Introduction

This document sets out the Specialised Healthcare Alliance's (SHCA's) draft response to NICE's consultation on proposed changes to its processes for health technology assessment. It should be read alongside the proposals, which are available here.

The document is structured in line with the four themes of the consultation, as follows:

- Theme 1: Alignment of the current guidance development process
- Theme 2: Opportunities for new process improvements and ways of working
- Theme 3: Commercial and Managed Access Processes
- Theme 4: Objectives & vision of the Highly Specialised Technologies programme

For each theme, a general response has been provided that summarises the SHCA's views, with responses on specific proposals following below.

Draft response

Theme 1: Alignment of the current guidance development process

Would you like to provide general comments in relation to the proposals to align guidance development processes?

The SHCA supports NICE's proposals to remove inconsistencies and strengthen the support available to patients and patient organisations to engage across its health technology guidance development process.

The SHCA represents people living with rare and complex conditions. Many of our members are primarily focused on providing support to individuals and families and therefore have limited capacity and resources to dedicate towards participation in the development of NICE guidance. This challenge is compounded for some SHCA members by the complexity associated with NICE's guidance, particularly in relation to technology appraisals, and the difficulty in accessing clear information on processes.

In a previous survey of the SHCA's members, 30% of respondents said they had found it challenging to provide information in the course of an STA, while 60% reported encountering difficulties in finding information on the STA process. The challenge in participating in appraisals was more pronounced in the HST programme, with 18% reporting having found it very challenging and 36% stating it had been challenging.

The SHCA therefore welcomes NICE's proposals to provide additional guidance to patients and patient groups in the form of the summary of information for patients and the dedicated stakeholder relationship manager. However, we are concerned that the benefits of these changes could be undermined by proposals to reduce the consultation length for some topics. The SHCA asks that NICE introduces appropriate safeguards to ensure that reducing the consultation length does not limit the ability of patients and patient groups to participate in the development of NICE guidance.

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

Scoping consultation length will be flexible from 5-20 days dependant on the needs of the topic (para 49)

Disagree

The SHCA believes that scoping consultation timelines are already relatively brief. Given the importance of scoping in ensuring that technologies are assessed through the most appropriate route, it is vital that all stakeholders have sufficient time to review proposals and contribute their perspectives. There is a risk that shortening the timelines for consultation could reduce the ability of patient organisations to provide responses, or reduce the quality of responses. This is particularly strong risk in the pandemic/post-pandemic context, given many patient organisations/single-condition charities have seen a significant squeeze on their resources (income, staffing numbers) at the same time as an increase in need in their communities, and therefore have limited capacity to engage externally. As such, shortening consultation timelines could in turn impact on the appraisal itself, and ultimately the outcome.

The SHCA understands NICE's desire to reduce inefficiencies in its approach to guidance development but we do not consider that reducing the time available for scoping to be an appropriate way in which to implement changes. Seeking to shorten the scoping stage could lead to misinformed decision-taking on technologies and appeals, which may then create inefficiencies if the resulting guidance is challenged.

Scopes for simple topics will not be consulted on (para 51)

Disagree

The SHCA recognises that there may be cases in which all parties agree that there isn't a need for a formal scoping exercise and that this could create efficiencies in NICE's work programme and enable earlier access for patients. However, we believe that it is vital that appropriate safeguards are put in place to ensure that any decision to expedite the process would not impact on the ability of patients and patient organisations to contribute their views.

We do not consider that the proposal in NICE's consultation as it is currently worded provides sufficient assurances on how 'simple' topics would be defined, the circumstances in which an expedited process would be used or how the involvement of all parties in taking decisions would be ensured. We therefore cannot support the proposal in its current form.

Companies will provide a 'Summary of Information for Patients' with their evidence submission (para 59)

Strongly agree

We welcome the proposal for companies to develop a summary of information for patients (SIP). We hope that this will provide patients and patient organisations with a clearer understanding of the technology and the supporting evidence as well as the issues that are likely to be important in any given appraisal.

We would welcome a commitment from NICE to review the SIP process one year after its introduction, including working with patients and patient organisations to understand how the SIP could be strengthened.

NICE will provide dedicated stakeholder relationship managers for patient and carer organisations (para 63)

Strongly agree

The SHCA supports the proposal to provide dedicated stakeholder relationship managers to support patient and carer organisations to engage with the evaluation process from start to finish. We believe that this will be an important step forward in enabling and empowering patient and carer organisations to engage with the guidance development processes.

