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# MCDA in the Real World: case studies in Europe and Americas

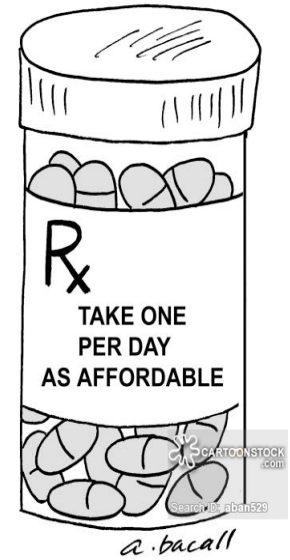
Vladimir Zah PhD

CEE Network Chair 2015-17

ISPOR Health Science Policy Council member



# HEOR Evolution



International  
Reference  
Pricing  
(IRP/ERP)

- Based on other countries, what is the maximum price that can be charged in my country

HTA

- Cost Effectiveness / Budget Impact Decision Analysis

VBA / MEA

- (CE- / BIM+ | CE+ / BIM- | CE- / BIM-)  
Confidentially create lowest based price either by health outcome or non-outcome

MCDAs in Late Stage  
Oncology / Rare  
Diseases

- Transparent Decision Making (not cost-effective/negative BIM)

Hospital  
Tenders  
MCDAs



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# MCDA – Oncology & Rare Diseases

How did we get here?

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"We're looking for someone with the wisdom of a 50-year-old, the experience of a 40-year-old, the drive of a 30-year-old and the pay scale of a 20-year-old."

# MCDA – Late Stage Oncology & Rare Diseases



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Healthcare MCDA  
Decisions  
(Patient perspective)

- 1) Immediate Cure
- 2) Most Effective
- 3) Most Expensive
- 4) No Side Effects
- 5) Unmet Need fulfilled (rare diseases)
- 6) Always Available
- 7) No Risk of Death
- 8) No Disease Recurrence
- 9) No Waiting
- 10) No Responsibility  
(Adherence/Compliance)
- 11) No Life Style Change

# MCDA – Late Stage Oncology & Rare Diseases



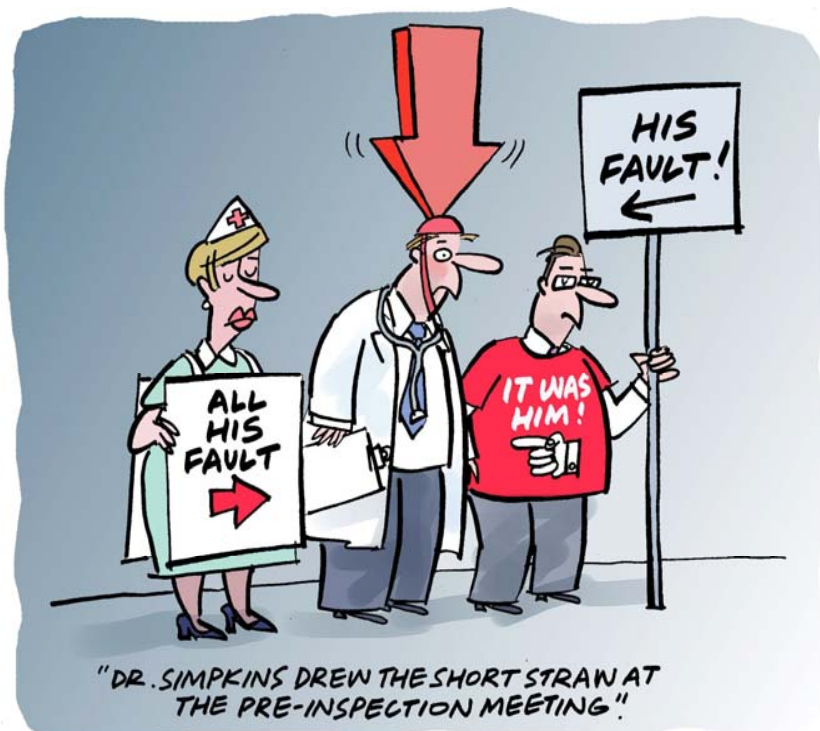
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## 7 P's MCDA Panel mix – Vlad's Inclusion Rule



- Payer
- Patient
- Pharma
- Physician
- Politician
- Pharmacist
- Public (General Public representative in case of NIF/MoH)





## MCDA Steps

- **Identify criteria (consultation with stakeholders)**
- **Identify criteria weights (inter-criteria comparison) through responders (7P's)**
- **Introduce alternatives (comparators)**
- **Get Responses (7P's)**
- **Results: ranking among alternatives (comparators)**

