Do We Always Need a New Preference Study for Patient-Focused Drug Development?

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MICHAEL BUI[1]

[1] Department of Health Technology and Services Research, Technical Medical Centre, University of Twente (m.bui@utwente.nl)

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Introduction

Patient preference studies

- Time-consuming and expensive
- Findings rarely used beyond objective of original study
- Transferring past findings to new settings (e.g., same disease but different countries) could improve resource usage[1]

1 Veldwijk J, Ozdemir S, Bui M, et al. Transferability of Preferences; for Better or ….? Patient. Published online February 2, 2025. doi:10.1007/s40271-025-00728-8

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Transferring Preference Information

BENEFIT TRANSFERS

- Method to reuse preference information from existing studies in new decision contexts through meta-regression to adjust for contextual differences
- Commonly used in environmental economics, where preferences for environmental goods inform policy decisions (e.g., determining acceptable levels of green taxes)
- Unclear whether the patient preference study landscape is suitable for benefit transfers
- Aim: assess readiness of patient preference field for adopting benefit transfers

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Methods

Scoping Review

- PubMed, Scopus, Web of Science
- Included quantitative patient preference studies examining risks/benefits of interventions
- Assessed study landscape against methodological criteria for benefit transfers[2]
  - Number of studies across indications
  - Comparability of study designs
  - Availability of parameter estimates

2 Johnston RJ, Boyle KJ, Loureiro ML, Navrud S, Rolfe J. Guidance to Enhance the Validity and Credibility of Environmental Benefit Transfers. Environ Resource Econ. 2021;79(3):575-624. doi:10.1007/s10640-021-00574-w

