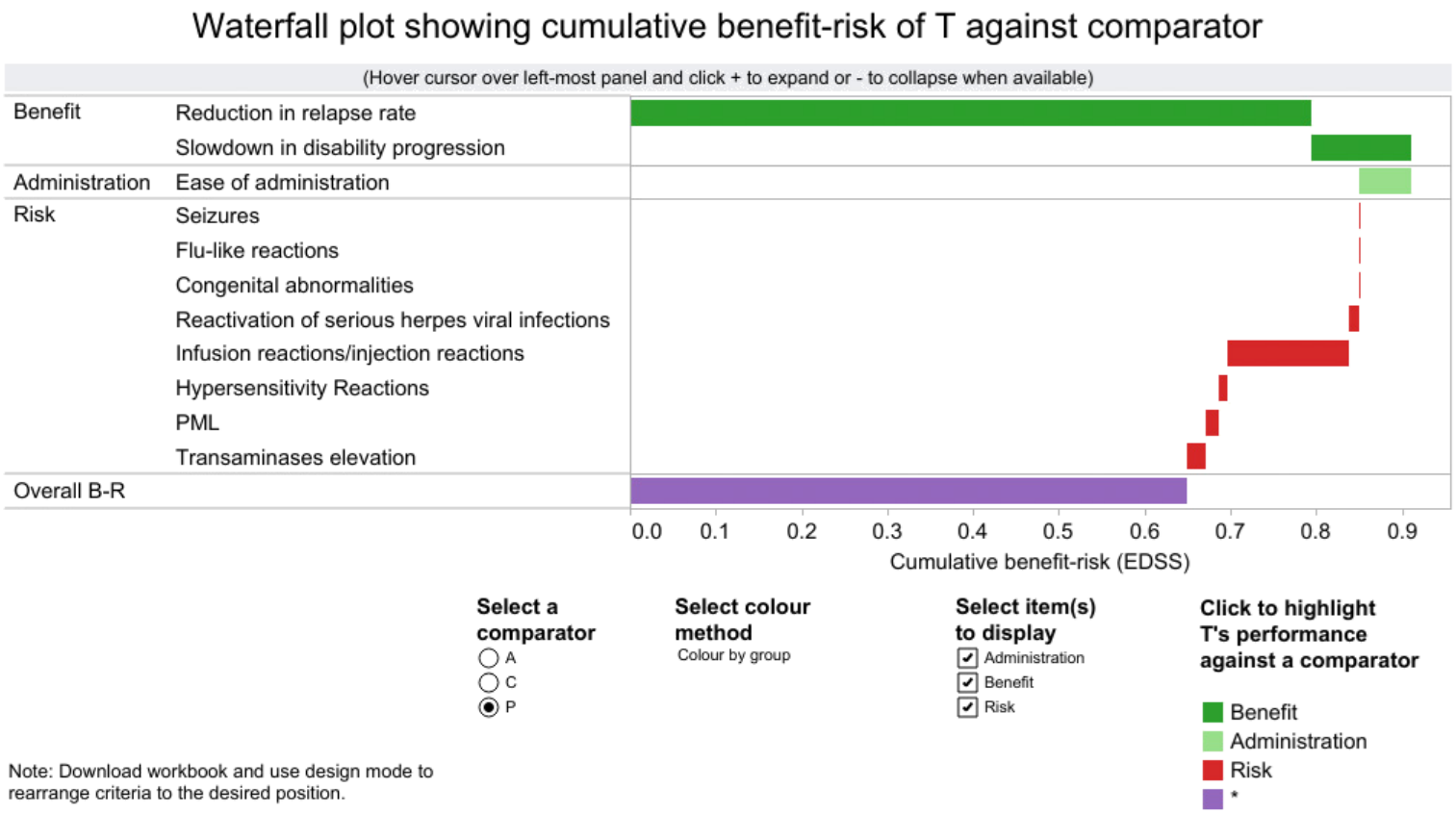
Patient Preferences Studies

Points to consider for patient organisations

Joint PCWP-HCPWP meeting, 3-4 February 2026, EMA

Starting from PPE study results

Natalizumab: criteria contribution



http://public.tableausoftware.com/views/T_Waterfall/WaterfallRisk

- The end of the previous bar determines the start of the next bar
- End of the last bar gives the overall benefit-risk.