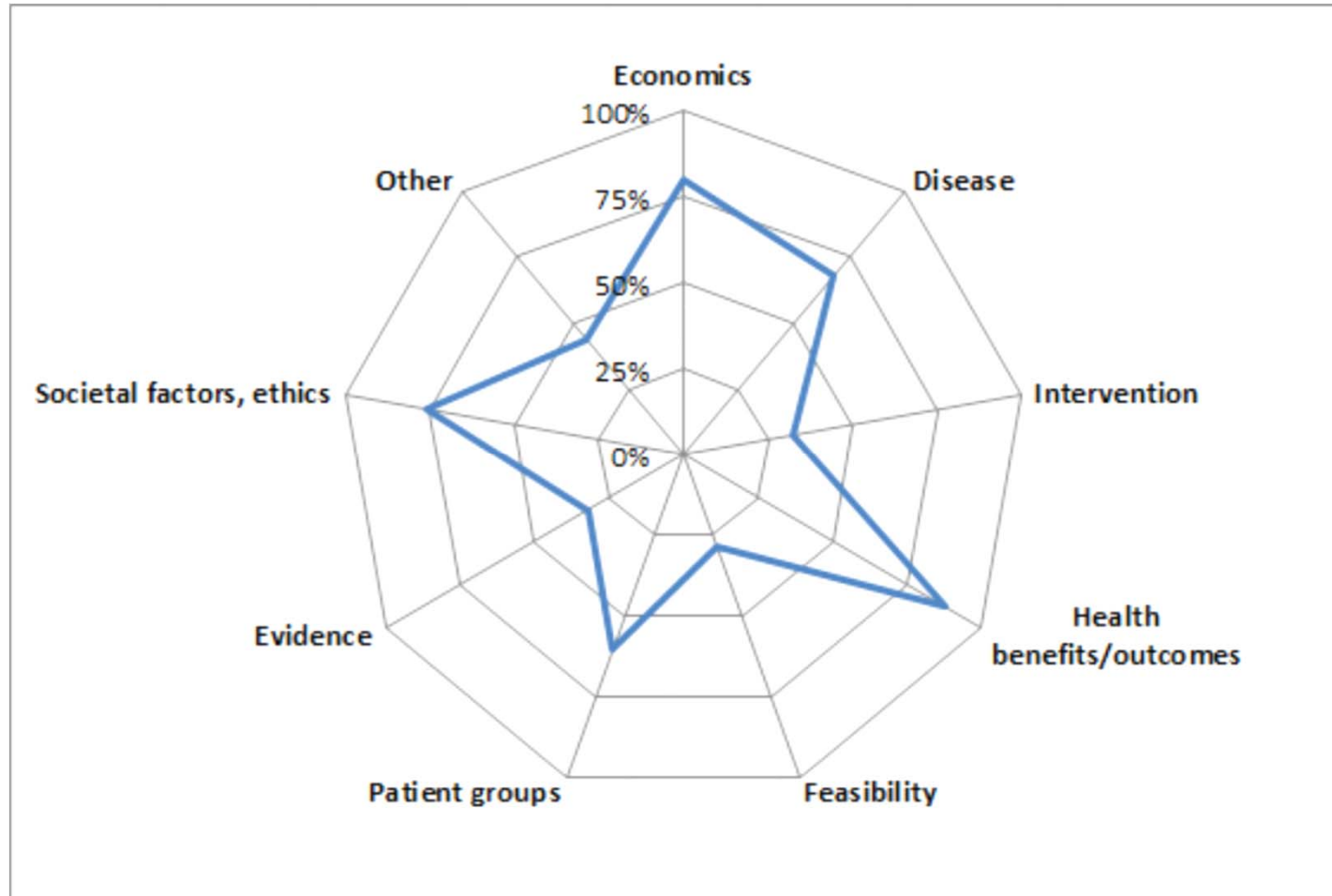




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# Identify criteria



Source: Piniashko O., Nemeth B. An analysis of the criteria used in existing or proposed MCDA models. *ISPOR 21<sup>st</sup> Annual International Meeting Research Abstracts*, May 21-25, 2016, Washington, DC, USA. *Value in Health*. 2016. 19(3): A106.

# Identify criteria weights

## Data Collection Software utilized



ZRx Multiple Criteria Decision Making Tool

ENTER ►

### ZRx Outcomes Research

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# MCDA Methodology

METHODS utilized for the criteria assessment:

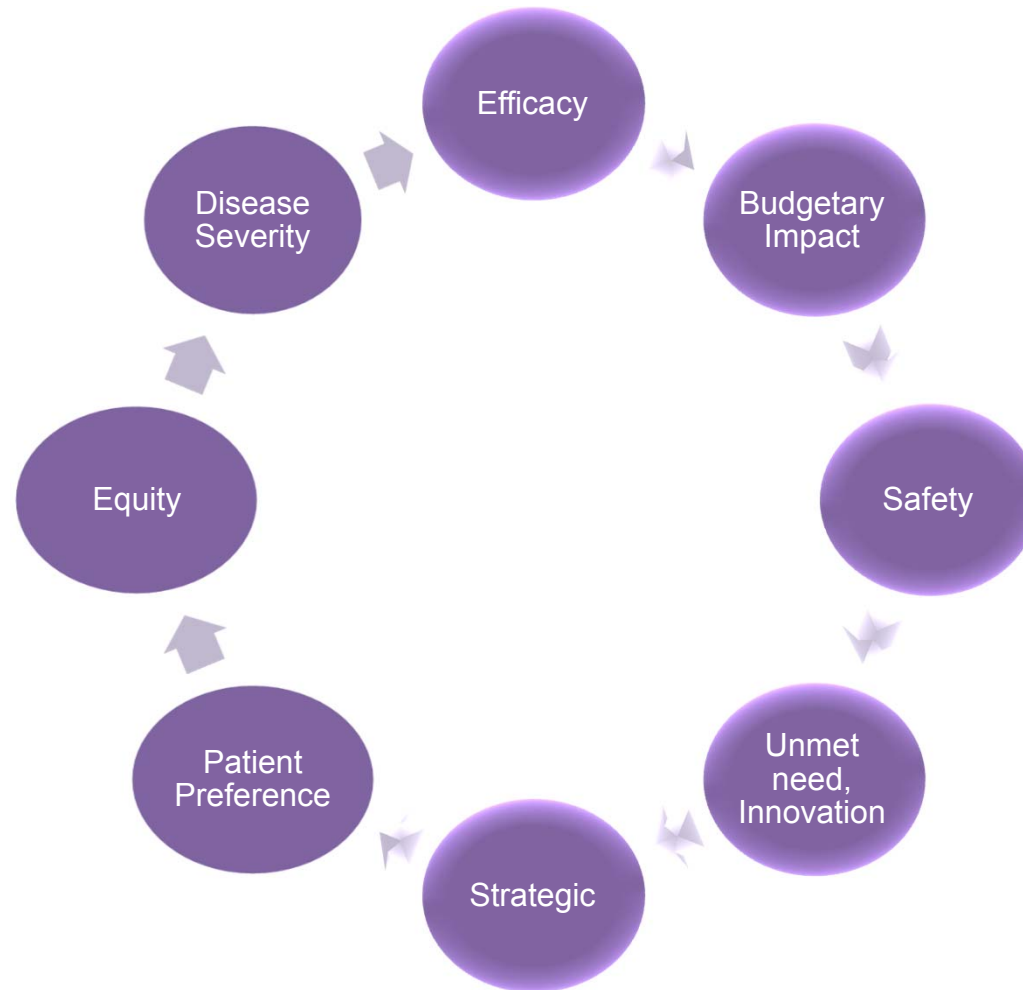
- In applying MCDM methods users are initially required **to define the decision problem**, including the objectives of the analysis and the alternatives being compared. In this study, from each country, three representatives of each of the seven stakeholder groups contributed to the criteria identification. The performance criteria on which to compare the alternatives was defined.
- The list of criteria should be as comprehensive as possible and each criteria as defined should be independent.
- Criteria were ranked and subsequently weighted by the committee according to their relative importance to the overall decision.
- Finally, how to measure the performance of each of the criteria was defined. **Direct rating methods using a visual analogue scale (VAS) were chosen.** For the purpose of informing the performance measures (or ratings), **decision makers were provided with explanation of criteria descriptors**, which they could combine with local experience.
- **The ratings were collected via an online survey.**



