Clinical Trials & Medical Devices

Patient Engagement Advisory Committee

Owen Faris, Ph.D.
Director, Clinical Trials Program
FDA/CDRH
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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.
Devices Are Different from Drugs

- Many devices are highly dependent on clinician knowledge, experience, and skill
- Devices and techniques iteratively and rapidly improve (sometimes even during a trial)
- Gold-standard RCT often not practical
What are the opportunities?

Flexibility
- “Can’t always get what you want….”
- But if we are flexible, we can “get what we need”

Innovation
- Modeling
- Adaptive designs
- Real-world evidence

Collaborations
- NEST
- Industry groups
- Patient and clinician groups
Clinical Trial Innovation


Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders


Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices

Guidance for Industry and Food and Drug Administration Staff


Evaluation and Reporting of Age-, Race-, and Ethnicity-Specific Data in Medical Device Clinical Studies

Guidance for Industry and Food and Drug Administration Staff

Document issued on September 12, 2017.

Adaptive Designs for Medical Device Clinical Studies

Guidance for Industry and Food and Drug Administration Staff

Evidence in Regulatory Decisions

Traditional Regulatory Pathway

Pre-Clinical Testing → Clinical Studies → Pre-Market Application → Post-Market

Hypothesis Generation

Device Innovation

Informed Clinical Decision Making

Non-Traditional Clinical Data Generation

Real-World Device Use

Physician and Patient Experience

Healthcare Information

Claims Databases

Pharmacy Data

Social Media

Electronic Health Records

Laboratory Tests

Patient Experience

Registries

Hospital Visits

Claims

Databases

Pharmacy

Data

Social

Media

Electronic

Health

Records

Hospital

Visits

Patient

Experience

Registries

Non-Traditional Clinical Data Generation

Evidence in Regulatory Decisions

Pre-Clinical

Clinical

Studies

Pre-Market

Application

Post-Market
Regulations

- 21 CFR 812  Investigational Device Exemptions
- 21 CFR 50  Protection of Human Subjects (Informed Consent)
- 21 CFR 54  Financial Disclosure of Investigators
- 21 CFR 56  Institutional Review Boards (IRBs)
- 45 CFR 46  “Common Rule”
Regulatory changes

The Common Rule, Updated
Jerry Menikoff, M.D., J.D., Julie Kaneshiro, M.A., and Ivor Pritchard, Ph.D.

IRB Waiver or Alteration of Informed Consent for Clinical Investigations Involving No More Than Minimal Risk to Human Subjects

Guidance for Sponsors, Investigators, and Institutional Review Boards

Central IRBs allowed for device Trials
Primary Groups in Clinical Trials

- FDA
- Sponsor
- Investigators
- IRB
- Patient Subject
Who are we in CDRH?

Engineers
- Biomedical
- Mechanical
- Electrical

Clinicians
- Cardiology
- Orthopedics
- Neurology
- Gastro-Renal –OB
- Ophthalmology
- Surgery
- Pulmonology
- Dental
- Anesthesiology

Scientists and Specialists
- Chemistry
- Toxicology
- Software
- Microbiology
- Animal Studies

Statisticians
- Epidemiologists
- Informaticists

• Regulatory
• Compliance
• Program support
“Are the right patients going to be enrolled in this study?”

“Will patients be able and willing to adhere to the follow-up visit schedule?”

“Are there ways the trial could be modified to be less burdensome for patients?”

“Will the trial measure outcomes that matter to patients?”

“Do patients understand the potential risks and benefits?”

“Does study success equal patient success?”

Clinical Trial Design
“Some patients experienced a certain adverse event at a higher than expected rate. Should we still approve?”

“Are the benefits highly meaningful for patients?”

“How do patients view this risk in the context of their disease and treatment alternatives?”

“Are there mitigations that can be put in place to address this risk?”

“Can post-market tools help fill the gap?”

**Marketing Application**
Informed Decisions → Patient Perspectives!

www.fda.gov
Patient Perspective Information: Fit for purpose

Patient-preference information
Focus groups
Carepartner engagement
Patient-reported outcomes
Medical professional engagement
High-quality surveys
Patient organization engagement
Patient Perspective Information: The earlier the better!
Thank you!

Owen Faris, Ph.D.
owen.faris@fda.hhs.gov