



Key Research Questions

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Four Key Research Questions

Q1. Desirability: Is a PBRSA the appropriate way forward given uncertainty and alternatives to the use of a PBRSA?

The general question is whether a PBRSA can effectively and efficiently address the uncertainties remaining following marketing authorization. This is a *de facto* prospective value-of information (VoI) question. What is the issue/problem? What are the alternatives to using a PBRSA to tackle the problem?



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Q2. Evidence collection: Which PBRSA research design is most appropriate to collect evidence, conduct analyses of the evidence, and how should results be reported?

The answer will depend on the kind of problem the PBRSA is trying to solve:

- Uncertainty around whether the medicine will be used in the right patients, e.g., responder schemes, or via value-based pricing schemes where the price differs across indications or patient groups.
- Uncertainty at launch around clinical or economic outcomes (effectiveness vs. efficacy, final outcomes vs. surrogates, questions around the ICER).

There are many possible research designs.



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Q3. Implementation: How should the PBRSA be organized /operationalised?

For a selected PRSA, how will the research (under Q2) be linked to the mechanics of the scheme, e.g., any price or revenue adjustment; rebates to be paid during the course of the scheme (in the case of responder based reimbursement), extension of restriction of the subgroups of patients who will have access to the treatment; process for review of reimbursement status? Fulfilment of adverse event reporting requirements.



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Q4. Evaluation: Has the PBRSA achieved its objectives? Was it good value from a societal perspective?

This is the *ex post* question. It links back to expectations /assumptions in Q1. Are we more knowledgeable about the technology in question? Have patients benefited from access? How do the costs of the scheme relate to the value of the benefits?