U.S. FOOD & DRUG ADMINISTRATION

Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda

Session 2:
Patient Perspectives & Preferences on Benefit-Risk

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NASEM Workshop
May 9, 2018

How Patient Preferences Contribute to Regulatory Decisions for Medical Devices

Posted on **September 25, 2017** by **FDA Voice**

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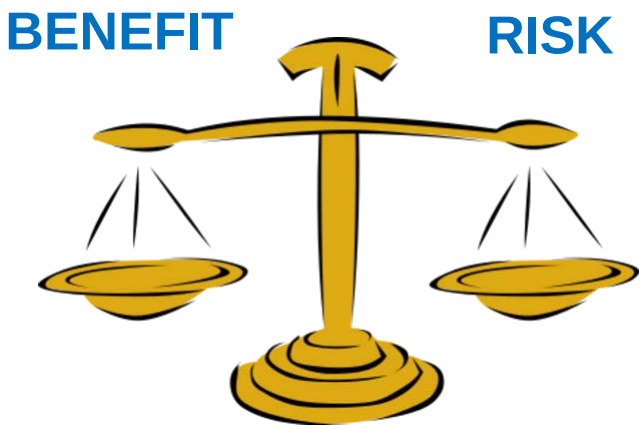


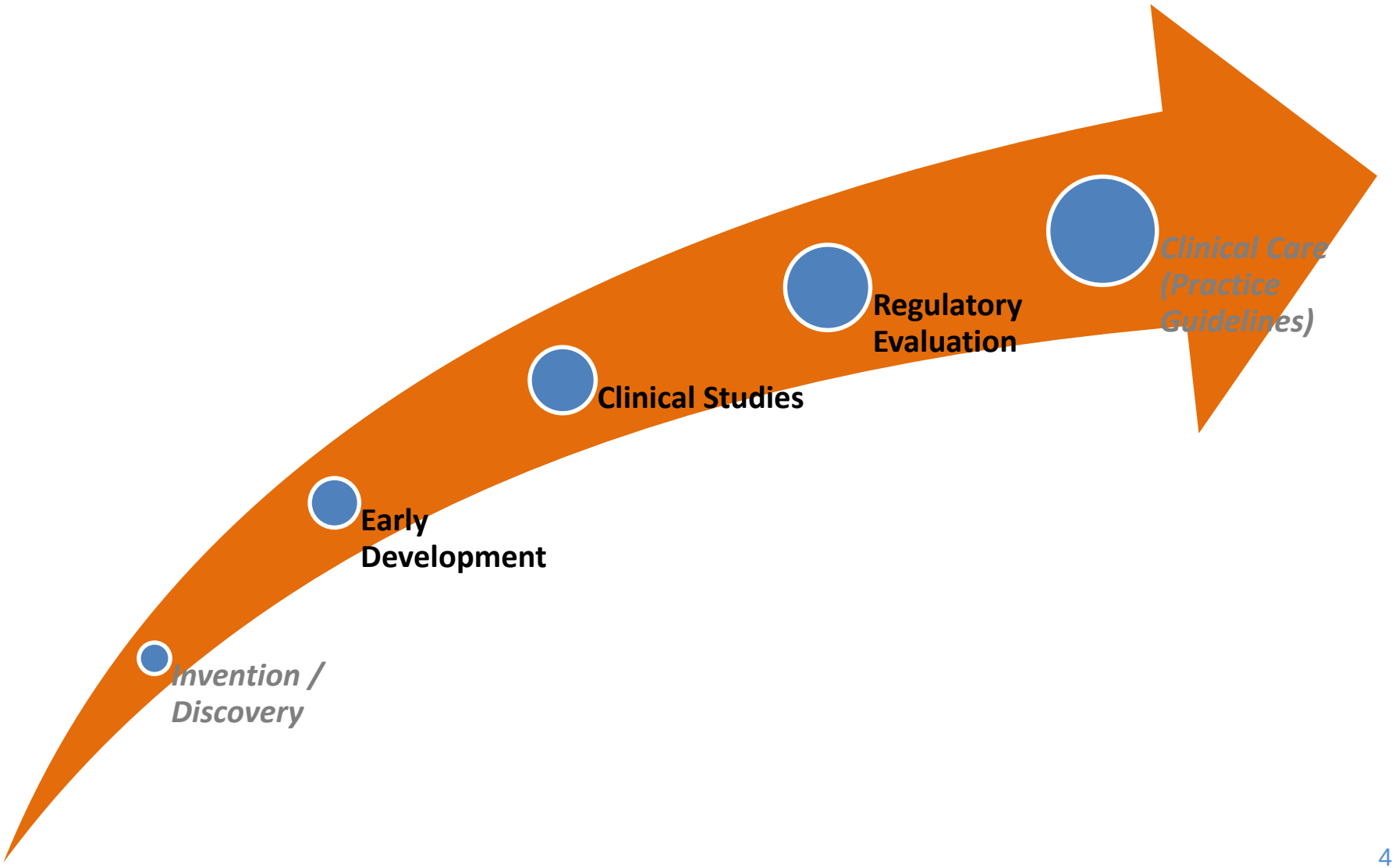
- **Weight loss**
  - Patient-informed trial design
  - PMA approval
- **At home dialysis**
  - Patient risk tolerance
  - Expanded indication for solo at home use
- **Diabetes care**
  - Risk management for pediatric population
- **Ongoing studies**
  - Neurology
  - Oncology
  - Ophthalmics
  - Prosthetics
  - Women's health
  - Urology
  - Pediatrics