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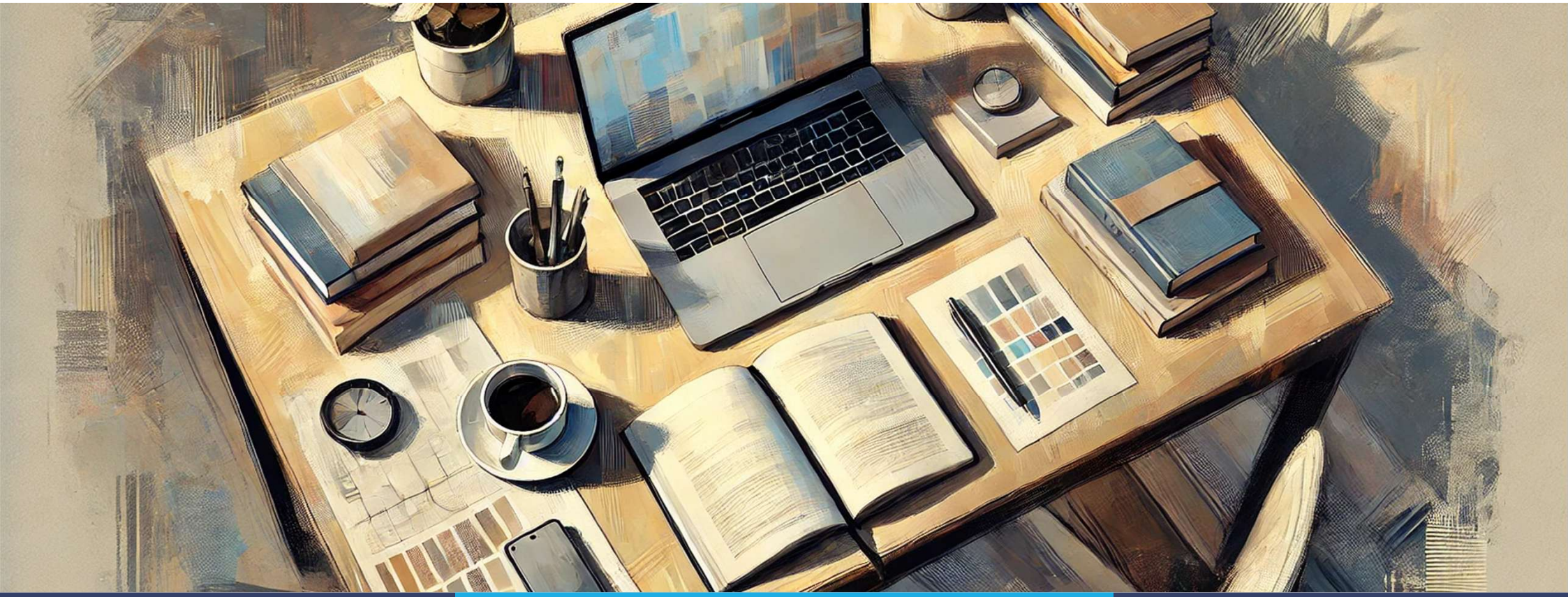
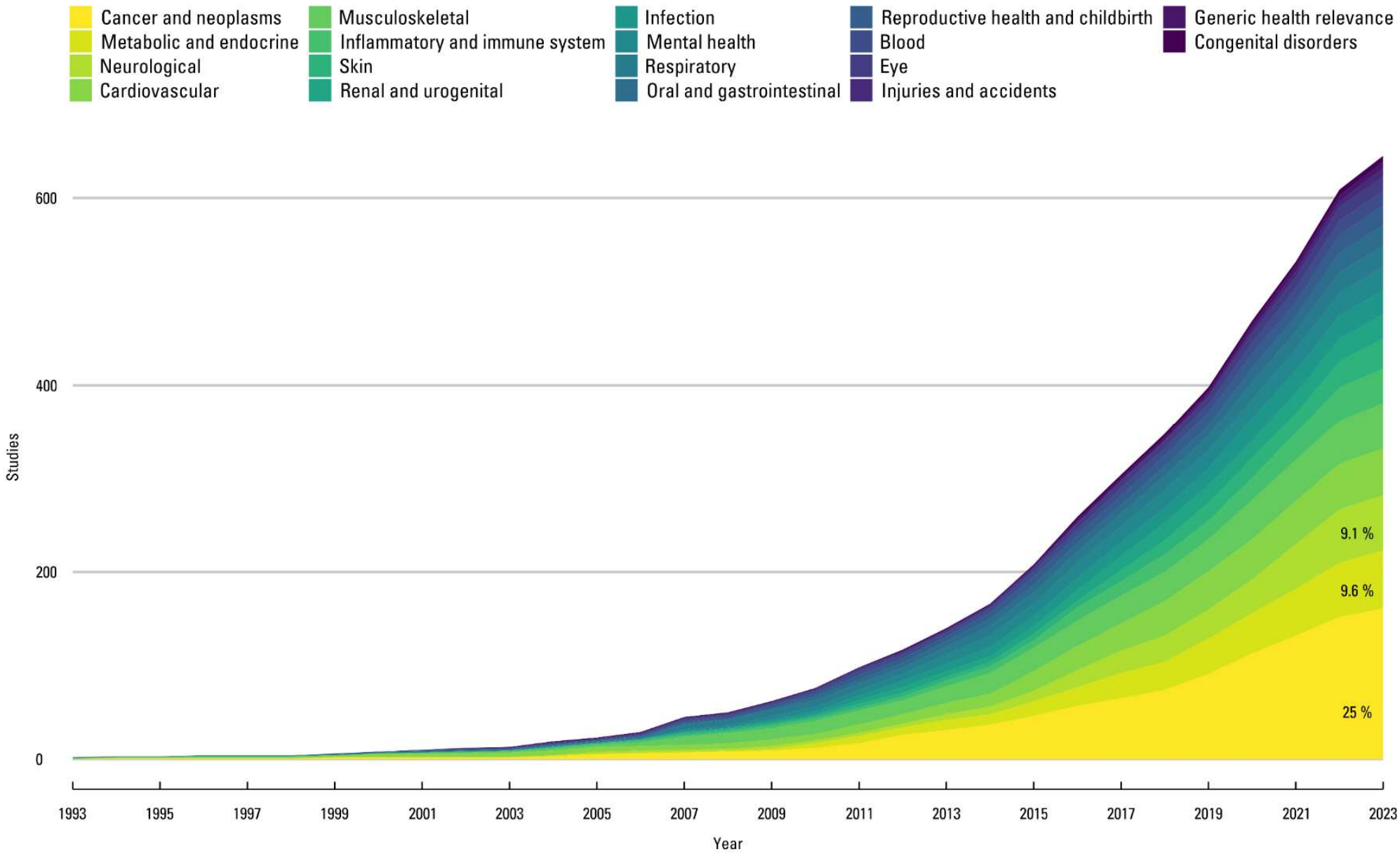


