

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE
CENTRE FOR HEALTH TECHNOLOGY EVALUATION

Note to describe procedures at NICE to support the Early Access to Medicines Scheme

Introduction to the Early Access to Medicines Scheme

1. In April 2014, the Government announced the launch of the Early Access to Medicines Scheme (EAMS). EAMS provides an opportunity for important drugs to be used in UK clinical practice in parallel with the later stages of the regulatory process. It is anticipated that medicines with a positive EAMS opinion could be made available to patients 12-18 months before formal marketing authorisation.
2. Under the scheme, the Secretary of State for Health, acting through the Medicines and Healthcare products Regulatory Agency (MHRA) issues a scientific opinion on the benefits and risks of a new medicine or indication. This opinion provides additional information for clinicians and patients to assist in treatment decisions in areas of unmet medical need.

Key stages of EAMS and NICE's role in them

3. The following paragraphs outline the key stages of EAMS and NICE's role in them.
 - i. **Promising Innovative Medicines (PIM) designation and NICE Topic Selection** – A PIM designation gives an early signal that, based on the evidence to date, the medicine may be a possible candidate for the Early Access to Medicines Scheme and thus has the potential to be of value in areas of unmet medical need. The MHRA operates the process resulting in a PIM designation.

Companies signal their intention to apply for a PIM designation and EAMS at an early stage by completing the relevant fields in UK PharmaScan. The MHRA advises NICE of the award of a PIM designation, subject to this information being treated as commercial in confidence unless and until the information is made public or otherwise disclosed by the company.

When NICE becomes aware of a PIM designation, the topic progresses through the usual NICE Topic Selection process (unless the technology is already covered in existing NICE guidance or is not within NICE's remit, for example a vaccine), except that products with a PIM designation are prioritised.

- ii. **Joint scientific advice** – A PIM designation may be granted to products at an early stage in clinical development. It is strongly recommended that companies which receive a PIM designation seek joint scientific advice. This gives companies an opportunity to meet with both NICE and the MHRA to discuss their development plans at length from both a HTA and a regulatory perspective, and then to receive a bespoke written advice report addressing key questions. NICE Scientific Advice is offered on a not-for-profit fee-for-service basis.
- iii. **Pre-submission meeting (MHRA)** – The MHRA holds one pre-submission meeting to ensure that all the information they need to reach an EAMS opinion is available before a company formally submits evidence for an EAMS opinion.
- iv. **NICE EAMS meeting** – The NICE Office for Market Access offers companies the opportunity to have a supplementary meeting with NICE to discuss the company's data collection plans during the EAMS period, in order to help ensure the company is well prepared for a potential Technology Appraisal (TA) or Highly Specialised Technologies (HST) evaluation. In some cases, data on clinical and cost effectiveness may

need to be generated during the EAMS period to address the uncertainties in the clinical effectiveness evidence or anticipated resource use. These data will inform any subsequent submission to NICE. The NICE EAMS meeting aims to help companies gain insight into the NICE processes and evaluation frameworks, as well as to learn more about the option to develop and propose managed access arrangements and patient access schemes to support adoption following the EAMS period and NICE appraisal.

NICE EAMS meetings are optional and offered on a fee-for-service, not-for-profit basis. It should be noted that NICE EAMS meetings are not a substitute for joint NICE and MHRA scientific advice, where much more detailed engagement on prospective clinical development plans is offered.

In planning for the EAMS period, including potential additional evidence generation, an arrangement between the company and NHS England will be necessary. NHS England is invited to the NICE EAMS meeting to discuss the necessary arrangements.

If a ministerial referral to NICE has already been made for the topic, a company may consider the standard TA decision problem meeting to be a sufficient forum to discuss plans for their submission to NICE, particularly if further evidence generation is unlikely to be feasible at this late stage. The decision problem meeting is a standard part of the NICE TA and HST processes and no charge is made for this meeting. A company may however believe that further evidence generation during the EAMS period would be both meaningful and feasible, and may ask for a separate NICE EAMS meeting.

Detailed procedures for NICE EAMS meetings are in the appendix.