Begin with the End in Mind: How will this information be used?

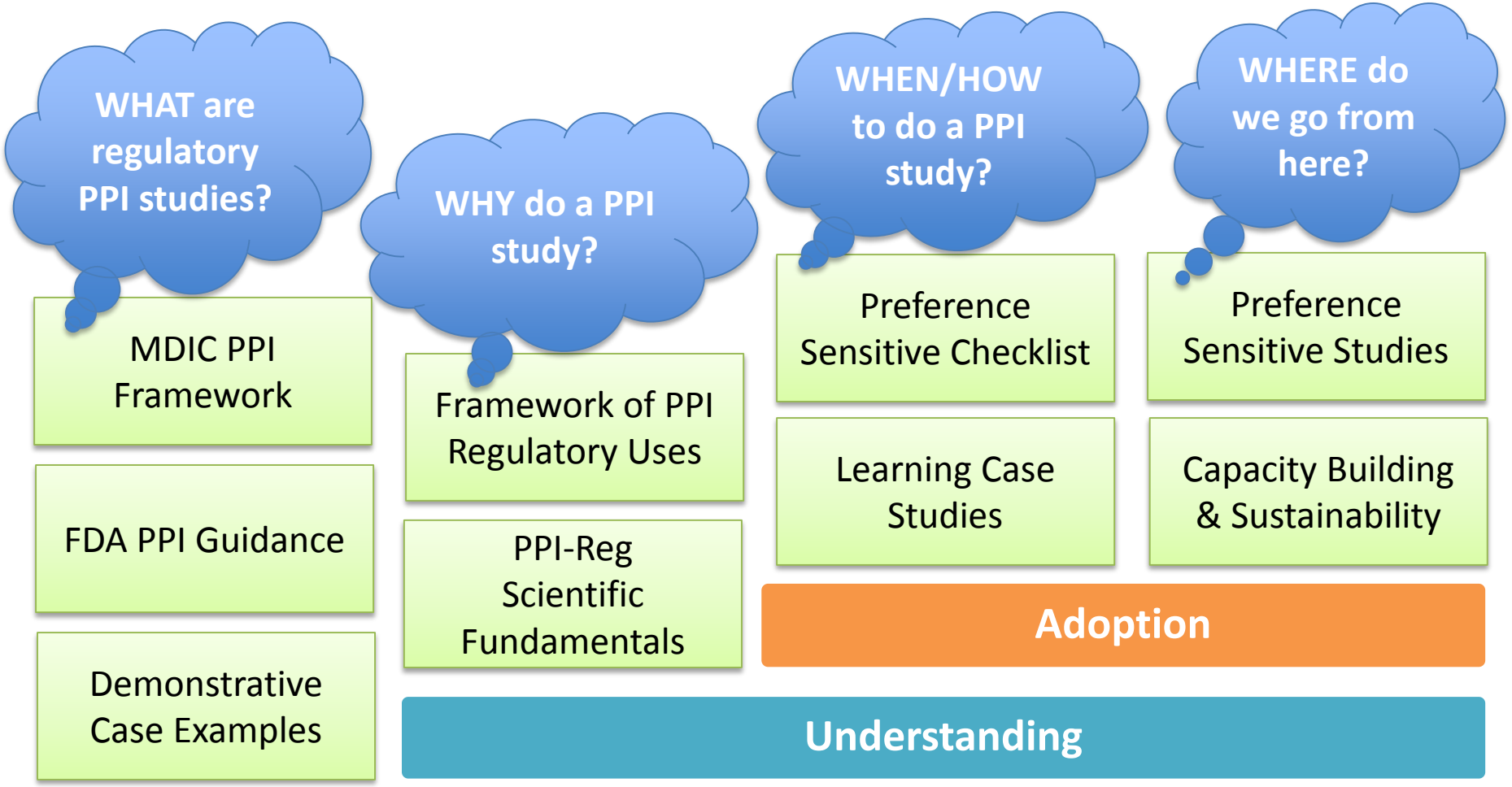
Framework for Potential Uses of PPI in Medical Product Development

