

RTI HEALTH SOLUTIONS®

ELICITATION OF STAKEHOLDER PREFERENCES TO ESTIMATE ADHERENCE AND ADOPTION OF PERSONALIZED DRUG TREATMENT

**3rd Annual Health Economics &
Personalized Medicine Symposium
Amsterdam, 13 November 2013**

A. Brett Hauber, PhD
Senior Economist and Vice President
Health Preference Assessment
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Personalized medicine is designed to meet the unique needs of a patient as defined by the personal characteristics of his or her disease in order to maximize treatment benefits or minimize treatment risks

Patient Preferences

- **Most patient-preference studies focus on eliciting tradeoffs among individual endpoints or outcomes**
- **Some patient-preference studies focus on tradeoffs among attributes of healthcare delivery**

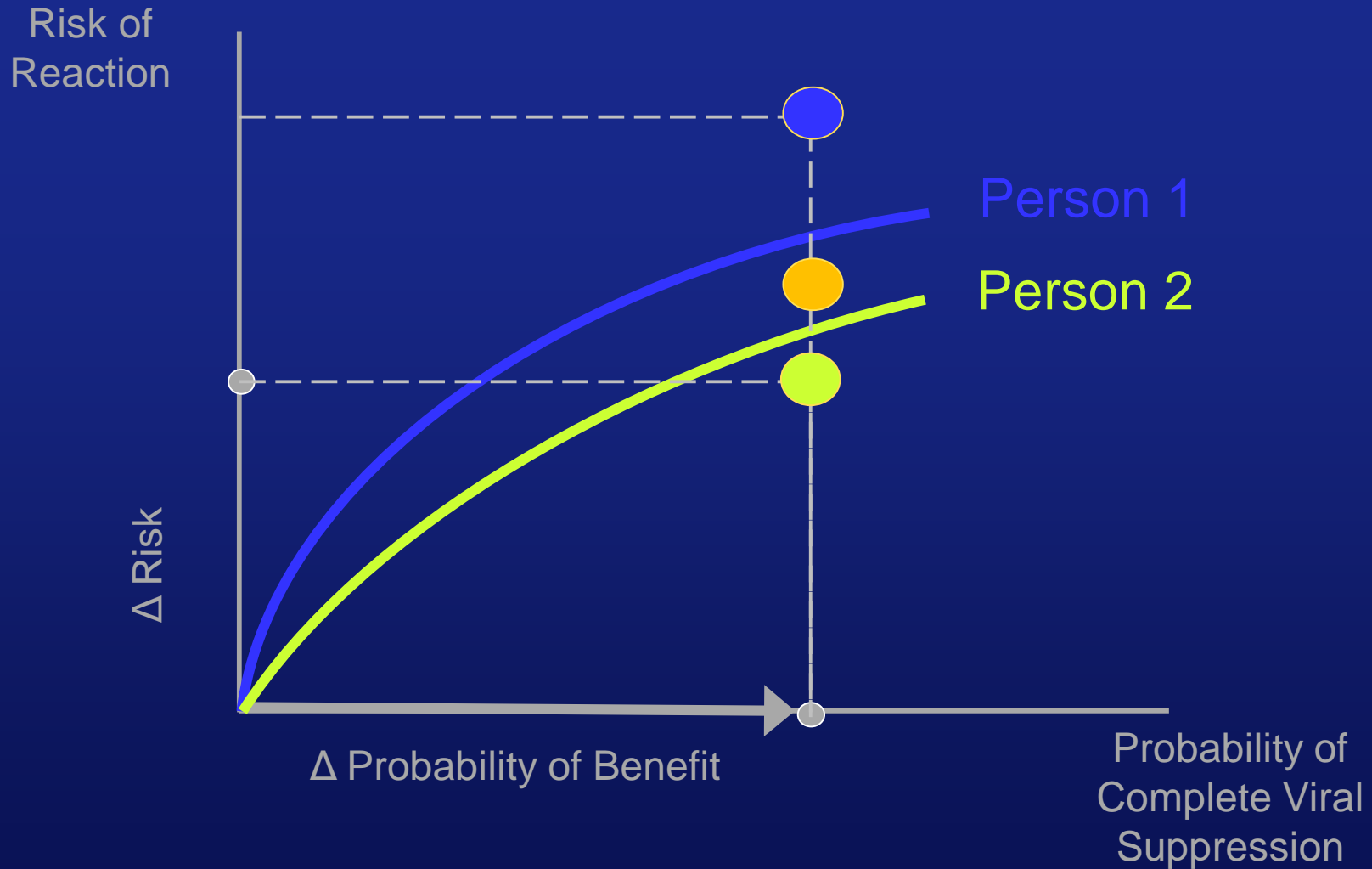
Patient Preferences

