



# Minimum Clinically Important Difference Workshop

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## FDA Perspective Pre-Market and Benefit-Risk

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Mark Melkerson, Director

Division of Orthopaedic Devices, FDA



# Pre-Market

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- **CDRH Strategic Priority #2**

“To improve public health and foster trust among our employees and with our constituencies, CDRH will provide meaningful and timely information about the products we regulate and the decisions we make, through strategic outreach and systems that support transparency and two-way communication”

<http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHVisionandMission/UCM288736.pdf>

**Strategy 2.1 – Enhance Communication and Transparency with Our Stakeholders**



# Pre-Market

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- **Clinical Trial Study Design**

Draft Guidance for Industry, Clinical Investigators, and Food and Drug Administration Staff: Design Considerations for Pivotal Clinical Investigations for Medical Devices

<http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM267831.pdf>

- Effect Size → Sample Size → Study Power
- Patient Success Criteria → Study Success Criteria → **Composite Endpoints of Success**
- What is clinically meaningful benefit? → **Benefit/Risk**



# Pre-Market

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- “A **subject-reported outcome instrument** can be used when the outcome of interest and desired intended use are best measured from the subject’s perspective (e.g., pain reduction). In such cases, it is important to select a scoring assessment that is **validated** for the subject population and condition being treated, and consistent with the desired **intended use.**”
- Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims.  
<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM193282.pdf>.



# Patient Reported Outcome (PRO) Research

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- Patient-Reported Outcome (PRO) Consortium
  - Critical Path Institute (C-Path) in cooperation with FDA 1<sup>st</sup> Annual Meeting March 23, 2010
  - <http://c-path.org/PRO.cfm>
- NIH Patient Reported Outcomes Measurement Information System (PROMIS)
  - Advancing PRO Science in Clinical Research and Patient Care
  - <http://www.nihpromis.org>



# Patient Data Collection

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- **ICOR** - International Consortium of Orthopaedic Registries
- **Force-TJR** - Function and Outcomes Research for Comparative Effectiveness in Total Joint Replacements
- **Kaiser Permanente Implant Registries**



# Benefit-Risk

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- Guidance for Industry and Food and Drug Administration Staff - **Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications**
  - <http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM296379.pdf>
  - Premarket Approval (**PMA**)
  - **De Novo** Classifications

# Benefit-Risk

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- “This guidance clarifies this process for industry, which will provide manufacturers with greater **predictability, consistency and transparency** in FDA decision-making while allowing manufacturers and the FDA to use a **common framework** for benefit-risk determinations,”

*Jeffrey Shuren, M.D., J.D.  
CDRH Director*





# Benefit-Risk

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- Assessing Benefit
  - The **Type** of benefit(s)
  - The **Magnitude** of the benefit(s)
  - The **Probability** a patient will experience the benefit(s)
  - The **Duration** of the benefit(s)



Thank you