

Validation of Patient Preference Data

FDA reflects on the previous panels regarding patient identification, methodology, limitations, and how patient preference information can be validated to be used for regulatory submissions.

***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:

William Maisel, M.D., M.P.H.

Deputy Director for Science and Chief Scientist

CDRH

Panel:

Carolyn Neuland, Ph.D.

CDRH/Office of Device Evaluation

Greg Campbell, Ph.D.

CDRH/Office of Surveillance and Biometrics

Juan Marcos Gonzalez, Ph.D.

Research Triangle Institute (RTI-Health Solutions)

Randall Brockman, M.D.

CDRH/Office of Device Evaluation

Sahar Dawisha, M.D.

CDRH/Office of In Vitro Diagnostics and Radiological Health

Scott Braithwaite, M.D.

Society of Medical Decision Making (SMDM)

Carolyn Neuland, Ph.D.
Chief, Renal Devices Branch
CDRH/Office of Device Evaluation

Greg Campbell, Ph.D.

Director, Division of Biostatistics

CDRH/Office of Surveillance and
Biometrics

Randall Brockman, M.D.

Chief Medical Officer

CDRH/Office of Device Evaluation

Sahar Dawisha, M.D.

Medical Officer

**CDRH/Office of In Vitro
Diagnostics and Radiological
Health**

Scott Braithwaite, M.D.

President

Society of Medical Decision Making

Discrete-Choice Experiments: Validation of Preference Data

**Patient Preference Initiative
Workshop**

Juan Marcos Gonzalez, Ph.D.

September 18-19, 2013

Evidence Standards for Patient-Preference Data

- **Well-informed respondents**
 - Relevant to clinical endpoints
 - Common understanding of benefits and risks
- **Controlled experiment**
 - Randomization of experimental stimuli
 - Utility-theoretic measurement
- **Representative sample**
 - Condition severity
 - Treatment and outcome experience
 - Sample size
- **Statistical analysis of effect sizes**
 - Data-quality evaluation
 - Correct statistical modeling
 - Formal hypothesis testing

Types of Validation

- **Data Validation** - Review response patterns to identify inconsistencies across responses
- **Results Validation** – Review of estimated weights to identify inconsistencies with empirical literature or prior expectations

Data Validation

- **Test respondents' attention and understanding of the choice task**
 - Task tutorial and follow-up comprehension questions
 - Test consistency of answers to repeated questions
 - Evaluate variation in responses
 - Include questions with known response based on theoretic principles and review respondents' answers to these questions

Results Validation

- **Test results' consistency with behavior as measured in other contexts**
 - Consistency with strongly held ideas about human behavior (e.g., results that show that, all else equal, people prefer to die)
 - Consistency with theoretic principles behind choice analysis (e.g., weak monotonicity and exogeneity of outcomes)

Panel Questions

- How to validate patient preference data and information?
- What methods and tools can be used for validation?
- Who should validate patient preference information?
- How will reviewers judge the validity of patient preference data?

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