Patient Preferences Studies

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Patients are at the Heart of What We Do

Center for Devices and Radiological Health Vision:
Patients in the U.S. have access to high-quality, safe, and effective medical devices of public health importance first in the world

Patient Preference

- Patient preferences are defined as qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions
- Relevant preferences of care-partners (e.g., parents) and health care professionals may also be considered

Guidance: Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling

What PPI Can Provide and How It Can Be Used

- PPI data can provide valuable information about:
  - Which benefits and risks are most important to affected patients
  - What benefit-risk tradeoffs are acceptable from the patient perspective
  - How do these patients think about these tradeoffs
  - Are there clinically-relevant subgroups of patients that would accept a particular benefit-risk profile and/or choose one treatment option over other alternatives

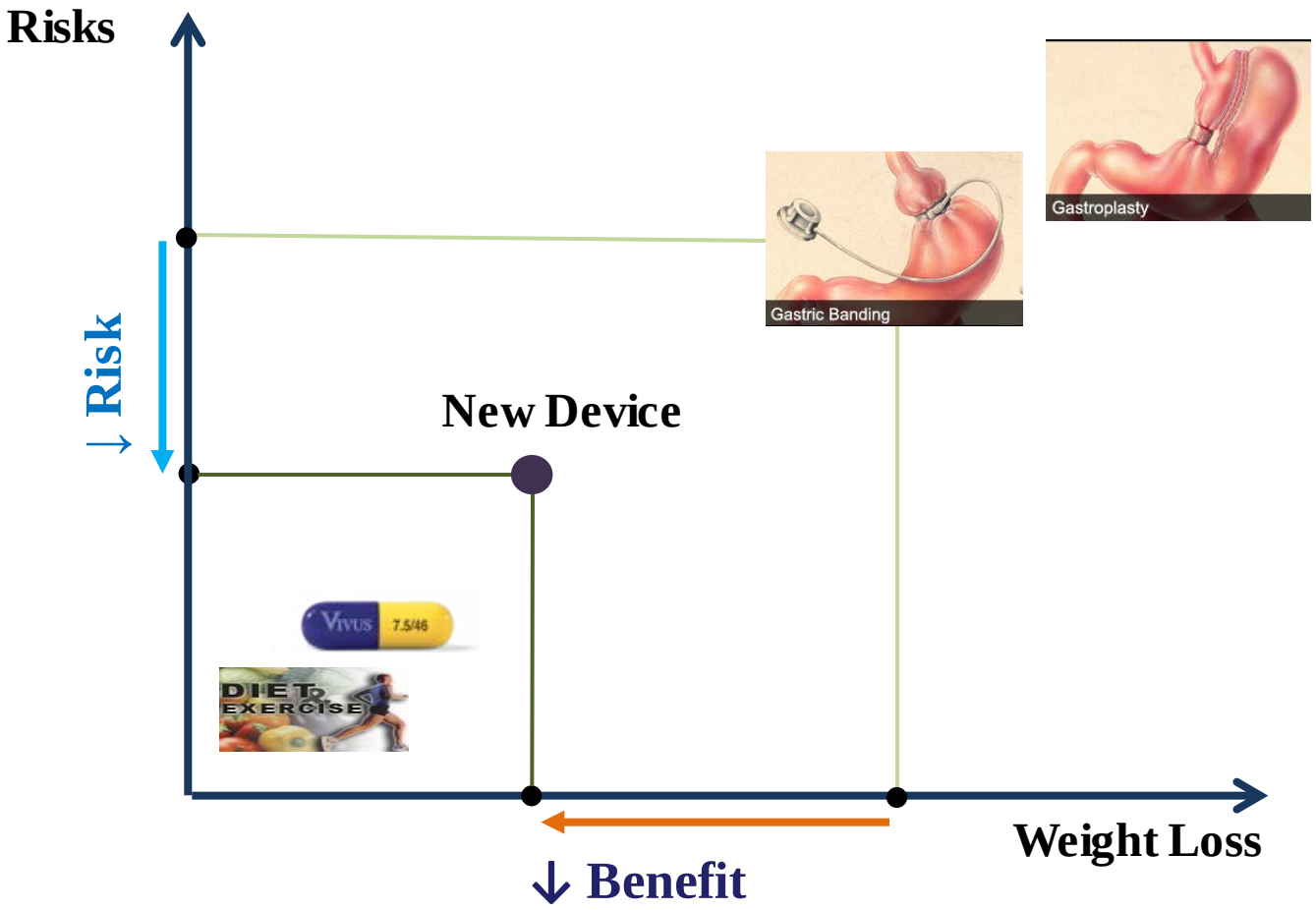
- Potential Uses of PPI:
  - Inform endpoints or effect size for regulatory studies
  - Inform subgroup considerations
  - Labeling changes / expanded indications

