NICE consultation on proposals to increase capacity within the technology appraisals programme

The NICE technology appraisals programme recently consulted publicly on proposals to increase capacity within the work programme.

This report presents the response received and changes made.

NICE, January 2018

Executive summary

1. The consultation received a generally supportive response to the proposals put forward, with the proposals for involvement of clinical experts and patient representatives at the committee meeting as the (only) exception.

2. We propose to make the following changes to the proposals for:

    - membership of the 'technical team';
    - attendance of clinical expert and patient representatives at the appraisal committee;
    - arrangements for consultation on optimised recommendations, and;
    - publication of final appraisal guidance relative to marketing authorisation.

Background

The proposals

3. The TA programme held a public consultation on proposals to change the existing process, that sought to:

    - Provide clear, recognisable milestones for companies and other stakeholders, linking them to key stages in regulatory pathways, providing more time for NICE to engage with companies early in the appraisal process;
    - Release capacity for the appraisal committees as more of the scientific and technical elements are pulled forward into the workup of topics. This should allow us to publish up to 75 appraisals per year, using the same committee resource that is now available;
    - Enhance our ability to deliver the ambitions set out in the Accelerated Access Review and the emerging Life Sciences Strategy, when required to do so.

The consultation

4. In October 2017, NICE published a public consultation containing proposals to change aspects of the NICE Technology Appraisal programme.

5. In summary, the proposals covered:

- Defining more points within the process for formal discussions with companies on the technical and commercial elements of their submission.
- Allowing more opportunities for scientific and technical issues to be addressed before a topic reaches the appraisal committee decision stage.
- Providing for more efficient consultation mechanisms.
- Alignment of the timeliness targets for all technology appraisal output, with guidance within 90 days of marketing authorisation for all new drugs.
- Altering the attendance of patient, clinical and commissioning experts at appraisal committee meetings.

6. The public consultation, which closed on 16 November 2017, received 78 responses. In addition, a webinar for stakeholders (80 people registered to attend in total) and a face-to-face event in Manchester (40 attendees in total) were held, along with a number of individual meetings with key stakeholder groups. Figure 1 shows the breakdown of groups/organisations that submitted consultation responses, figure 2 shows the breakdown of responses by subject heading:

Figure 1

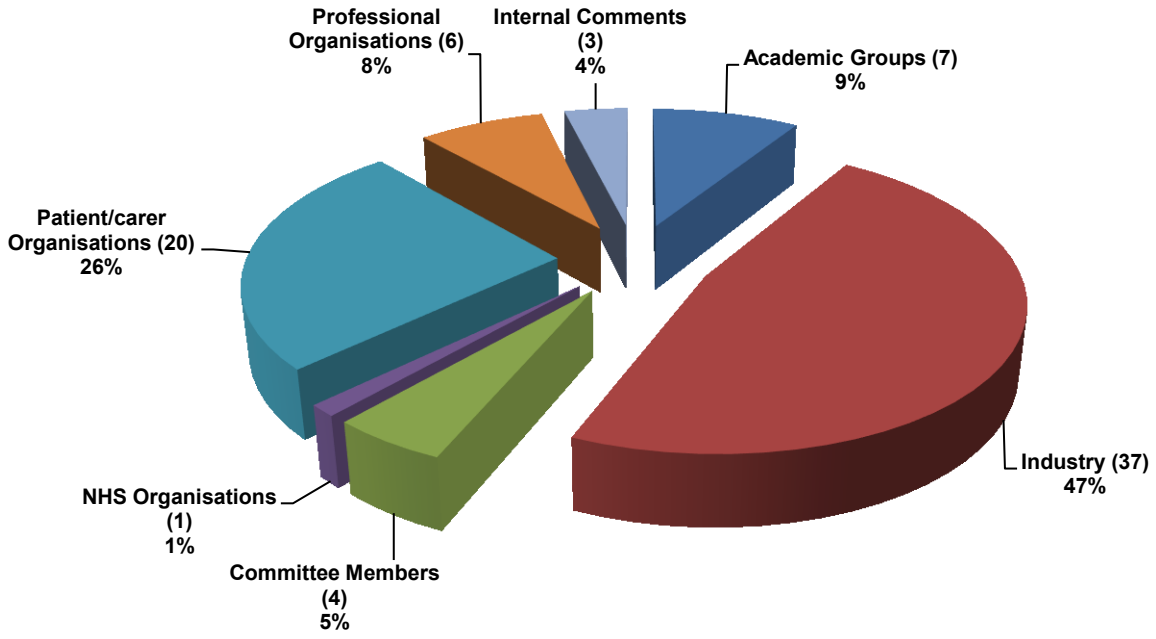
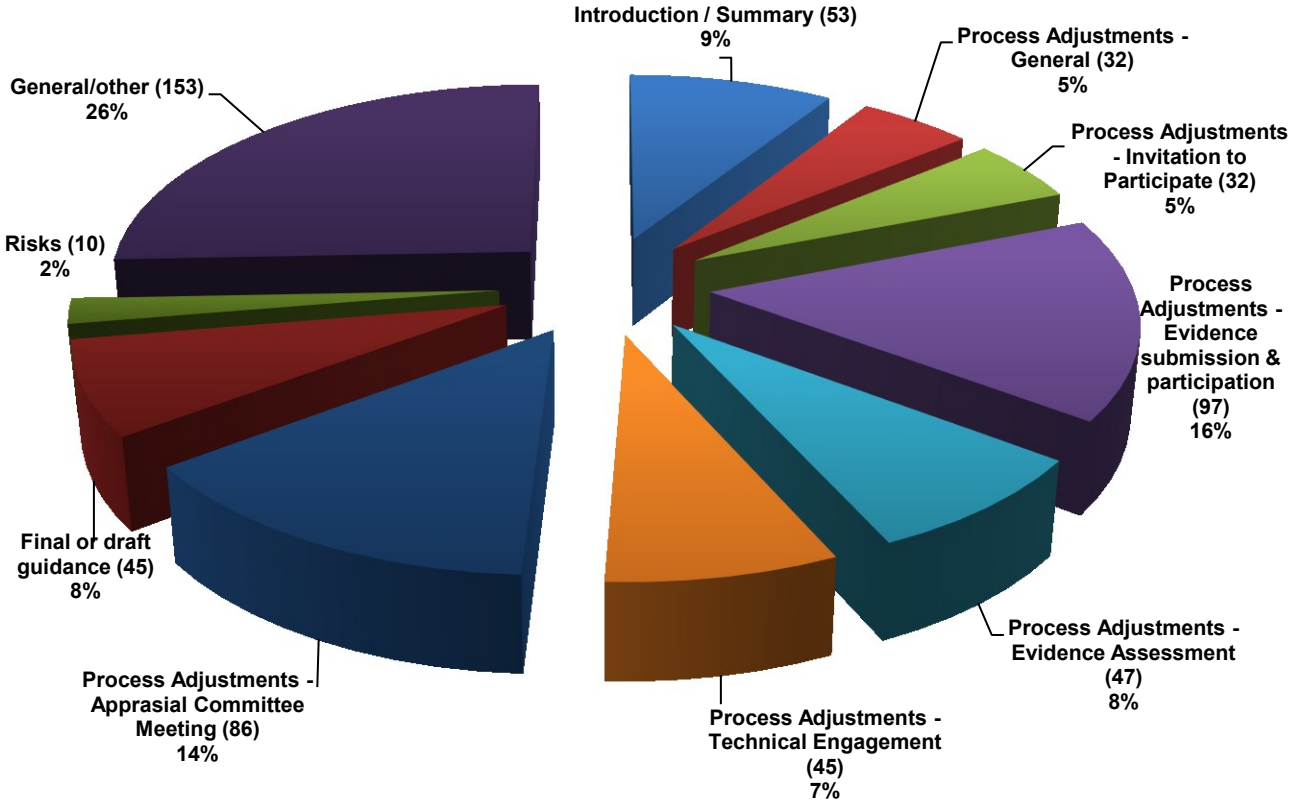


Figure 2



General Points

7. Virtually all respondents support the concept that the NICE technology appraisals programme needs to increase its capacity in order to be able to publish 75 appraisals per year. The Department of Health emphasise the importance of clarity about the resources being in place to support the increase. Some urge NICE to go further and consider opportunities to select technologies for appraisal that wouldn't meet the current topic selection criteria. Others suggest that the concepts put in place to increase capacity within the technology appraisals programme should also be considered within the highly specialised technologies programme in order to increase capacity from the current 3 per year.

