Academic perspective on patient preference research

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Overview

To present the results of a **survey** of **Prader-Willi Syndrome** (PWS) caregivers that sought to:

1. Identifying **caregiver priorities** for outcomes
2. Quantifying **caregiver preferences** for treating PWS
3. Assessing the potential impact of **hyperphagia** on the **quality of life** of the PWS patients

We will also discuss:

4. What is a **valid preference study**?
5. What types of study do **patients want**?

Acknowledgement

- The project is support by **International Consortium to Advance Clinical Trials for Prader-Willi Syndrome**
- We would like to thank the members of our executive team, **Sophie Tsai**, **Theresa V. Strong** and **Nathalie Kayadjanian**.
- We are grateful for the guidance of the **PWS Community Advisory Board**
- Special thanks to the **Prader-Willi Syndrome Association and Foundation for Prader-Willi Research** for assisting with recruitment, and especially to **Susan Hedstrom**, **Jessica Bohonowych**, and **Lauren Roth**

Demographics (n=450)

Caregiver age (mean, range)	49.0 (20-83)
Parents	96%
Females	84%
Caucasian	87%
Familiar with FDA	46%
Annual household income >$100,000	52%
Private insurance	68%
Patient age (mean, range)	15.9 (4-54)
Diagnosed via genetics (DNA/blood)	97%
Genetic subtype	
Deletion	50%
UPD	37%

Potential treatment benefits

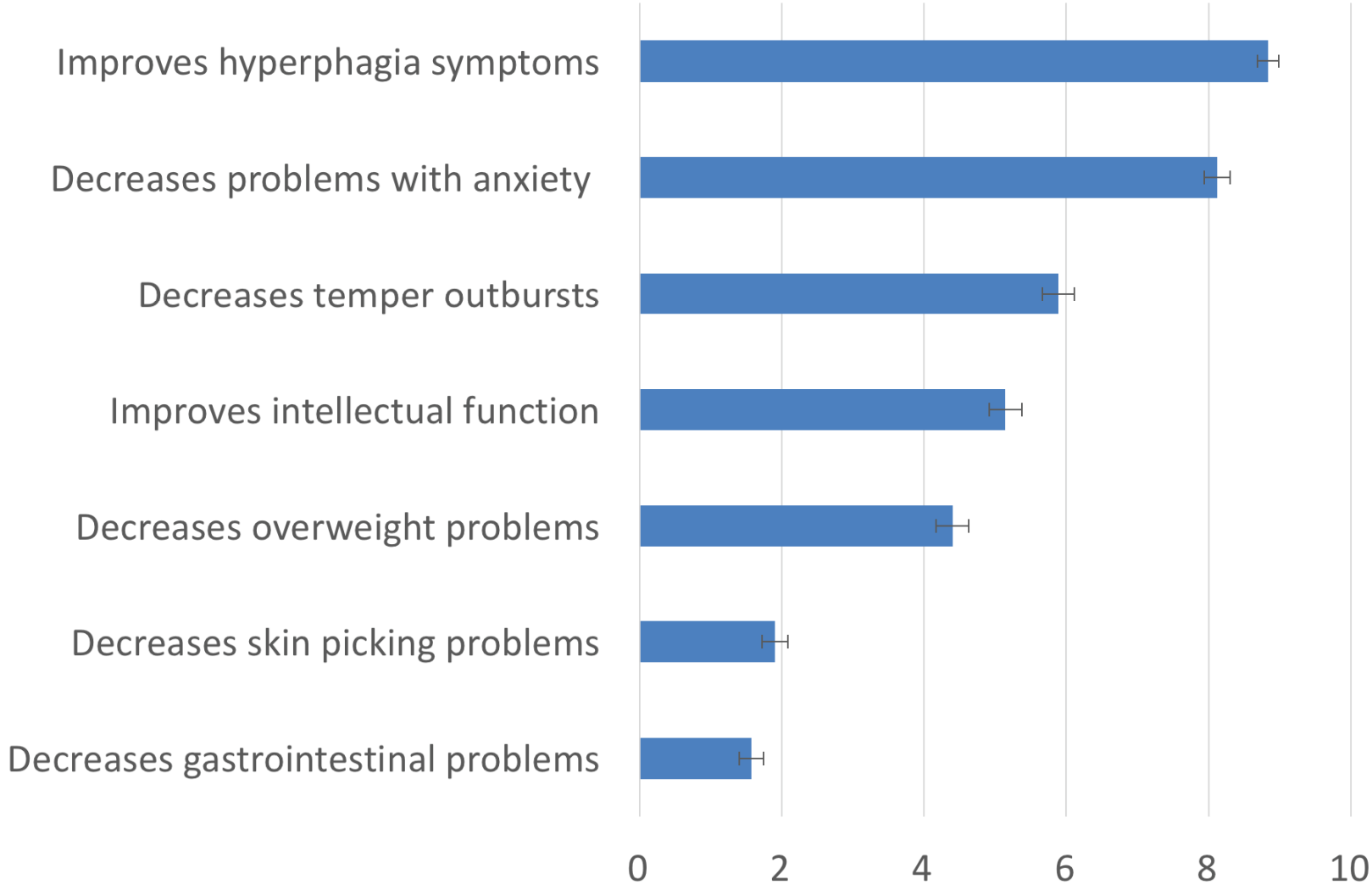
- The set of potential treatment benefits was identified from prior research and discussions with the **PWS community advisory board**.
  - Improves **intellectual** function
  - Decreases **gastrointestinal** problems
  - Improves **hyperphagia** symptoms
  - Decreases **overweight** problems
  - Decreases problems with **anxiety**
  - Decreases **temper outbursts**
  - Decreases **skin picking** problems

BWS example task

What do you think is **most important and least important** when choosing a treatment for your family member with PWS?