Credits: IMI Protect Benefit/risks project team
Professor Deborah Ashby (Imperial College London)
Dr. Alain Micaleff (Merck Serono)
Richard Nixon, PhD, Novartis
Kimberley Hockley, Shahrul Mt-Isa

Example of comparing treatment options with Discrete Choice Experiments

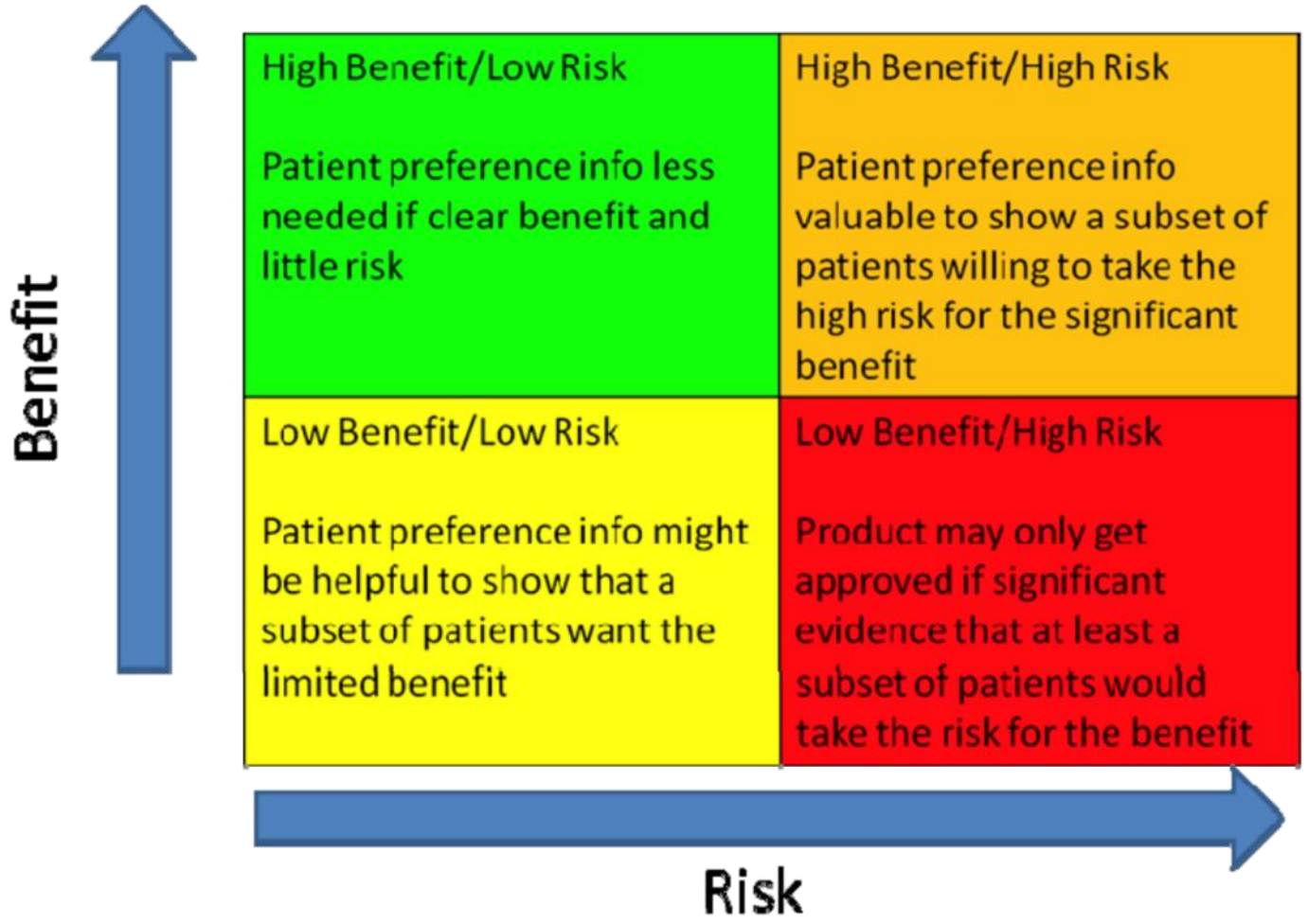
Treatment features	Treatment A	Treatment B
Number of relapses during the next 5 years	4 relapses	2 relapses
Time (from today) until your MS gets worse	3 years	3 years
Chance of dying from liver failure within 10 years	None would die	20 patients out of 1000 (2%) would die
Chance of dying or severe disability from PML within 10 years	5 patients out of 1000 (0,5%) would die	None would die
Chance of dying from leukaemia within 10 years	None would die	None would die
Which treatment would you choose?	Treatment A?	Treatment B?
	50%	50%

