

# Stakeholder Values in Decision-Making

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Presented to the Advisory Committee on Heritable Disorders in  
Newborns and Children

August 1, 2019

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# Factors that Play Into an Important Decision

Competing options



Outcome preference



Uncertainty



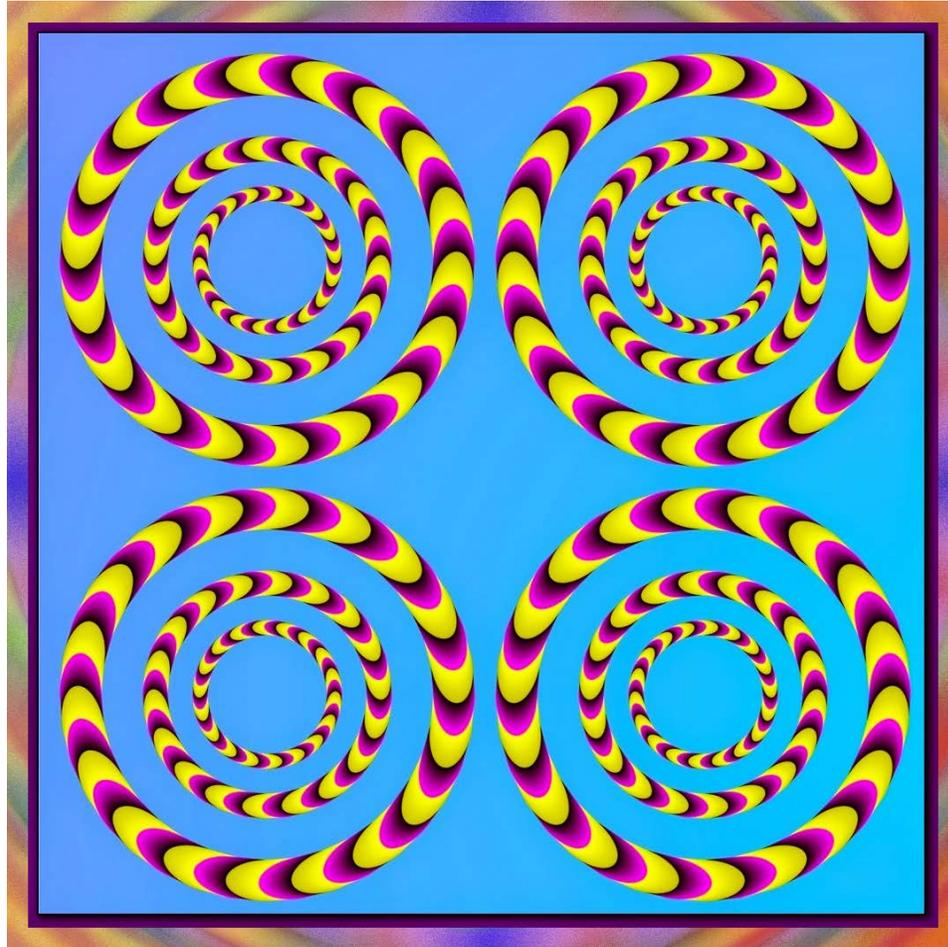
# The Evidence Review Process

- Describes options
  - Newborn screening vs. usual clinical case detection
  - Alternative strategies for newborn screening
- Characterizes outcomes
  - Immediate outcomes of screening
  - Individual level health impact
  - Impact on newborn screening systems
- Outlines uncertainty
  - Range of screening test accuracy
  - Distribution of potential outcomes
  - Gaps in evidence

# Challenges in Newborn Screening Decision-Making

- Competing options
  - The option for newborn screening is within public health, even though it affects individuals and their families
- Outcomes
  - Variation in magnitude of benefits and harms across the population
  - Differences in timing
  - Concerns about equity
  - Regret
- Uncertainty
  - Often significant for both benefits and harms, with insufficient evidence to fully describe
  - Fast-moving advances in screening and treatment
  - Even if the benefit of early detection is unclear, it could potentially help define new treatments or affected individuals might be more likely to benefit from future treatments

# Stakeholder Perspectives



# Values

“Values and preferences is an overarching term that includes ***patients’ perspectives***, beliefs, expectations, and goals for health and life. More precisely, they refer to the process that individuals use in considering the potential benefits, harms, costs, limitations, and inconvenience of the management options in relation to one another. For some, the term “values” has the closest connotation to these processes. For others, the connotation of “preferences” best captures the notion of choice. Thus, we use both words together to convey the concept.”

Andrews JC, Schunemann HJ, Oxman AD, et al. Grade guidelines: 15. Going from evidence to recommendation – determinants of a recommendation’s direction and strength. *J Clin Epidemiol.* 2013;66:726-735.

