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Incorporating Patient Preferences in HTA

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Overview

- What are patient preferences
- The use of Patient Preferences in a Regulatory Setting
- The role of Patient Preferences in Reimbursement Setting
- Current practice in incorporating patient preferences in the HTA process
- Implications

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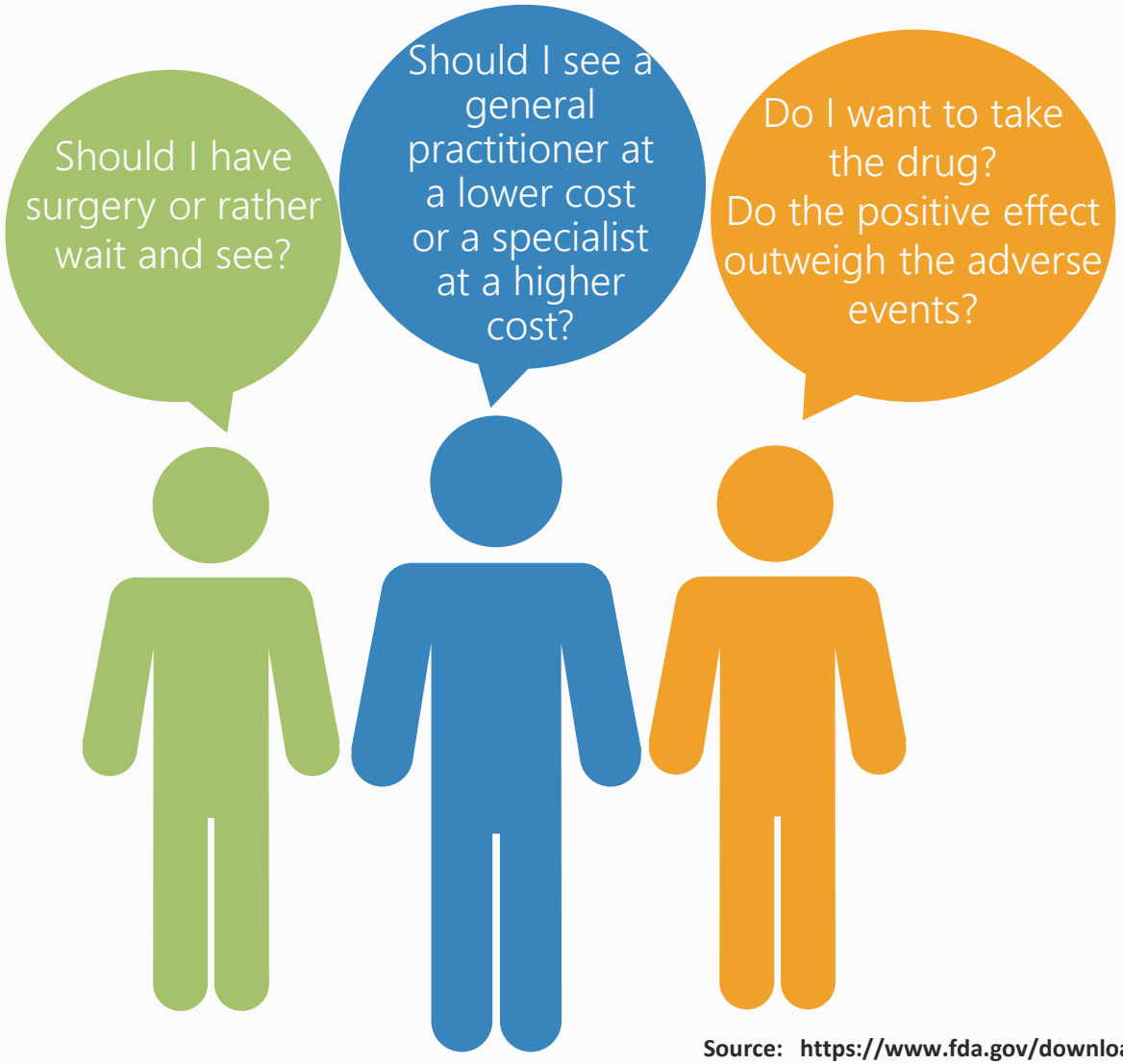
What are patient preferences

