Improved methods and actionable tools for enhancing HTA

# Checklist for a Rare Disease Treatment
# Is an Outcomes-Based Managed Entry Agreement Feasible?

Criteria for use by a Health Technology Assessment (HTA) body or Marketing Authorisation Holder (MAH) to determine whether an Outcomes-Based Managed Entry Agreement (OBMEA) with mandatory data collection for re-appraisal (Coverage with Evidence Development) is feasible for a rare disease treatment (RDT):

**Answer "yes" to all statements.**

1. A price has been agreed for the RDT that has been developed responsibly to support sustainability of the health system (as stated in the pricing and reimbursement/ commercial agreement).

2. High therapeutic benefit is predicted, but there are major uncertainties that affect internal or external validity of the clinical effectiveness, or the economic evaluation, such that the treatment would not be recommended by the appraisal process.

3. Decision-relevant uncertainties in clinical effectiveness that drive determination of therapeutic benefit and/or cost-effectiveness can be resolved or substantially reduced with additional data collection within a reasonable timeframe.

4. Planned or ongoing studies, or post-licensing data collection activities will not resolve all the decision-relevant uncertainties for this RDT at the time of re-appraisal.

5. Additional data collection is feasible and of value:

    5.1 A data collection plan/protocol can be developed with stakeholders that includes clear research questions related to the decision-relevant uncertainties and outlines the study design, data sources and analytical plans.
    (This should be approved by the HTA/Payer to ensure it is likely to provide data of sufficient quality to resolve the uncertainties whilst limiting the clinical and administrative burden placed on all stakeholders.)

    5.2 If needed, ethical approval can be obtained timeously.

    5.3 Patients, clinicians and the MAH will commit to participation in the OBMEA for the required timeline (recognizing that if a new treatment becomes available, clinicians/patients may wish to alter treatment).

    5.4 Data of sufficient quality and quantity can be collected within the timeframe of the OBMEA (and combined with other data generated internationally since the initial HTA) to inform re-appraisal or future reimbursement decisions, recognizing that some rare diseases may have small, heterogeneous populations and study durations may need to be longer.

6. The manner in which the data from the OBMEA will be used in re-appraisal is clearly specified and agreed by all parties.

7. There is a mechanism agreed with patients and clinicians to enact disinvestment or delisting after the re-appraisal, if required, with explanation of what happens to patients currently on treatment.

**A "no" to any statement means that an OBMEA is not likely to resolve the decision-relevant uncertainties. More clinical trial evidence, or a price reduction, or other inputs to the determination of value are needed.**

This checklist has been developed as part of the IMPACT HTA project: Work Package 10 Appraisal of Rare Disease Treatments. It was developed following a targeted literature search of criteria for determination of when to use OBMEA, discussed with HTA/Payer experts in OBMEA workshops and consulted upon in the international HTA community.

Other tools to support use of OBMEA for RDTs can be found at Impact HTA | Health Technology Assessment | Work Package 10 (impact-hta.eu).

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