groups are:
- developing eligible patient and longitudinal disease registries to facilitate clinical trials
- training patient advisors to improve clinical study design and conduct to be more patient-friendly and efficient
- harnessing the power of social media and mobile tech
- developing research-ready networks like PCORnet PPRNs that can be a rich source of direct-from patient “real world” data
- and more
Patient Preference Initiative

- The goal of this initiative is to develop a systematic way of eliciting, measuring, and incorporating patient preference information where appropriate throughout the Total Product Life Cycle, to drive more patient-centric device development, evaluation and delivery

- CDRH seeks to:
  1. Understand the barriers patients have faced in trying to participate in the regulatory process
  2. Incorporate patient perspectives to inform benefit-risk decisions, e.g., as valid scientific evidence in regulatory contexts
  3. Advance the state of the science of measuring patient preferences
Where can patient perspectives inform the medical device TPLC?

- Patient-Informed Needs
- Patient-Informed Clinical Trial Design
- Patient Preference Benefit-Risk Information
- Communicating Benefit-Risk Information to Patients
- Patient-Centered Outcomes
Collaborative Building Blocks

- CDRH/CBER Draft Guidance: PPI in Benefit-Risk
- MDIC Methodology Catalog
- MDIC Framework: PPI in TPLC
- CDRH-RTI Obesity Case Study
- Device Patient Preference Initiative
- Patient Reported Outcomes
Which is a Favorable Benefit-Risk Tradeoff?

Risks ↓ Benefit↓

New Device

Weight Loss

Gastroplasty

Gastric Banding

Diet:
Exercise
Study on Devices to Treat Obesity

- Explore how to elicit and incorporate patient preferences into regulatory decision making
- Treatments involve difficult benefit-risks tradeoffs
- Broad array of potential devices with diverse benefit-risk profiles
- Used Discrete-choice experiment survey
- Published in Surgical Endoscopy *
- CDRH Impact:
  - Maestro System, a vagus nerve stimulator indicated as a weight-loss treatment was approved on January 14, 2015

Patient Preference Information – Submission, Review in PMAs, HDE Applications, and De Novo Requests, and Inclusion in Device Labeling (May 2015)

1. Encourage voluntary submission of patient preference information
2. Recommended qualities of patient preference studies which may be valid scientific evidence
3. Recommendations for collecting patient preference information
4. Recommendations for including patient preference information in labeling for patients and health care professionals

The draft guidance is applicable to sponsors and other stakeholders including patient groups.

**Voluntary Submission**

- PPI may not be relevant or appropriate for all device types

- Could be useful for those product types and diseases or conditions where usage decisions by patients and health care professionals are “preference-sensitive”

**Examples of preference-sensitive decisions:**

- Devices with a direct patient interface
- Devices intended to yield significant health and appearance benefits
- Devices intended to directly affect quality of life
- Certain life-saving but high-risk devices
- Devices developed to fill an unmet medical need or treat a rare disease or condition
- Devices with novel technology
- Framework for Patient Centered Benefit-Risk Assessment
- Catalog of Patient Preference Assessment Methods
- Agenda for Future Research in Patient Preferences
Submissions with PROMs have increased from ~20/yr prior to 2009 to >120 in 2014.

Patient preference draft guidance expected to lead to increased usage of patient preference information.
CDRH is committed to integrating the patient voice into our regulatory decision-making.

CDRH encourages patient groups, industry, and others to interact with us early to discuss how patient preference information can be used for regulatory purposes.

Other ways to incorporate patient input:
- Improve communication of benefit-risk information to patients
- Incorporate patient input to inform clinical trial design
- Encourage PRO development and use (e.g., MDDT qualification)
Laying the Groundwork
Patients are at the heart of everything we do.

Patients have unique perspectives about the value of benefits and impact of risks of medical devices.

CDRH is committed to integrating the patient voice into our regulatory decision-making.

We have made significant progress in laying the groundwork for this burgeoning field.

Patients, researchers, industry, FDA and others have a role to play in driving more patient-centric device development, evaluation and delivery.