Development	Clinical Trial Design	Pre-Market Benefit-Risk Assessment	Post-Market
1. Identify unmet medical need 2. Understand what matters most to patients about their disease or treatment	1. Inform endpoint selection 2. Inform performance goal or effect size	1. Analysis of condition 2. Current treatment options 3. Patient perspective on benefit-risk tradeoffs 4. Population subgroup considerations	1. Inform interpretation of new data affecting benefit-risk assessment 2. Inform studies of new / expanded use populations 3. Communicate benefit-risk information to patients

Strength of Evidence Needed Depends on Context of Use



Dec. 2017 CERSI-FDA Workshop: Advancing Use of PPI as Scientific Evidence for Medical Product Evaluation

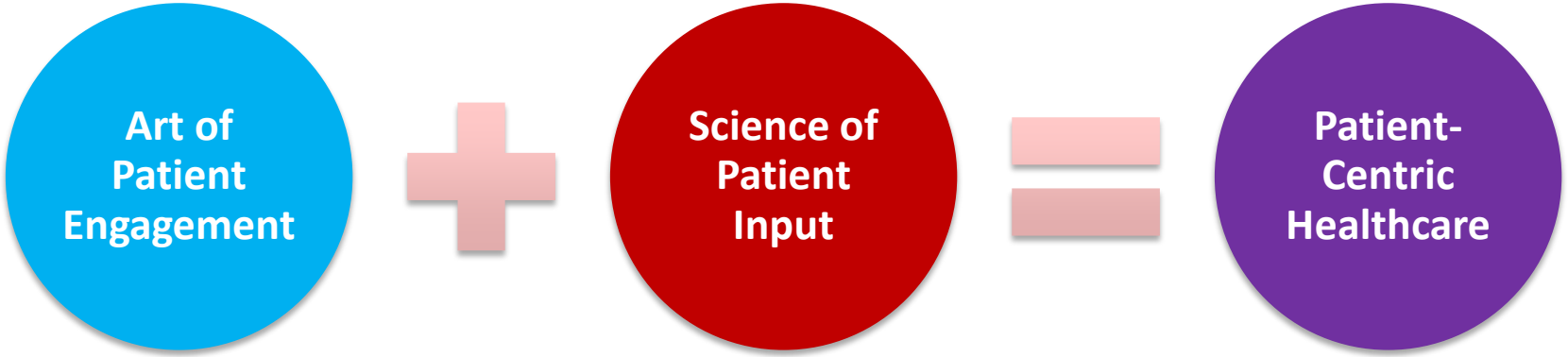


Recommended Early Focus: Barriers & Potential Strategies

- Limited system capacity to perform rigorous, high quality PPI studies
- Goal is to channel these resources where most valuable
- Where is the sweet spot?
  - "Preference-sensitive" patient decision re: diagnostic / treatment options (no clear single best option for all)
  - Relevance. Patient preferences relate to measures of effectiveness, safety, other attributes relevant to product developers and regulators, AND
  - We understand enough about a) patient perspectives on disease and existing options, and b) characteristics of the proposed new option

Shared Goal

Improve patient health by better understanding patient needs, experiences and preferences



Patient Reported Outcomes (PRO)

- Can be Endpoints in Regulatory studies
- Outcomes to monitor in clinical care
- Interest to patients, providers, payers

Patient Preference Information (PPI)

- Inform endpoints or effect size for Regulatory studies
- Inform subgroup considerations
- Inform studies of new / expanded uses

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ADDITIONAL MATERIAL

Patient Input as Evidence*

- **Final Guidance***
- Hiring & training staff
- Access to external SMEs
- Expanding collaborative networks