Results


Cancer and neoplasms
Metabolic and endocrine
Neurological
Cardiovascular
Musculoskeletal
Inflammatory and immune system
Skin
Renal and urogenital
Infection
Mental health
Respiratory
Oral and gastrointestinal
Reproductive health and childbirth
Blood
Eye
Injuries and accidents
Generic health relevance
Congenital disorders
600
400
200
0
Studies
9.1 %
9.6 %
25 %
1993
1995
1997
1999
2001
2003
2005
2007
2009
2011
2013
2015
2017
2019
2021
2023
Year

Results

TOP 5 MOST STUDIED INDICATIONS

Table 1. Overview of the number of available patient preference studies in the top five most studied indications. The study counts were stratified by whether discrete choice experiments (DCEs) or non-DCE methods were used.

Indication	DCE			Non-DCE		
	Studies	Total sample	Sample range	Studies	Total sample	Sample range
Type 2 diabetes	42	37,592	58-11,883	7	2,546	114-818
Psoriasis	22	8,897	126-1,608	8	2,522	126-600
Multiple sclerosis	20	7,873	60-1,862	7	1,399	50-350
Breast cancer	15[a]	4,164	78-641	8	1,419	41-310
Prostate cancer	14[b]	3,843	58-1,381	6	894	18-401

[a] Six studies with exclusive focus on metastasised cancer
[b] Three studies with exclusive focus on metastasised cancer