Most Important	Benefits	Least Important
	Improves intellectual function	
	Improves hyperphagia symptoms	
	Decreases skin picking problems	
	Decreases problems with anxiety	

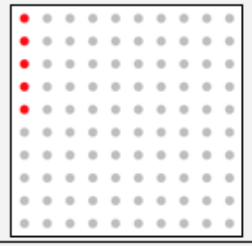
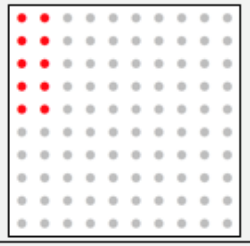
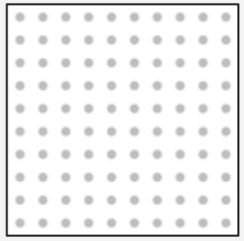
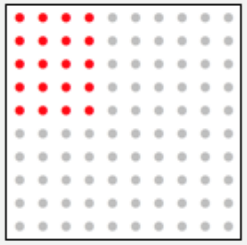
Best-worst scores



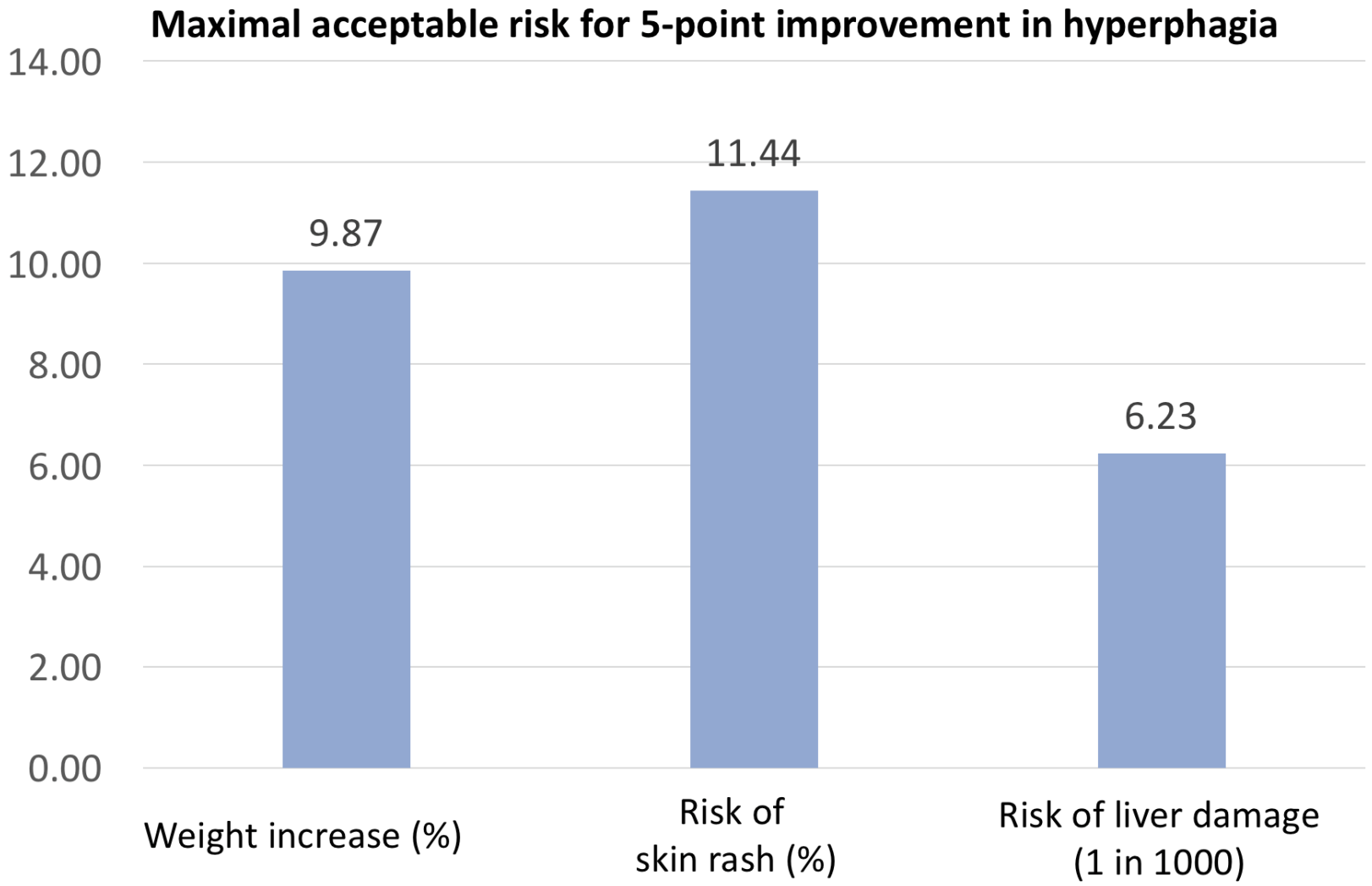
Benefit-risk assessment

- There is a paucity of available treatment options directly targeting **hyperphagia**
- The FDA weighs the **benefit** of a drug (effectiveness) against the **risk** of a drug (side effects) when making regulatory approval decisions.
- **Discrete-choice experiment (DCE)** is a stated-preference method used by the FDA to quantify patient benefit-risk preferences.
- Benefits were described as 5 and 10 point differences on the **hyperphagia questionnaire for clinical trials (HQ-CT)** scale

DCE example task

	Drug A	Drug B
Improvement in Hyperphagia	5-point improvement	10-point improvement
Improvement in obesity (weight loss)	5% weight loss 	10% weight loss 
Increased risk of skin rash	No additional risk 	20% higher risk 
Risk of liver damage	10 in 1000 risk 	1 in 1000 risk 
Which is the better drug?	☐	☐

