EUROPEAN FEDERATION OF STATISTICIANS IN THE PHARMACEUTICAL INDUSTRY
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From Trials to Target Populations: Extending and Extrapolating Evidence for HTA Decision-Making

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What is the challenge?

- Clinical trials enroll highly-selected populations that are not always aligned with the population that would be eligible for treatment in the real world following a positive health technology assessment (HTA) recommendation.
- This divergence may be necessary or desirable for reasons of safety, ethics, trial logistics and/or statistical power, and the uncertainty this introduces into the HTA decision problem must be tackled with other methods.
- The real-world population that would receive treatment in routine clinical practice, or equivalently the population covered by the HTA recommendation, may be considered the 'target' population.
- HTA decision makers routinely consider analyses of clinical trial data to make recommendations for a target population, and increasingly rely on non-randomised evidence to supplement trial data.
- Consequently, there is a need to consider the best use of data to inform decision-making for target populations.

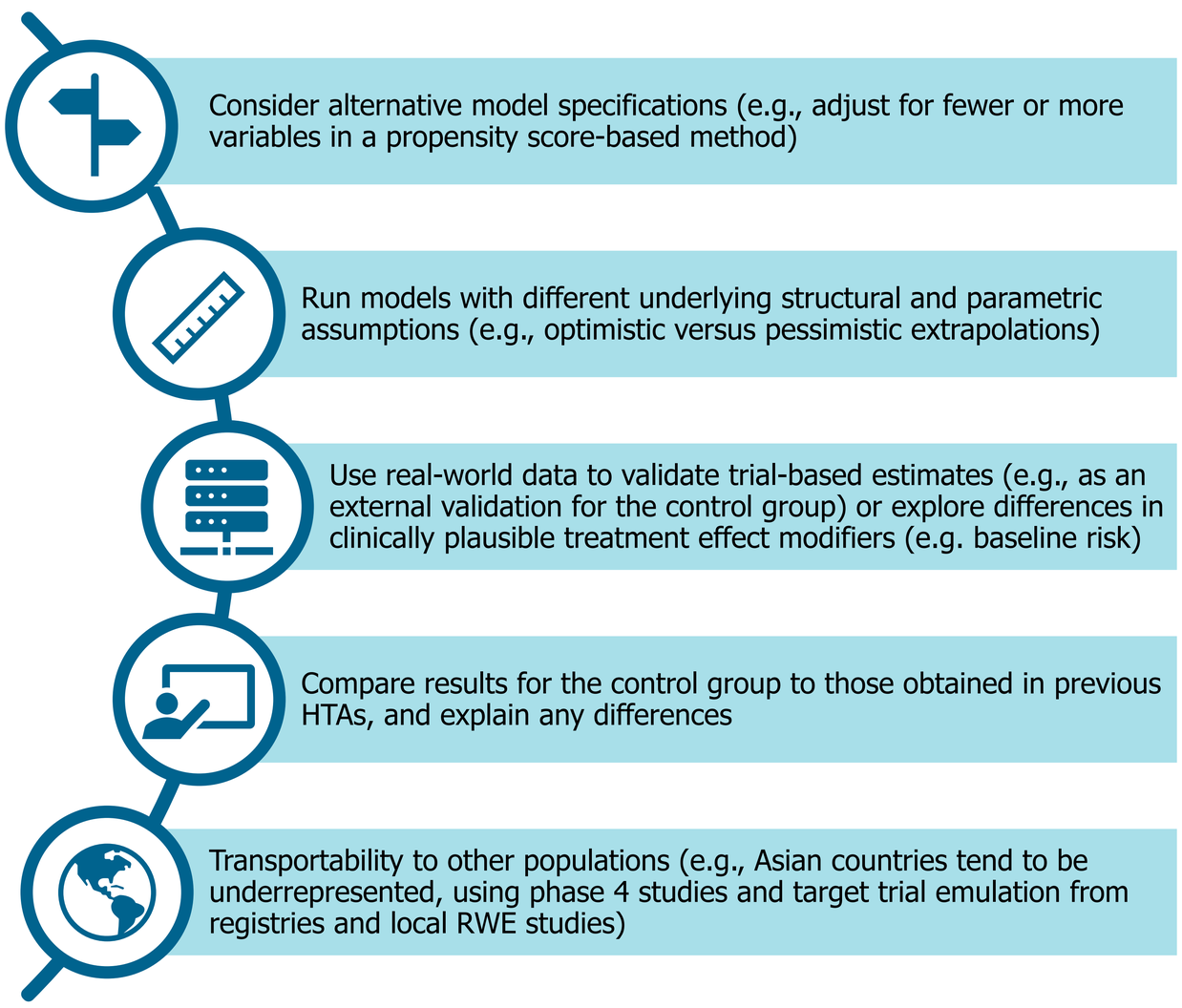
What can be done?

Statistical techniques and related HTA activities:

There are a range of different methods and activities that may help guide estimates of clinical outcomes and treatment effects.

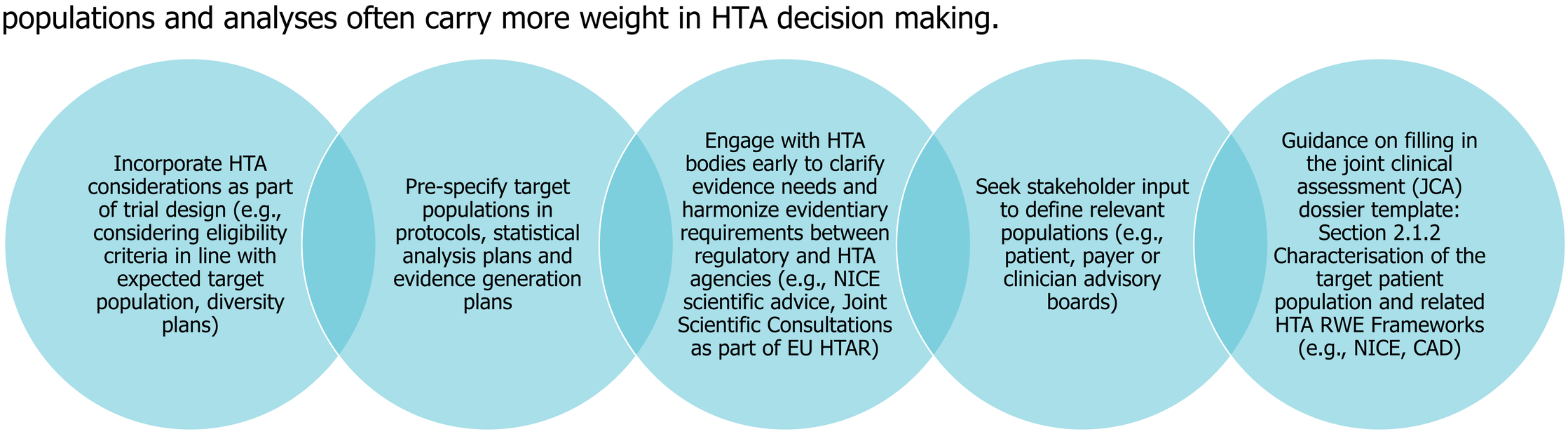
Sensitivity analysis:

For any analysis method performed, it should be possible to consider sensitivity analyses to stress test various settings and/or assumptions.



Early planning:

Outside of specific analyses, it is important to plan early for HTA as part of evidence generation plans. Pre-specified populations and analyses often carry more weight in HTA decision making.



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