- v. **Positive EAMS opinion** – This is granted by the MHRA and is expected towards the end of the development process (typically at the end of phase

III trials). It can exceptionally happen earlier. The EAMS opinion enables prescribers and patients to decide if the product might be suitable for an individual patient. Products with a positive EAMS opinion could be available to NHS patients 12-18 months before marketing authorisation is granted. The MHRA expect that EAMS products will be provided by the company to the NHS free of charge during this period.

- vi. **EAMS period and NICE TA or HST evaluation** – NICE anticipates that before the EAMS period starts, all EAMS products will already have been selected as topics for a NICE TA or HST evaluation.

In order to develop timely guidance, NICE starts the TA or HST evaluation during the EAMS period (before marketing authorisation), and the company prepares its submission during this period. Any data collected during the EAMS period may be included in the company submission.

A NICE TA or HST evaluation of an EAMS product follows the normal published processes and methods. If NICE is notified of the PIM designation and positive EAMS opinion at least 12 months before expected receipt of marketing authorisation, these products are planned as a priority into the work programme so as to allow the first Committee decision to be published within 3 months of marketing authorisation, rather than 6 months under the usual process.

Prioritising EAMS products for evaluation may have the effect of deprioritising other products if there are late changes in the regulatory timelines for the EAMS product.

- vii. **Marketing authorisation and NICE TA or HST evaluation recommendations** – In line with standard processes, NICE issues preliminary recommendations for public consultation or final recommendations (where there are no issues requiring public consultation) as soon as possible after marketing authorisation.

If the Appraisal Committee or HST Evaluation Committee agrees that the product can be recommended, these recommendations are subject to the 2013 Regulations. These provide for a legal duty for NHS England, clinical commissioning groups and local authorities to make treatments recommended by NICE available to patients. The regulations normally require that products recommended by NICE are commissioned within 3 months of publication of the guidance. NHS England reduces this to 30 days for EAMS products.

4. The components of the EAMS process are outlined in Figure 1.

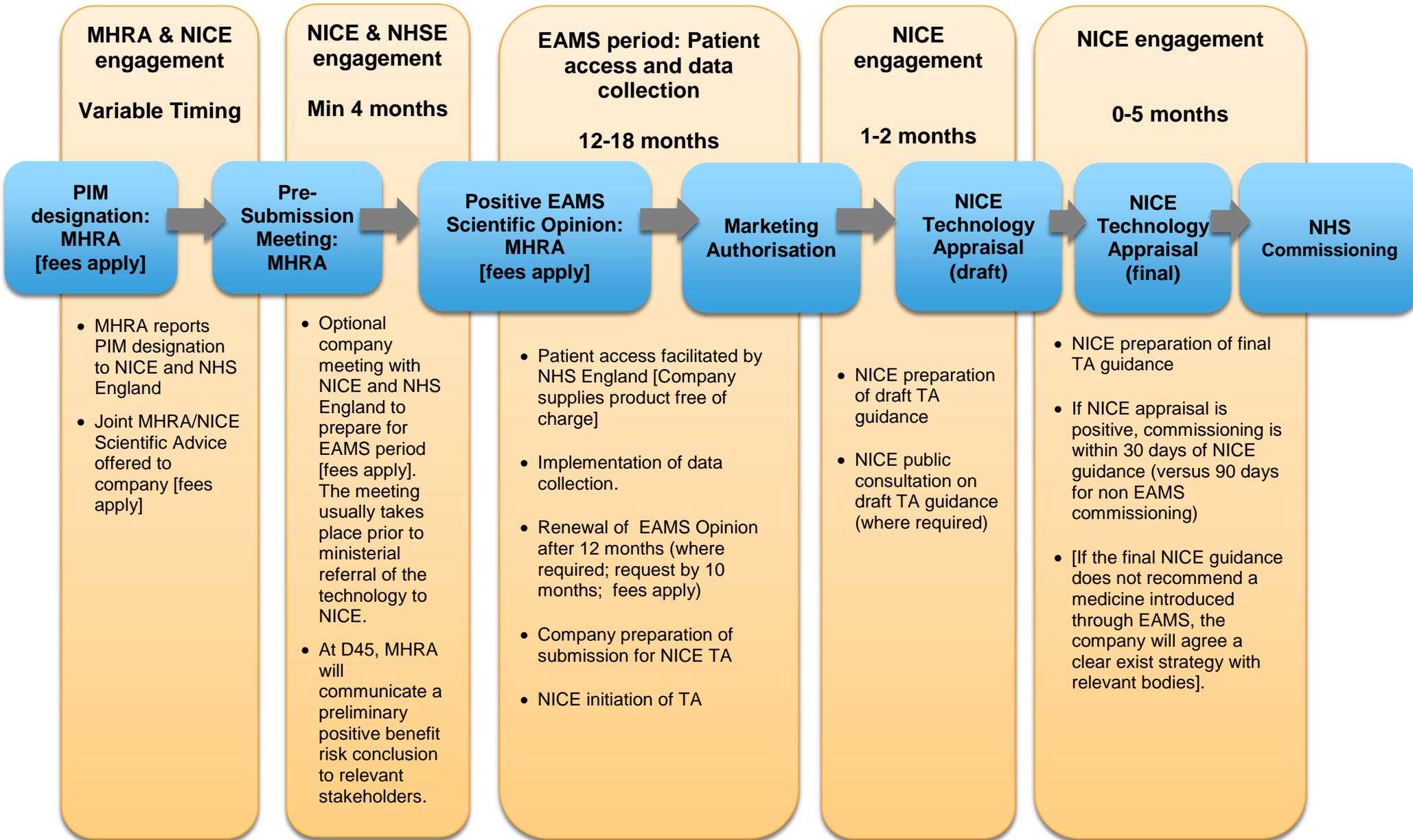
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Figure 1: EAMS Schematic