- **Most patient-preference studies focus on eliciting tradeoffs among individual endpoints or outcomes**
 - Important for understanding individual-level or disease-level treatment decisions
 - May have implications for reimbursement (but probably only if it provides a monetary measure of value)
- **Some patient-preference studies focus on tradeoffs among attributes of healthcare delivery**
 - Important for understanding optimal healthcare delivery including budget allocation

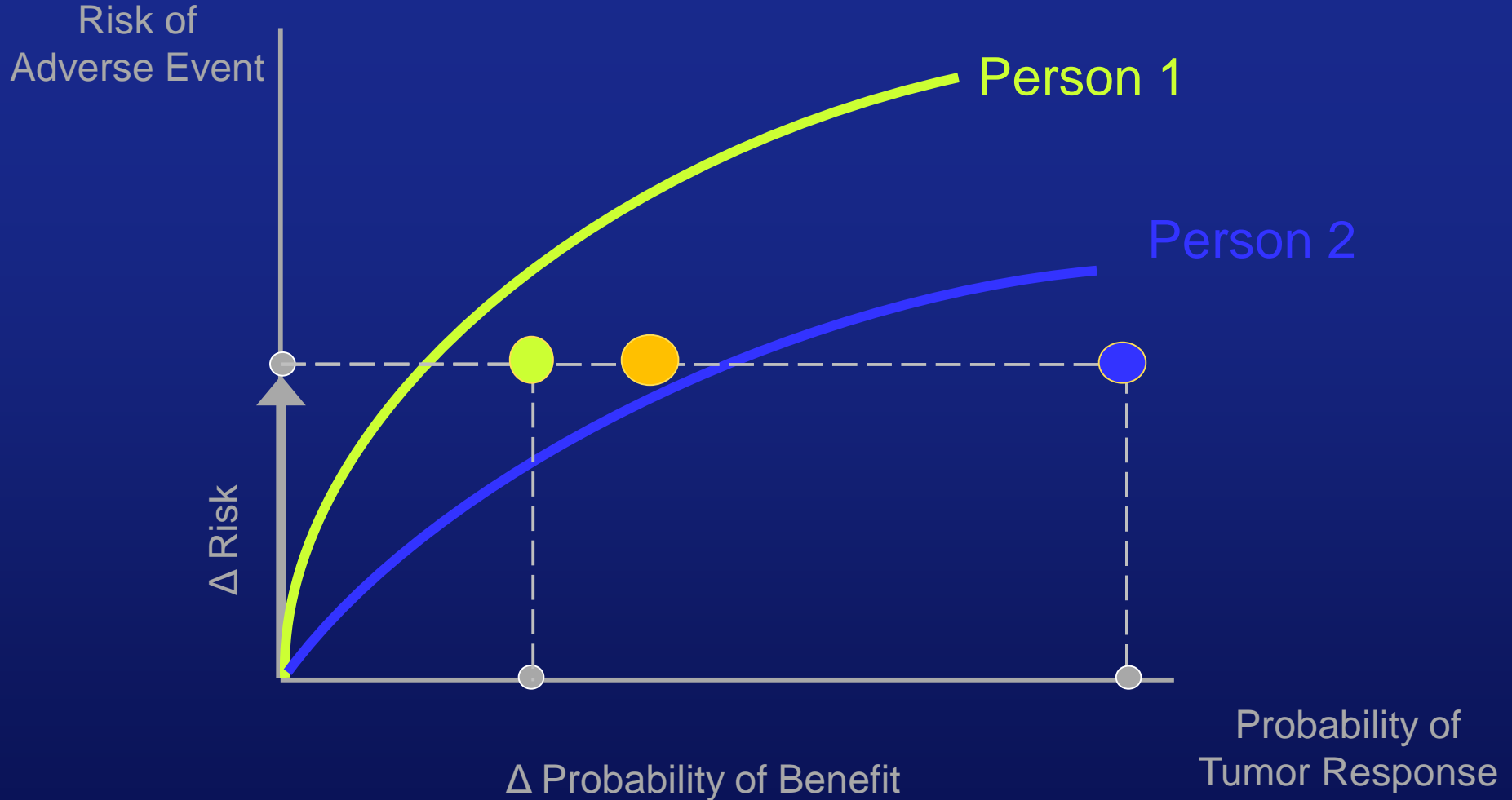
Consider 2 Cases for Genetic Testing

- **Increased Risk of Adverse Event**
 - Hypersensitivity reaction and abacavir in HIV (HLA-B*5701)
 - Increases the risk for a given benefit
- **Increased Probability of Benefit**
 - HER2+ breast cancer and targeted therapies (IHC or ISH)
 - Increases the probability of benefit for any given risk

