CDRH Preference Sensitive Areas Discussion: Diseases and Conditions where patient preference studies could be useful
MDIC is a 501(c)(3) non-profit organization and is the first-ever public-private partnership created with the sole objective of advancing regulatory science of medical devices for patient benefit.

MDIC HIGHLIGHTS

- 65 participating member organizations
- Leading resource on issues important to the Medtech innovation ecosystem
- 6 Projects have been initiated
- Congressional testimony on modernizing clinical trials
- Over $35m funding from grants and contracts for Program initiatives
MDIC Patient Preference Framework

Framework for Incorporating Patient Centered Benefit Risk Assessment into Regulatory Submissions

- Resource for CDRH, MDIC members, and industry on when and how to collect patient preference information for incorporation into the regulatory process
- Incorporates Catalog of Methods as appendix

Published in 2015, the Framework is the collaborative effort of industry, CDRH and patient preference experts

- An initial thought piece in an emerging area
- To be updated as industry, FDA, and patient groups gain experience with collecting/using patient preference information

The Value of Patient Preference Information

- Identify benefits and harms most important to patients
- Frame the benefit-risk issues and tradeoffs from the patient perspective
- Identify whether there are subgroups of patients that would choose to use the technology over other alternatives
- Support quantitative benefit-risk modeling

The value of patient preference information as a function of benefit and risk

<table>
<thead>
<tr>
<th>Benefit/Low Risk</th>
<th>High Benefit/High Risk</th>
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<tbody>
<tr>
<td>Patient preference info less needed if clear benefit and little risk</td>
<td>Patient preference info valuable to show a subset of patients willing to take the high risk for the significant benefit</td>
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<table>
<thead>
<tr>
<th>Low Benefit/Low Risk</th>
<th>Low Benefit/High Risk</th>
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<tbody>
<tr>
<td>Patient preference info might be helpful to show that a subset of patients want the limited benefit</td>
<td>Product may only get approved if significant evidence that at least a subset of patients would take the risk for the benefit</td>
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Identifying preference sensitive areas

- Patients as stakeholders
  - Examples: Patient preference or risk tolerance different from other stakeholders; heterogeneity in patient preference

- Benefit-risk tradeoffs
  - Examples: Marginal benefit-risk scenarios

- Regulatory novelty
  - Example: Familiarity with the use of a particular technology

Areas that may be less preference sensitive

- When the patient is not a major stakeholder or decision-maker
- When the disease state/technology are generally understood by sponsors and FDA staff and there is significant regulatory precedent for approval
- When the treatment is clearly superior to existing therapies with no tradeoffs in risk
- When the treatment meets an unmet medical need with poor outcomes such that the risk of treatment will not be greater than the risks of the untreated disease

Considerations for Patient Preference

• There is not a “cookbook” or algorithmic approach to identifying preference sensitive areas.

• There are factors that characterize situations where patient preference information could be useful, in particular, in the context of regulatory benefit-risk assessments.

• Let’s not limit ourselves to the regulatory considerations. Patient preferences can provide valuable insight across the medical device development lifecycle.
Patient Preference Information Resources

For more information, visit: http://mdic.org/SPI/resources

Upcoming and archive webinars: http://mdic.org/mdicx