Appendix

Detailed procedures for NICE EAMS meetings

1. When NICE is notified that a PIM designation has been given and that the company has requested a pre-submission meeting with the MHRA, the NICE Office for Market Access offers the company an optional NICE EAMS meeting.
2. Companies wishing to take up the offer are advised to give the NICE Office for Market Access details about when the company anticipates engaging in the pre-submission meeting with MHRA. The NICE EAMS meeting is scheduled to align with the timelines provided for the MHRA pre-submission meeting. The NICE EAMS meeting usually takes place before Ministerial referral of the topic to NICE.
3. The company is asked to provide the pre-submission template submitted to the MHRA and to complete a NICE pro-forma to describe any plans for further evidence collection, for patient access schemes or flexible pricing.
4. The NICE Office for Market Access team considers the plans as set out in the pro-forma. Where evidence development during the EAMS period is likely to be important in reducing uncertainty prior to a NICE appraisal or evaluation, the Office for Market Access draws together a bespoke team of experts from NICE Scientific Advice, Technology Appraisals or the Highly Specialised Technologies programme as appropriate. The NICE EAMS meeting therefore provides an opportunity for tailored engagement and expert advice on plans for further evidence development.
5. The meeting is chaired by an Associate Director or Programme Director from the Centre for Technology Evaluation (CHTE).
6. Representatives from NHS England are invited to the NICE EAMS meeting. This enables NHS England to explore whether a commissioning circular would facilitate patient access during the EAMS period. It also allows discussion about

arrangements that may need to be put in place to facilitate and coordinate data collection during the EAMS period. If a company does not wish to take up the NICE EAMS meeting offer, it may still wish to engage with NHSE separately.

7. Where there is likely to be high uncertainty at the point of NICE appraisal or evaluation, the NICE EAMS meeting provides an opportunity to discuss the options for 'managed access arrangements' after the EAMS period. This could take the form of continued data collection and the introduction of patient access schemes. In this case, the Office for Market Access draws in a representative of the Patient Access Scheme Liaison Unit to attend the NICE EAMS meeting.
8. NICE EAMS meetings are provided by the Office for Market Access on a fee-for-service, not-for-profit basis. Further details on fees are provided in the NICE EAMS meeting invitation letter to the company.