State of the Science: Methods to Collect and Use Patient Preference Data

Overview of methods to collect patient preference information and active research regarding patient collecting patient preferences.

For the online webcast: Please submit your questions to the panel via the chat box. The online hosts will be collecting the questions during the session to be brought to the panel moderator during the panel discussion.
Moderator:  
Telba Irony, Ph.D.  
Chief, General Surgical Devices Branch  
CDRH/Office of Surveillance and Biometrics

Panel:  
A. Brett Hauber, Ph.D.  
Research Triangle Institute (RTI-Health Solutions)

Bennett Levitan, M.D., Ph.D.  
Janssen Research & Development (Johnson and Johnson)

Sonal Singh, M.D., M.P.H.  
Johns Hopkins University

Scott Braithwaite, M.D.  
Society of Medical Decision Making (SMDM)
Methods for Eliciting Benefit-Risk Preference Data

Patient Preference Initiative Symposium

A. Brett Hauber, Ph.D.
Senior Economics and Vice President
Health Preference Assessment

September 18-19, 2013
Benefit-Risk Tradeoff Metrics

- **Net Safety Benefit**
- **Maximum Acceptable Risk**
- **Δ Risk**
- **Δ Effectiveness**
- **Patient Benefit-Risk Threshold**
- **TREATMENT A**
- **Minimum Acceptable Benefit**
- **Net Effectiveness Benefit**

Source: Hauber et al., Appl Health Econ Health Policy (2013)
Benefit-Risk Preference Elicitation Methods

- Weighting methods for single decisions
  - Standard Gamble
  - Threshold Technique
- Generalized weighting methods
  - Best-Worst Scaling
  - Discrete-Choice Experiments (DCE)
- Decision support methods
  - Analytic Hierarchy Process (AHP)
  - Multi-Criteria Decision Analysis (MCDA)
Benefit-Risk Preference Elicitation Methods

• **Weighting methods for single decisions**
  – Standard Gamble
  – Threshold Technique
Standard Gamble

\[ U(\text{Asthma}) = p \cdot U(\text{Perfect Health}) + (1-p) \cdot U(\text{Death}) \]

\[ U(\text{Asthma}) = p \cdot (1) + (1-p) \cdot (0) \]

\[ \text{MAR (Death)} = 1-p \]

Source: Bernie O'Brian, personal communication
## Threshold Technique

<table>
<thead>
<tr>
<th>Treatment:</th>
<th>OPTION A (Reference)</th>
<th>OPTION B (Target)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Pain:</strong> Pain</td>
<td>5</td>
<td>3</td>
</tr>
<tr>
<td>experienced while walking after taking pills daily, on a 0-10 scale is…</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Out of Pocket Cost:</strong></td>
<td>$0</td>
<td>$0</td>
</tr>
<tr>
<td><strong>Risks and Side Effects:</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Stomach Bleed</td>
<td>2 %</td>
<td>2 %</td>
</tr>
<tr>
<td>Feeling unwell, vomiting blood. Treatment involves hospitalization, sedation for tests, a tube inserted down the throat, and blood transfusion. Hospital stay will be for 2-7 days. You will be tired for about 3-4 weeks, on medication for 6 months. A small proportion of people may die from stomach bleeding.</td>
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<td>These conditions usually require hospitalization and may cause long-term disability. About 1 in 10 to 1 in 5 patients will die after heart attack or stroke.</td>
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<td>High Blood Pressure</td>
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• **Generalized weighting methods**
  - Best-Worst Scaling
  - Discrete-Choice Experiments (DCE)
### Best-Worst Scaling (Object Case)

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<tr>
<th>MOST Important (Please click ONE)</th>
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<th>LEAST Important (Please click ONE)</th>
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<td>Chance of dying from getting the weight loss device</td>
<td>1% (10 out of 1,000)</td>
<td>15% (150 out of 1,000)</td>
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</tbody>
</table>

- Greater weight loss
- Longer duration of side effects
- Lower Risk
DCE Question: Renal-Cell Carcinoma

**Efficacy**
- How long you will live after starting to take this medicine and how long the medicine will keep the cancer from getting worse

**Mild-to Moderate Side Effects**
- Feeling tired
- Diarrhea
- Redness and sores on your hands and feet
- Sores in your mouth or throat

**Serious Adverse-Event Risks**
- Chance of liver failure: 3 people out of 1,000 (0.3%)
- Chance of getting a blood clot: 30 people out of 1,000 (3.0%)

Which medicine would you choose if these were the only options available?