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# RARE DISEASE CRITERIA CEE Region



# MCDA – Late Stage Oncology & Rare Diseases



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## RARE DISEASE CRITERIA DESCRIPTION

- 1. Efficacy:** Evidence on clinical efficacy: Good quality evidence with a low degree of statistical uncertainty and methodological limitations. It is possible to determine the actual clinical value according to relevant, validated clinical outcomes.  
*(-Very low-Low-Moderate-High-Very high) <options>*
- 2. Budgetary Impact:** What is the budget impact of this NEW drug vs. available medication, if any?  
(-Average annual treatment cost per patient, -Target population - number of patients, and -Is the inclusion of this drug sustainable from Insurance system perspective?)  
*(-Significantly higher-Moderately higher-No difference-Moderately lower-Significantly lower)*
- 3. Safety:** What is the safety profile (side effects and adverse effect) of this NEW technology vs. standard of care? (benefits of the drug exceeds its risks, while preserving appropriate standards for safety, especially when these patients have unmet needs - \* same ethical and safety standards apply to rare and common disease drugs)  
*(Much worse safety profile-Somewhat worse safety profile-The same safety profile as standard of care-Somewhat better safety profile-Much better safety profile)*
- 4. Unmet Need/Innovation:** Potential of the drugs to address unmet medical needs  
-Is there any available medication? -To what extent patients receive provision in relation to their needs?  
*(Not at all influential-Slightly influential-Somewhat influential-Very influential-Extremely influential)*

# MCDA – Late Stage Oncology & Rare Diseases



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## RARE DISEASE CRITERIA DESCRIPTION

5. **Strategic:** How high is the political pressure for reimbursement?  
Political pressure to ensure that patients have access to high quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs  
*(Major negative implications-Moderate negative implications-Neutral-Moderate positive implications-Major positive implications)*
  
6. **Patient Preference:** What is the patient preference towards treatment in this rare disease?  
Whether a general societal preference for prioritizing treatment of this particular rare disease over common ones exists and could provide a justification for accepting higher cost for orphan drugs?  
(It is difficult to assess choice alternatives with no prior experience.)  
*(Not preferred at all-Slightly preferred-Moderately preferred-Very much preferred-Extremely preferred)*
  
7. **Equity:** Equity of access  
Does the illness or required care justify a claim for solidarity, given the context in society?  
(Societal considerations that may matter to the principle of social solidarity in which vulnerable groups receive support; that orphan drugs tend to target life-threatening diseases with no alternative therapy and that they have considerable impact on patients' health care expenditures)  
*(Significant decrease in equity-Minor decrease in equity-No improvement in equity-Minor improvement in equity-Significant improvement in equity)*
  
8. **Disease Severity:** How severe is the illness or the required care from societal perspective?  
(in relation to the disease's clinical characteristics (e.g., shortened lifespan or sensory impairment) and severity ratings of the individual characteristics)  
*(Less importance-Mild-Moderate-Severe-Profound)*