8. Most respondents react positively to the idea that the increase in capacity is best achieved by ensuring that more opportunities are provided for resolving issues of scientific uncertainty before the committee meets. Allowing for expert advice to be sought, engagement on the technical aspects, and commercial interactions before committees meet, is welcomed as a means of reducing the number of occasions where more than one committee meeting is required for the majority of topics. Some respondents acknowledge that multiple committee meetings resulting in multiple consultation documents leads to significant resource issues at their end.

9. Industry respondents argue that the consultation should have gone further and set out proposals for more extensive changes to methods, beyond those needed to increasing efficiency. Their suggestions include consideration of 'multi-indication and combination pricing', governance arrangements for patient access schemes, topic selection for the highly specialised technologies programme, adjustments to the appeal process to accommodate the proposals laid out in consultation, and NICE's plans for cost recovery. These suggestions go beyond the scope of the current consultation and would require extensive engagement with the Department of Health and NHS England, amongst other stakeholders, before being taken forward.

Defining more points within the process for formal discussions with companies on the technical and commercial elements of their submission

Summary of comments received

10. There is strong support from respondents for the proposal to incorporate more points within the process for formal discussions with companies on the technical and commercial elements of their submission. Industry in particular welcome the opportunity to increase dialogue with NICE (and NHS England) before the 1st appraisal committee meeting.

Response, including amendments to the proposals

11. The consultation document did not contain any procedural information about the type of discussion or indicative timelines of when, where and the particular format in which these would take place. As noted above, phase 1 of the consultation was to explore the principles of the changes that we propose to make to the existing processes to ensure that there wasn't a fundamental issue that stakeholders would find unacceptable. Respondents ask for more information on the frequency and format of any engagement/dialogue opportunities and so we have provided more detail in the process guide (as originally intended).

Allowing more opportunities for scientific and technical issues to be addressed before a topic reaches the appraisal committee decision stage

Summary of comments received

12. The majority of respondents express support for introduction of this principle. In particular, the suggested new process step of 'technical consultation' ahead of the 1st appraisal committee meeting is well received. A recurrent theme is

the need for companies to take seriously the engagement with NICE, and others in the system, treating it as the definitive opportunity to present a robust analysis and to submit their best offer/price. The process must provide the incentives, and disincentives, for a commercial offer to be introduced and evaluated before the committee meeting; for example, NICE should not accept model alterations and new evidence after the full committee meeting is held, or FAD is issued.

13. In the consultation document, we introduced the idea of 'technical team', consisting of NICE staff, the evidence review group (ERG) and key appraisal committee members, that would take a lead in managing the evidence submission phases of the process. We indicated that the technical team would be developing a report for committee along the lines of the 'pre-meeting briefing' that is currently used, but with an additional expectation that this report would include scientific 'judgements' to be reached by the technical team, which in turn would allow stakeholders, and in particular the company, to respond in consultation before committee meets with further evidence, clarification or explanation, or seek a commercial solution.

14. A number of respondents express concern that the 'technical team' appears to be functioning as an unofficial appraisal committee. They ask for the roles and responsibilities of the team in general, and of specific members in particular, to be made clear, and rules and approaches to decision-making to be identified. For example, respondents from industry point out that ERGs approach their work in different ways – some put more emphasis on providing a worse-case scenario, where others focus on establishing what might be seen as a most plausible case – which will not be helpful where they are expected to also be co-authors of a technical report expressing scientific and technical 'conclusions' before committee. The ABPI suggests that adding a company representative to the 'technical team' could be another way of addressing these concerns. We have also heard from the ERGs that their involvement in a technical team could compromise their role in providing an independent assessment of the company's evidence, and the subsequent input at the committee meeting.

15. A recurrent theme in the responses received is to ensure that committees must continue to feel engaged if more of the (scientific) judgements are to be made before it meets: the chairs of committee note that 'for the committee to have confidence in the information put before it in the amended process, it has to be satisfied that the reasoning behind both a potential positive and negative decision have been explored in a balanced way with meaningful input from clinical and patient experts'. A number of respondents call in this context for NICE to look carefully at the skills and competencies of its staff to ensure that they are capable and (feel) empowered to come to the (scientific) judgements

required. Others raise concerns about the capacity of individual committee members to participate in the technical team; both in terms of time commitment and skills/competencies.