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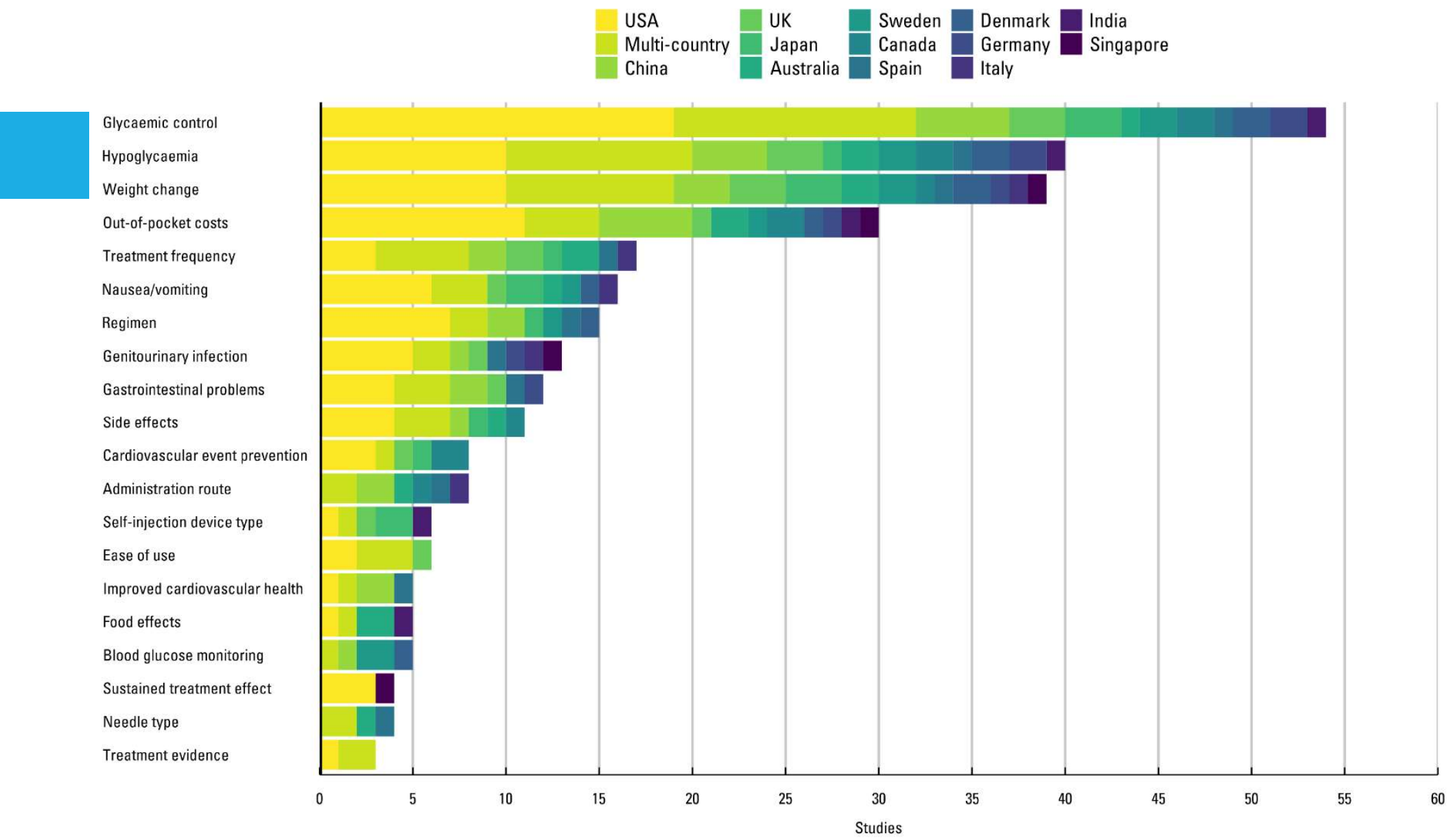
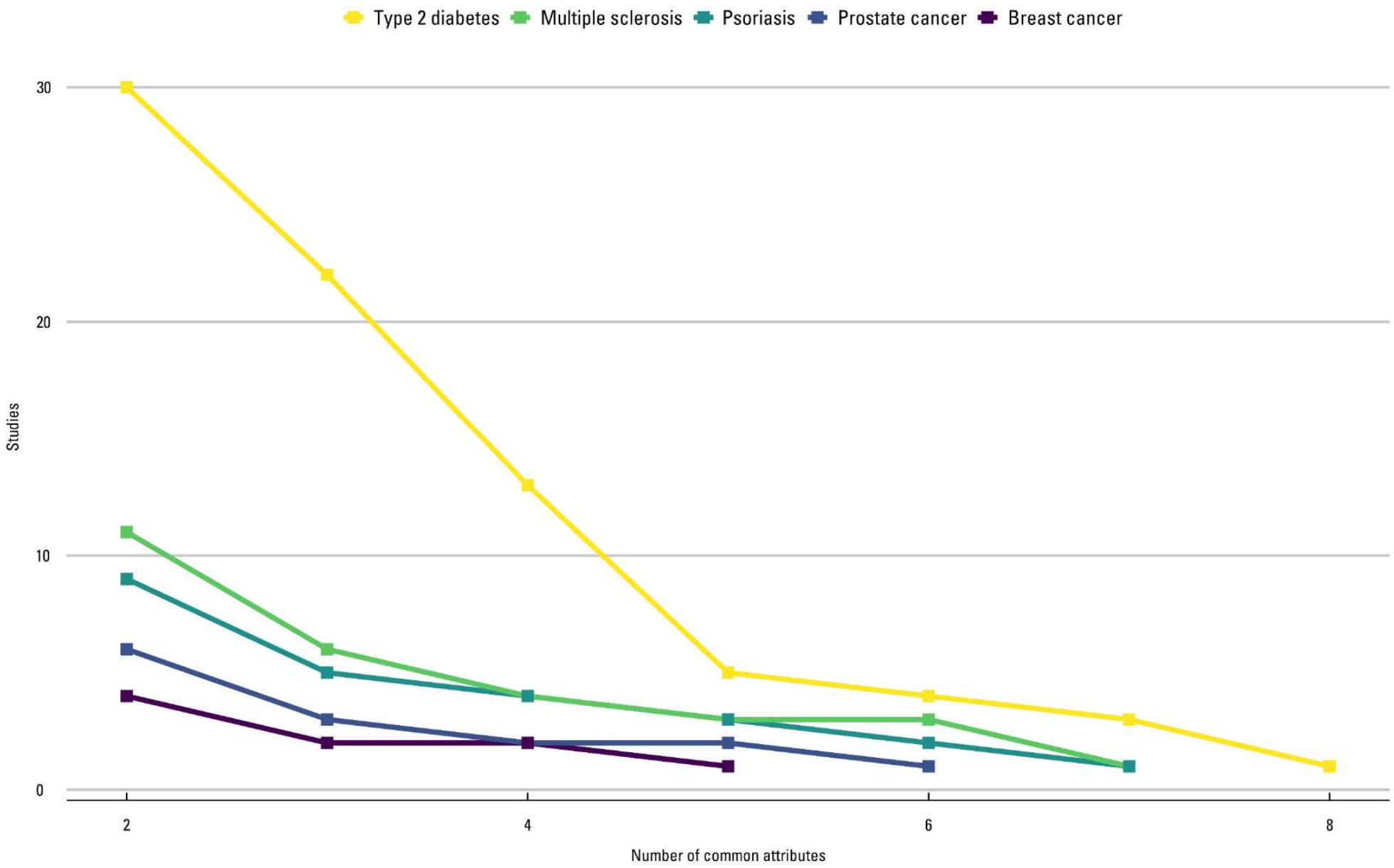