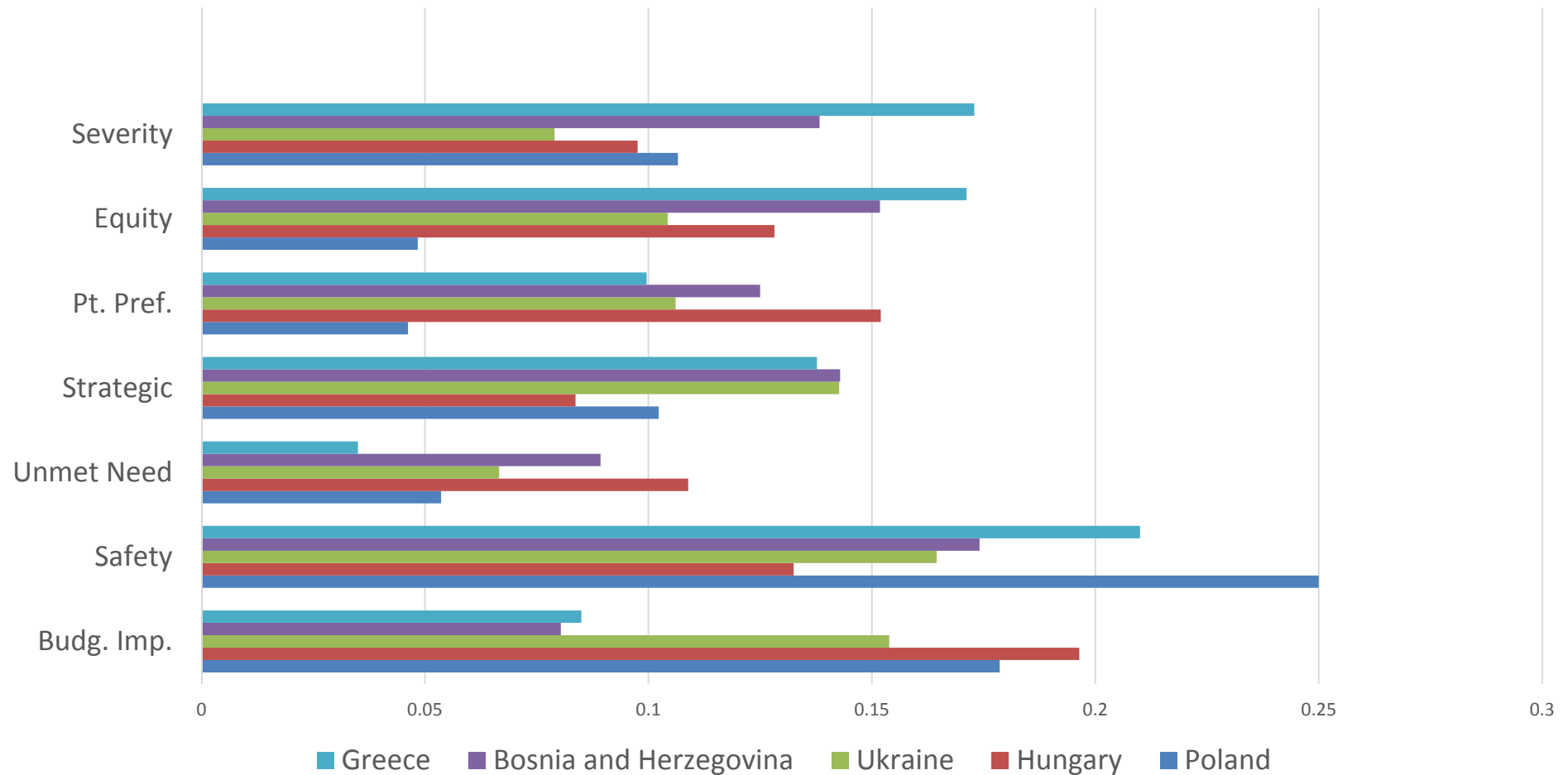


# RARE DISEASES INTER-CRITERIA COMPARISONS (Trade-off analyses)

Greece, Poland, Hungary, Ukraine, Czech  
Republic & Bosnia and Herzegovina project