16. Some respondents identify the increased need for consistency between committee streams in handling and approach to regularly returning issues of scientific (value) judgements. Examples given are the consideration of utility values for common cancers like breast cancer, costs for intravenous chemotherapy administration and costs of palliative care. It is suggested that this would not only aid consistency in decision making, but would also be much more efficient than having the same debates time after time in every appraisal. In the same vein, it is suggested that there will be circumstances where multiple technology appraisals are both more efficient and more useful to the NHS than single technology appraisals. Plus it would be in the interest of all parties if tighter constraints could be placed on the length of company submissions and ERG reports, as well as input from the new technical team, including presentations to committee.

17. Respondents also express a desire to receive more information on the content and phrasing of the report that is expected to be issued for consultation. They also ask for NICE to uphold its principle regarding transparency of decision making, arguing that transparency may be compromised if the committees do not discuss technical and scientific issues in depth at their meetings. Some suggest that providing 20 working days to respond to the consultation may not be sufficient, and that NICE may need to consider an extension to this timeframe on a case by case basis to allow for availability/generation of relevant evidence.

18. One respondent, the director of ScHARR-TAG, supported by colleagues from the group of ERGs, proposes for the current process up to the first committee meeting to be largely maintained, with some scope for efficiencies in terms of presentation slides, and a slightly more elaborate factual error check before committee, but add to this a much more focussed set of process steps after the meeting. They describe their proposal as: ‘At the end of the first meeting, the appraisal committee would arrive at their preferred assumptions, and assuming that NICE can restrict the submission of new (non-price related) evidence following a negative recommendation, the only option for a company (excluding appeal) would be to submit a commercial offer’. They go on to propose that: ‘once submitted, the NICE technical team can evaluate the new incremental cost effectiveness … which can be shared with the appraisal committee who, via email exchanges, can decide whether to accept that the revised incremental cost effectiveness ratio is sufficiently low, and robust, to allow a positive recommendation to be made’.

19. The ERGs further note that not evaluating submissions where a company submission involves a large incremental cost effectiveness ratio would (also) increase the number of available appraisal committee slots.

Response, including amendments to the proposals

20. The broad support for the proposals suggests that we do not need to make substantive changes to the principles laid out in consultation. We accept that we need to be clearer about the roles and responsibilities of those involved in the adjusted processes, and in particular those involved with the new 'technical team'. We intend to make two changes in this regard: 1) not include the ERG as a formal member of the 'technical team', and 2) put a greater emphasis on participation of the committee chair in the 'technical team'.

21. We accept that ERG membership of the 'technical team' is not a prerequisite for meeting the capacity challenges put forward in this consultation, and we therefore will not include them in the technical team when we operationalise the proposals. Nor do we consider the addition of a company representative to the 'technical team' as appropriate or necessary. Other opportunities exist for the company's position to be clearly articulated and the presence of a representative would compromise the objectivity and independence of the process.

22. An enhanced role of the chair of the appraisal committee in the new 'technical team' is expected to elicit the required response by companies to the scientific judgements put forward before getting to committee; because of the independence of the committee from NICE, companies are likely to treat the chair's judgement on what committee will or will not accept as a stronger signal for what to do next than a similar signal given from the NICE technical lead or the ERG. The chair's involvement at this stage further allows committee members to feel comfortable that their interests are represented, their independence maintained, and should allow them to continue feel engaged with the process under the new proposals. Finally, we will continue to support the meetings in which chairs and vice-chairs share experience and learning from individual appraisals, and will strengthen the governance surrounding these meetings.

23. We acknowledge the concern raised by some respondents about the ability of the proposed technical teams to get to the depth of scientific understanding that would otherwise be achieved by committee if it was allowed to continue to have the time to discuss a topic it now has. Although we absolutely accept that we will need to continue to support our staff in gaining the skills and competencies required to support the new technical teams, of which they are a key member, we do not subscribe to the notion that committees will have no time to come to sound scientific judgements. Indeed, we envisage committees

spending only marginally less time on individual topics in the meeting than they do now, and rather focus the effort of the technical team on preventing more than one meeting to be required for a topic to be discussed. Technical teams will have to be skilled and experienced enough to ensure that the pre-work identifies all the key uncertainties, and responses to them, but it doesn't detract from the very important role individual committee members have in coming to their own judgements about science and value.

24. We will keep under review the arrangements for participation by individual committee members as part of 'lead teams'; in terms of time commitment, potential for some form of remuneration, and governance arrangements regarding conflicts of interest.