We would welcome clarity from NICE on how the system will work – for example, it is not clear from the consultation document whether these managers would be assigned to individual organisations, guidance programmes or specific appraisals. It will be important that the new system is communicated clearly to patient and carer organisations. This should take place directly, with a communication setting out the designated manager when stakeholders are invited to participate in appraisals, and through a page on the NICE website which clearly sets out their roles, responsibilities and contact details.

A shorter (less than 20 working days) consultation length can be used for some topics (para 72)

Disagree

The SHCA does not support proposals to enable consultation timeframes to be shortened in any circumstance for all guidance programmes, given the impact on our charity members, many of whom find it challenging to respond within NICE's existing consultation deadlines. As set out above, we believe this challenge has been compounded by the impact of the pandemic on resourcing in charities.

However, where guidance for the use of a technology is positive and does not contain any optimisations or restrictions, we recognise that there may be benefits in adopting a shorter consultation timeframe to accelerate the publication of final guidance and access for patients to the new technology.

The proposals as drafted do not specify the circumstances in which a condensed timeline would be introduced, and we cannot support them in their current form. We would welcome further clarity on this point from NICE and a commitment that shortened timelines would not be pursued where guidance is negative or optimised.

Committees will make recommendations on different types of guidance (TA, MTG, HST, DG) (para 67-68)

Agree

The SHCA welcomes the proposal to enable the highly specialised technologies committee to develop and make guidance on technology appraisals when required. The HST committee has a strong understanding of the challenges in assessing technologies for rare and complex conditions. The committee is therefore well placed to lead technology appraisals for these conditions. We would welcome guidance from NICE setting out when technology appraisals would be routed to the HST committee – we believe it would be appropriate for technologies that meet some, but not all, of the criteria for the HST programme to be routed to the HST committee, where capacity allows.

The option of a multiple technology assessment for highly specialised technologies (para 78)

Disagree

The SHCA does not support the proposal to introduce the option of a multiple technology assessment for highly specialised technologies. The consultation notes that the purpose of the proposal is to "increase flexibility, maximise resource, align working practices and provide timely guidance for the NHS".

However, it is not clear how the proposal to introduce a multiple technology assessment would support timely guidance, as the majority of technologies that are likely to qualify for HST assessment will not have clear comparators, given the rarity of the conditions involved. Where there may be two similar products on the horizon there is a risk that routing a topic to an MTA could lead to a delay in guidance as the licensing timeframes are unlikely to be identical. A delay of months or even weeks can be hugely significant for patients with ultra-rare diseases, especially in cases where conditions are progressive.

There may be cases where there is one or more alternative treatment already in use by the NHS for a new technology assessed through HST, but it is not clear from the consultation document why the current approach to assessment, incorporating appropriate comparators, is not suitable in these circumstances.

We are therefore concerned that, contrary to the stated aims of the change, the introduction of MTAs could result in unnecessary complexity and potential delays for patients who already face significant challenges.

Routing topics to clinical guidelines (para 81)

Strongly disagree

The SHCA believes there is a need for clarity and transparency in relation to this proposal. Since 2012, many technologies for rare and complex conditions were assessed by NHS England through its policy development and prioritisation process. This approach had evolved organically over time, as it was recognised that NICE's traditional assessment process was not suitable for some of these treatments. The 2019 Voluntary Scheme for Branded Medicines Pricing and Access signalled a shift in approach, with all licensed medicines expected to be assessed by NICE except in certain unspecified circumstances.

The SHCA has consistently called for clarity on the appropriate route for the assessment of these technologies – we believe that an updated NICE technology appraisal process, including additional flexibilities for the assessment of technologies for rare and complex conditions, represents the most robust and transparent approach to assessment. The existence of the mandatory funding direction for NICE technology appraisal guidelines also provides assurances for patients that they will be able to access treatments that NICE recommends.

We believe that introducing a separate quasi-assessment route through clinical guidelines would be a backwards step that would risk introducing uncertainty around the status of NICE's recommendations and patients' rights to treatment. We also have concerns about the timelines associated with the development of guidelines compared to technology appraisals and the differences in the approach to unpublished data between guideline development and technology appraisals.