Source: Mohamed et al., Pharmacoeconomics, 2011
Benefit-Risk Thresholds

Source: Johnson et al., Chapter 4. Quantifying Patient Preferences to Inform Benefit-Risk Evaluations (In Press)
• Decision support methods
  – Analytic Hierarchy Process (AHP)
  – Multi-Criteria Decision Analysis (MCDA)
# Multi-Criteria Decision Analysis (MCDA)

<table>
<thead>
<tr>
<th>Alternative 1</th>
<th>Criterion 1</th>
<th>Criterion 2</th>
<th>Criterion 3</th>
<th>Criterion 4</th>
<th>Criterion 5</th>
</tr>
</thead>
<tbody>
<tr>
<td>a₁c₁</td>
<td>a₁c₂</td>
<td>a₁c₃</td>
<td>a₁c₄</td>
<td>a₁c₅</td>
<td></td>
</tr>
<tr>
<td>Alternative 2</td>
<td>a₂c₁</td>
<td>a₂c₂</td>
<td>a₂c₃</td>
<td>a₂c₄</td>
<td>a₂c₅</td>
</tr>
<tr>
<td>Alternative 3</td>
<td>a₃c₁</td>
<td>a₃c₂</td>
<td>a₃c₃</td>
<td>a₃c₄</td>
<td>a₃c₅</td>
</tr>
<tr>
<td>Alternative 4</td>
<td>a₄c₁</td>
<td>a₄c₂</td>
<td>a₄c₃</td>
<td>a₄c₄</td>
<td>a₄c₅</td>
</tr>
<tr>
<td>Alternative 5</td>
<td>a₅c₁</td>
<td>a₅c₂</td>
<td>a₅c₃</td>
<td>a₅c₄</td>
<td>a₅c₅</td>
</tr>
<tr>
<td>Weights</td>
<td>W₁</td>
<td>W₂</td>
<td>W₃</td>
<td>W₄</td>
<td>W₅</td>
</tr>
</tbody>
</table>

where $a_{ij}$ is the criteria score (performance) of alternative $i$ on criterion $j$

\[
\text{Value for alternative } i = \sum_{j=1}^{n} w_j a_{ij}
\]
Analytic Hierarchy Process (AHP)

Criteria Weighting:
Pairwise comparisons on a 9-point scale

AHP Scoring:
Applying weights to criteria values
Source: Dolan JG. The Patient 2012

<table>
<thead>
<tr>
<th>Major criterion</th>
<th>Effectiveness</th>
<th>Adverse effects</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td>Priority score</td>
<td>0.64</td>
<td>0.26</td>
<td>0.1</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Intra-criterion priority</th>
<th>Symptom relief</th>
<th>Hall disease progression</th>
<th>Serious</th>
<th>Common</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>0.17</td>
<td>0.83</td>
<td>0.88</td>
<td>0.12</td>
<td>1</td>
</tr>
<tr>
<td>Global priority</td>
<td>0.11</td>
<td>0.53</td>
<td>0.23</td>
<td>0.03</td>
<td>0.1</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Option scores</th>
<th>Total score&lt;sup&gt;a&lt;/sup&gt;</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug A</td>
<td>0.64</td>
</tr>
<tr>
<td>Drug B&lt;sub&gt;1&lt;/sub&gt;</td>
<td>0.26</td>
</tr>
<tr>
<td>Drug C</td>
<td>0.10</td>
</tr>
</tbody>
</table>

