Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

GENETIC ALLIANCE UK

Strengthening the patient voice in NICE's decision-making

Introduction

The National Institute for Health and Care Excellence (NICE) is the independent assessment body with responsibility for taking decisions on which new treatments and technologies should be made available by the NHS in England. In January 2022, NICE published its updated programme manual setting out changes to its ways of working – the conclusion of a long period of consultation as part of its methods and processes review.[1] The objective of the review was to ensure NICE keeps pace with developments in science, enabling it to evaluate new technologies fairly, efficiently, and robustly.

The review led to a series of changes to NICE's approach to health technology assessment (HTA), from the introduction of the 'severity modifier' to give additional weight to health benefits in the most severe conditions, to the acceptance of higher degrees of uncertainty for treatments where it is difficult to generate evidence. NICE also introduced a range of reforms intended to improve engagement with patients and patient representatives. The goal of the reforms was to make the highly complex HTA process more transparent and accessible to patients, helping to ensure expert insights from patients is used to help inform NICE's decision taking, and that guidance has a greater focus and relevance for the people most directly affected by its recommendations.

Specific changes to strengthen the patient voice included:

- Reforms to the Public Involvement Programme (PIP) and support provided by the public involvement adviser assigned to each evaluation
- A requirement that companies include a 'summary of information for patients' within their submissions

Patient involvement is particularly important in HTAs for rare disease treatments. These treatments tend to have higher levels of uncertainty associated with their evidence base, with challenges in data collection due to small patient numbers. This uncertainty makes it particularly important that people living with the condition and their family have the opportunity to input into NICE's decision-making processes.

The Specialised Healthcare Alliance (SHCA), in partnership with Genetic Alliance UK, has developed this paper to set out SHCA members' reflections on NICE's engagement with patient groups representing people living with rare and complex conditions.

To examine the impact of the changes that NICE introduced, we disseminated a survey to the SHCA's membership to collect quantitative feedback on members' experiences working with NICE. We then held a series of one-on-one interviews with members who have been through a HTA since the final programme manual came into effect. Based on that engagement, the report focuses on:

- SHCA members' experiences working with the NICE PIP team
- Increasing support for patients in providing written submissions
- Ensuring NICE committee meetings are made more accessible to rare disease patient groups

The SHCA and Genetic Alliance UK have welcomed NICE's approach to engagement as part of its methods and processes review and looks forward to continuing to work with NICE in considering how the recommendations made in this report can be implemented.

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SHCA members' experiences working with the NICE PIP team

Background

As part of its methods and processes review, NICE set up a specific workstream to consider the public involvement aspects of its work, with the aim of co-designing realistic proposals to strengthen participation in the development of its medicines and non-medicines guidance.[2] It identified a series of areas where improvements could be made, from increasing opportunities for public and patient involvement, providing greater feedback on patient group submissions, and delivering greater training on how NICE's methods and processes work.

These recommendations offer a helpful starting point for future progress, and some were actioned as part of the review. The final programme manual set out guiding principles for patient groups to consider in their submissions and highlights the support of the public involvement adviser in working alongside the evaluation team to support the involvement of patients. Meanwhile the review also asked that companies provide a 'summary of information for patients' using a template provided by NICE, to support patient engagement with an appraisal. The document should provide detail relating to the treatment, the condition it is intended to treat and the existing patient pathway.

Members' reflections on patient involvement and transparency in decision making

To assess the impact of these changes, we asked SHCA members to share their feedback on how patient involvement in NICE decision-taking has evolved following the publication of the programme manual. Whilst the focus was on members who had gone through, or were in the process of going through, a HTA following the conclusion of the review, we also asked members for wider reflections on NICE's approach to transparency and ensuring patient involvement in decision-making. Five patient groups responded to our survey. The results found that:

- **Only one of five respondents found NICE's methods and processes easier to engage with** following the conclusion of the review. Two said they have found NICE harder to engage with, whilst a two were unsure
- **Two in five felt the NICE review has had no impact on transparency in decision-making**, whilst one suggested transparency had improved, and one said NICE had become less transparent

We also asked SHCA members to share information relating to whether they had received a 'summary of information for patients' from the manufacturer, and if so, if it had helped them in their engagement with NICE's processes. Concerningly, none of the patient groups we spoke to were familiar with the summary of information provided by manufacturers, and members were unsure what the role of the document was and how it was intended to support patient engagement. **Four of five respondents to our survey found that they did not know whether the summary of information made it easier for patient and carer organisations to understand industry submissions**.

Working with NICE's PIP team

PIP refers to the team at NICE that develops and supports patient, service user, carer and public involvement. Support ranges from informal advice to providing workshops and supporting engagement with the HTA process.

SHCA members welcomed the support of the NICE PIP team and the public involvement advisor in helping them navigate NICE's processes. One member spoke about the value of having regular calls with their advisor, who was open and responsive to questions. Some advisors have joined calls with the community directly and disseminated information relating to the TA – easing the responsibilities of the patient group.

Two in five respondents to our survey noted the introduction of a public involvement adviser assigned to each technology evaluation has helped make NICE's methods and processes more accessible.