Pharmaco-epidemiological Research on Outcomes of Therapeutics by a European ConsorTium (IMI PROTECT). EMA with 31 public and private partners, 2009-2014

Prof Deborah Ashby OBE FMedSci, Professor of Medical Statistics and Clinical Trials, School of Public Health, Imperial College London

Preference sensitive situations

Preference sensitive benefit-risk profiles



Medical Device Innovation Consortium MDIC, 2015

In practice

feasibility

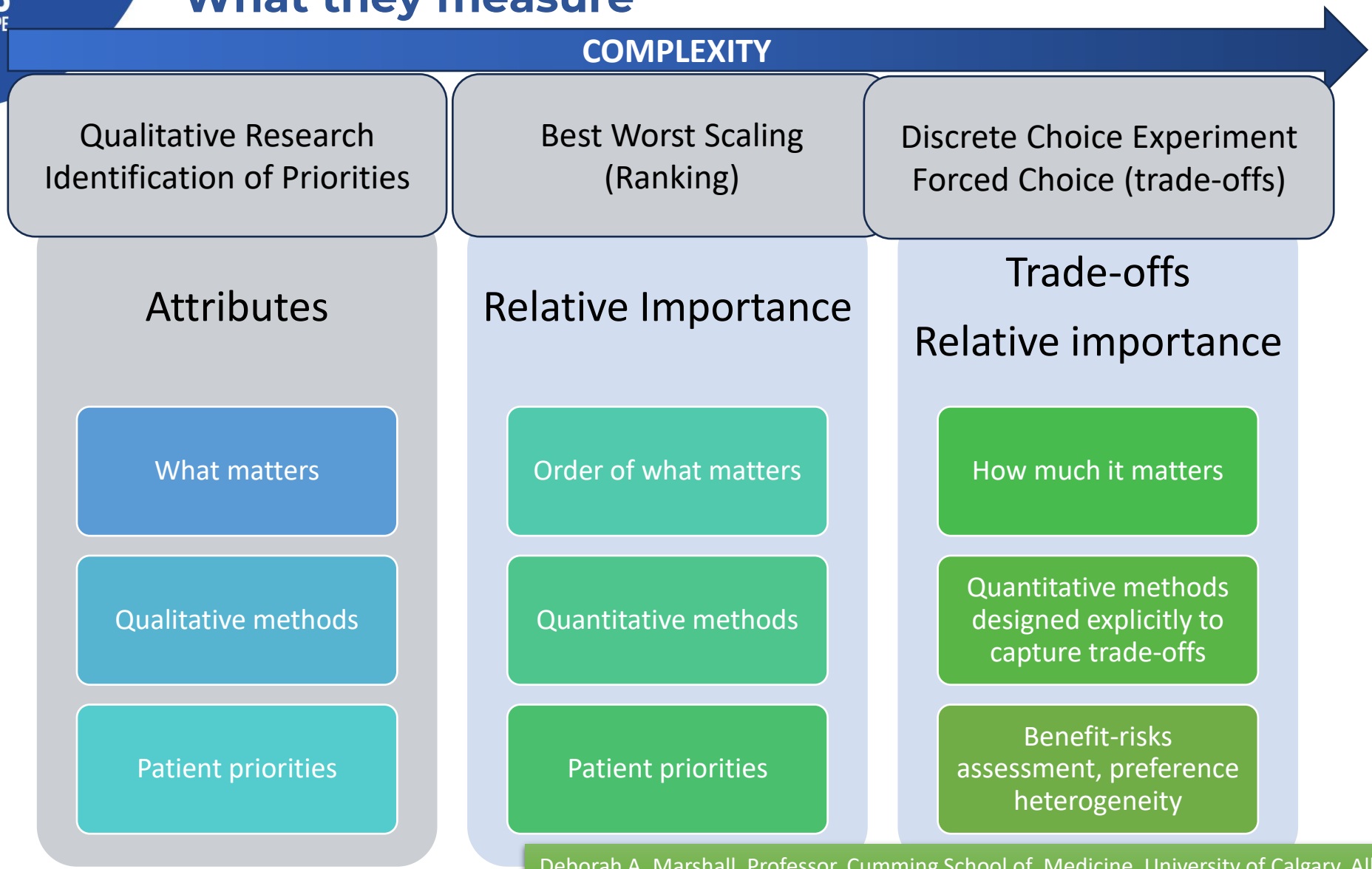
- Can patient organisations run such studies themselves? Or partnership with academia? Specific guidance?
  - Identify what matters, ranking attributes: possibly. DCE ?
  - Same rigour as for a clinical study (consent, IRB, representative sample…)
  - As Third-party contribution?
- How many patients needed? Sub-groups of patients? Cultural preferences?
- Costs? Timelines? Do we want to see the results in the Medicine Overview?