<sup>a</sup> The total score is calculated by multiplying each option's score by the global priority for each criterion, summing over all criteria, and then normalizing the results. For example, drug A's score = (0.64 × 0.11) + (0.09 × 0.53) + (0.65 × 0.23) + (0.70 × 0.03) + (0.74 × 0.1).
Multi-criteria Decision Analysis for Patient Preference Assessment

Bennett Levitan, MD-PhD
Department of Epidemiology
Janssen Research and Development, Titusville, NJ

FDA CDRH Patient Preference Initiative Workshop
September 18, 2013
Topics

- Multi-criteria Decision Analysis for Patient Preferences
- Industry Considerations for Eliciting Patient Preferences
Multi-criteria Decision Analysis (MCDA)

- A generic approach to aid decision-making by
  - Decomposing a complex problem into pieces
  - Applying judgment to the pieces
  - Reassembling them into a coherent whole
- Generally conducted by a facilitator with a small group of experts
- Long and successful history in Decision Analysis
- Limited application to benefit-risk or patient considerations until recently

- European Medicines Agency Benefit-Risk Methodology Project, EMA/213482/2010
Steps in Multi-criteria Decision Analysis (MCDA)

Clinical Context → Treatments → Endpoints → Data → Tradeoffs

- Uncertainty
- Risk Tolerance
- Linked Decisions

PrOACT URL Decision-making framework
Steps in Multi-criteria Decision Analysis (MCDA)

What is the problem I am trying to solve?

- What is the disease and who has it?
- What is it like to have the disease?
- How well do existing treatments work?
- What treatments are we considering?
- Over what time period am I considering treatment?
- Who is seeing this analysis?
Steps in Multi-criteria Decision Analysis (MCDA)

Clinical Context → Treatments → Endpoints → Data → Tradeoffs

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How do I want to characterize treatment performance?

Identify and pare down the key endpoints (attributes)
Steps in Multi-criteria Decision Analysis (MCDA)

How do I want to characterize treatment performance?

- Critical step for benefit-risk
- Organizes endpoints into an interpretable tree
- Distinguishes between critical and less important endpoints
- Can find differences between what patients consider important and what is measured in clinical studies
- Enables identifying symptoms or functional outcomes that can be used for PRO development
Example Value Tree: Statins for Coronary Heart Disease

Benefits

Cardiovascular Issues
- Coronary heart disease death
- Angina requiring CABG
- Nonfatal myocardial infarction
- Lipid levels meet target

Benefit-Risk Balance

Ischemic Stroke
- Fatal ischemic stroke
- Nonfatal ischemic stroke

Risks

Liver Damage
- Liver failure
- Hepatitis with hospitalization
- Hepatitis without hospitalization
- Persistently elevated liver enzymes

Muscle Damage
- Myopathy (weakness)
- Rhabdomyolysis (breakdown)
- Severe rhabdomyolysis leading to kidney failure

Steps in Multi-criteria Decision Analysis (MCDA)

Which endpoints are important and by how much?

- Two considerations:
  - What changes are important?
  - Which endpoints make the biggest difference?
- Example:
  - Does 40% vs. 50% chance hepatitis really matter?
  - Which is worse:
    - Heart attack vs. stroke?
    - Muscle weakness vs. hospitalization for hepatitis?
Steps in Multi-criteria Decision Analysis (MCDA)

Clinical Context → Treatments → Endpoints → Data → Tradeoffs

- Uncertainty
- Risk Tolerance
- Linked Decisions

What changes are important?

How much it matters to me (Utility)

Chance I have a non-fatal stroke

All I care about are changes from 0% to 10%:

The lowest and high values relevant to the decision
Steps in Multi-criteria Decision Analysis (MCDA)

Clinical Context → Treatments → Endpoints → Data → Tradeoffs

Uncertainty → Risk Tolerance → Linked Decisions

Which endpoints make the biggest difference (weighing!)?

