SESSION 2

Part 1: Scientific Fundamentals of PPI Studies

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FDA-CERSI Patient Preference Workshop
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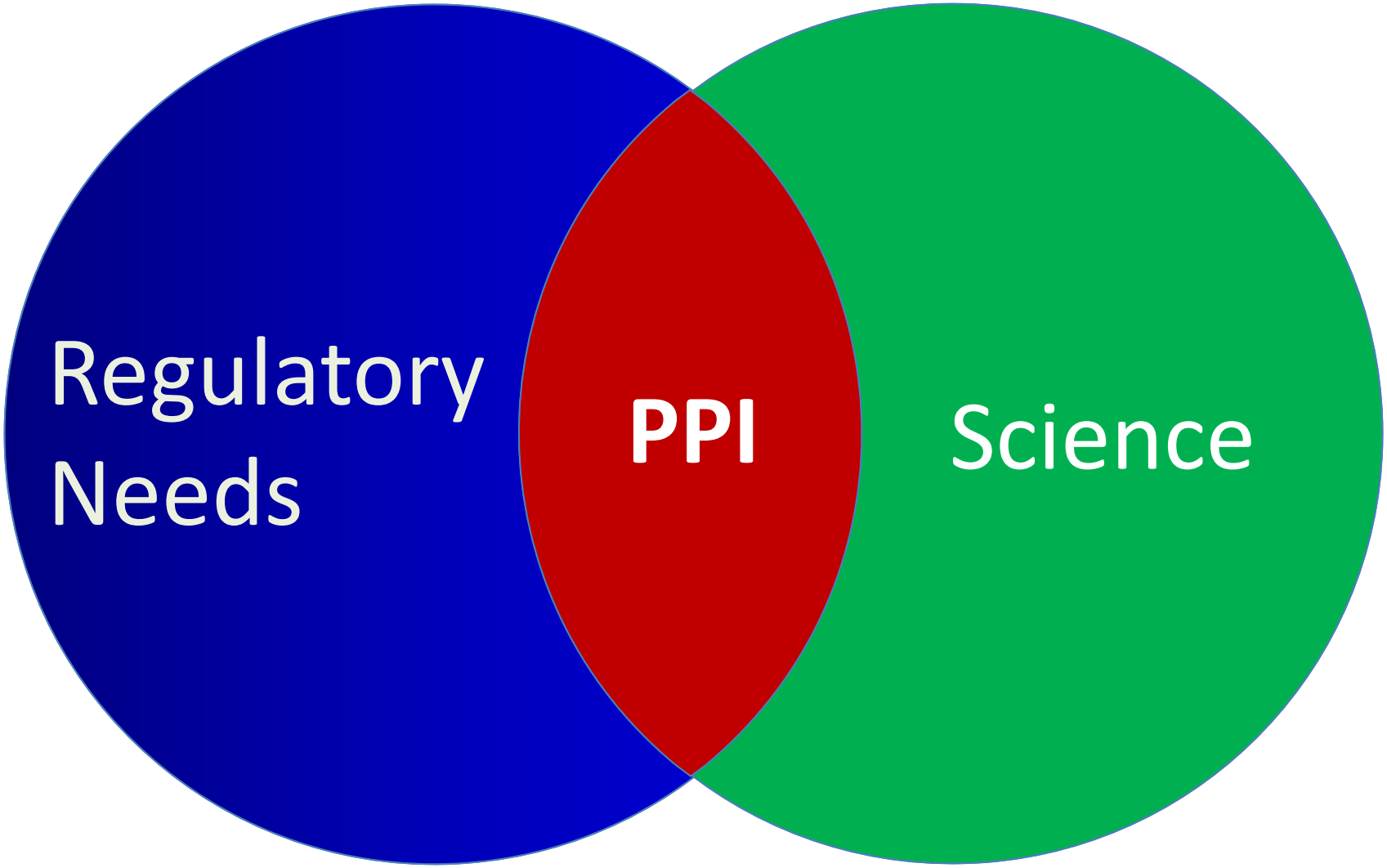
Objectives

1. Science: Introduction to science of PPI
2. Scope: Where science and regulatory-needs meet
3. Context: Explain why study quality matters
4. Approach: How CERSI and FDA can collaborate to advance science of PPI

PPI: Where Regulatory Needs Meet Science



Regulatory Considerations of PPI Studies

Context specific to individual regulatory decisions

1. Chosen method informs benefit-risk assessment.
2. Patients can understand & answer the questions.
3. Descriptions of treatment options (benefits & risks etc.) are accurate with minimum cognitive bias.
4. Study sample is sufficiently large and diverse that reflects the population of interest.

Some Useful Resources

- Your FDA review divisions (come early and often!)
- CBER-CDRH Patient Preference Information Guidance (https://goo.gl/N1BDTw)
- Patient Reported Outcome Guidance's Qualitative Section (https://goo.gl/UpJ6EV)
- Science of Patient Input at MDIC (http://mdic.org/spi/)
  - Framework and Catalog (https://goo.gl/SqrZLR)
  - NEW **Qualitative Steps** (https://goo.gl/owAABB)
- PFDD Patient Experience Data Workshop, Dec. 18 (https://goo.gl/ZMX6Lr)