with industry

- Launch preparation of PPE studies early in R&D → to inform the development (ample time)
- Scientific Advice should always include question whether PPE study needed or not (or at pre-submission stage?)
- When b/r evaluation approaches: refine study and questionnaire (gain time)
- What if Patient Preference Study results favour the comparator?
- Re-use survey for regulatory purposes, add attributes relevant to HTA? (organisational, ethical…)

Different PPE study methods

What they measure



Deborah A. Marshall. Professor, Cumming School of Medicine, University of Calgary, Alberta Children's Hospital Research Institute, *HTAi Annual Meeting, June 2023, Adelaïde*

Towards Patient preferences in EPARs

Ritlecitinib - B7981048: **Alopecia Areata** Benefit-risk trade-off study in adults

Patient preferences were elicited using a **discrete choice experiment** and **applied to the trial results** in alopecia areata in a quantitative Benefit/Risk analysis.

The main objectives of this study were to: elicit patient preferences for AA treatment attributes and to estimate **maximum acceptable risks (MARs) of potential safety concerns** associated with JAK Inhibitors (upper respiratory infections, nasopharyngitis, nausea, headache, and acne) that AA patients are **willing to tolerate** for specific treatment benefits, and to assess the **net benefit-risk profile** of oral ritlecitinib 50 mg QD compared to 30 mg QD and to placebo.

- the probability of 80% to 100% **scalp hair regrowth** over 24 weeks of treatment,
- the probability of moderate or **normal eyebrows** after 24 weeks of treatment
- probability of moderate or **normal eyelashes** after 24 weeks of treatment,
- 3-year **risk of serious infection** due to treatment,
- 3-year **risk of cancer** (including non-melanoma skin cancer) due to treatment,
- and 3-year risk of **blood clot** due to treatment.

Conclusion: **Given the high value patients with severe AA placed on scalp hair regrowth** in the patient preference studies in adults and adolescents, the net B/R for ritlecitinib 50 mg, compared to no treatment, is considered **positive** from the patient's perspective

https://www.ema.europa.eu/en/documents/assessment-report/litfulo-epar-public-assessment-report_en.pdf

THANK YOU!

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