Hyperphagia - acceptable tradeoffs

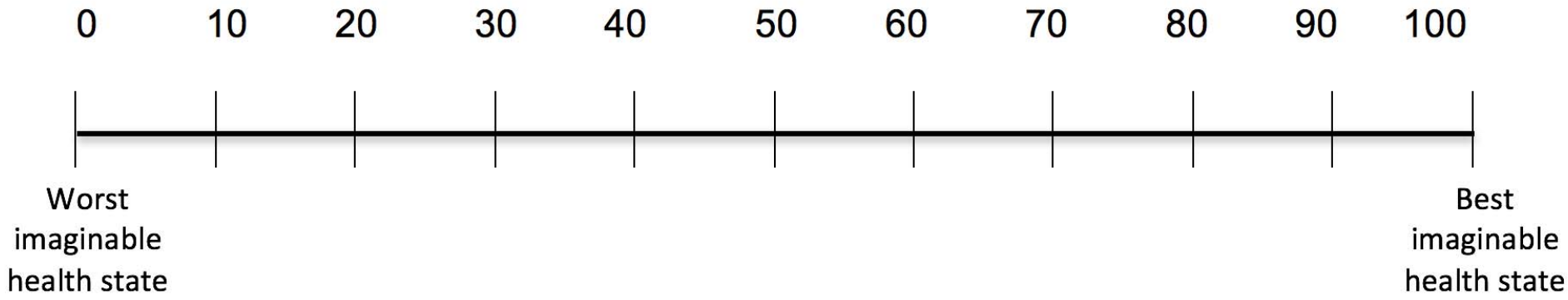


Impact of hyperphagia on QoL

- We sought to quantify the impact of treating hyperphagia (relative to treating obesity) on quality of life by estimating quality-adjusted life years (QALYS)
- **Visual Analogue Scale (VAS)** and **Time-trade-off (TTO)** were used to estimate QALYS.
- Caregivers were ask to evaluate **three different health states** for a individual (Alex) who was 18 years old:
    - PWS without treatment
    - PWS with no obesity
    - PWS with no obesity nor hyperphagia

VAS example task

On a scale of 0 to 100, 0 being the worst health that you can imagine, 100 is the best health you can imagine, how would you rate Alex's current health?



TTO example task

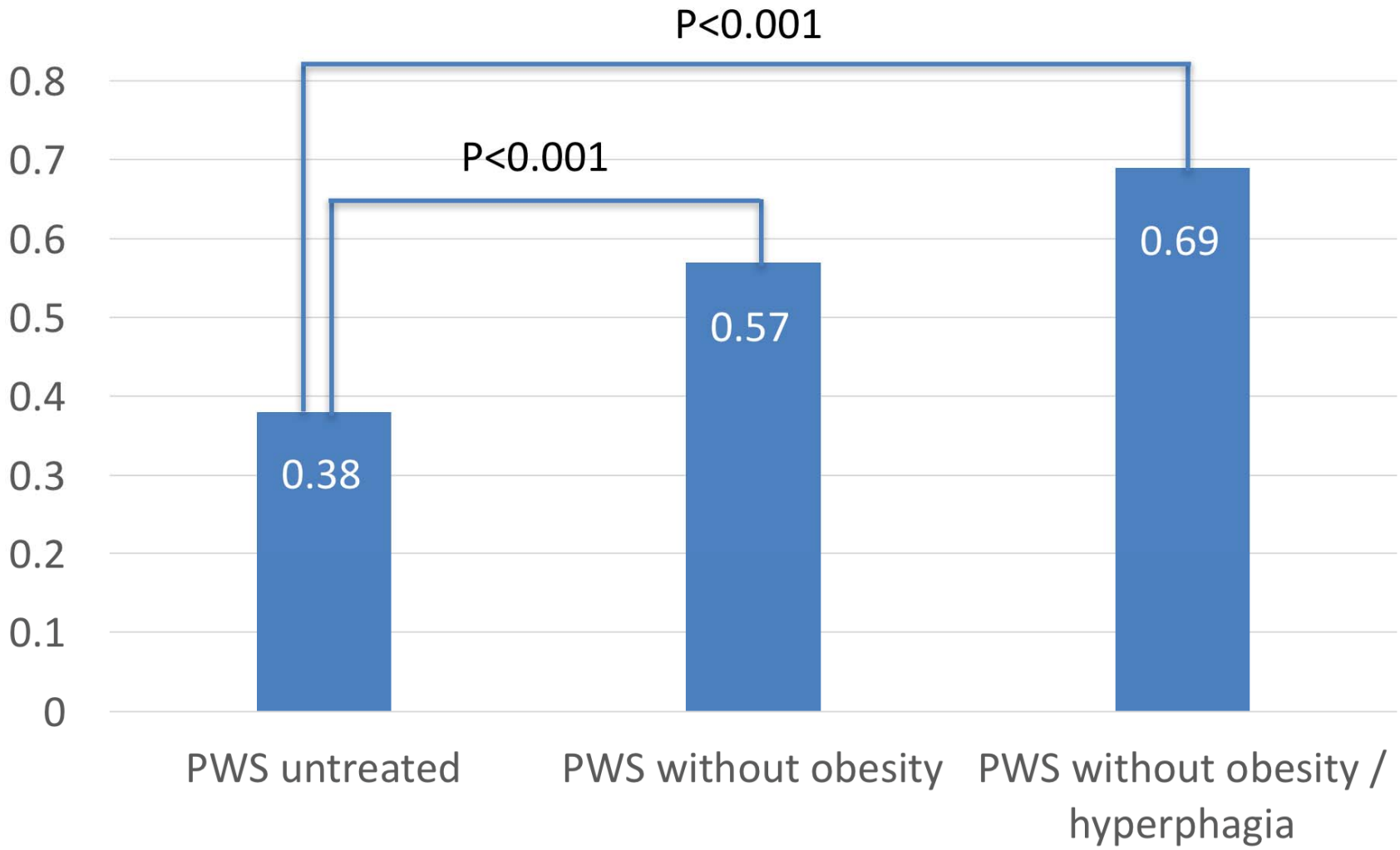
Would it be better for Alex to:

Live in the current condition until age 38 (that is an additional 20 years)

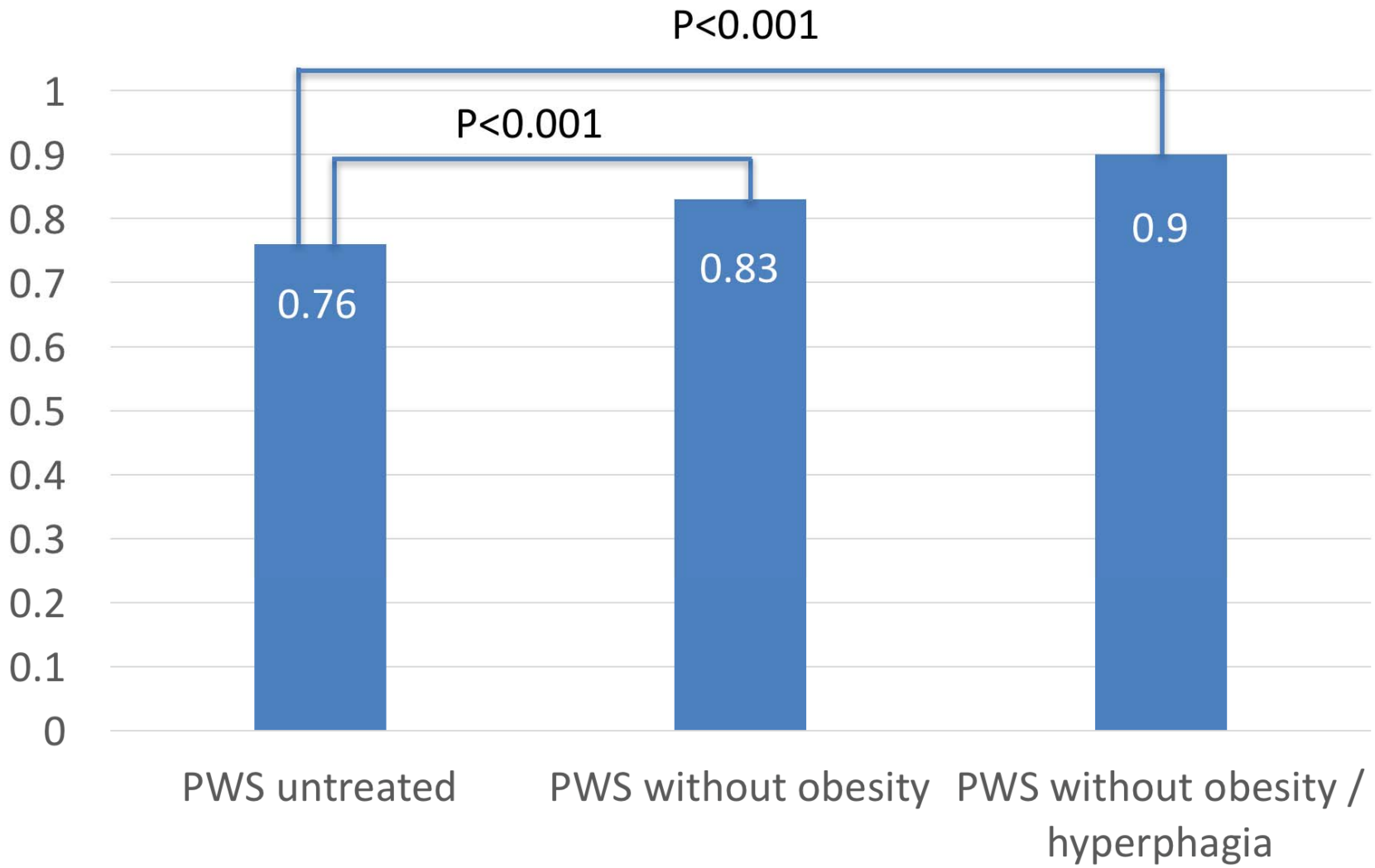
OR

Live FEWER years but be completely healthy without excessive appetite for the rest of Alex's life? Alex should give up _____ years (write the years) to live completely healthy for all of them.

QALY results (VAS)



QALY results (TTO)



Acknowledgements

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- I am grateful for the guidance of the **Diabetes Action Board (DAB) and Johns Hopkins Community Research Advisory Committee (C-RAC).**