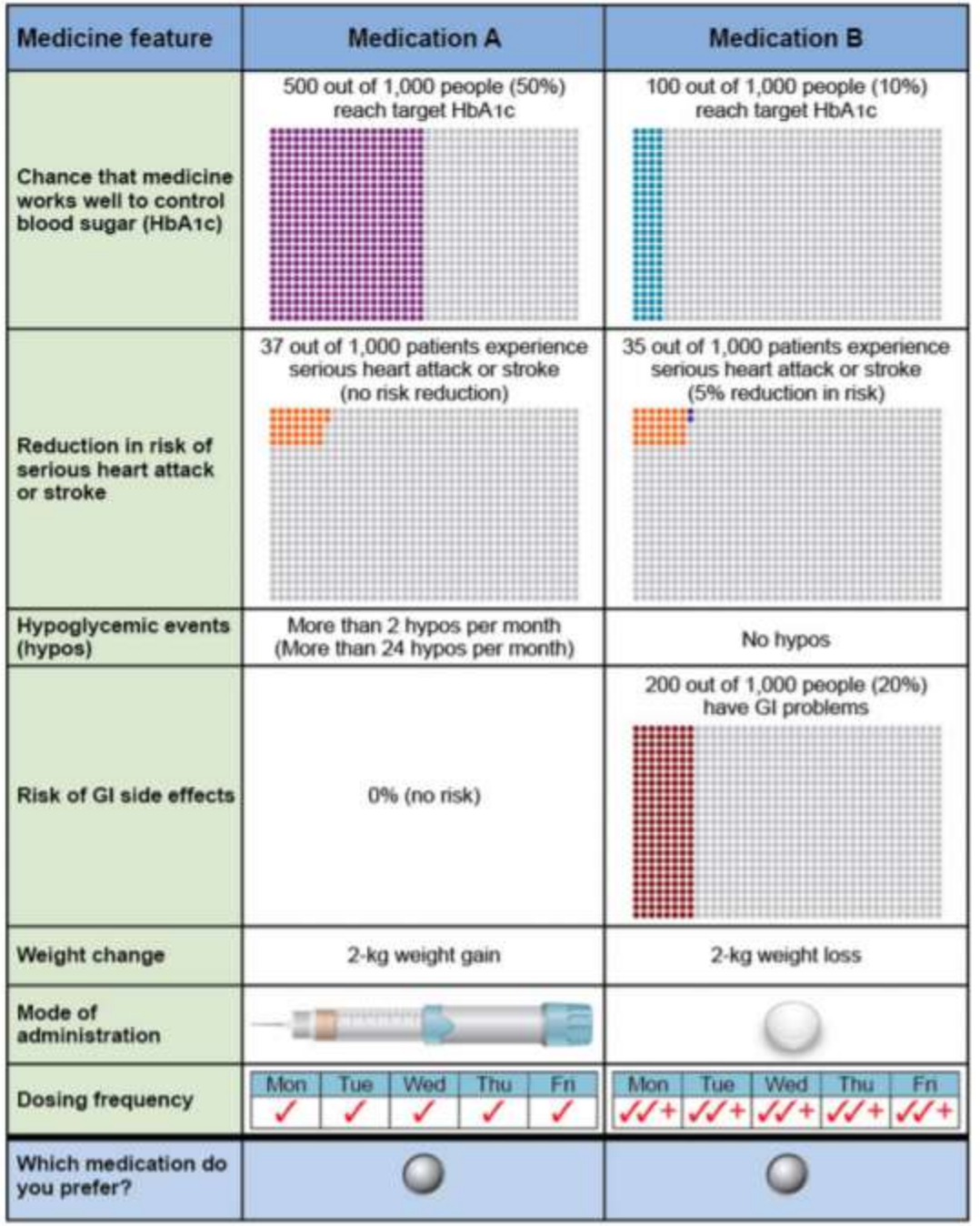


- **Patient preference information (PPI)** is defined as: **qualitative** or **quantitative** assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions (FDA 2015).
  - ✓ **Qualitative PPI**… may be useful in identifying **which** outcomes, endpoints or other attributes are valued most by patients and which factors affect patients' perspectives on risk and benefit.
  - ✓ **Quantitative PPI** can provide estimates of **how much** different outcomes, endpoints or other attributes are valued by patients, and the **tradeoffs** that patients state or demonstrate they are willing to make among them.

