Health Technology Assessment Innovation Laboratory report: rapid entry to managed access

1. Background

As part of NICE’s implementation of a [proportionate approach to technology appraisals](https://www.nice.org.uk/about/what-we-do/proportionate-approach-to-technology-appraisals) (TAs) from 2023 to 2024, [NICE's new Health Technology Assessment Innovation Laboratory (HTA Lab)](https://www.nice.org.uk/news/blog/a-safe-space-for-addressing-complex-health-technology-assessment-challenges) is addressing the challenge when a topic is known from the outset to have uncertainty that can be resolved by further data collection.

Presently, a full TA based on uncertain evidence is done before such technologies can enter [managed access](https://www.nice.org.uk/about/what-we-do/our-programmes/managed-access). A second full TA is done when the managed access period ends.

The HTA Lab has been exploring if technologies that are identified as highly likely to enter managed access could follow a rapid entry to managed access (REMA) process, taking a new approach to the first TA before entry to managed access. The value signals from the TAs done on these technologies are typically highly uncertain, so an accelerated process may offer a more proportionate approach that supports timely patient access to promising new treatments.

This report summarises the work undertaken by the HTA Lab to date and outlines the proposed next steps.

1. Scoping work

A review of NICE evaluations that led to a managed access agreement between January 2019 and June 2022 was done to understand trends and patterns for predicting a managed access recommendation. This identified 33 topics (27 cancer topics, and 6 non-cancer topics or highly specialised technologies).

Analysing the topic characteristics revealed that being part of the [Project Orbis programme](https://www.gov.uk/guidance/guidance-on-project-orbis) increased the likelihood of a managed access recommendation for cancer topics. Project Orbis is coordinated by the US regulatory body, the Food and Drugs Administration (FDA), as an expedited process for reviewing and approving promising cancer drugs.

The scoping exercise also showed that NICE’s managed access team in partnership with NHS England (NHSE) could identify topics requiring managed access early in the process. Over 90% of the 33 topics were triaged as moderate or high potential for managed access based on information provided at the NICE decision problem meeting, held around 6 months before the appraisal committee meeting.

1. Stakeholder engagement events

To conceptualise a REMA process, the HTA Lab used a collaborative way of working using a ‘policy sandbox’ approach (see [Leckenby et al. [2021]](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8545721/)). This involved doing multistakeholder engagement events to co-develop and pilot a potential approach to achieve the key objectives outlined in the section on background.

The sandbox workshops were facilitated by NICE HTA Lab staff, who presented the business case for a novel REMA process and initial ideas of what such a process might look like to stimulate discussion from participants.

These engagement events generated valuable insights. The discussions were thematically analysed to capture the key messages. These are shown in Box 1.

In summary, stakeholders agreed that:

1. each REMA topic should have at least 1 full appraisal, either at entry into or at exit from a managed access period
2. the process needs to generate a value signal
3. the process should not encourage or indirectly increase use of managed access
4. the process should align with the [Innovative Medicines Fund principles](https://www.england.nhs.uk/publication/the-innovative-medicines-fund-principles/).
5. Focused engagement events

NICE also held focused engagement events with NHSE in addition to the stakeholder engagement events. It was clear that a key NHSE requirement, which aligns with the principles of the [NHS Commercial Framework for new medicines](https://www.england.nhs.uk/medicines-2/commercial-medicines/nhs-commercial-framework-for-new-medicines/), is for the NICE evaluation process to define the range of plausible incremental cost-effectiveness ratio (ICER) estimates to inform its commercial discussions to manage the identified risk within managed access. This meant that radically redesigning the process of entry to managed access to one that does not involve economic modelling would be difficult because it would also require an overhaul of the NHSE commercial negotiation process and the policies upon which it is based.

An engagement event was also held with the Association of the British Pharmaceutical Industry (ABPI), representing an industry perspective. The ABPI was supportive of a streamlined process that results in a managed access recommendation for promising new treatments. It expressed willingness to collaborate on designing an alternative approach including the potential submission of early economic models and/or exploring alternative approaches to pricing during the managed access period.

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| Box 1: Summary of key themes from the sandbox events**Business case for REMA*** There are NICE business and system-level needs to evaluate technologies that receive early marketing authorisation.
* Technologies likely to require managed access can be identified at an early stage.

**Broad approach to REMA*** Technologies should have at least 1 full technology appraisal, either at managed access entry or exit.
* For REMA, some form of early value assessment is required to inform the price during managed access.

**Potential risks and challenges*** REMA should not encourage or indirectly increase the use of managed access. Simple pricing arrangements are preferred and will remain suitable for most technologies.
* REMA should not undermine other health technology assessment initiatives and criteria should align with the [Innovative Medicines Fund principles](https://www.england.nhs.uk/publication/the-innovative-medicines-fund-principles/).
* Resources required to implement REMA and identify appropriate topics may offset some efficiency savings from doing fewer appraisals.