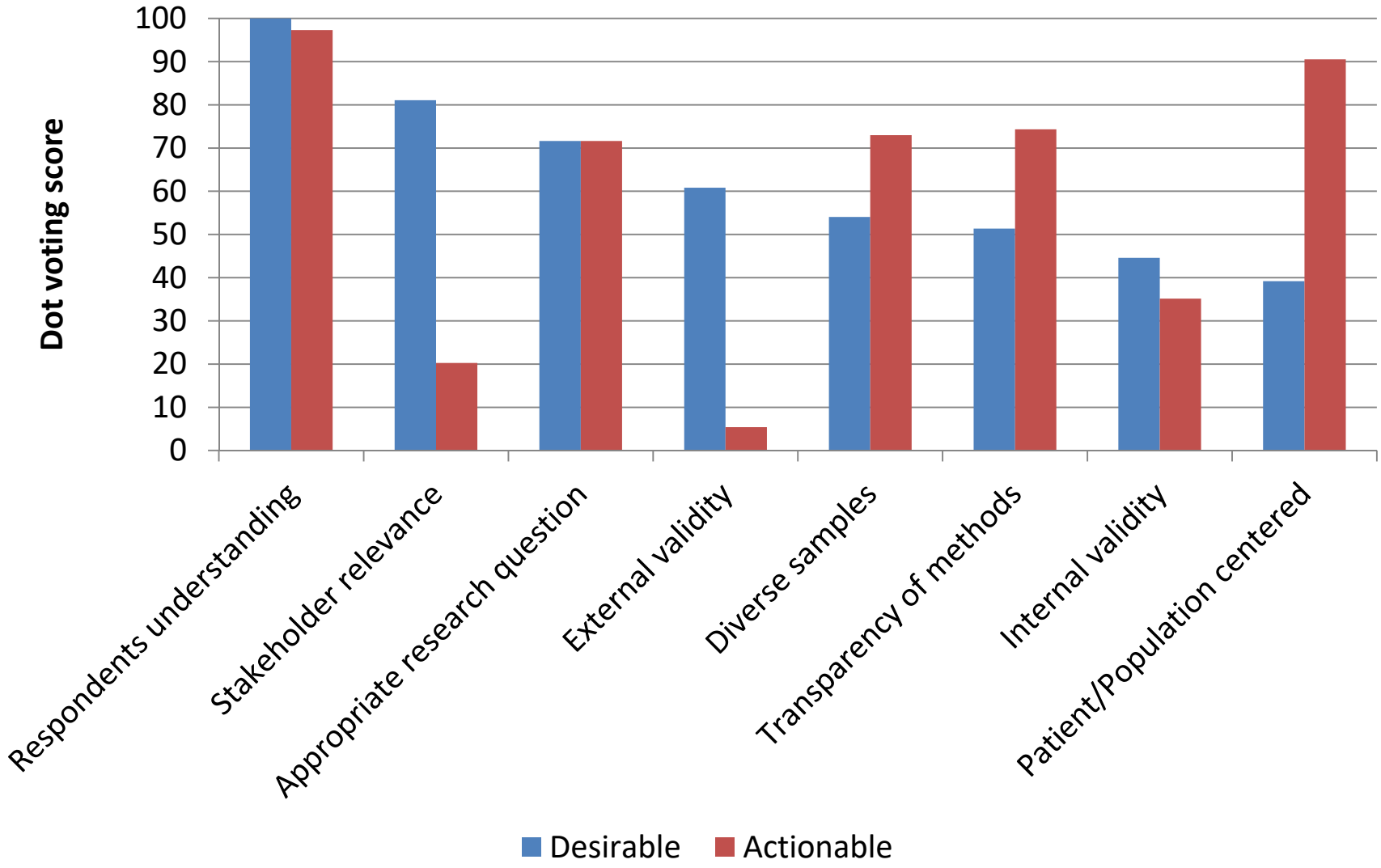
Dot voting process

- In small groups:
  - Discuss important characteristics of preference studies
  - Create a list of the five most important characteristics
  - Present list to large group

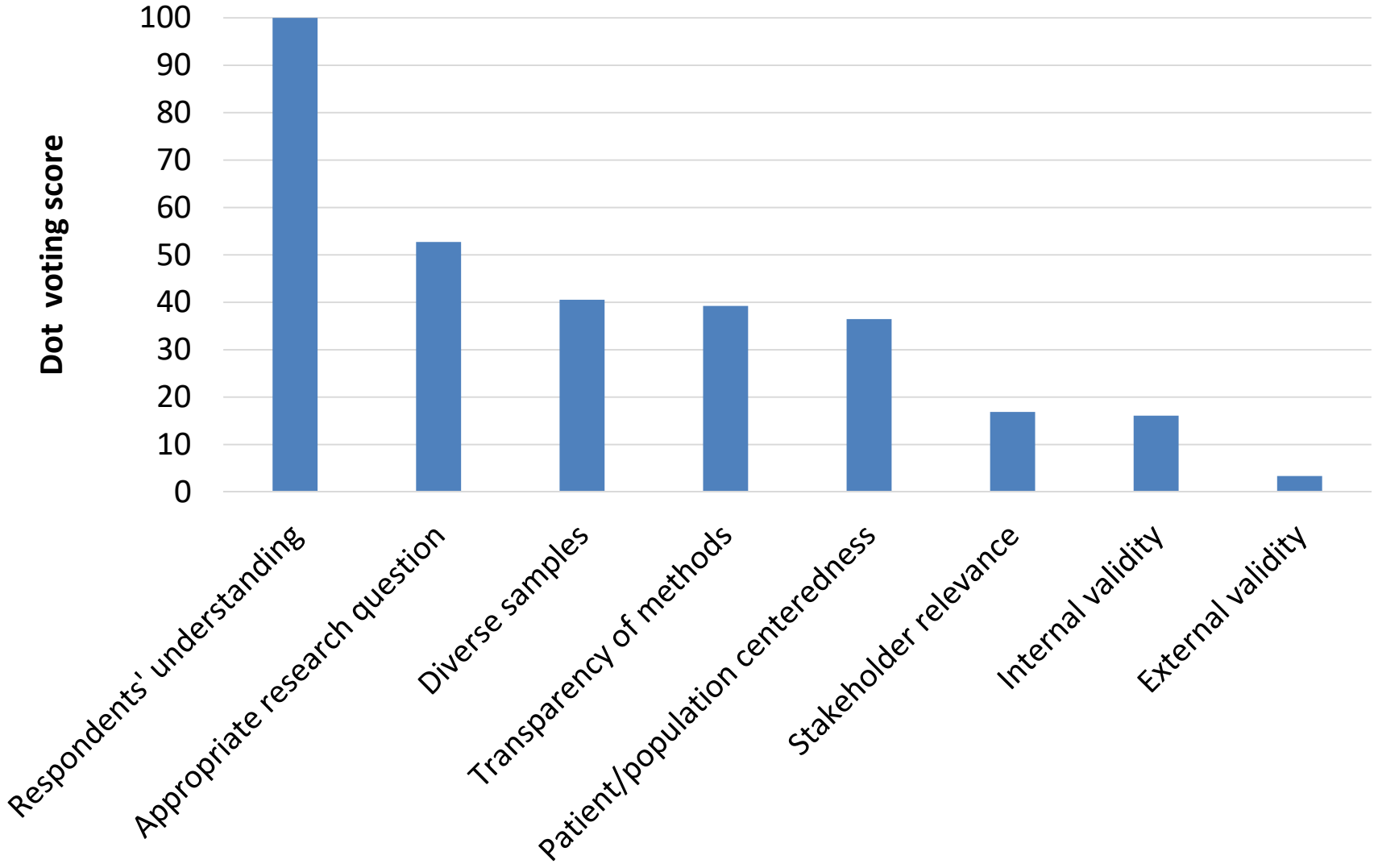
- Individually:
  - Take 12 red dots
  - Consider all study characteristics identified by all groups
  - Allocate dots to the most **desirable** study characteristics