25. We accept that in order for the new approach to succeed in limiting the number of committee meetings required to discuss a topic, one of the key areas of 'process discipline' for NICE will have to be the acceptance, or not as the case may be, of new evidence after committee has met, with the exception of commercial offers using the preferred assumptions expressed by committee in the meeting. We understand that this may mean that some companies will be reluctant to provide a submission to NICE at the time we invite them to. As is the case now, we will be open to discussing individual circumstances with individual companies when they arise, and will endeavour to seek solutions that allow NICE to produce timely guidance, while at the same time ensuring we are able to consider as much as possible of the evidence base that has been collected for a new technology (see also below under 'alignment of timeliness targets').

26. Finally, as stated previously, a technical report will be released to stakeholders before the 1st appraisal committee meeting, and before regulatory approval. In order for the technical engagement to be as meaningful as possible for all parties involved, NICE will need to provide maximum transparency at this step. Therefore we are proposing that all clinical documents and analyses designated confidential are shared with consultees and commentators in their un-redacted form during the technical engagement step. We will continue to keep commercial discounts and commercial access arrangements confidential to protect commercial confidentiality between companies.

27. All consultees and commentators sign a confidentiality, undertaking and acknowledgement form before we send documents to them. This provides a 'confidentiality club' to allow NICE to distribute the clinical information and analyses. The signed confidentiality agreement provides the necessary level of security and commitment to handling confidential information. The papers published on the NICE website will, as now, have all confidential information redacted. The information owner will retain the opportunity to check for correct redactions before information is put into the public domain.

Providing for more efficient consultation mechanisms

Summary of comments received

28. We received varying levels of support for the proposals regarding more efficient consultation mechanisms after the first committee meeting. There looks to be consensus that a requirement for consultees and commentators to respond to an ACD consultation within 10 working days would not be achievable. Some respondents suggest that this should be increased to 15 working days, and others stated that the standard 20 working days should be maintained. Some stakeholders indicate that an optimised recommendation does mean a particular group of patients will be declined access. For these patients, this is a negative recommendation and should be subject to the same consultation standards. Stakeholders would like more clarity to be provided as to the criteria to determine whether a second committee meeting will be held.

29. A number of respondents raise concern about the proposal for responses from a targeted ACD consultation to be reviewed (only) by the appraisal committee chair and members of the technical team. Conversely, the group of ERGs supports the proposal for committee to apply a light touch to consultation responses if they lead to the incremental cost effectiveness ratio being low enough to allow a positive recommendation; conditional on the committee having expressed their preferred assumptions at the first meeting, and the company being allowed to only submit a commercial offer as part of consultation.

Response, including amendments to the proposals

30. In light of the responses received, it is proposed that we do not continue to explore shorter, targeted ACD consultation arrangements for optimised recommendations. We will revert to the standard 20 working day stakeholder and public consultation.

31. We acknowledge the concerns raised about transparency of decision making when comments received in consultation are considered, but remain confident that with the right conditions in place, it should be feasible for the process to be run more efficiently. In circumstances where the committee is clear about its expectations after the first meeting, and where the company responds by making only an updated commercial offer, the Chair (with support from the technical team) should be given a mandate by members to decide, on their behalf, whether what is being proposed by the company is likely to result in positive guidance. Where this scenario applies, the Chair may decide that a second formal appraisal committee meeting is not required and a FAD is

drafted with the final recommendations agreed by the appraisal committee electronically.

Alignment of the timeliness targets for all technology appraisal output, with guidance within 90 days of marketing authorisation for all new drugs

Summary of comments received

32. The desire to align the timeliness targets for all technology appraisal output is welcomed by respondents, with some directly acknowledging the link to recommendations within the Accelerated Access Review and the vision within the Life Sciences Industrial Strategy. There is also broad recognition of the alignment of process steps with that of the regulatory process for medicines.

33. Respondents note that in order for NICE to allow for engagement on technical and scientific issues ahead of the 1st appraisal committee meeting, the appraisal would need to start earlier than the current process allows. Some companies raise particular concerns with this due to the increased potential for uncertainty in the evidence and the final regulatory decision. Some indicate that submissions could only be realistically received from day 121 of the regulatory process at the earliest, with some companies suggesting that day 150 or even day 180 would be a more appropriate point for submission of evidence.