Source: https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf#page=8&zoom=100,0,593

Eliciting patient preferences

Discrete Choice Experiments Design



Medicine feature	Medication A	Medication B
Chance that medicine works well to control blood sugar (HbA1c)	500 out of 1,000 people (50%) reach target HbA1c	100 out of 1,000 people (10%) reach target HbA1c
Reduction in risk of serious heart attack or stroke	37 out of 1,000 patients experience serious heart attack or stroke (no risk reduction)	35 out of 1,000 patients experience serious heart attack or stroke (5% reduction in risk)
Hypoglycemic events (hypos)	More than 2 hypos per month (More than 24 hypos per month)	No hypos
Risk of GI side effects	0% (no risk)	200 out of 1,000 people (20%) have GI problems
Weight change	2-kg weight gain	2-kg weight loss
Mode of administration		
Dosing frequency	Mon ✓ Tue ✓ Wed ✓ Thu ✓ Fri ✓	Mon ✓✓+ Tue ✓✓+ Wed ✓✓+ Thu ✓✓+ Fri ✓✓+
Which medication do you prefer?		

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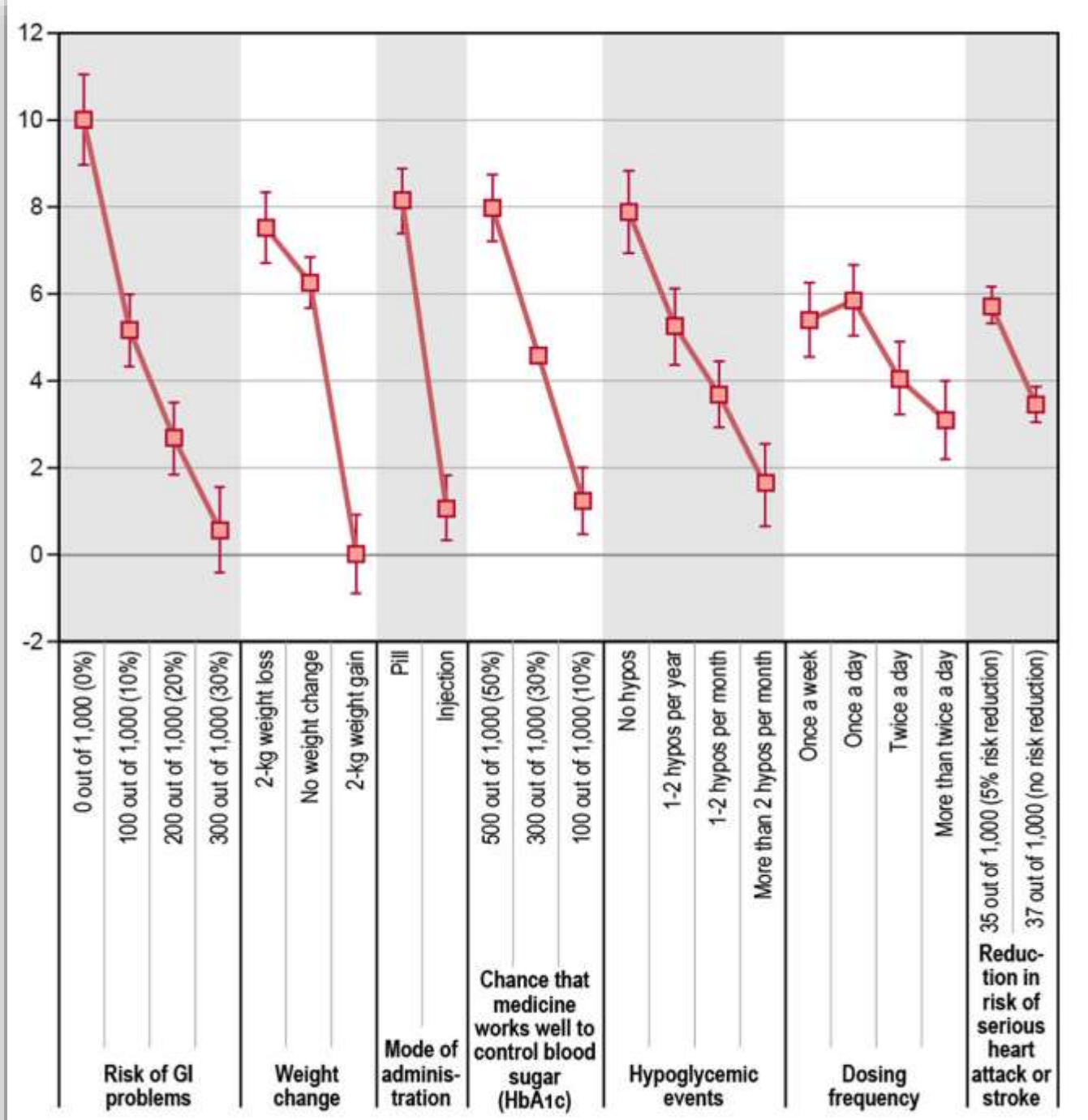
Source: Mansfield et al, 2017 Diabetes Ther. Dec;8(6):1365-1378