- Individually:
  - Take 12 green dots
  - Consider all study characteristics identified by all groups
  - Allocate dots to the most **actionable** study characteristics

Desirable and Actionable



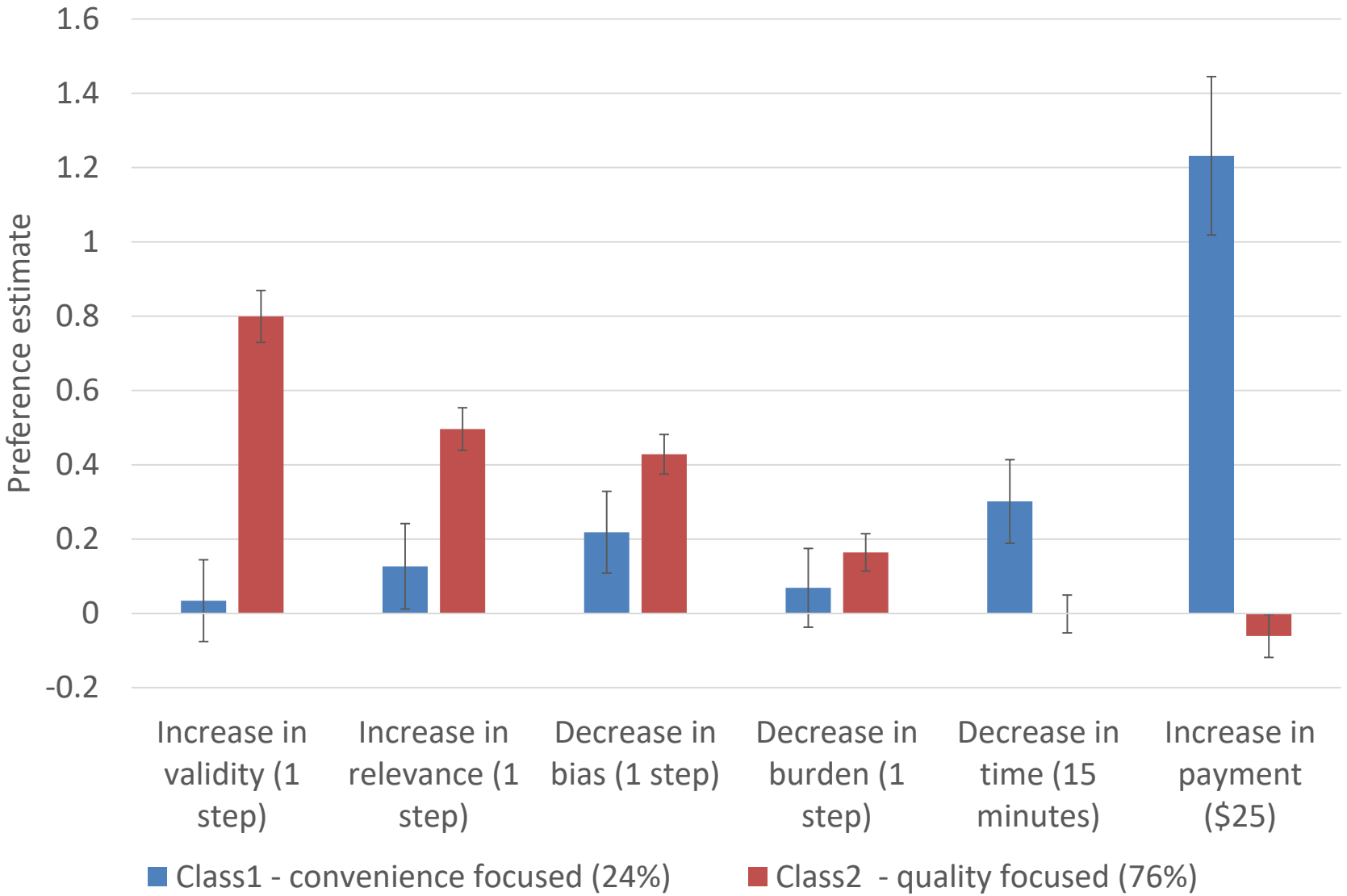
Desirable and actionable combined



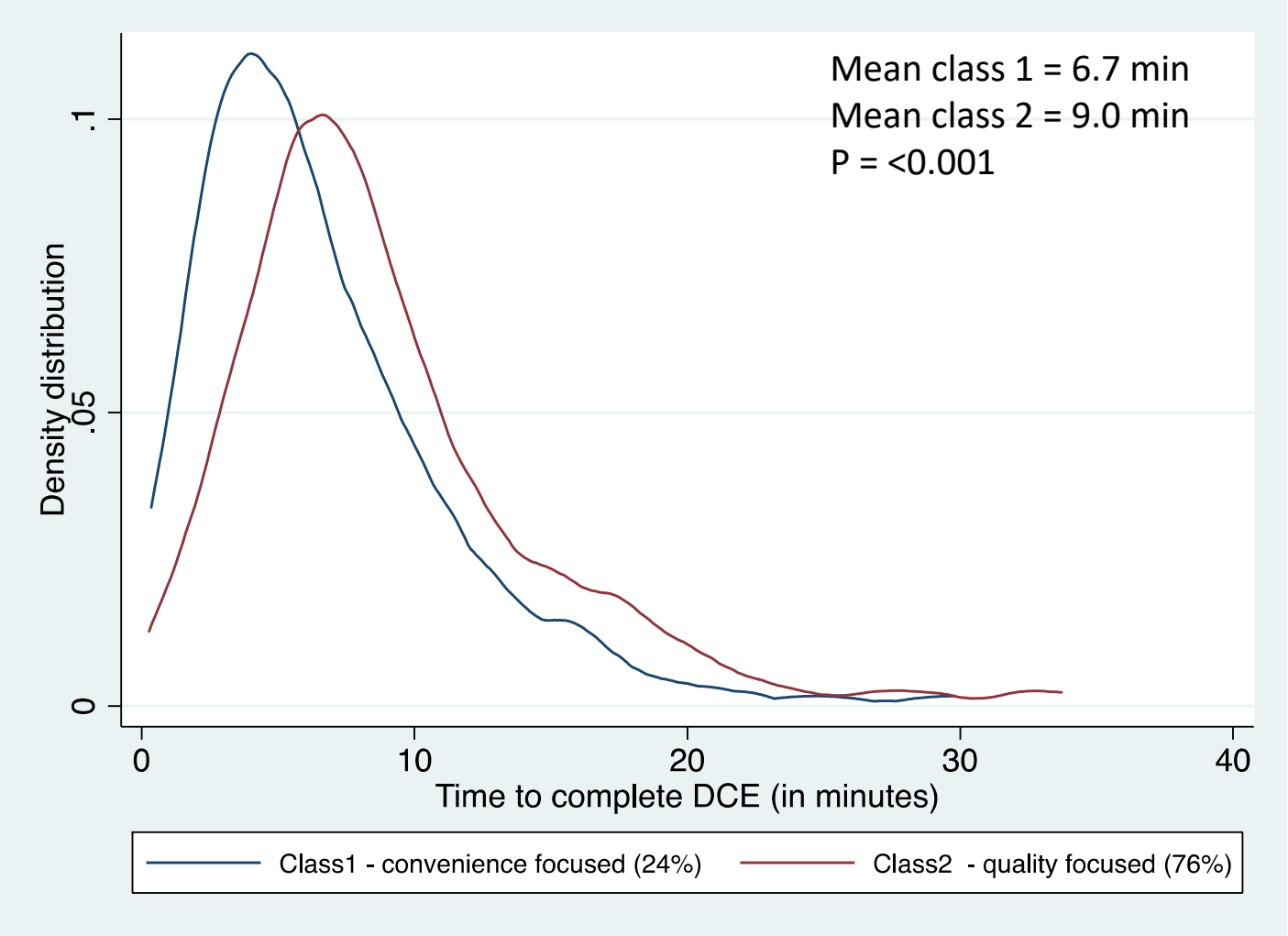
Sample DCE task

A local hospital is conducting a study to learn about the preferences of patients. Choose the study that you think is better.		
Attributes	**Study A**	**Study B**
Validity	High Validity (★★★★☆)	Low Validity (★★☆☆☆)
Relevance	Low Relevance (★★☆☆☆)	High Relevance (★★★★☆)
Bias	Medium Bias (★★★☆☆)	Medium Bias (★★★☆☆)
Burden	Low Burden (★★★★☆)	High Burden (★★☆☆☆)
Time	45 minutes	30 minutes
Payment	$50	$25
What study do you think is better?	☐	☐