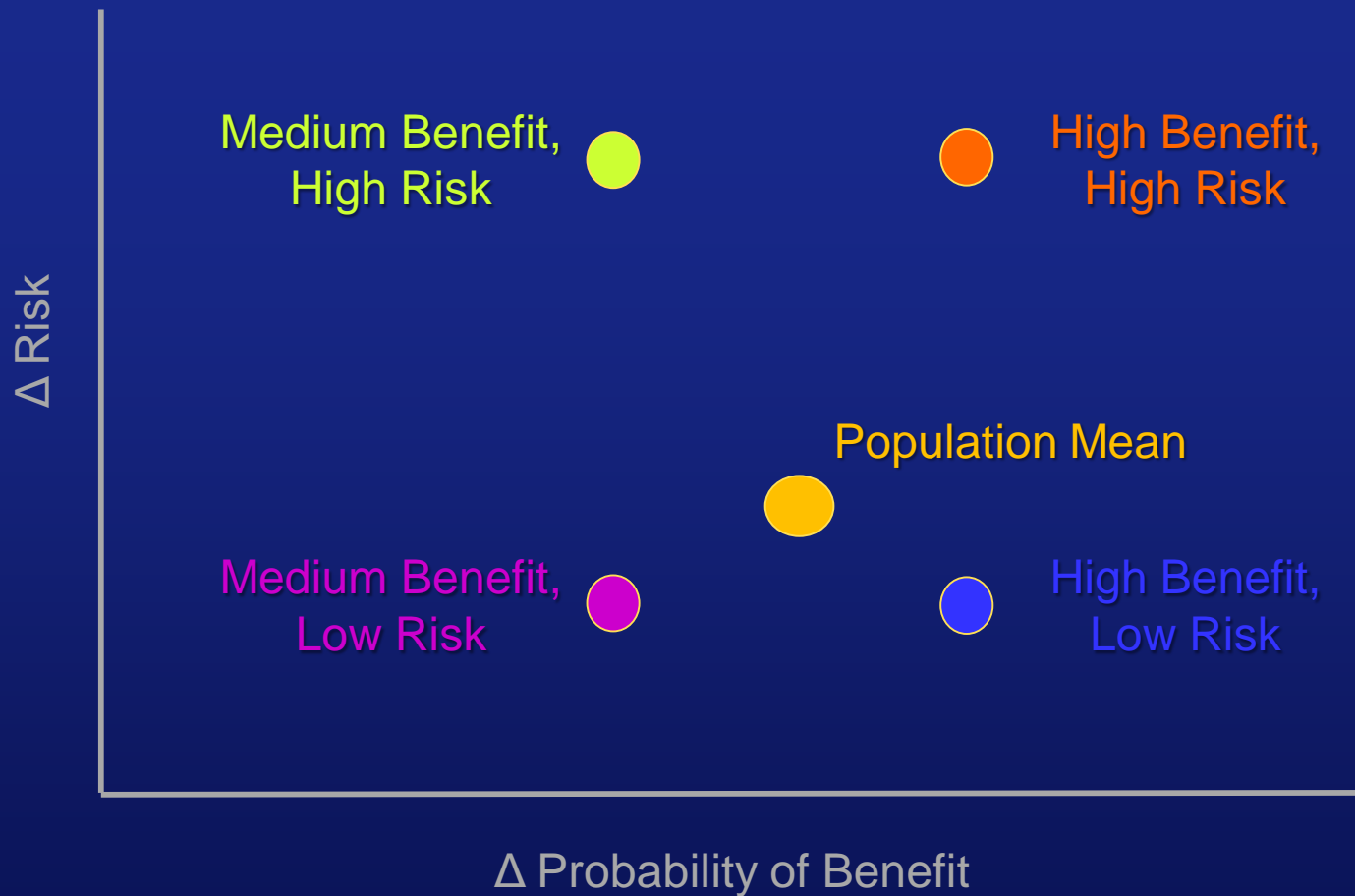
Hypersensitivity Reaction



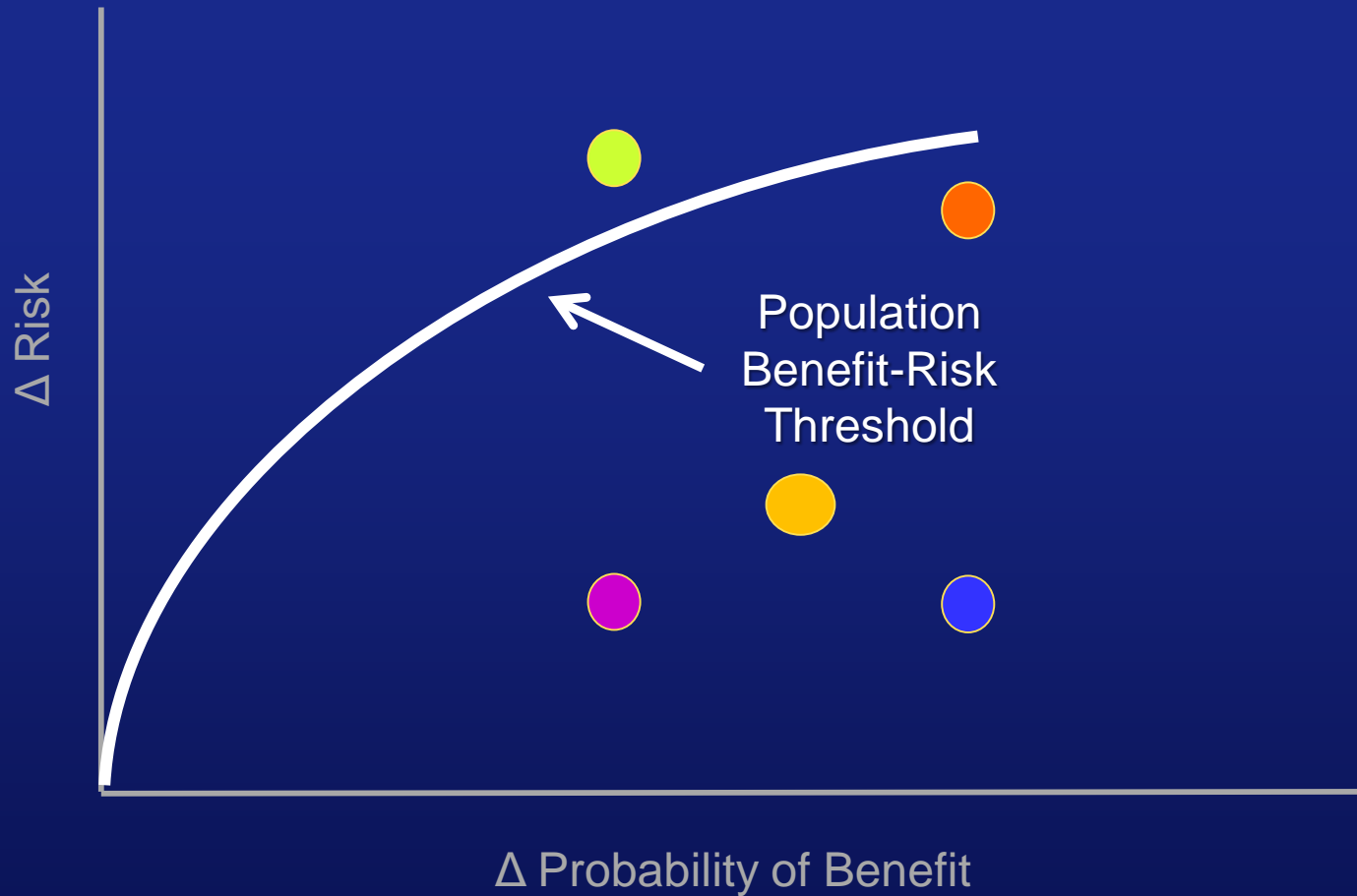
HER2+ Breast Cancer



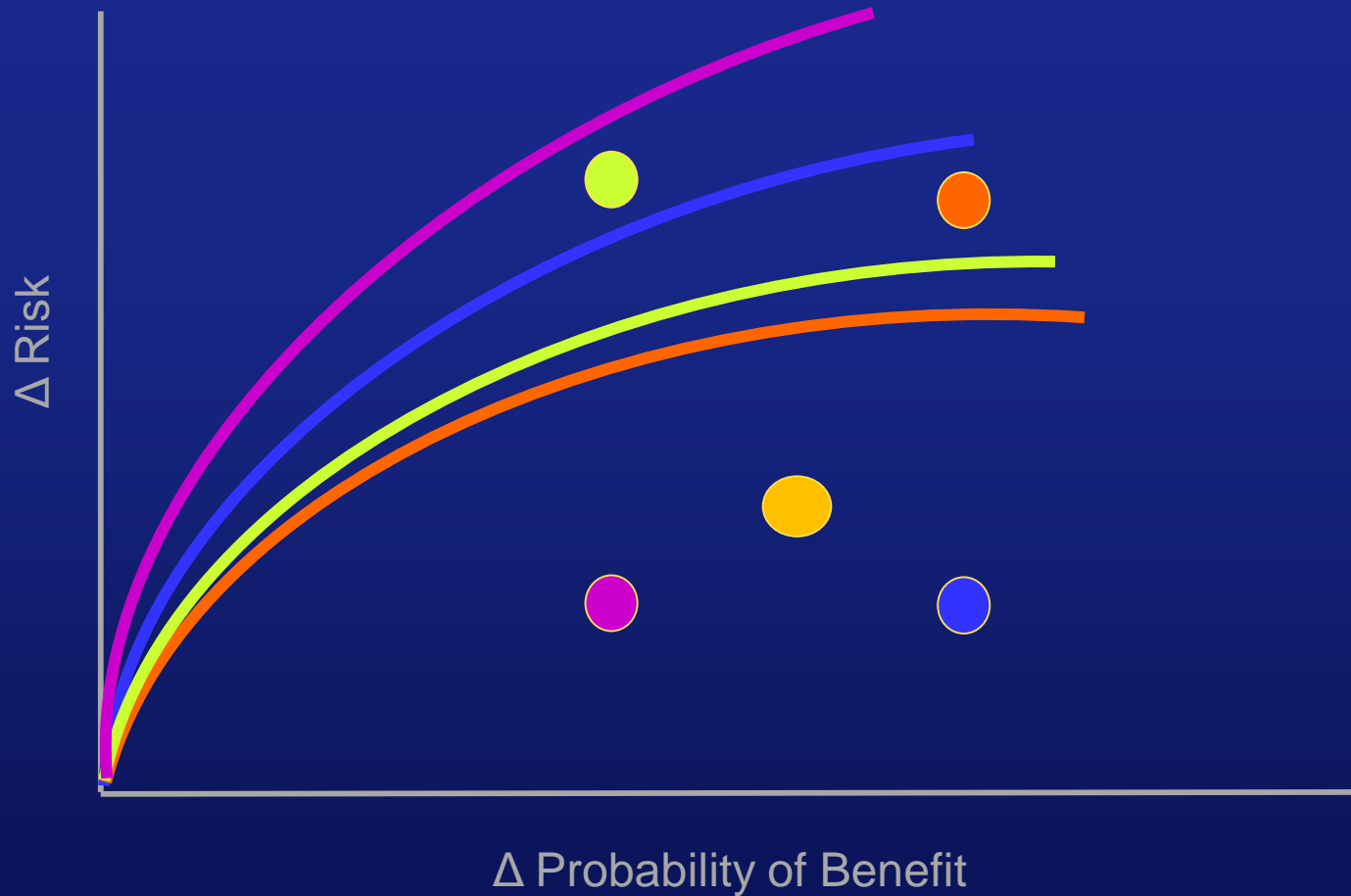
Consider 4 outcomes for a given treatment



Consider 4 outcomes for a given treatment



Consider 4 outcomes for a given treatment



Patient Preferences and Personalized Medicine

“Traditional” View

- **Most studies present population means**
 - Randomized controlled trials
 - Preference studies
- **Personalized medicine allows us to understand and exploit heterogeneity in clinical outcomes**
- **Preference heterogeneity also matters**
 - Individuals at highest risk may have high risk tolerance
 - Individuals who stand to benefit the most may not value the benefits very highly
- **When discussing patients, the challenge is to match individual-level preferences with individualized expected outcomes**

Effectively implementing personalized medicine in the real world may also require a better understanding of the multiple stakeholders who may be involved in the treatment process

Preferences influence decisions

Patients
Caregivers

Seeking treatment
Taking treatment
Adhering to treatment
Persisting with treatment

Healthcare providers

Prescribing treatment
Monitoring treatment

Regulators

Market authorization
Needs for additional data
Post-marketing surveillance

Payers

Reimbursement

Patients and caregivers

Patients
Caregivers

Seeking treatment
Taking treatment
Adhering to treatment
Persisting with treatment

Individual-level decisions, informed by individual-level preferences, subject to:

- Individual budget constraints
- Available technologies

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Who's preferences matter?

