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ACCESSIBILITY TO HIGH-VALUE MEDICINES

THE NEW FRONTIER?

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Social Cost Value Analysis

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Notes on “Social Willingness to Pay”

The Case for an Alternative Method for the Valuation of Health and Health Care

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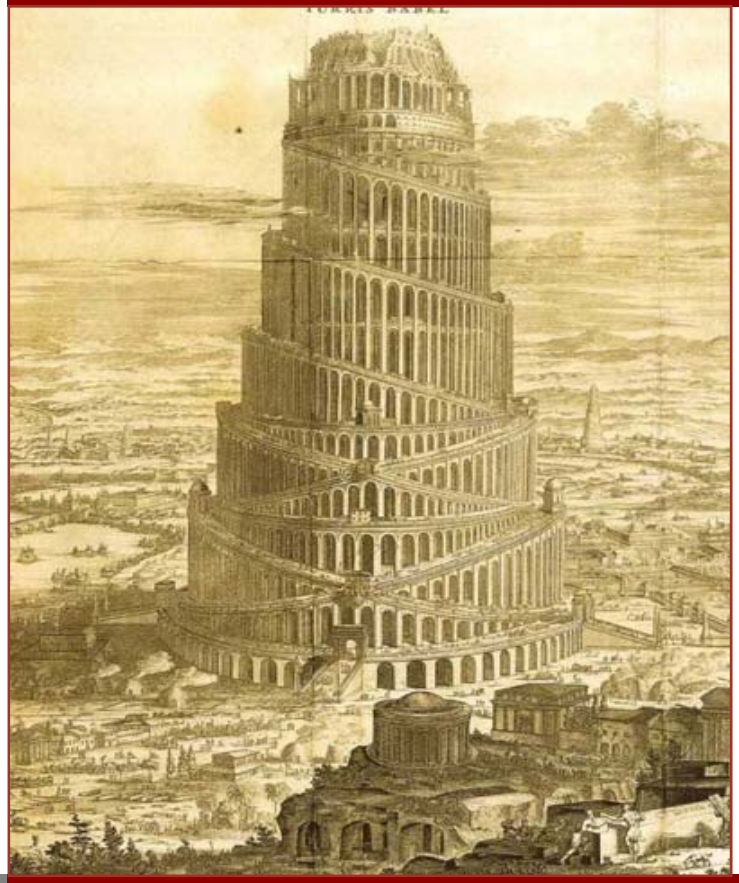
dkfz.



"Values Talk" - A Tower of Babel[1]

- Referral to many different and often incommensurate things…
- **A key paradox**:

  The discourse about values is both very important and very ambiguous.
- Stakeholders may be tempted to react to this problem with either

  reductionism
  (focusing on one particular definition of values to the neglect of other relevant types)

  or

  nihilism…
  (either rejecting all values analyses as equally unreliable, or accepting all as equally credible)

[1]based on a Canadian policy analysis by Mita Giacomini et al. (2004)

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Cost-Benefit Analysis (CBA)

$$B_i > C_i$$

$$NSB_i > 0$$

$$NSB_i = B_i - C_i$$

$$NSB_i = \sum_{t=1}^{n} \frac{B_i(t) - C_i(t)}{(1+r)^{t-1}}$$

- The primary goal of CBA is to identify projects where NSB > 0.
- For allocation within a fixed budget, projects would be ranked according to their NSB.

NSB, Net Social Benefit; I = 1, …, I, number of possible investments (programs); $B_i(t)$, benefits (in money terms) derived in year t; $C_i(t)$, costs (in money terms) in year *t*; *r*, annual interest rate; *n*, life time of project in years.

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From CBA to Cost-Effectiveness Analysis (CEA)

CBA

$$B_1 > C_1$$

B, benefit
C, (opportunity) cost

$$P_1 \bullet E_1 > C_1$$

P, price (valuation) of effect
E, effect

$$\frac{P_1 \bullet E_1}{C_1} > 1$$

Note that this excludes all potential sources of value other than those captured in the definition of "effect."

$$\frac{P_1 \bullet (\Delta)E_1}{(\Delta)C_1} > \frac{P_2 \bullet (\Delta)E_2}{(\Delta)C_2}$$

Alternative formulation, introducing a **budget constraint** which limits how much costs can be expended.

CEA

$$\frac{\Delta E_1}{\Delta C_1} > \frac{\Delta E_2}{\Delta C_2}$$

Eliminating the pricing of effects, thus **introducing the requirement of $P_1 = P_2$** (which is considered valid in a CEA since one is comparing a common effect E with the two interventions[1]).