- Several methods to elicit weights
- Common approach is “swing weighting”
- Facilitated process that can take a few hours
- Results in weights that reflect the relative impact for each endpoint “swinging” from its lowest to highest value
Multi-criteria Decision Analysis: Putting it all together

- Most of the value from MCDA comes from the collective discussion during the facilitated meetings
- Modeling results can inform decision-making by handling all the assumptions and data
- The endpoints, data, utility functions and weights result in scores and graphics that show how each endpoint contributes to the value of a treatment

**Example of MCDA Model Results**

<table>
<thead>
<tr>
<th>Treatments</th>
<th>Study Drug</th>
<th>Comparator</th>
</tr>
</thead>
<tbody>
<tr>
<td>CV</td>
<td>Stroke</td>
<td>Liver</td>
</tr>
<tr>
<td></td>
<td>CV</td>
<td>Stroke</td>
</tr>
</tbody>
</table>

Higher scores reflect better performance
Agenda

- Multi-criteria Decision Analysis for Patient Preferences
- Industry Considerations for Eliciting Patient Preferences
Some Sponsor Considerations for Preference Studies

- Is a preference study needed for benefit-risk?
- When to commit to a preference study?
- What type of study?
- Whose preferences?
- Challenges with multiple regions
- Patients and physicians from a trial or a panel?
- How will the study be viewed by health authorities?
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Net Safety Benefit
Maximum Acceptable Risk

Δ Risk

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Minimum Acceptable Benefit

Patient Benefit-Risk Threshold
TREATMENT A

Source: Hauber et al., Appl Health Econ Health Policy (2013)
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- **Weighting methods for single decisions**
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- **Generalized weighting methods**
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<tbody>
<tr>
<td>Pain:</td>
<td>Take 1 to 3 pills, spaced through the day</td>
<td></td>
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<td>5</td>
<td>3</td>
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- **Greater weight loss**
- **Longer duration of side effects**
- **Lower Risk**
## DCE Question: Renal-Cell Carcinoma

### Medicine Features

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<th>Medicine</th>
<th>How long you will live after starting to take this medicine and how long the medicine will keep the cancer from getting worse</th>
<th>Feeling tired</th>
<th>Diarrhea</th>
<th>Redness and sores on your hands and feet</th>
<th>Sores in your mouth or throat</th>
<th>Chance of liver failure</th>
<th>Chance of getting a blood clot</th>
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</thead>
<tbody>
<tr>
<td>Medicine A</td>
<td><img src="image.png" alt="Graph showing efficacy" /></td>
<td>Moderate</td>
<td>Moderate</td>
<td>Moderate</td>
<td>Mild-to-moderate</td>
<td>3 people out of 1,000 (0.3%)</td>
<td>30 people out of 1,000 (3.0%)</td>
</tr>
<tr>
<td>Medicine B</td>
<td><img src="image.png" alt="Graph showing efficacy" /></td>
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<td>Moderate</td>
<td>Moderate</td>
<td>Mild-to-moderate</td>
<td>No Chance</td>
<td>No Chance</td>
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### Efficacy

- **Mild-to Moderate Side Effects**
- **Serious Adverse-Event Risks**

### Source
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Source: Johnson et al., Chapter 4. Quantifying Patient Preferences to Inform Benefit-Risk Evaluations (In Press)
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<th>Criterion 1</th>
<th>Criterion 2</th>
<th>Criterion 3</th>
<th>Criterion 4</th>
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<tbody>
<tr>
<td>Alternative 1</td>
<td>$a_1c_1$</td>
<td>$a_1c_2$</td>
<td>$a_1c_3$</td>
<td>$a_1c_4$</td>
<td>$a_1c_5$</td>
</tr>
<tr>
<td>Alternative 2</td>
<td>$a_2c_1$</td>
<td>$a_2c_2$</td>
<td>$a_2c_3$</td>
<td>$a_2c_4$</td>
<td>$a_2c_5$</td>
</tr>
<tr>
<td>Alternative 3</td>
<td>$a_3c_1$</td>
<td>$a_3c_2$</td>
<td>$a_3c_3$</td>
<td>$a_3c_4$</td>
<td>$a_3c_5$</td>
</tr>
<tr>
<td>Alternative 4</td>
<td>$a_4c_1$</td>
<td>$a_4c_2$</td>
<td>$a_4c_3$</td>
<td>$a_4c_4$</td>
<td>$a_4c_5$</td>
</tr>
<tr>
<td>Alternative 5</td>
<td>$a_5c_1$</td>
<td>$a_5c_2$</td>
<td>$a_5c_3$</td>
<td>$a_5c_4$</td>
<td>$a_5c_5$</td>
</tr>
<tr>
<td>Weights</td>
<td>W1</td>
<td>W2</td>
<td>W3</td>
<td>W4</td>
<td>W5</td>
</tr>
</tbody>
</table>