- Patients
- Caregivers
- Household
- Healthcare providers

Healthcare providers

Healthcare providers

Prescribing treatment
Monitoring treatment

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Regulators

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Market authorization
Needs for additional data
Post-marketing surveillance

Patient-population-level decisions, informed by population-level, societal-level, and regulators preferences, subject to:

- Available technologies

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Market authorization
Needs for additional data
Post-marketing surveillance

Patient-population-level decisions, informed by population-level, societal-level, and regulators preferences, subject to:

- Available technologies

Who's preferences matter?

- Patients
- Caregivers
- Household
- Healthcare providers
- Regulators
- Society

Payers



Societal decisions informed by societal and payer preferences, subject to:

- Budget constraints
- Available technologies
- Competing priorities

Payers

Payers

Reimbursement

Societal decisions informed by societal and payer preferences, subject to:

- Budget constraints
- Available technologies
- Competing priorities

Who's preferences matter?

- Patients, caregivers, household
- Healthcare providers
- Society
- Payers

Why are payer preferences different?

- **Payer decisions are primarily budget-allocation decisions**
- **Cannot evaluate a single technology outside the context of**
 - Alternative technologies for the same disease
 - Technologies for other diseases
 - Budget
- **Most patient, healthcare provider, and regulator decisions only consider**
 - Alternative technologies for the same disease

Who's preferences matter in payer decisions?

Societal decisions informed by societal and payer preferences, subject to:

- Budget constraints
- Available technologies
- Competing priorities

Who's preferences matter?

- Patients, caregivers, household
- Healthcare providers
- Society
- Payers

- **Regulatory decisions influence available technologies (constraint)**
- **Regulators' preferences likely do not play a role in payer decisions**

Who's preferences matter in payer decisions?

Societal decisions informed by societal and payer preferences, subject to:

- Budget constraints
- Available technologies
- Competing priorities

Who's preferences matter?

- Patients, caregivers, household
- Healthcare providers
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- Payers

- **Regulatory decisions influence available technologies (constraint)**
- **Regulators' preferences likely do not play a role in payer decisions**
- **How can other stakeholders' preferences influence payer decisions?**

Stakeholder preferences and reimbursement

- **Payer decisions are primarily budget-allocation decisions**
- **Two approaches to elicit non-payer stakeholder preferences for budget-allocation decisions.**

Who's preferences matter?

- Patients, caregivers, household
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Stakeholder preferences and reimbursement

- **Payer decisions are primarily budget-allocation decisions**
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Who's preferences matter?

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How can relevant preferences be elicited?

- Contingent valuation
- Budget allocation exercises

Contingent Valuation

- **Also known as willingness-to-pay**
- **Direct elicitation of value of a single technology**
 - Multiple elicitation formats
 - Capable of providing individual-level and group-level estimates of value
 - Can be elicited from multiple stakeholder groups
- **Budgets allocated by conducting benefit-cost analysis and maximizing net benefits given a budget constraint**

Budget allocation

- **Also known as point-allocation or person tradeoff**
- **Indirect elicitation of value of individual technologies within a portfolio of options**
 - Multiple elicitation formats
 - Not capable of providing individual-level estimates of value
 - Can be elicited from multiple stakeholder groups
- **Provides a direct estimate of budget-allocation preferences**

Implications

- **For reimbursement**

- Relevance of stakeholder preferences depends on the goal of the system
 - *Maximize individual health outcomes subject to a budget constraint*
 - *Maximize societal health outcomes subject to a budget constraint*
 - *Maximize societal welfare subject to a budget constraint*
- If maximizing health outcomes is the goal...
 - Preferences have less relevance
- If maximizing societal welfare is the goal...
 - Stakeholder preferences matter
 - Otherwise resources may be misallocated result in sub-optimal outcomes