Thus, *formally* CEA can be regarded as a special type of CBA under restrictive assumptions: 1. a single effect must be the outcome of interest, and 2. this effect must be exactly the same for both interventions.

Note implied linearity of "price" / WTP.

[1]Adapted from R.J. Brent (2003); note that this formal treatment is simplifying the differences between CBA, CEA, and CUA, for example with regard to the issue whose preferences (/WTP) should count.

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From CEA to Cost-Utility Analysis (CUA)

CEA

$$\frac{\Delta E_1}{\Delta C_1} > \frac{\Delta E_2}{\Delta C_2}$$

$$\frac{\Delta C_1}{\Delta E_1} < \frac{\Delta C_2}{\Delta E_2}$$

CUA

$$\frac{\Delta C_1}{\Delta QALY_1} < \frac{\Delta C_2}{\Delta QALY_2}$$

$$\frac{\Delta C_1}{\Delta QALY_1} \leq ICER\_Threshold$$

CMA

$$C_1 < C_2$$

Formally CEA can be regarded as a special type of CBA under restrictive assumptions: 1. a single effect must be the outcome of interest, and 2. this effect must be exactly the same for both interventions.

Note implied linearity of "price" / WTP.

If we want to compare entirely different effects (as with headache pain relief and the precision of a diagnostic test), and if we do not want to use prices explicitly, then all effects need to be converted into a common unit. This is usually the QALY.

Thus (CUA) is a restricted version of CEA (and thus of CBA), adding **E = QALY** for each intervention, in addition to $\mathbf{P_1 = P_2 = P}$, with P now relating to the **price of a QALY**.

In cost-minimization analysis (CMA), consequences play no part in the evaluation as they are assumed to be identical: $E_1 = E_2$.

Note: Unless consequences are identical across interventions, a CMA would not constitute a valid evaluation of these interventions.

[1]Adapted from R.J. Brent (2003); note that this formal treatment is simplifying the differences between CBA, CEA, and CUA, for example with regard to the issue whose preferences (/WTP) should count.

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From CUA to [Health-Related] Social "Utility"

$$U = f(H, W, \ldots)$$

$$U = f(H) + f(W, \ldots)$$

"The **principal objective** of the **National Health Service** ought to be to maximize the aggregate improvement in the health status of the whole community."[1]

$$QALYs = \sum_{h=1}^{n} u_h \times t_h$$

$$QALYs = \sum_{t=1}^{n} \frac{u_t}{(1+r)^{t-1}}$$

$$Social\_Health\_Gain = m \times \sum_{t=1}^{n} \frac{\Delta u_t}{(1+r)^{t-1}}$$

$$ICER = \frac{c_A - c_B}{e_A - e_B} = \frac{\Delta costs}{\Delta effects} = \frac{\Delta costs}{\Delta QALYs}$$

Usual HTA Perspective:

- incremental cost ***per patient***
- health insurance or NHS perspective
  [sometimes incl. social insurance / PSS / …; controversial: caregiving / productivity loss]
- incremental gain in ***individual*** "utility"
  (health-related quality of life x length of life)

$$ICER = \frac{\Delta C}{\Delta E} = \frac{\Delta C}{\Delta QALY} < \lambda$$

"The Silence of the Lambda"[2]

"Information Created to Evade Reality"[3]

[1] A.J. Culyer (1997); also M.C. Weinstein and W.B. Stason (1977): "*The underlying premise of CEA in health problems is that for any given level of resources available, society (or the decision-making jurisdiction involved) wishes to maximize the total aggregate health benefit conferred.*"
[2] A. Gafni, S. Birch (2006)
[3] S. Birch, A. Gafni (2006)

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Increasing Uneasiness with Thresholds

HTA Agencies

- NICE (England): end-of-life treatments, ultra-orphans
- TLV (Sweden): adjustments for severity

Research-Based Biopharmaceutical Industry

- Barriers to access
- Innovation (dealing with uncertainty and dynamic efficiency)

Payers

- NHS England: Cancer Drugs Fund
- A "prescription for uncontrolled growth in expenditures"[1]?

Academics

- Increasing literature on the importance of "other criteria"
- Scientific foundations of actual benchmarks for cost effectiveness: might be too high[2] / too low[3] / non-existent[4]?

[1]A. Gafni, S. Birch (1993)
[2]K. Claxton et al. (2013)
[3]M. Schlander et al. (2017)
[4]when social preferences are taken into account

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Valuation of Health: A Framing Issue?