### Physicians on all criteria for RD



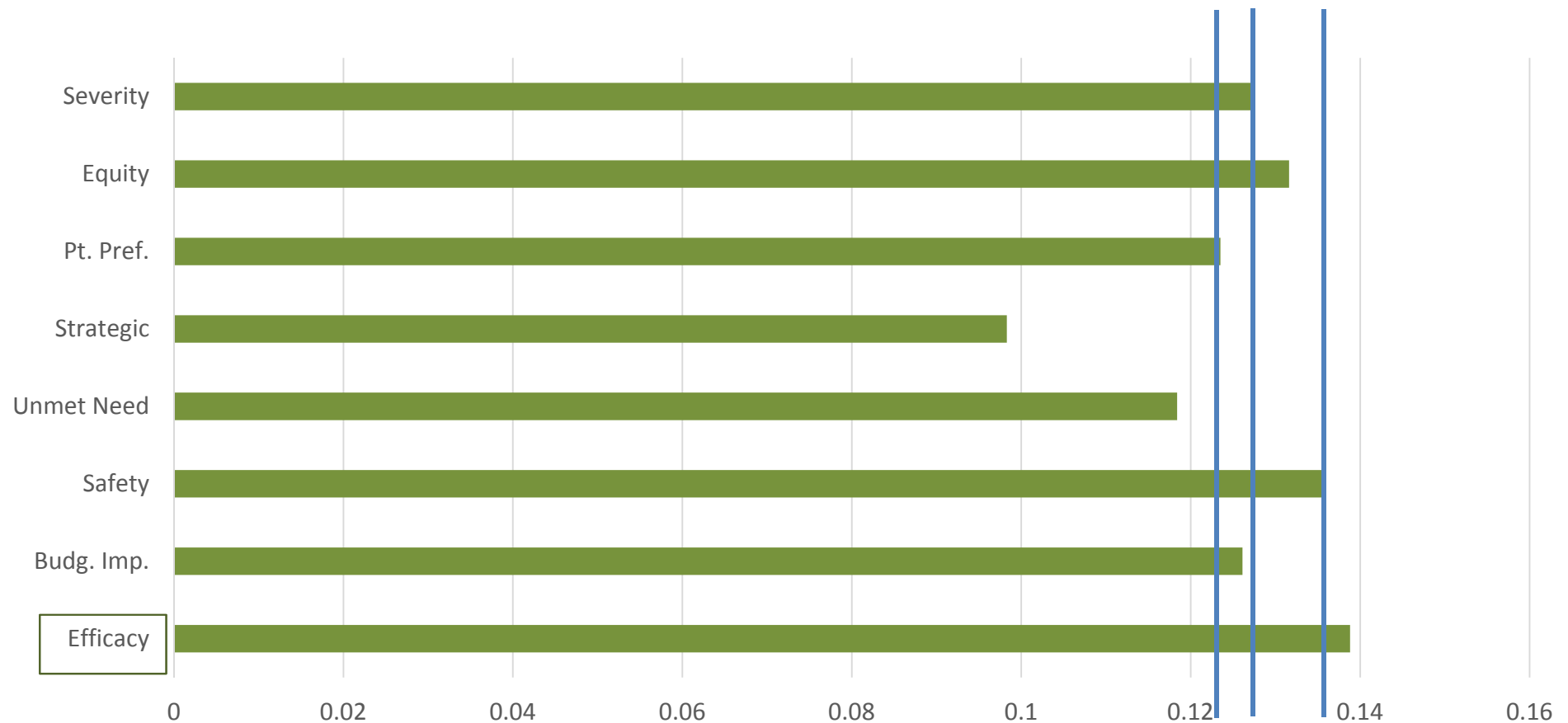




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## Median on all criteria from all representatives from all included countries for RD



# MCDA –Oncology



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## LATE STAGE ONCOLOGY CRITERIA





# MCDA – Late Stage Oncology & Rare Diseases

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## LATE STAGE ONCOLOGY CRITERIA DESCRIPTION

- 1. Efficacy:** Is this NEW technology superior to standard of care and by how much?  
*(Much lower –Lower- About the same as standard of care – Higher – Much higher)*
- 2. Budgetary Impact:** What is the budget impact of this NEW technology vs. standard of care, with the same number of patients treated? Is the inclusion of this drug sustainable from Insurance system perspective?  
*(Significantly higher –Moderately higher –No difference –Moderately lower –Significantly lower)*
- 3. Safety:** What is the safety profile (side effects and adverse effect) of this NEW technology vs. standard of care? (benefits of the drug exceeds its risks, while preserving appropriate standards for safety, especially when these patients have unmet needs - \* same ethical and safety standards apply to rare and common disease drugs)  
*(Percentage: 0%– 100% )*
