

09 May 2018

# Patient Perspectives and Preferences on Benefit-Risk

Advancing the Science of Patient Input in Medical Product R&D

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The power of **knowledge.**  
The value of **understanding.**

# Patient Preference Information

## Definition

*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<sup>1</sup>*

## Multiple Methods

Group	Method
Structured-weighting	<ul style="list-style-type: none"> <li>• Simple direct weighting</li> <li>• Ranking exercises</li> <li>• Swing weighting</li> <li>• Point allocation</li> <li>• Analytic hierarchy process</li> <li>• Outranking methods</li> </ul>
Health-state utility	<ul style="list-style-type: none"> <li>• Time tradeoff</li> <li>• Standard gamble</li> </ul>
Stated-preference	<ul style="list-style-type: none"> <li>• Direct-assessment questions</li> <li>• Threshold technique</li> <li>• Conjoint analysis and discrete-choice experiments</li> <li>• Best-worst scaling exercises</li> </ul>
Revealed-preference	<ul style="list-style-type: none"> <li>• Patient-preference trials</li> <li>• Direct questions in clinical trials</li> </ul>

## IMI-PREFER List of Stated Preference Methods

- Divides methods into 2 categories
  - Preference exploration (qualitative) methods
  - Preference elicitation (quantitative) methods
- Preference Exploration
  - Individual techniques (3)
  - Group techniques (6)
  - Individual/group techniques (1)
- Preference Elicitation
  - Discrete choice techniques (4)
  - Threshold techniques (7)
  - Rating techniques (7)
  - Ranking techniques (4)

1 CDRH Guidance <https://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM446680.pdf>

2 MDIC Benefit-Risk Guidance: <http://mdic.org/spi/pcbr-framework-report-release/>

3 IMI-PREFER Work Package 2.4: <http://www.imi-prefer.eu/>

# Two Recent and Very Different Examples

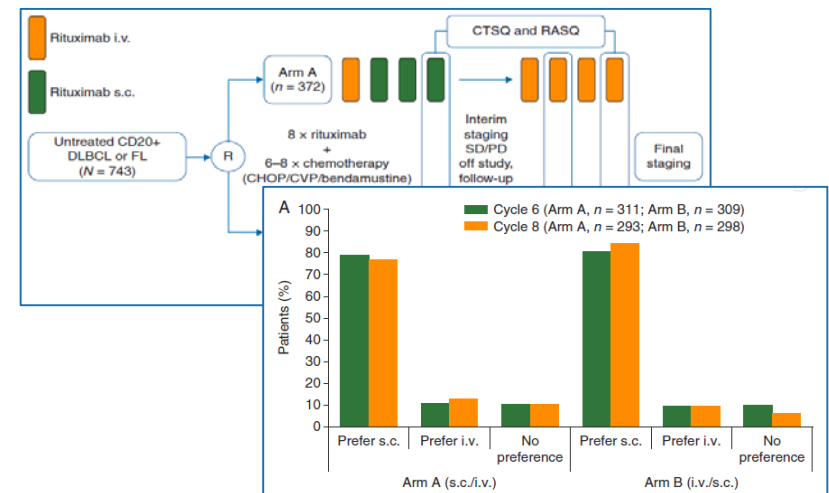
## Obesity Devices

- FDA developed a stated-preference survey to elicit benefit-risk preferences for multiple attributes of hypothetical obesity devices<sup>1</sup>
- Used the results in evaluating the Maestro Rechargeable system<sup>2</sup>



## Oncology Mode of Administration

- Genentech sponsored an interventional study to elicit preferences for subcutaneous and IV rituximab<sup>3</sup>
- Results included in the label for Rituxan HYCELA<sup>4</sup>



1 Ho et al., Surgical Endoscopy (2015)

2 <http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm430223.htm>

3 Rummel et al., Annals of Oncology (2017)

4. [https://www.gene.com/download/pdf/rituxan\\_hycela\\_prescribing.pdf](https://www.gene.com/download/pdf/rituxan_hycela_prescribing.pdf)

# Patient Preference Challenges

## Differences between the two examples:

- FDA-sponsored vs industry-sponsored
- Hypothetical scenarios vs prior exposure
- Attribute-based vs decision-based
- Applicable to multiple decisions vs applicable to a single decision

## Challenges in Collecting and Using Patient Preference Information:

- Whenever we collect patient preference information, we need to first understand
  - What decision will this information support?
  - What are the information needs of the end user?
  - What is the appropriate methods for a given situation?
  - How will we evaluate or demonstrate the quality and usefulness of the information that is collected?