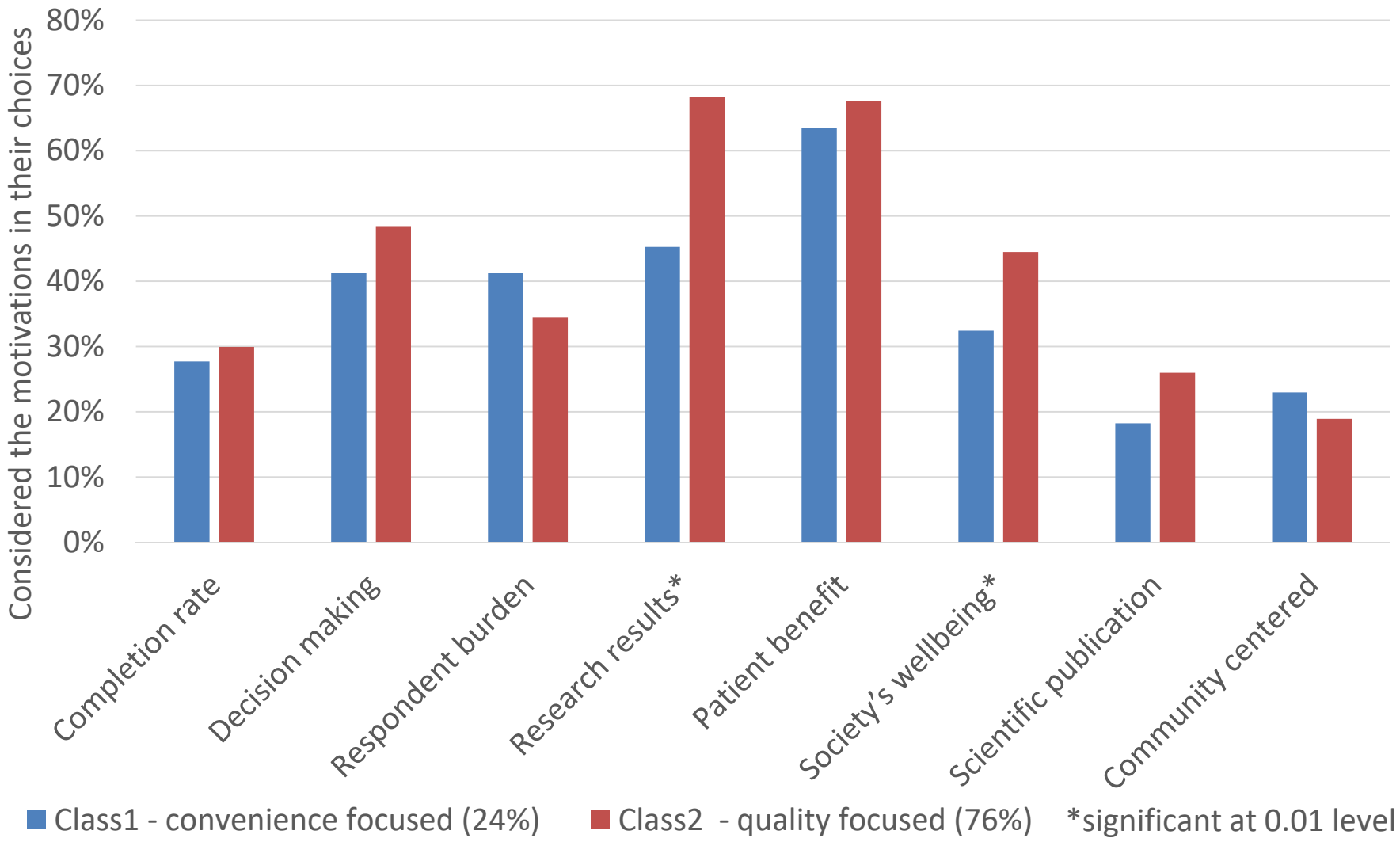
Latent class results



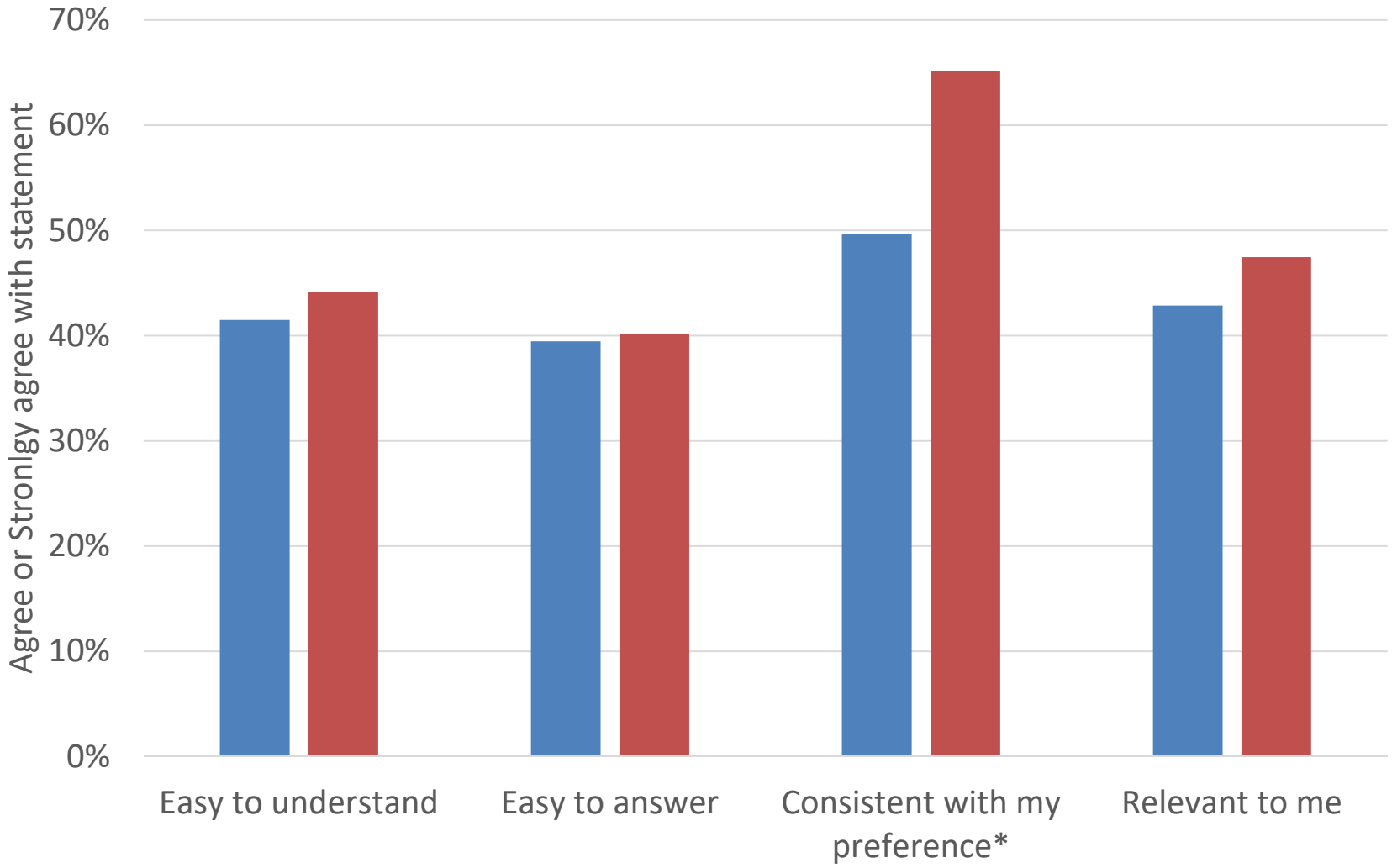
DCE completion time, Latent Class



Motivating factors, Latent Class



DCE evaluation, Latent Class



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