- 4. Unmet Need/Innovation:** To what extent patients receive provision in relation to their needs in the therapeutic area of NEW technology? Potential of the drugs to address unmet medical needs? Is there any available medication?  
*(Far below needs –Below needs –Met expectations –Influential –Extremely Influential)*

# MCDA – Late Stage Oncology & Rare Diseases



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## LATE STAGE ONCOLOGY CRITERIA DESCRIPTION

- 5. Strategic:** What are strategic/policy implications of reimbursement of NEW technology vs. standard of care? Political pressure to ensure that patients have access to high quality care, including effective drugs. (- Major negative implications –Moderate negative implications –Neutral –Moderate positive implications, - Major positive implications)
- 6. Patient Preference:** What is the patient preference towards this NEW technology vs. standard of care? Essential for obtaining values or weights indicating patients' trade-off preferences for health outcomes, health-care processes and treatment convenience features. (-Not preferred at all –Slightly preferred – Moderately preferred –Very much preferred –Extremely preferred)

~~Equity~~

~~Disease Severity~~

~~Cost Effectiveness~~



# LATE STAGE ONCOLOGY INTER-CRITERIA COMPARISONS (Trade-off analyses)

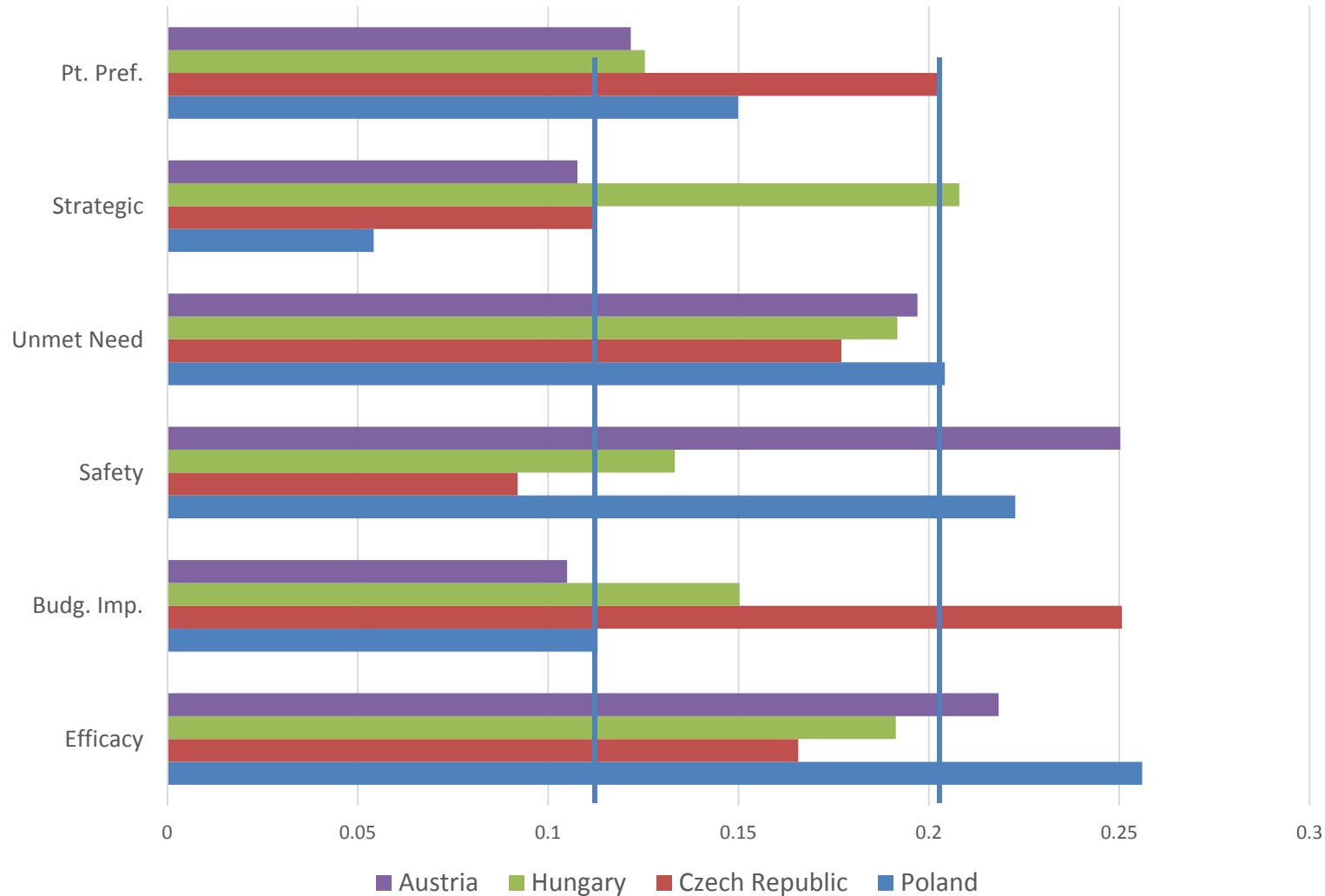
## Austria, Poland, Hungary & Ukraine project