Eliciting patient preferences

Discrete Choice Experiments Results- Interpretation



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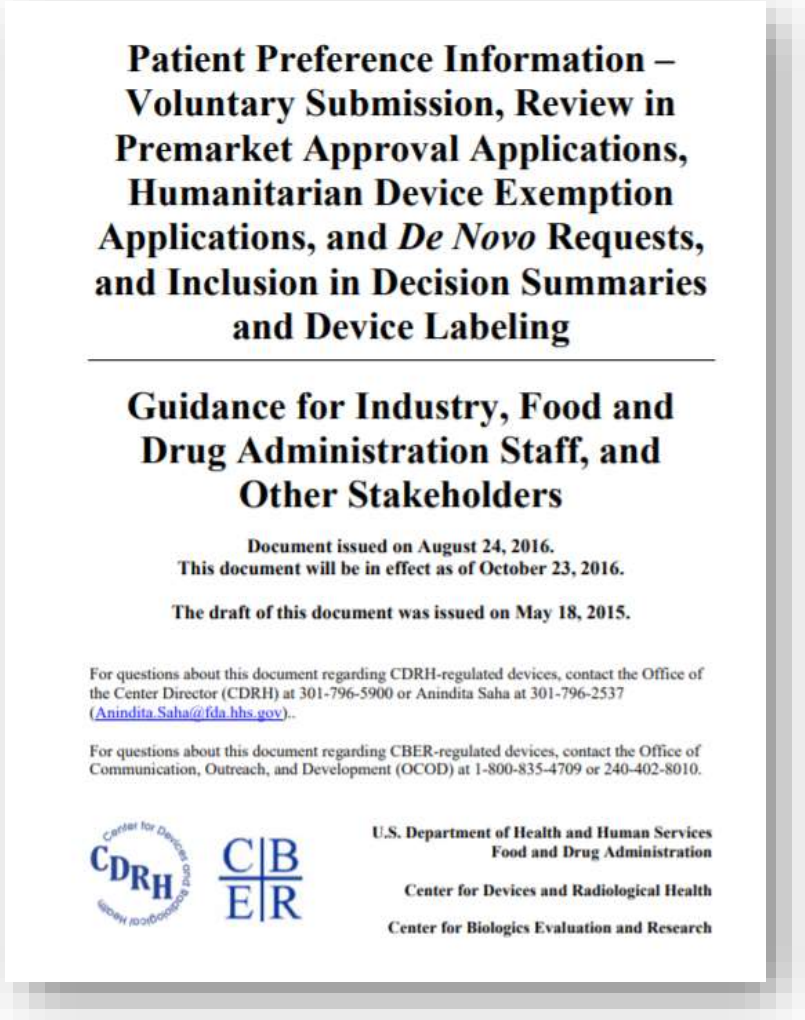
Source: Mansfield et al, 2017 Diabetes Ther. Dec;8(6):1365-1378

Incorporating patients preferences Regulatory environment



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

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

Document issued on August 24, 2016.
This document will be in effect as of October 23, 2016.