USA
Multi-country
China
UK
Japan
Australia
Sweden
Canada
Spain
Denmark
Germany
Italy
India
Singapore
Glycaemic control
Hypoglycaemia
Weight change
Out-of-pocket costs
Treatment frequency
Nausea/vomiting
Regimen
Genitourinary infection
Gastrointestinal problems
Side effects
Cardiovascular event prevention
Administration route
Self-injection device type
Ease of use
Improved cardiovascular health
Food effects
Blood glucose monitoring
Sustained treatment effect
Needle type
Treatment evidence
0
5
10
15
20
25
30
35
40
45
50
55
60
Studies


Type 2 diabetes
Multiple sclerosis
Psoriasis
Prostate cancer
Breast cancer
30
20
10
0
Studies
2
4
6
8
Number of common attributes

Results

REPORTED PARAMETERS

Table 2. Reported parameters in discrete choice experiments within the top five most studied indications. For each result, the relative frequency is provided.

Indication	β	OR	RAI	Marginal rate of substitution				Predicted uptake
				MAB	MAR	WTP	Other	
Type 2 diabetes	32/43	6/43	19/43	1/43	4/43	15/43	0/43	3/43
Psoriasis	19/22	2/22	12/22	2/22	4/22	4/22	2/22	1/22
Multiple sclerosis	18/20	3/20	13/20	3/20	7/20	2/20	0/20	1/20
Breast cancer	13/15	0/15	9/15	5/15	1/15	3/15	2/15	1/15
Prostate cancer	14/14	1/14	6/15	6/14	0/14	1/14	1/14	2/14

β regression coefficient, *OR* odds ratio, *RAI* relative attribute importance, *MAB* minimum acceptable benefit, *MAR* maximum acceptable risk, *WTP* willingness-to-pay

Conclusion

- Discrete choice experiments on type 2 diabetes treatments offer the most promising starting point for methodological explorations of benefit transfers

Challenges

- How to select covariates for benefit transfers in healthcare?
  - Systematic review of subgroup differences in preferences for attributes of type 2 diabetes medication

Preference Heterogeneity in Studies

STUDY DESIGNS

- 33/43 diabetes studies performed subgroup analyses, using a combination of the following methods:
  - Testing interactions (N=16)
  - Split-sample analyses (N=8)
  - Latent class models (N=5)
  - Unclear subgroup analyses (N=5)
- Amongst studies examining subgroup differences, risk of underpowered tests is present:
  - Median number of attributes: 6 (IQR: 5-7)
  - Median number of levels per attribute: 3 (IQR: 2-4)
  - Median sample size: 407 (IQR: 227-643)