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## CEE NETWORK

Pharma on all criteria for oncology



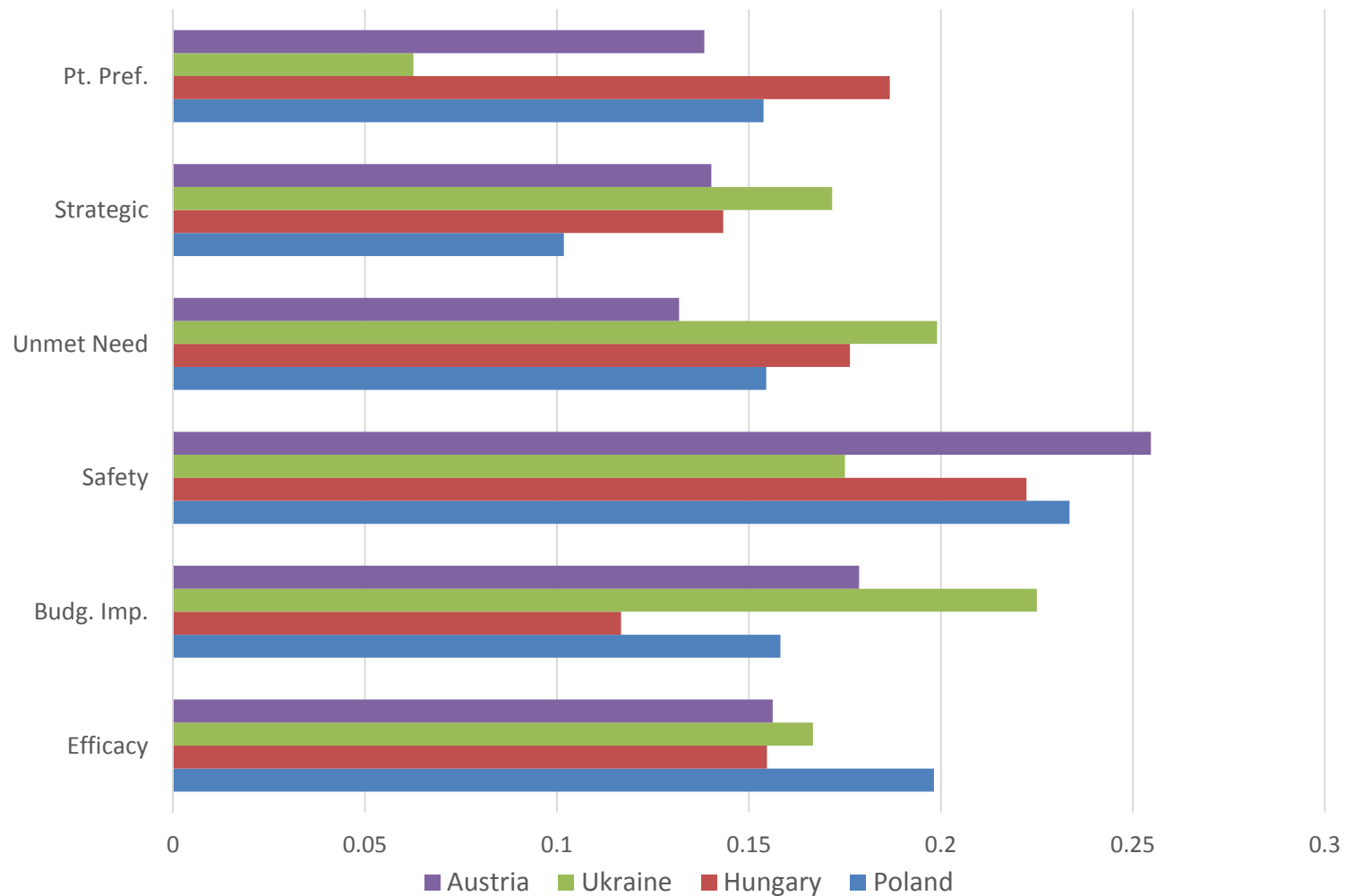




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## CEE NETWORK

Payers on all criteria for oncology

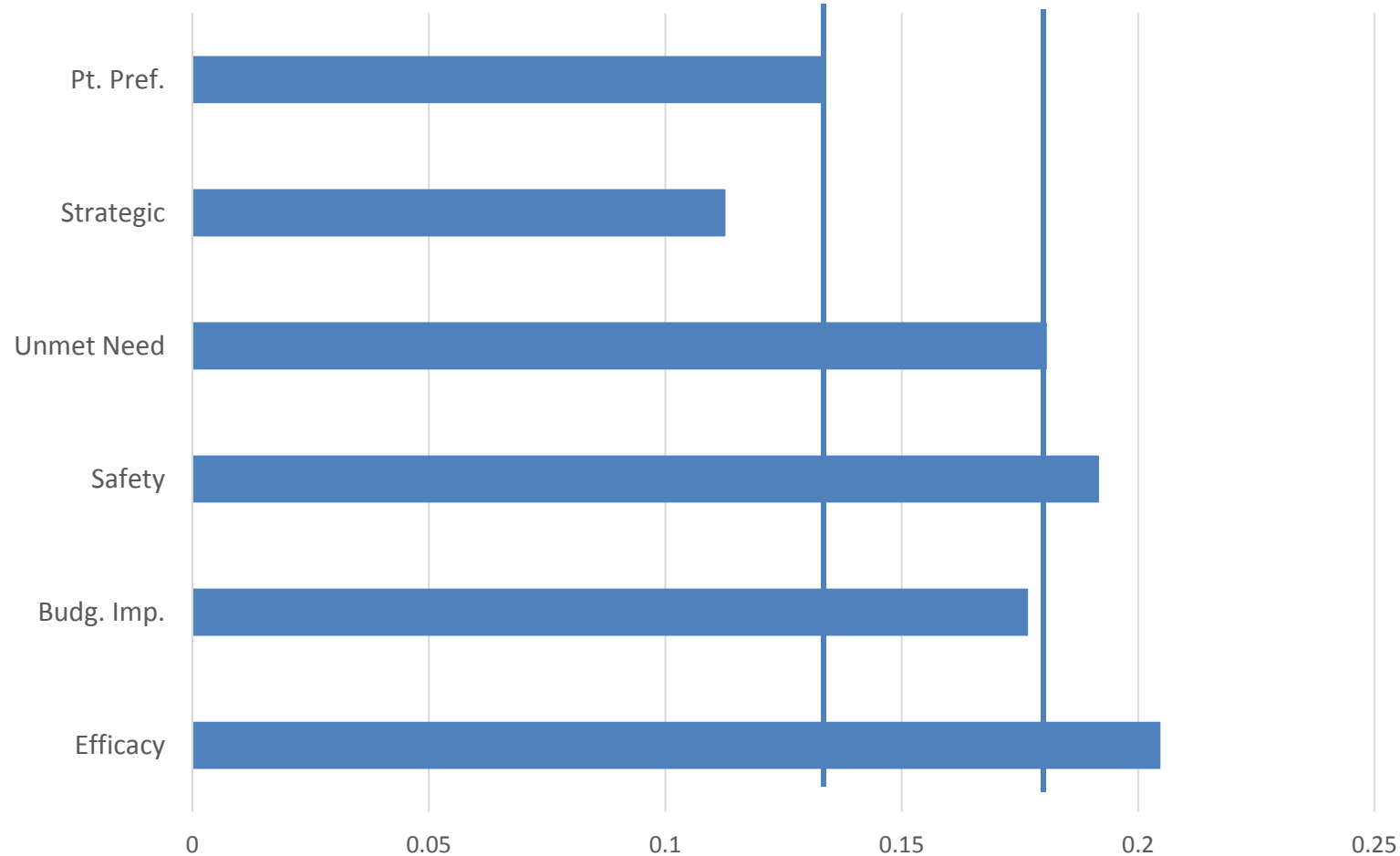




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### Median on all criteria from all representatives from all included countries for oncology





## NEXT STEPS

1. Populate alternatives
2. Run SLAM – Simple Linear Additive Model
3. Run MAVT – Multi Attribute Value Theory  
(adjust weights on responses)

**ZRx MCDM** ← Log Out MODEL HURWICZ SLAM MAVT ELECTRE AHP

Model Save Load Reset Help

Outcomes Research Function Results VIP

Please click Save in order to store your changes. MAVT - Multi-Attribute Value Theory

**MAVT - Value function**

Attribute	0.0	0.1	0.2	0.3	0.4	0.5	0.6	0.7	0.8	0.9	1.0
Efficacy	Very low (0)	Low (0.25)	Moderate (0.5)	High (0.75)	Very high (1)						
Budg. Imp.	Significantly higher (0)	Moderately higher (0.25)	No difference (0.5)	Moderately lower (0.75)	Significantly lower (1)						