- PPI in IDE Benefit-Risk
- PROs & Outcomes that Matter Most to Patients

- *PPI & PRO in Marketing Application Benefit-Risk

- PPI in Compliance Benefit-Risk
- PPI & PRO for new uses

Factors to Consider in Medical Device Benefit – Risk Determinations

- Worksheet with questions to guide evaluation of each factor
- Patient Perspectives are an as important factor:

PPI Factors	Questions
Patient-Reported Outcomes	• Do benefit(s) and risk(s) include effects on patients' health-related quality of life?
Benefit-Risk Considerations	• Which benefits and risks are most important to affected patients? • What benefit-risk tradeoffs are acceptable from the patient perspective? • Are there clinically-relevant subgroups of patients that would choose a particular benefit-risk profile over other alternatives? • Does PPI capture diverse preference across the spectrum of indicated population and thus, generalizable?

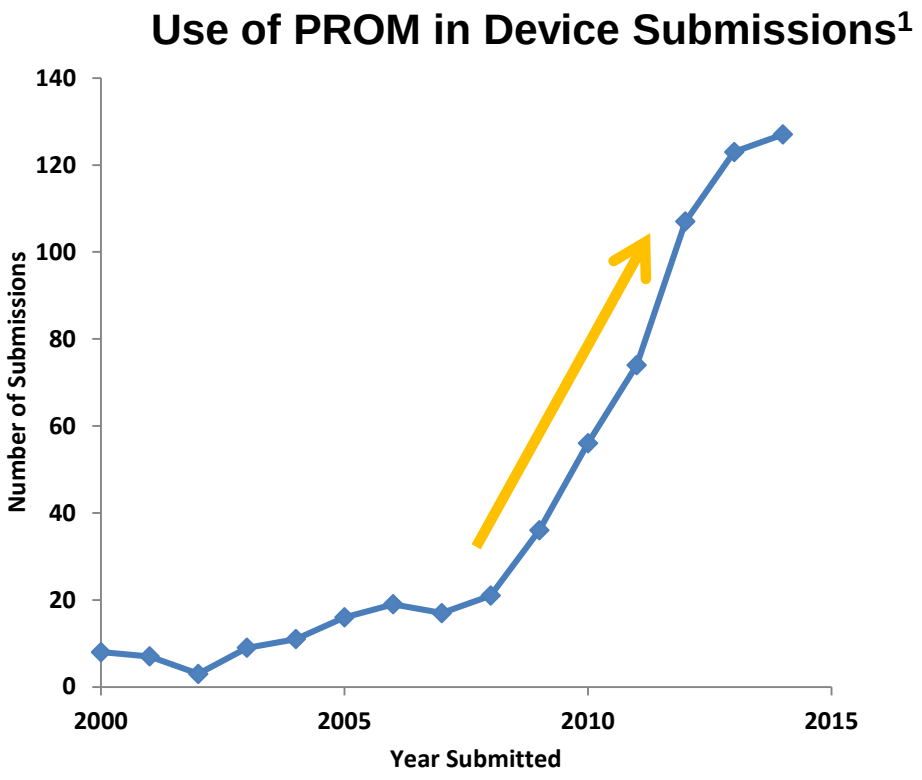
Patient Preference Information (PPI)

- Qualitative PPI may be useful
  - identifying which outcomes, endpoints or other attributes are valued most by patients
  - which factors affect patients' perspectives on risk and benefit
- Quantitative PPI
  - provide estimates of how much different outcomes, endpoints or other attributes are valued by patients
  - tradeoffs that patients state or demonstrate they are willing to make

Significant Increase in Patient Perspective Studies

- **>500% increase** submissions with PROs
  (2009-2015)

- **>75% of clinical protocols** include PROs
  (FY17 pivotal study approvals)



[1]*Submitted to CDRH as of FY2015*

"The FDA's work requires us to establish objective, consistent criteria on which we base our decisions. But ultimately, the criteria we use to judge benefit and risk turn on the parameters that matter to patients.

"Involving the end-user – the patient – in identifying health priorities and outcomes desired from health interventions is critically important.

"The bottom line is this: When assessing whether valid scientific evidence shows that a device's probable benefit outweighs its likely risks, the FDA can also consider rigorous, systematically gathered patient preference information as a part of the totality of the evidence from clinical and nonclinical testing. "

- ***FDA Commissioner Scott Gottlieb***, *Oct 11, 2017*

Thank You

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