34. Whilst industry colleagues support the principle to publish final NICE guidance within 90 days of receipt of a marketing authorisation, they note that there will be occasions where a company may wish to delay an appraisal, and that there should be flexibility to reschedule the process for a later date. Industry colleagues express the desire that individual companies should be responsible for proactive engagement with NICE in order to agree the most efficient and feasible timeline possible for a technology appraisal, based upon the expectation of mature data.

35. In order to help speed up the process of final guidance publication, respondents request that NICE consider reducing the time frame between close of appeal and the final guidance publication from 21 calendar days to 14 days.

36. The Department of Health has indicated that it wishes to consider the proposal to align the publication schedule for non-cancer and cancer topics in the context of the forthcoming renegotiation of the Pharmaceutical Price Regulation Scheme (PPRS).

Response, including amendments to the proposals

37. The TA programme already applies a degree of flexibility in scheduling of the work programme and this will be maintained within the new process. NICE remains open to discussion with companies to determine the optimum alternative timelines for an appraisal in certain circumstances. For example, where the release of a product into the UK market will take place sometime after the date of marketing authorisation, or where the pivotal evidence for clinical effectiveness, without which no reasonable value assessment can be performed, is not available. Where timelines are altered as a result, NICE will require the company to agree to complete transparency to allow other stakeholders to be informed and understanding of the requirement for the change.

38. In view of the Department of Health's position on the matter, we will not proceed with the proposal to align the publication schedule for non-cancer and cancer topics at this time.

39. In response to the request to reduce the time frame between close of appeal and the final guidance publication from 21 calendar days to 14 days, NICE are still considering whether this can be accommodated and the impact that it might have on the publishing schedule and links to the appeal process.

Altering the attendance of patient, clinical and commissioning experts at appraisal committee meetings

Summary of comments received

40. Virtually all respondents express a very strong concern about the proposal to change patient and clinical expert attendance at the appraisal committee meeting. They indicate that the proposal for a 'need assessment' to be conducted ahead of the appraisal committee meeting to determine whether clinical, patient and commissioning experts should attend is unacceptable.

41. Respondents indicate that patient and clinical experts add an invaluable insight into medical conditions and how a product may be of benefit. As such, they should be able to attend the meeting and not merely have their views represented in a written statement as the messages and insights may become diluted by their non-attendance. Furthermore, it is considered crucial that experts are available to answer queries of individual committee members to aid understanding.

42. Some stakeholders indicate that where direction of travel may be clear (or the committee decision highly predictable) from the technical engagement step, they would be willing to reconsider their attendance at the committee meeting.

Response, including amendments to the proposals

43. Some of the challenges may have revealed a misunderstanding that NICE were seeking to remove patient and clinical input from the process. This was certainly not the intention. The intention was to increase patient and clinical input into the appraisal process at a much earlier point, rather than a reliance on input at the appraisal committee meeting.

44. In light of the responses received, we propose that patient, clinical and commissioning experts would be invited to attend the 1st appraisal committee meeting. However, they will be provided with an opportunity to 'opt-out' of attendance at the meeting should they feel that their views are adequately reflected in the technical report, particular areas of uncertainty have been addressed, and they feel that attendance in person may not add any additional benefit.

45. Expert attendance at committee should not be a substitute for input into the technical consultation stage prior to the 1st appraisal committee meeting. The process guide has been updated to specify that along with consultee and commentator input at the technical consultation stage, it will be expected that the experts will also fully engage at this stage. Following this, NICE will liaise with the experts regarding their opportunity to 'opt-out' of attendance at the meeting, if that is their preferred approach.

Other changes to the process guide

46. Since publication of the current process guide in September 2013, the programme has published 2 addendums to the guide to address process elements of the new arrangements for the Cancer Drugs Fund and the introduction of the Fast Track Appraisal. We have also published a procedure as to how the Budget Impact Test will be applied. We have used the opportunity of this consultation on an updated process guide to combine the addenda and procedure note to create one new process guide. It is expected that this will provide greater clarity to users of the guide. The drafting of a new process guide further allows us to make reference to commercial and managed access arrangements.

Next steps

47. A 6 week targeted consultation on the updated Guide to the processes of technology appraisal, incorporating the changes proposed in the original consultation, and any amendments presented in this paper.

48. Phased implementation of the proposals from 1 April 2018 onwards.

National Institute for Health and Care Excellence

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