1. **Use value** (consumer perspective)
2. **Option value** (due to uncertainty and risk averse citizens)
3. **Externalities** (caring externalities and altruistic behaviors)

Perspective on incremental costs and WTP:

1. direct out-of-pocket payments
2. private (voluntary) health insurance premiums
3. public (compulsory) health insurance premiums (or tax)

$$WTP_{direct\_oop} \leq WTP_{private\_ins} \leq WTP_{public\_tax}$$

¬ But can we expect this additive relationship to be (always) true?[1]

[1]cf. D. Gyrd-Hansen (2013)

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Key Elements of the Conventional Logic

Use value: Quality-Adjusted Life Years (QALYs)

- (fully) capture the value of health care interventions;
- are all created equal ("a QALY is a QALY is a QALY…").

Aggregation: Maximizing the number of QALYs produced

- ought to be the primary objective of collectively financed health schemes,
- leading to the concept of thresholds (or benchmarks) for the maximum allowed cost per QALY gained.

Decreasing cost per QALY

- implies increasing social desirability of an intervention.

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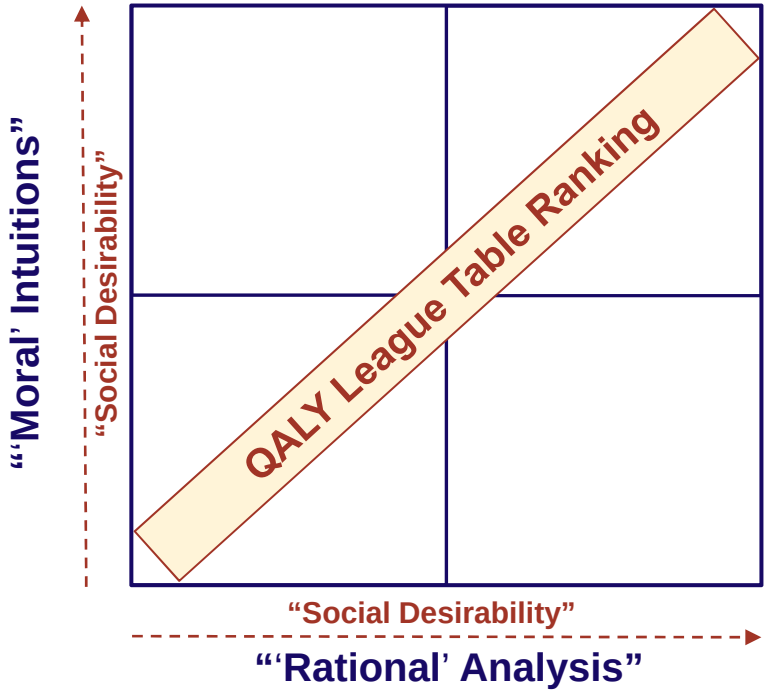
Reflective Equilibrium I



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Textbook Example: "QALY League Table"[1]

Ranking [original]	**Intervention** [abbreviated; comparator not stated in original table]	**Cost / QALY [£ (1990)]**
3	G.p. advice to stop smoking	£ 270
5	Antihypertensive therapy to prevent stroke	£ 940
6	Pacemaker implantation	£ 1,100
7	Valve replacement for aortic stenosis	£ 1,140
8	Hip replacement	£ 1,180
9	Cholesterol testing and treatment	£ 1,480
11	Kidney transplant	£ 4,710
12	Breast cancer screening	£ 5,780
15	Home hemodialysis	£ 17,260
18	Hospital hemodialysis	£ 21,970
20	Neurosurgery for malignant intracranial tumors	£ 107,780
21	Epoetin alfa therapy for anemia in dialysis patients	£ 126,290