where $a_{i}c_j$ is the criteria score (performance) of alternative $i$ on criterion $j$

Value for alternative $i$ = \[ \sum_{j=1-n}^{n} w_j a_{i}c_j \]
Analytic Hierarchy Process (AHP)

Criteria Weighting:
Pairwise comparisons on a 9-point scale

AHP Scoring:
Applying weights to criteria values
Source: Dolan JG. The Patient 2012

<table>
<thead>
<tr>
<th>Major criterion</th>
<th>Effectiveness</th>
<th>Adverse effects</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td>Priority score</td>
<td>0.64</td>
<td>0.26</td>
<td>0.1</td>
</tr>
<tr>
<td>Intra-criterion priority</td>
<td>0.17</td>
<td>0.83</td>
<td>0.88</td>
</tr>
<tr>
<td>Global priority</td>
<td>0.11</td>
<td>0.53</td>
<td>0.23</td>
</tr>
<tr>
<td>Option scores</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Drug A</td>
<td>0.64</td>
<td>0.09</td>
<td>0.65</td>
</tr>
<tr>
<td>Drug B</td>
<td>0.26</td>
<td>0.25</td>
<td>0.29</td>
</tr>
<tr>
<td>Drug C</td>
<td>0.10</td>
<td>0.65</td>
<td>0.06</td>
</tr>
</tbody>
</table>

The total score is calculated by multiplying each option's score by the global priority for each criterion, summing over all criteria, and then normalizing the results. For example, drug A's score = (0.64 × 0.11) + (0.09 × 0.53) + (0.65 × 0.23) + (0.70 × 0.03) + (0.74 × 0.1).
Role of the analytic hierarchy process in measuring patient preferences

Sonal Singh MD MPH
Johns Hopkins University
When is AHP useful as a tool

- Stakeholder engagement and incorporation of experts’ and patient preferences at any stage of health care decision-making
- Uncertainties about benefit and risk decisions
Overview of Analytic Hierarchy Process

Step 1: Defining the regulatory decision context

Step 2: Assembling and organizing outcomes

Step 3: Making comparisons
   a) Comparison among options relative to criteria
   b) Comparisons among criteria

Step 4: Comparing judgements to see how alternatives meet goal

Step 5: Sensitivity Analysis
Step 1. Decision Context

- Goals, alternatives, criterion by which alternatives are judged to meet the goal
- Stated goal: Best (safe and effective) treatment for type 2 diabetes
- Comparators: Product A vs B (standard)
- Criteria: Maximize benefits via glucose reduction and minimize adverse effects (hypoglycemia, CV effects, fractures, lactic acidosis)
- Refined after stakeholder input
AHP Model for Type 2 Diabetes
Step 2. Data Inputs: Assembling & Organizing Outcomes in an Evidence Matrix

• Determine the most reliable source of evidence on each outcomes

• Regulatory context may require critical attention to data sources- Evidentiary standards of the harm? Single clinical trial, meta-analysis of trials, observational studies, Adverse Event Reports

Step 3. Comparison among alternative criteria using AHP pairwise comparisons

• Comparisons among criterion: AHP Pairwise comparisons conducted to determine the priorities of the criteria relative to decision goal.