- Other potential uses outside regulatory context, such as shared medical decision-making.

Recommended Qualities of Patient Preference Studies

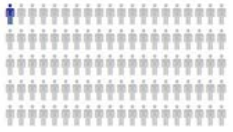
Study sample	Study design	Study conduct and analysis
Well-informed patients	Questions are meaningful and relevant to patients	Well-documented instrument development process and study conduct, including the initial qualitative work
Representative sample for generalizable results	Minimize cognitive bias	Logical soundness (e.g., internal consistency)
Capturing heterogeneity	Effective benefit-risk communication	Robustness of study results (e.g., sensitivity analysis)
	Demonstrated comprehension by patients	

Example: Obesity Devices



Ho MP, Gonzalez JM, Lerner HP, Neuland CY, Whang JM, McMurry-Heath M, Hauber B, Irony T. Incorporating patient-preference evidence into regulatory decision making. *Surgical Endoscopy*; 2015.

Discrete Choice Question

Attribute	Device A	Device B
Type of operation	Endoscopic Surgery	
Recommended diet restriction	Wait 4 hours between meals	
On average, how much weight is lost	30 lbs.	60 lbs.
On average, how long weight loss lasts	5 years	1 year
Average reduction in dose of prescription drugs for diabetes at the lower weight	Eliminates the need for prescription drug	
On average, how long side effects last *(Remember that side effects will limit your ability to do daily activities several times a month)*	1 month	1 year
Chance of a side effect requiring hospitalization	None	
Chance of dying from getting the weight loss device	 10% (10 out of 100)	 1% (1 out of 100)
Which weight loss device do you think is better for people like you?	☐ Device A	☐ Device B

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Example Questions

High intensity focused ultrasound (HIFU) for ablation of prostate tissue in men with localized prostate cancer

- Prostate cancer is the most common malignancy and the third leading cause of cancer-related deaths in men in the United States and Europe.

- FDA recently allowed to market two HIFU tools for prostate tissue ablation after rejecting prior PMA indicated to treat prostate cancer, as they did not demonstrate cancer-specific effectiveness.

- *How to make decisions in the absence of relevant clinical effectiveness data, but in light of known potential adverse events and 12 month post-treatment prostate biopsy data*

Charles Viviano, MD, PhD, Joyce Ting-Hsuan Lee, MHS, Olufemi Babalola, MHS

What amount of adverse event risk are patients willing to tolerate for increased effectiveness for this treatment?

Rationale / Public Health Need

- Patient perspective on available benefit data or tolerance of risks associated with HIFU may inform future premarket device evaluation of ablation tools.
- Delivery of better ablation devices sooner to patients.

CDRH Strategic Priority of Partnering with Patients:

"Increase use and transparency of patient input as evidence in our decision making."

Submission of PPI to FDA

FDA encourages sponsors and other stakeholders to have early interactions with the relevant FDA review division if considering collecting and submitting PPI.

- Request an informational pre-submission meeting to discuss plans for designing or submitting a patient preference study
- Request participation from Martin.Ho@fda.hhs.gov and Anindita.Saha@fda.hhs.gov

Thank You

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