# “Going from Evidence to Recommendation”

- GRADE has identified
  - Magnitude of estimates of effect on important outcomes
  - Confidence in these estimates
  - Estimates of typical values and preferences
  - Confidence in these estimates
  - Variability of values and preferences
  - Resource use

# Perspectives

- Whose values do we value?
- How do we facilitate the process to understand the values of all stakeholders?

How can values and preferences be assessed?

How can values and preferences be incorporated into the decision-making process?

# Measuring Utility Over Time: The Quality-Adjusted Life Year

- Standardized measurement of health outcome, facilitating comparisons across health conditions and populations
- Calculated as  $\text{Time} \times \text{Utility}$
- Utility ranges from 0 (death) to 1 (perfect health)
- Strategies for measurement include
  - Time trade-off
  - Standard gamble
  - Visual analog scale
  - Conversion from another quality-of-life instrument

# Utility Assessment

- One study recruited parents from pediatric practices, urgent care centers, health fairs, and other events including the Indiana State Fair
- Some findings (5<sup>th</sup>-95<sup>th</sup> percentile) using the standard gamble
  - Mild ADHD – 0.94 (0.72-1.00)
  - Monocular blindness 0.88 (0.50-1.00)
  - Severe bilateral vision loss 0.81 (0.39-1.00)
  - Severe intellectual impairment 0.59 (0.10-1.00)

Carroll AE, Downs SM. Improving decision analysis: Parent preferences (utility values) for pediatric health outcomes. *J Pediatr.* 2009;155:21-25.

# Challenges in Eliciting Utility

- Understanding the health condition
- Situating the perspective
- Considering the role of contextual factors

# Citizens' Jury

- Typically up to 20
- Demographically representative
- Given information and substantial time to deliberate
- Substantial risk of bias if not done properly

Wise J. Citizens' jury for health policy. *BMJ*. 2017;35:7:j2650.

# Application of Citizens' Juries

- Legislative reform of insurance for injury compensation after motor vehicle collisions in Australia
- Taxing soft drinks in Australia
- Ethics of mitochondrial donation using assisted reproductive technology in Australia
- Extent of patient control of their medical records for secondary research in the UK
- Cystic fibrosis carrier screening in Italy
- Bariatric surgery in Australia
- Government funding of adolescent vaccinations in Australia
- Screening for prostate cancer in Italy

# Public Surveys

- More feasible to administer surveys to nationally representative panels
- Can assess preferences using sophisticated approaches used in marketing
- One such study found that amongst adults presented various characteristics related to newborn screening
  - “the impact of newborn screening on treatment success was not associated with the recommendation for or against newborn screening for a profiled condition”
  - Cost was the “most important attribute” and then the age at which treatment would start

Tarini BA, Simon N-J, Payne K, et al. An assessment of public preferences for newborn screening using best-worst scaling. *J Pediatr.* 2018;201:62-68.e1.

# Multicriteria Decision Analysis

- EVIDEM

- Value of an intervention is determined by domains including
  - Need for intervention
  - Comparative outcomes of the intervention
  - Economic consequence of the intervention
  - Knowledge about the intervention
  - Population priorities
- Situated within contextual factors such as alignment with priorities, environmental sustainability, system capacity, and the political, historical, and cultural context

Wagner M, Khoury H, Willet J, Rindress D, Goetghebeur M. Can the EVIDEM framework tackle issues raised by evaluating treatments for rare diseases: analysis of issues and policies, and context-specific adaptation. *Pharmacoeconomics*. 2016;285-301.

# Evidem Framework – Value of the Intervention

- Need for intervention (severity, size of population, unmet needs)
- Comparative outcomes (effectiveness, safety, patient perceived health)
- Type of Benefit (preventive, therapeutic)
- Economic consequences (medical and nonmedical interventions)
- Knowledge about intervention (evidence, expert consensus)
- Scoring has been adapted for rare diseases

[evidem.org](http://evidem.org)

# Applications of Evidem

- Therapeutic interventions
  - Pulmonary arterial hypertension
  - Gastroenteropancreatic neuroendocrine tumors
  - Non-Hodgkin Lymphoma
  - Thyroid cancer
  - Dementia
  - Prader-Willi syndrome
- Prevention
  - Cervical cancer screening (liquid based cytology compared to conventional cytology) in South Africa

# For Discussion

- Why assess values and preferences? How will this information be used in the decision process?
- Who are the stakeholders?
- What values and preferences are needed to facilitate the decision-making process? What are the key points that need this assessment?
- How can the relevant values and preferences be elicited?
- When in the review process should values and preferences be elicited?

# Summary and Next Steps