• *Baseline analysis assume all criteria equally important*

• Pair wise comparisons are conducted among the alternative drugs with regard to fulfilling each criterion
# AHP Pairwise comparison scale

<table>
<thead>
<tr>
<th>Verbal judgment of preferences</th>
<th>Numerical rating</th>
</tr>
</thead>
<tbody>
<tr>
<td>Extremely preferred</td>
<td>9</td>
</tr>
<tr>
<td>Very strongly to extremely</td>
<td>8</td>
</tr>
<tr>
<td>Very strongly</td>
<td>7</td>
</tr>
<tr>
<td>Strongly to very strongly</td>
<td>6</td>
</tr>
<tr>
<td>Strongly preferred</td>
<td>5</td>
</tr>
<tr>
<td>Moderately to strongly</td>
<td>4</td>
</tr>
<tr>
<td>Moderately preferred</td>
<td>3</td>
</tr>
<tr>
<td>Equally to moderately</td>
<td>2</td>
</tr>
<tr>
<td>Equally preferred</td>
<td>1</td>
</tr>
</tbody>
</table>
Comparison among options/criteria

- Compare the ability of alternative options to achieve the decision goal by making comparison among alternatives for each criterion
- Standard AHP pairwise comparisons among alternatives for each of the criterion
- Same pairwise comparison to determine the priorities of the each of the criteria relative to decision goal.
Combining judgments to evaluate how alternatives meet goal

- Standard AHP process weighting to combine results of judgment made in Step 3 to determine relative ability of drugs to meet stated goal
- After all the comparisons are made they are combined to create a normalized, ratio scale that summarizes the results of the direct and indirect comparisons made among the decision elements
- Relative differences > 1.1 considered significant
## Overall Results

<table>
<thead>
<tr>
<th>Alternative</th>
<th>All Participants (-1)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Exenatide (GLP-1 analog)</td>
<td>21.68 %</td>
</tr>
<tr>
<td>Metformin</td>
<td>24.91 %</td>
</tr>
<tr>
<td>Pioglitazone</td>
<td>16.31 %</td>
</tr>
<tr>
<td>Sitagliptin (DPP-4 inhibit)</td>
<td>19.82 %</td>
</tr>
<tr>
<td>Sulfonylureas</td>
<td>17.28 %</td>
</tr>
</tbody>
</table>
## Priority of Objectives

<table>
<thead>
<tr>
<th>Objectives</th>
<th>Local Priority</th>
<th>Global Priority</th>
</tr>
</thead>
<tbody>
<tr>
<td>Goal</td>
<td>100.00 %</td>
<td>100.00 %</td>
</tr>
<tr>
<td>Maximize benefits</td>
<td>54.83 %</td>
<td>54.83 %</td>
</tr>
<tr>
<td>Reduce HbA1c</td>
<td>100.00 %</td>
<td>54.83 %</td>
</tr>
<tr>
<td>Minimize harms</td>
<td>45.17 %</td>
<td>45.17 %</td>
</tr>
<tr>
<td>Minimize non-serious harms</td>
<td>32.74 %</td>
<td>14.79 %</td>
</tr>
<tr>
<td>Minimize risk of fracture</td>
<td>17.40 %</td>
<td>2.57 %</td>
</tr>
<tr>
<td>Minimize weight gain</td>
<td>51.71 %</td>
<td>7.66 %</td>
</tr>
<tr>
<td>Minimize GI symptoms</td>
<td>30.89 %</td>
<td>4.57 %</td>
</tr>
<tr>
<td>Minimize serious harms</td>
<td>67.26 %</td>
<td>30.38 %</td>
</tr>
<tr>
<td>Minimize risk of severe hypoglycemia</td>
<td>46.12 %</td>
<td>14.01 %</td>
</tr>
<tr>
<td>Minimize risk of CHF</td>
<td>26.20 %</td>
<td>7.96 %</td>
</tr>
<tr>
<td>Minimize risk of acute pancreatitis</td>
<td>14.68 %</td>
<td>4.46 %</td>
</tr>
<tr>
<td>Minimize risk of bladder cancer</td>
<td>12.99 %</td>
<td>3.95 %</td>
</tr>
</tbody>
</table>
Sensitivity analysis