We would welcome further details on the circumstances in which NICE believes that an update to guidelines would be appropriate rather than a technology appraisal – depending on the nature of the circumstances, it may be more appropriate for an update to be made to existing technology appraisal guidance, for an expedited appraisal or for a policy similar to

that used for biosimilars to be followed. However, without a clear understanding of the issue that NICE is attempting to address it is not possible to support its proposal.

Theme 2: Opportunities for new process improvements and ways of working

Would you like to provide general comments in relation to the proposals?

The SHCA welcomes the confirmation that expert involvement remains an integral part of health technology guidance development. We note that NICE has committed to exploring how and when patient evidence is presented to the committee. We are disappointed that NICE has not developed specific proposals in this area in time for the consultation and we look forward to additional details on NICE's plans being provided separately in advance of the consultation on the draft manual.

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

Professional, patient & carer organisations to nominate for all guidance topics (para 86)

Agree

The SHCA supports the proposal for patient and carer organisations to continue to nominate experts for all guidance topics. It is vital that NICE takes steps to ensure that patient-focused evidence is examined and reflected during the guidance development process and we welcome the commitment to engage with stakeholders at an earlier stage to provide additional time to source appropriate expertise.

Technical engagement shall become an option in Technology Appraisals and other guidance programmes (para 102)

Disagree

The SHCA understands that the technical engagement step has been a helpful mechanism to deliver earlier alignment on key issues in technology appraisals. We are therefore concerned that it may not be available in all technology appraisals and particularly those in which it is likely to be of most value, including in relation to technologies for rare and complex conditions.

We would welcome additional clarity from NICE on the circumstances in which a technical engagement stage would not be used. We would also welcome consideration of the value in involving patient and carer organisations in the technical engagement process. While we understand that there are likely to be issues of commercial confidentiality that will not be possible to share with the consultees, we believe that there is value in ensuring that all involved parties understand the issues that have been identified during technical engagement at an early stage.

This is important for patient and carer groups as there may be evidence that they can provide or generate to help to address challenges, if provided with sufficient understanding of the problems and advanced warning of the opportunity to address challenges.

Engagement with stakeholders on reducing health inequalities (para 133)

The SHCA welcomes NICE's commitment to exploring how it can change its processes to help to reduce health inequalities. We would welcome the opportunity for further discussion with NICE on this issue, including opportunities for individual SHCA charity members to share their experiences of health inequalities and ways in which the system could be improved.

Theme 3: Commercial and Managed Access Processes

Would you like to provide general comments in relation to the proposals for Commercial & Managed Access Processes?

The SHCA welcomes NICE's recognition of the need to consider opportunities to expand managed access arrangements to technologies outside of the Cancer Drugs Fund and to align with the MHRA's Innovative Licensing and Access Pathway. Technologies for rare and complex conditions are often associated with similar levels of uncertainty to new cancer medicines, but the absence of a clear assessment pathway for these technologies has created challenges and delays in access for patients.

The SHCA hopes that the creation of the Innovative Medicines Fund will enable a clearer route to facilitate earlier access to promising technologies for patients with rare and complex conditions. We welcome the proposals to clarify the process for entry into managed access, introduce a single approach to guidance reviews and to ensure that data collection arrangements are consistent and high quality.

As a patient-focused coalition, the SHCA is not well placed to respond to the technical questions on commercial processes, however we do have concerns that the proposal to review the status of managed access arrangements could result, at best, in uncertainty for patients and, at worst, patients being denied access to technologies.

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

The status of a recommendation for managed access (para 150)

Neither agree nor disagree

The SHCA notes that NICE states that the status of a recommendation for managed access is unclear and that it is proposing that there is a need to work with stakeholders to confirm the status of recommendations in relation to the mandatory funding direction. While the SHCA supports the proposal for further engagement on this issue, we are concerned at the prospect of any potential dilution of the status of managed access arrangements.

For many patients with rare and complex conditions, some form of managed access arrangement will be the primary route through which they will be able to access treatment. As a minimum, we believe there is a need to ensure that any clarification of the status of managed access arrangements does not act as a barrier to access for those patients who are provided with a treatment during the period of managed access.

More broadly, we are concerned that a decision to place managed access arrangements outside the terms of the funding requirement outlined in sections 7 and 8 of NICE regulations could act as a barrier to the timely implementation of recommendations.

There is a need for transparency in engagement with stakeholders on this issue and for all parties to agree at the outset on shared principles in relation to the objective of using managed access arrangement to support faster and wider access to treatment for promising technologies, including those for rare and complex conditions.