[1]A. Maynard. *Economic Journal* 1991; 101 (408): 1277-1286

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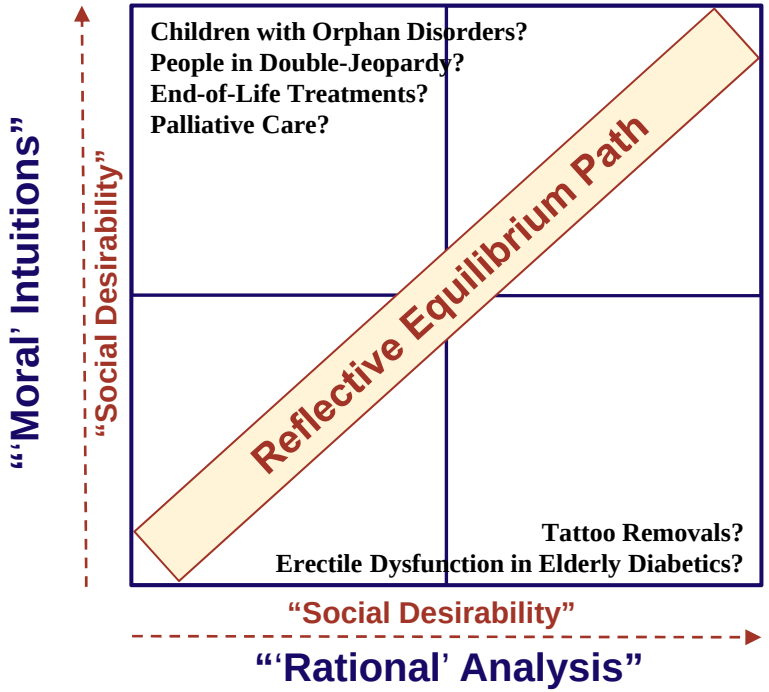
Reflective Equilibrium II



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Loopholes of the Conventional Logic

Effectiveness and Efficiency

Need to justify the appropriateness of the chosen effectiveness criterion

- by definition, "efficiency" is a secondary or instrumental objective,
- whereas the "effectiveness" criterion invariably represents the primary objective.

Efficiency

Need to distinguish explicitly between

- technical efficiency, productive efficiency, and allocative efficiency;
- static and dynamic efficiency.

Social Value ("Utility")

Existence of

- components different from individual utility and its aggregation;
- social (and non-selfish) preferences; rights and duties.

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Multi-Criteria Decision Analysis (MCDA)

There are many **definitions** of Health Technology Assessment (HTA).

Some Commonalities:

- A Multidisciplinary Endeavor:
  Clinical Medicine, Epidemiology, [Health] Economics, „*Policy Makers*"
- Systematic Evaluation of **Evidence of Clinical Benefit**
  of medical interventions and clinical strategies

Some Differences:

- Systematic Inclusion of Costs (…)
  of medical interventions and clinical strategies
- Types and Roles of **Economic Evaluation**

All definitions have in common that HTA (by definition) represents a variant of multi-criteria decision making.

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Multi-Criteria Decision Analysis (MCDA)

There are many **methods** for Multi-Criteria Decision-Making.

Some Strengths:

- Integration of multiple (sometimes conflicting) objectives
- Decomposing complex decision problems
- Comprising a broad set of methodological approaches
- Building on many disciplines
  (incl. operations research, decision sciences, economics, psychology, …)

Some Problems:

- It is doubtful if any identification of the "best" MCDA method can be performed
- Appropriate consideration of opportunity cost?

Some Commonalities:

All need to be informed by

- criteria,
- weights,
- and ranking principles.

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Economic Literature: Preferences for Health

Contingent Valuation (CV) of Health[1]

- Smith and Sach identified 265 CV Studies
  (published from 1985 – 2005):
  - Focus on **Use Value** of Health only, 73%
  - Focus also on **Option Value**, 13%
  - Focus also on **Externalities**, 5%
  - Focus including **Option Value and Externalities**, 9%

- Arguably, **Option Value** and **Externalities** will be most important when access to high technology and/or high cost interventions is at stake – *i.e., in practice, when most*
- **Health Technology Assessment**s *(HTAs) are conducted*

[1]cf. R.D. Smith, T.C. Sach, *Health Economics, Policy and Law* 2010; 5: 91-111.

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A Rapidly Growing Economic Literature

on a Broad Range of Characteristics[1]

contributing to **Social Value Judgments**, such as

- Attributes of the Health Condition
  - individual valuation of health conditions
  - severity of the condition
  - unmet medical need
  - urgency of an intervention
  - capacity to benefit from an intervention
- Attributes of the Persons Afflicted
  - non-discrimination (and claims-based approaches)
  - age (and fair innings)
  - other patient attributes
  - fairness objectives; aversion against *all-or-nothing* decisions

[1]cf., for example, M. Schlander, S. Garattini, S. Holm, et al., *Journal of Comparative Effectives Research* 2014; 3 (4): 399-422.

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Social Preferences in the Economic Literature:

An Early Intuition…

"The taste for improving the health of others appears to be stronger than for improving other aspects of their welfare."[1]

[1]Kenneth Arrow (1921-2017)
Uncertainty and the Welfare Economics of Medical Care (1963; p. 954)

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Research Need: "Social Preferences"

- many studies of social preferences …
  - most of them small
  - many studies limited in scope
  - many studies likely to be impaired by framing effects
  - other study types (not choice-based experiments)
  - some studies of questionable methodology
- … very difficult to generalize
  - severity probably best documented contextual variable
  - distinct difficulties to quantify effects observed
  - if measures of willingness-to-pay were incorporated, they typically reflected maximal individual WTP
  - social willingness-to-pay in exchange for health care programs covered under a collectively financed health scheme might be more relevant

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Research Need: "Social Preferences"

Studies addressing social preferences (externalities)

- caring externalities
- altruism ("warm glow", sympathy, or moral constraints?)