• Explore impact of different judgments on relative importance of criteria varying priorities from 0 (no importance) to 1 (most important).
• Input on relative importance from various stakeholders.
• Conduct additional sensitivity analysis to determine impact on decision goals
• Vary probability inputs
• Vary preferences
  – Input on relative importance (priorities) from various stakeholders
Consistency

- Measure consistency among comparisons to estimate consistency ratio
- Perfect consistency occurs when judgments are transitive and numerically consistent
  - i.e. if a/b = 2 and b/c = 3, a/c should = 6
- AHP does not require transitivity or numerical consistency
- Generally 10 (or 15%) inconsistency (ratio <=1) is tolerated because it is one order of magnitude smaller than the scale (0-1)
Validation

- AHP has been extensively validated
- AHP is a method of measurement
- If the underlying assumptions met and it is technically AHP is considered industry standard
Advantages of AHP

• Helps to improve judgement
• Low cognitive stress
• Hierarchial structure
• Flexible, easy to apply
• Possible to use us a group consensus building tool in small groups
Limitations of AHP

• Task sometimes seen as less realistic
• Some assumptions, such as those of independence may be unrealistic in certain scenarios
Facilitating preference-concordant decisions by patients

R. Scott Braithwaite, MD, MS, FACP
President, Society for Medical Decision Making
Chief, Division of Comparative Effectiveness and Decision Sciences
Professor of Medicine and Population Health
New York University School of Medicine
Disclaimer

• Not an expert on preference elicitation though many in SMDM are
  – Methods concierge service

• Will focus on gathering and presenting information to facilitate patient-centered decision making
  – Preference-concordance
First steps towards preference-concordance

• What do patients expect of FDA?
  – “Safety” is context-dependent
  – May be easy place to hide but hard place for patient decision making
  – Does risk aversion of FDA match risk aversion of consumers?
    » Societal versus individual preference?

• Establish explicit systematic quantitative criteria for harm/benefit assessment
  – Risk tolerance may vary depending on presence of alternatives and seriousness of untreated risk
Does FDA try hard enough to pick up “harm” signal?

• Add generic QOL measure (e.g. EQ 5-D) to the more typical disease-specific measures
  – May pick up “harm” signal otherwise undetected

• Be explicit about whether studies have power sufficient to pick up amount of harm that would offset hypothesized benefit

• Are comparators the next best alternative?
Does one size fit all?

- Preference-concordant decisions may require different messaging for different harm/benefit
- Should FDA have different “labels” reflecting
  - _Magnitude_ of potential treatment harm?
  - _Size_ of harm/benefit ratio
  - _Certainty_ of harm/benefit ratio
  
    » Statistical uncertainty (insufficient power to detect harms sufficient to offset benefits),
    
    » Evidence uncertainty (study design limitations or uncertainty regarding effect of comparator)
    
    » Uncharacterized heterogeneity of treatment effect

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What different “labels” could look like: one vision

• “Safe”
• “Benefits proven to exceed risks for most patients”
• “Benefits proven to exceed risks for some patients”
• “Benefits likely to exceed risks for most patients”
• “Benefits likely to exceed risks for some patients”
  – +/- “harms may be severe”
  – May complement drug “fact box”
FDA as “learning institution”

• Important to test whether information from FDA facilitates patient-centered and preference-concordant decisions

• Should FDA routinely conduct research using standard instruments for decision quality?
  – Decision satisfaction,
  – Decision regret
  – Decision conflict
  – Knowledge
Panel Questions

• What methods and tools can be used to collect patient information?
• What are the relative strengths and limitations of these methods and tools?
• What approaches should be used to collect patient preference information?
• Who should collect patient preference information?
• What solutions do you see going forward?

**For the online webcast:** Please submit your questions to the panel via the chat box. The online hosts will be collecting the questions during the session to be brought to the panel moderator during the panel discussion.