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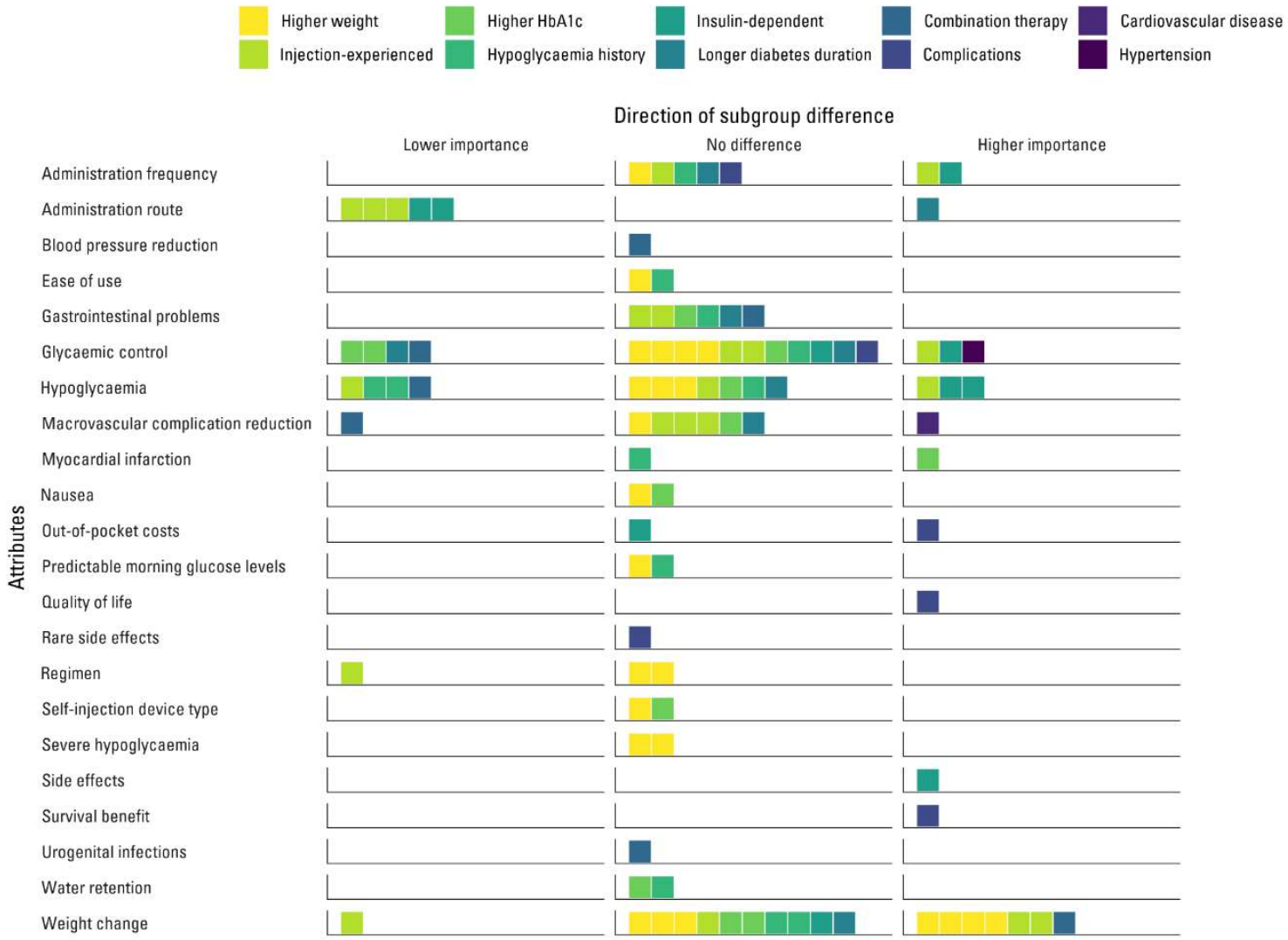
Preference Heterogeneity in Studies

SUBGROUP DIFFERENCES

- Univariate tests for differences by sociodemographic subgroups: 32/148 (21.6%) were significant, e.g.,
  - Men cared less about weight change than women (N=5)
  - Patients with lower income cared more about medication costs (N=2)
  - Older patients were more risk averse (N=1)
- Univariate tests for differences by clinical subgroups: 38/102 (37.3%) were significant, e.g.,
  - Injection naïve patients cared more about oral vs. injectable administration (N=5)
  - Patients with higher weight cared more about weight change (N=4)
- Latent class models corroborated these findings, especially older patients' risk aversion (N=4)


Higher weight
Injection-experienced
Higher HbA1c
Hypoglycaemia history
Insulin-dependent
Longer diabetes duration
Combination therapy
Complications
Cardiovascular disease
Hypertension
Direction of subgroup difference
Lower importance
No difference
Higher importance
Attributes
Administration frequency
Administration route
Blood pressure reduction
Ease of use
Gastrointestinal problems
Glycaemic control
Hypoglycaemia
Macrovascular complication reduction
Myocardial infarction
Nausea
Out-of-pocket costs
Predictable morning glucose levels
Quality of life
Rare side effects
Regimen
Self-injection device type
Severe hypoglycaemia
Side effects
Survival benefit
Urogenital infections
Water retention
Weight change

Next Steps

- Develop meta-regression models to assess the explanatory power of the proposed covariates in performing benefit transfers

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Thank you

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