Initial stakeholder engagement workshops were also highlighted as particularly helpful. One SHCA member noted that at the start of a TA they were invited to a two-and-a-half-hour workshop with NICE that included all of the key stakeholders involved, which set out key processes of the TA and accompanying timelines.

However, it was felt that support can vary depending on your individual advisor, with members' experience of support differing as a result. We were also told that existing support is often reactive, with NICE responding to specific queries rather than proactively reaching out to offer support. We heard that smaller charities may miss out because of this, as they are less likely to be aware of what types of support are available from the PIP team and their public involvement advisor. Given the limited resources of these charities, it is important NICE proactively reaches out to them to identify forms of support required.

Recommendations

Following our engagement with members examining the effectiveness of changes introduced as part of the NICE methods review, we would recommend:

- At the start of a TA for a rare disease treatment, NICE's PIP team should work with the relevant patient group to understand support that might be needed, to ensure that smaller patient groups do not miss out on available support
- NICE should develop and publish online a short, succinct step-by-step guide to the TA process for patient organisations representing rare disease patients, outlining what is expected from patient organisations from the beginning. It should also set out a list of key stakeholders involved in the TA, such as the PIP team and the project manager
- NICE should undertake a review of the effectiveness of summaries of information for patients. NICE should also ensure they are sent to patient groups as early as possible to support their engagement with a TA, whilst manufacturers should ensure they are as accessible as possible for patient groups so they can be used effectively

Increasing support for patients and patient representatives in providing written evidence

Background

Evidence submitted by patient groups provides a vital part of the NICE HTA process. Patient groups are able to set out the impact of a condition on a patient's day to day life, the existing patient pathway based on currently available treatments, if any, and the impact a new treatment option could have on disease severity and quality of life. Given the uncertainty in evidence that often accompanies rare disease treatments, the patient voice is particularly important when considering new treatments.

NICE's updated programme manual sets out the importance of qualitative data from patient groups and what committees are looking for from submissions, including patients' experience and quality of life living with a condition, their views on the acceptability of different types of treatments, and the

considerations of their carers. This submission also helps to supplement data and evidence provided by clinical consultees and the manufacturer of a new treatment.

The process of developing a written submission

NICE defines which topics to assess, the scope of the TA and who is consulted during it. NICE also defines the condition and any potential subgroups that the technology may be licensed for. Consultees are then asked to comment on the appraisal consultation document (ACD), with timelines for when evidence is asked for and required by set by NICE.

As part of our survey, we asked SHCA members to share their reflections on the process of submitting evidence to NICE before probing this further in our one-on-one interviews. Themes from those discussions included:

- The need for sufficient notice and time to prepare submissions
- Support on the scope of the submission
- Reassuring patient groups of the role their evidence plays in decision-making

Timelines

One SHCA member we spoke to highlighted that whilst they had considerable notice that a multi technology appraisal (MTA) was in the pipeline, no advanced notice was provided by NICE on timelines for the scoping consultation, which made advanced planning difficult. They were then informed of an eight-week timeline for evidence to be submitted, though they had hoped they would have twelve weeks based on previous experience.

Four out of five respondents to our survey stated that NICE does not provide sufficient notice to patient groups to submit evidence as part of a technology appraisal, whilst 3 felt they did not have sufficient support from NICE to draft their written submissions.

Whilst patient groups can request additional time where needed, many of the SHCA members we spoke to are not aware of this and it was suggested the NICE PIP team are clearer that extensions are possible where circumstances require. Members also highlighted that, given it is not uncommon for there to be a six-month gap between the deadline for submissions and the first committee meeting, they were unsure why these timelines could not be extended. Providing sufficient time and support is particularly important for rare disease charities, who juggle multiple responsibilities and often lack the capacity required to submit a detailed written submission within standard timelines.

Scope

We also asked SHCA members to share their thoughts on support available to draft written submissions. The support of the public involvement advisor was again commended here, as well as the template submission document provided by NICE. However, we did hear that due to the short timelines and limited resource of members, some didn't feel they had sufficient time to involve the PIP team in their submission and take advantage of the support they can offer.

Some SHCA members also suggested NICE should look to follow the Scottish Medicines Consortium (SMC) as a model of best practice in supporting written submissions, with one describing how the SMC PIP team offered to review their evidence, and whilst not directly amending content, providing advice and guidance on structure and areas where evidence should be focused.

We were told that more guidance from NICE on what a committee is likely to be looking for from their submission would be helpful, covering areas such as whether direct quotes from patients are helpful, how analysis should be themed, and the extent to which data collected by patient groups is valued.

Whilst some of this is provided in the final programme manual, it is important it is set out in a clear and accessible way by PIP teams. Finally, some members questioned the extent to which their submissions are taken into account in decision-making, when compared to that provided by the manufacturer and clinicians. NICE public involvement advisors should therefore use its engagement with patient groups to offer reassurance on this point.