# MCDA in Brazil



# Example: Brazil Conitec Training 2014

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Criterion	Weight	Levels (high-low)	Scoring
Cost-effectiveness	0.3	Dominates existing treatment	+2
		Attractive/low cost-effectiveness ratio	+1
		Similar in terms of costs and effects	0
		Unattractive/high cost-effectiveness ratio	-1
		Dominated by existing treatment	-2
Budgetary impact	0.15	Large budgetary saving	+2
		Small/moderate budgetary saving	+1
		Budget neutral	0
		Small/moderate budgetary increase	-1
		Large budgetary increase	-2
Safety	0.15	Much safer than existing treatment	+2
		Minor/moderately safer than existing treatment	+1
		Similar safety	0
		Minor/moderately less safe than existing treatment	-1
		Much less safe than existing treatment	-2
Unmet need/innovative	0.09	Significant unmet need/innovation in disease/indication area	+3
		Moderate unmet need/innovation in disease/indication area	+2
		Minor unmet need/innovation in disease/indication area	+1
		No unmet need/innovation in disease/indication area	0
Feasibility	0.08	Significantly easier to implement than existing treatment	+2
		Minor/moderately easier to implement than existing treatment	+1
		Similar implementation needs compared to existing treatment	0
		Minor/moderately harder to implement than existing treatment	-1
		Significantly harder to implement than existing treatment	-2
Equity	0.08	Large improvement in equity compared to existing treatment	+2
		Minor/moderate improvement in equity vs existing treatment	+1

Source: Ron Goeree,  
PATH Canada 2014

Ministério da Saúde  
Secretaria de Ciência, Tecnologia e Insumos Estratégicos  
Departamento de Gestão e Incorporação de Tecnologias em Saúde

Priorização de  
Protocolos e Diretrizes  
Terapêuticas para  
Atenção Integral às  
Pessoas com Doenças  
Raras

Setembro de 2014

Priorities for the development  
of protocols to treat rare  
diseases





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Ministério da Saúde  
Secretaria de Ciência, Tecnologia e Insumos Estratégicos  
Departamento de Gestão e Incorporação de Tecnologias em Saúde

Priorização de  
Protocolos e Diretrizes  
Terapêuticas para  
Atenção Integral às  
Pessoas com Doenças  
Raras

Setembro de 2014

- Describe the existing protocols for rare disease
- Describe a panel that was performed with specialists to prioritize, among more than 8.000 diseases those that would be studied first



# Major criteria used (CONITEC 2017)

MCDA required criteria:

- Burden of disease
- Severity
- Prevalence
- Availability of effective interventions (need)
- Availability of cost-effective interventions
- Equity – socioeconomic level
- Equity – geographic level

Methodology: EVIDEM core model & PAPRIKA (Potentially all Pairwise Rankings of all possible Alternatives) approach



# MCDA Applications

- **Drugs -**
  - A) **Late Stage Oncology** (to prioritize among different products)
  - B) **Rare Diseases** (to prioritize among rare diseases – national/regional priorities)
  - C) **HTA submissions quality comparison**
- **Medical Devices** – to establish both quantitative (e.g. CE/CUA, BIM) and qualitative (e.g. quality of support, implementation time, warranty)
- **Public Tender in Hospital setting** (to establish prioritization/ranking among different alternatives based on award criteria)



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# Japan

## recommendations

**Official guideline for Economic Evaluation of the Drugs / Medical Devices in Japan stipulates the importance of the following HTA Criteria:**

- |   |  |
|---|--|
| <ul style="list-style-type: none"><li>• Perspective</li><li>• Target population</li><li>• Comparator</li><li>• Additional benefit</li><li>• Method of analysis</li><li>• Results of analysis</li><li>• Subgroup analysis</li><li>• Time horizon</li><li>• Outcome measure</li><li>• Methods to derive QOL score</li></ul> | <ul style="list-style-type: none"><li>• Sources of clinical data</li><li>• Indirect comparison</li><li>• Costs to be included</li><li>• Sources of costs</li><li>• Estimation of productivity loss</li><li>• Discount rate</li><li>• Modeling</li><li>• Sensitivity analysis</li><li>• Reporting</li></ul> |
|---|--|

**MCDA - could be used to map Quality of submissions on each domain.**



“Problems with some **members** include: a lack of direction, poor accountability, lack of respect among members, pushing personal agendas, poor communication ...”

# MCDA – Late Stage Oncology & Rare Diseases

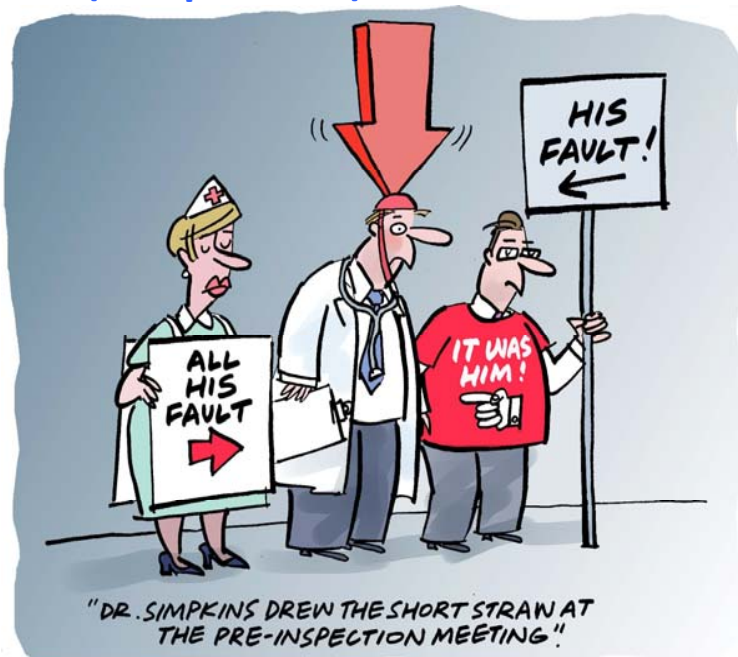


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- Results: ranking among alternatives (comparators)



## 7 P's MCDA Panel mix – Vlad's Inclusion Rule

- Payer
- Patient
- Pharma
- Physician
- Politician
- Pharmacist
- Public (General Public representative in case of NIF/MoH)



# Thank you