Some more limitations (many studies)

- zero sum assumption
- level of information offered
- cognitive overload use of simple heuristics …
  - dominant attributes
  - lexicographic rankings in some CV / DCE studies
  - "Thinking Fast" *versus* "Thinking Slow"
- unstable preferences

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ESPM Project: Research Objectives

1. To investigate systematically how the general public valuates selected characteristics ("attributes") of health care interventions,
   - ¬ and how they weigh them against each other (including their interaction).
2. To compare the valuation results obtained in the study with those based on the logic of cost effectiveness by means of a utility comparator.
3. To assess the sensitivity of weights to the level of information offered to respondents and to potential framing effects.
4. (in Phase II:) To identify international similarities and differences with regard to the valuation of the attributes tested.
5. (in Phase II:) to explore the agreement of respondents between their choices in the experimental setting, their policy implications, and their policy preferences.

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ESPM Project Governance:

Scientific Steering Committee

- **Silvio Garattini** (Mario Negri Institute, Milan / Italy)
- **Sören Holm** (U of Manchester / England)
- **Peter Kolominsky** (U of Erlangen / Germany)
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- **Keith Tolley** (Tolley HE, Buxton / England)
- **Mondher Toumi** (U of Lyon / France)

*Scientific Project Leaders.

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ESPM Project:

Attributes Investigated

1. **Severity** of the initial health state: lost life expectancy (i.e., *ex ante*, before / without an intervention)
2. **Severity** of the initial health state: lost quality of life (i.e., *ex ante*, before / without an intervention)
3. **Effectiveness** of an intervention: life expectancy gained
4. **Effectiveness** of an intervention: quality of life gained
5. **Age** of patients (or "fair innings")
6. **Rarity** of disorder (i.e., prevalence or number of persons benefitting)
7. **Cost** of intervention: perspective of a compulsory health scheme ("OKP"); payment vehicle = social willingness-to-pay

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ESPM Project: Design Elements

1. **Representative population sample**
   - 1,501 respondents from Switzerland in Study Phase I
2. **Discrete Choice Experiment (DCE) design**
3. **Initial Preference Formation Phase**
   - prior to DCE experiment
4. **Testing for framing effects** (by randomization):
   - different levels of information on implications of rarity
   - information on cost per patient (either provided or withheld)
5. **Perspective on costs**:
   - incremental compulsory health insurance premiums
6. **Utility comparator** (with generic health state descriptions)
7. **Econometric evaluation**
   - interaction of attributes; subsamples, latent class, random coefficient models

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From CUA to MCDA and SCVA

SCVA: Social Cost Value Analysis

- **Social WTP**
  capturing the will to share health care resources[1]
  (option value and externalities)

Potential attributes influencing the will to share may include

- **severity** of the initial health state
- certain **patient attributes**
- a strong dislike for "**all-or-nothing**" resource allocation decisions
- **rights**-based considerations

[1] cf. J. Richardson et al. (2012; 2017)

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SCVA: How Different is it from CUA?

Moving from CUA to SCVA would be of little consequence, if and when

- the QALY calculation algorithm offered an adequate proxy for individual [health-related] utility gains,
  - including the transformation of length and quality of life inherent in the QALY model and further assumptions,
- individual [health-related] utility gains mapped into social [health-related] utility gains,
- citizens were not risk averse,
- citizens had little (if any) consideration for others,
  - which would eliminate any non-selfish preferences (for sharing health care resources),
- citizens' WTP was proportional to the number of patients benefitting from the adoption of a health care program.

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SCVA: A Changing Perspective

shifting the focus
from cost per patient to cost at program level

- A **decision-makers**' (and payers') perspective
  has been traditionally overall **budgetary impact** (*transfer cost*)

- A **social value** perspective

  (instead of a narrow focus on QALYs as a proxy for individual health-related "utility" and their aggregation) corresponds to social **opportunity cost** (or [social] value foregone) being reflected by net budgetary impact (*transfer cost*)

- This reflects the type of decisions informed by HTAs,
  i.e., decisions on the adoption of health technologies
  at the level of programs (*not* at the level of individual patients)

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Thank You for Your Attention!

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