**Operationalising REMA*** Unmet clinical need for a technology and the usefulness of data collection should be key criteria for a technology to use a REMA process.
* Decision making within REMA should be transparent and fair.
* Cancer technologies may be most suited to pilot REMA.
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1. Proposed approach

Based on the scoping work, multistakeholder discussions and focused engagement events, it was clear that it will not be possible to implement a completely new process in the time available. NICE will therefore be working with system partners and stakeholders to ensure a robust policy option is developed moving forward.

In the meantime, a pragmatic short-term solution was to develop a streamlined decision making approach as a first step to improve entry to managed access. The proposed streamlined approach (referred to as streamlined entry to managed access [SEMA]) aligns with the proportionate approach to TA streamlined decision making. This process ensures that the key checkpoints in a traditional appraisal process are still followed, and the same outputs are produced.

The SEMA process follows the standard single TA process until the external assessment group (EAG) report stage, after which a decision is made about whether the appraisal could be streamlined, rather than going through a full appraisal committee meeting.

When deciding on the suitability for streamlined decision making, NICE will take into account the risks associated with the appraisal and the decision to streamline. For managed access specifically, this should include that the topic is highly likely to both meet all the normal criteria for entry into managed access **and** not be recommended for routine use.

This streamlined process removes the need for a full committee meeting while enabling a robust assessment to determine the appropriateness of managed access. A full evaluation upon exit from managed access will still be required to review all clinical and economic evidence including that collected during the period of managed access.

If at the progression decision point NICE decides that the technology is not appropriate for streamlined decision making, it will proceed as a standard single TA with a full committee meeting.

1. Retrospective pilot

Because of a lack of appropriate topics scheduled for evaluation, a simulated pilot was done. In the technology appraisal of trastuzumab deruxtecan for HER2-positive unresectable or metastatic breast cancer (TA862), technology appraisal committee A made a managed access recommendation in December 2022. It was agreed to pilot this topic through the proposed SEMA process.

A NICE technical team developed the committee papers and presentation. This documentation only included information that would have been available at the first committee meeting.

The subset of committee members agreed the technology could not be recommended for routine use and was suitable for managed access. They felt it demonstrated plausible potential to be cost effective but considered the external assessment group (EAG)-preferred assumptions may be more realistic. They confirmed that additional data collected through the ongoing trial and Systemic Anti-Cancer Therapy (SACT) dataset would help reduce this uncertainty. Overall, the subset of committee members was able to make a managed access recommendation based on the information available at that point in the process.

Overall, the SEMA process enabled decision making and culminated in committee conclusions that broadly aligned with the original appraisal and, based on the input from the NICE commercial liaison team, provided the same information needed for NHSE commercial negotiation. This suggests that the SEMA process would have been feasible and would have allowed faster patient access as well as efficiency savings for the technical team.

1. Recommendations for implementation

**Stakeholder input is key to ensuring topics are correctly identified as having potential for streamlined entry into managed access**

* Rationale: The effectiveness of the SEMA process is based on the ability to streamline decision making. In more complex cases a full committee discussion would be more appropriate. It is equally important that SEMA is not seen as a ‘less-robust’ appraisal process.
* The effectiveness of this process depends on a full understanding of the potential for managed access and the associated risks at the progression decision stage. Stakeholder input is therefore vitally important in supporting this decision. This is currently undertaken as part of normal NICE processes through company submission and NICE and NHSE feasibility assessment to ensure that NICE understands the willingness to enter managed access from all parties ahead of the decision to streamline.

**Members of the streamlined decision making committee should be given the same amount of preparation time as for a full committee meeting**

* Rationale: Even though the SEMA committee meeting was shorter than a full committee meeting, the committee members fed back that it still took the same time to read the papers beforehand.

**Representatives from NHSE and the EAG should be present at the SEMA committee meeting**

* Rationale: Because SEMA committee meetings are for technologies suitable for managed access, they have inherent uncertainty relating to clinical outcomes. For this reason, NHSE and the EAG should normally be present to provide input and explanation about the impact of this uncertainty, and support understanding and decision making by the committee subset.

**NICE should provide advice to the committee subset that it considers managed access appropriate**

* Rationale: This was received as feedback from the committee members doing the simulated SEMA pilot. NICE advice empowers the committee subset to be confident in the process, ensures all the relevant information for commercial negotiation and data collection agreement is collected, and minimises the risk of streamlined decision making.
* Further piloting this streamlined process with live cancer and non-cancer topics will allow for further lessons to be learned and identify necessary refinements that need incorporating.
1. REMA

The SEMA process allows a short-term pragmatic solution to support streamlined entry to managed access. However, there remains an ambition to develop a process to allow even faster entry to managed access (REMA), which aligns with key policy developments in this area to accelerate the regulation and evaluation of pharmaceutical products.

Further research will be undertaken alongside stakeholder engagement to examine potential approaches and inform this process redesign.

1. Conclusion

Extensive engagement with system partners and stakeholders has confirmed that there is a clear need for a more streamlined and accelerated process for entry into and exit from managed access agreements. This is to allow patients timely access to promising new treatments that have an uncertain evidence base. Streamlining decision making is the first pragmatic step in redesigning this process in advance of further activity to develop and enhance accelerated policy options. Suggestions and issues raised by system partners and stakeholders during the sandbox workshops will directly inform further development of a redesigned REMA process.