Theme 4: Objectives & vision of the Highly Specialised Technologies programme

Would you like to provide general comments in relation to the proposals for the Highly Specialised Technologies programme?

The SHCA welcomes NICE's vision for the Highly Specialised Technologies programme, including the recognition that the programme is a necessary departure from the standard approach to technology appraisal due to its unsuitability for patients with rare diseases.

While we recognise that the HST programme was not set up with the intention of appraising all rare disease technologies, we believe there is a need for NICE to explore how it can take a more flexible approach to the rare disease technologies that do not meet the criteria for entry to HST, in light of the challenges that it has highlighted with respect to standard approaches to assessment.

The SHCA has responded accordingly to NICE's consultation on changes to its methodology and we look forward to continuing to engage with NICE on this issue in advance of the publication of the updated manual.

While we support NICE's intention to update the entry criteria for the HST programme, we are concerned that the proposed principles set out in the consultation would not address all the existing challenges and would create new uncertainties, which could result in some new technologies for ultra-rare treatments being barred from entry. We believe that there is a need for more straightforward approach, whereby all technologies for ultra-rare conditions are eligible for HST assessment. This would be aligned with NICE's vision for the programme not to be used for all rare disease technologies but would provide much needed consistency and clarity for patients.

We would welcome the opportunity for more in-depth engagement with NICE on the principles, to ensure that they meet NICE's vision and deliver an equitable system for patients with ultra-rare diseases.

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

The key principles for the highly specialised technologies programme (para 186-190)

Strongly disagree

The SHCA is concerned that the adoption of new criteria on the basis of the principles set out in the consultation would act as a barrier to the equitable assessment of treatments for ultra-rare conditions through the HST programme.

We have particular concerns about principles a, c, g, h and j, as follows:

Principle a. We believe that this principle is inappropriate as there may be a valid reason for a condition to not be listed in the manual of prescribed services, or to have a recognised

highly specialised service. This is possible, and even likely, in the case of ultra-rare conditions for which there is no existing treatment option or method of prevention, as the NHS may not have had reason to create a specific service for a condition when the management of symptoms can be overseen by a specialised, but not highly specialised, service.

The principle provides a caveat in noting that technologies will be eligible if it is expected that a service will be in place at the time of guidance publication. However, the timeline and process to establish a highly specialised service is complex and lengthy. In the case of a 'Rare Disease Collaborative Network' specifically, there is no published guidance on the steps through which such networks can be proposed, developed or accredited, and only two networks currently exist in pilot form.

There is a need for NICE to revisit this principle to ensure that patients with under-served conditions are not unfairly discriminated against due to the absence of an existing service.

Principles c, g, j. We do not believe that these principles are appropriate as they may disqualify technologies that could be used to treat other conditions but which could 'plausibly' be used to treat another condition, even if in practice it would only be used in an ultra-rare population.

We believe that this approach would be both unfair and unequitable, as patients with high levels of unmet need could be effectively denied access to a treatment on the basis of a hypothetical scenario.

The SHCA recognises that NICE and NHS England wish to avoid circumstances in which multiple indications of a single technology are assessed though the HST and STA programmes due to the pricing complexities that this would create. However, we do not believe that the proposed solution is appropriate and would welcome revised proposals from NICE to ensure that patients with ultra-rare diseases are not unfairly penalised due to commercial challenges related to non-ultra-rare indications.

We would welcome clarity from NICE on the expected scale of this challenge, including the number of treatments that have gone through HST for which companies have subsequently sought to secure an STA appraisal in a separate indication and the number of treatments that NICE has identified from its horizon scanning in which companies could seek both HST and STA assessment.

Principle h. The SHCA does not understand the rationale for this principle. The nature of ultra-rare diseases means that there may be significant differences across a patient population in some cases or high levels of consistency in others and there may be limited levels of understanding as to the cause of variations in disease manifestations or response to treatment.

It would be consistent with NICE's usual practice for the differences in the patient population to be examined in detail in the course of an evaluation, informed by expert evidence. In some cases, this may result in an optimised recommendation, based on an assessment of the strength of clinical and cost effectiveness evidence.

Given the inherent uncertainties associated with treating these small groups of patients, we do not believe it is appropriate for restrictions to be placed on *entry* to the HST programme based on potentially limited levels of understanding about the heterogeneity of the patient population.