The draft of this document was issued on May 18, 2015.

For questions about this document regarding CDRH-regulated devices, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anindita Saha at 301-796-2537 (Anindita.Saha@fda.hhs.gov)..

For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

US Food and Drug Administration (FDA)

Center for Devices and Radiological Health

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Center for Biologics Evaluation and Research

- ❑ Guidance on how to collect patient preference
- ❑ Recommendation on incorporating data into a benefit-risk assessment framework
- ❑ Recommendation on including preferences information in labelling
- ❑ Voluntary submission of preference data

Source:
https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf

Incorporating patients preferences Regulatory environment

European Medicines Agency (EMA)

2009-2012: Benefit-Risk assessment (BRA) methodology project

Develop decision-making models that could to make the assessment of the benefits and risks of medicines more **consistent**, more **transparent** and **easier to audit.**

2009-2015: Innovative Medicines Initiative's PROTECT project (Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium)

To develop innovative tools and methodological standards, and contribute to helping enhance the monitoring of the safety of medicinal products. Improved evaluation and communication of their benefit-risk profile throughout medicines life cycle.

2016- present: Innovative Medicines Initiative's PREFER project (Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle). Develop a systematic approach for considering the use of patient preferences across the medical treatment life cycle. Final recommendations in 2021 on incorporating patient preferences for both BRA and HTA.

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Values for patient involvement in the HTA process

Fairness	Have the same rights to contribute to the HTA process as other stakeholders
Legitimacy	Facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process
Relevance	Knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA
Equity	Contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users
Capacity building	Addresses barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together

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Source: https://htai.org/interest-groups/pcig/values-and-standards/

Forms of patient involvement in decision-making based on HTA

- Possibility of appeal for patients/patient organisations against the final recommendations of the decision makers
- Involvement through public consultations
- Involvement in appraisal committees
- Patient evidence (patients submission) provided through HTA, being weighted in the decision and clearly included in public reports and communications
- Involvement in prioritising the research topics/topic selection/scoping

Source: http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf

How can patient preferences be incorporated in the HTA process: two different HTA paradigms

	NICE, England	IQWiG, Germany
Assessment method	Cost-utility analysis	Efficiency frontier
Decision space	Societal allocation of health care resources	Allocation within treatment area
Sources of preferences	General public	Patients preferences

IQWiG: "…. In order to better determine the weighting of different outcomes from the patient perspective, IQWiG tested the conjoint analysis (CA) and the analytic hierarchy process (AHP). It was shown that both methods can contribute to determining the most important outcomes for patients"

NICE: "We do see Discrete Choice Experiments (DCEs) being considered/applied to inform this decision problem, but eliciting general population preferences…"

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Source: http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf

How can patient preferences be incorporated in the HTA process

Additional ways to use DCEs preference data

- Process Utility: attributes related to health care processes (waiting time, frequency of visits, mode of administration)
- Willingness –to-pay (and Net monetary benefit)
- Uptake rate: likelihood that patient will adopt or continue to take a treatment (external validity?)

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Source: http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf

Implications

- ❑ Capitalize on the synergies of data requirement between regulatory and reimbursement purposes (i.e. Plan early)
- ❑ Consider the use of Discrete Choice Experiments at early stages of product development
- ❑ Communicate with payers
- ❑ Consider using DCEs as a source of real world evidence to support HTA submissions (alongside core items e.g. clinical, cost-effectiveness in a Value Dossier)

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Source: http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf

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