Recommendations

Following our engagement with SHCA members on the process of developing written submissions, we recommend that NICE:

- Offers tailored support to patient groups on the areas written submissions should cover. For smaller organisations, NICE's PIP team should offer to review drafts of submissions, so that they are steered in the right direction in setting out areas of interest to committees, in line with the approach taken by the SMC
- Individual NICE public involvement advisors should supplement the webinars that NICE runs by explaining to individual patient groups how their evidence forms an important part of the HTA process
- The NICE PIP team should make clear at the start of a HTA that, where required, patient groups are able to seek extensions to the deadline for submissions. Alongside this, it is important NICE proactively reaches out to patient groups to provide estimated timelines of the written submission process as soon as consultees for a new appraisal are determined
- NICE committees should be required to clearly and consistently set out how patient input and evidence has shaped their decisions in written guidance

SHCA members welcomed the existing support provided by the PIP team and the important role public involvement advisors play in supporting engagement with NICE. These recommendations are intended to further strengthen the support of the PIP team and address outstanding challenges faced by rare disease charities.

Ensuring NICE committee meetings are accessible

Background

NICE's TA recommendations are prepared by an independent advisory committee. Following the finalisation of the scope and the submission of evidence, committee papers are prepared which set out the evidence that will be looked at by the appraisal committee, before it then meets to consider the evidence. Consultee organisations, including patient groups, are invited to nominate patient experts or clinical specialists to speak at the meeting.

NICE's final programme manual describes the vital role of clinical and patient experts in the decisions of the committee, with experts chosen based on their experience of the technology and the condition that the technology is designed for. As part of our interviews with SHCA members, we asked how they found the process of nominating a patient expert and then supporting them to prepare for the meeting. Themes from those discussions included:

- Ensuring patient groups have sufficient notice on timelines
- Strengthening preparation support and making committees more accessible for patient experts

Ensuring patient groups have sufficient notice on timelines

SHCA members we spoke to raised the importance of continuous dialogue on the scheduling of committee meetings and prompt updates on any changes made to planned dates, given the significant

preparation required both in identifying a patient expert and supporting them to prepare for the meeting. However, one SHCA member we spoke to noted that in one instance, whilst a date for a meeting they were preparing for had been set there had been no communication from NICE around the meeting, and only after their public involvement adviser chased the NICE team directly did they hear it had been delayed. It was noted these delays in communications are felt particularly acutely, as in addition to liaising with the patient expert, members also need to provide updates to the wider patient community interested in the outcome of an appraisal.

Strengthening preparation support and making committees more accessible for patient experts

SHCA members we spoke to highlighted how the process of speaking at a committee meeting can often be very intimidating for patient experts, as the discussion is very technical and this can serve to discourage patients from sharing their experiences. It was recommended that NICE accept pre-recorded evidence from patients, for those who do not feel equipped to speak directly during a meeting. NICE should also consider allowing patient experts to provide an opening statement at the start of meetings to explain how their condition affects their day-to-day life and the impact the treatment under consideration could have, to help frame the subsequent agenda items and allow the expert an opportunity to speak before the discussion becomes more technical.

Alongside this, members we spoke to also felt that the extent to which patient experts are listened to varied by committee. In some meetings, committee members appear less engaged by the patient expert than in other parts of the meeting, and it was suggested NICE considers how it can educate committee members on the importance of the patient voice. It would also be helpful for the PIP team to routinely hold calls with the patient expert in advance of meetings, to help set expectations of the meeting and ease any nerves.

It is important that NICE sets out guidance to committees that it has the patient evidence provided in meetings, and the accompanying written submission, at the forefront of its deliberations on a new technology under consideration.

We also heard how the timing of committee meetings can make it difficult for patient groups and carers to engage with, as meetings are often held in the evenings when patients and carers have to balance caring responsibilities. We were told of one evening meeting that started 90 minutes late before running for two and a half hours, meaning the patient representative had to leave early for childcare.

Recommendations

Building on our engagement with SHCA members, we recommend that NICE:

- Produces a simple guide for all committee members that sets out the role the patient expert's testimony should play as part of the committee's deliberations
- Ensures NICE committee meetings involve a patient impact statement at the start of the meeting
- Enables patient experts to share pre-recorded testimony during a committee meeting, for those not able to attend the meeting, or not comfortable sharing their perspectives directly
- Invites patient organisations and patient experts to briefing calls in advance of committee meetings, to set expectations and support preparation

Conclusion

The SHCA and Genetic Alliance UK welcomed the consultative approach taken by NICE as part of its methods and processes review. From our conversations, it is clear the PIP team has already made improvements in how it involves patients in its methods and processes, and this has made a real difference in supporting patient engagement – particularly through the support of public involvement advisors.

We also heard there are a range of areas where further improvement can be made in ensuring patient groups are supported to engage with NICE's HTA process, from the support provided by the PIP team, to strengthening support for written submissions and ensuring NICE committee meetings are made more accessible.

We hope this paper is a constructive contribution towards the shared goal of strengthening the ability of small and specialist charities and support groups to provide their vital input and support into NICE's decision-making.

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[1] NICE, 2022, NICE publishes new combined methods and processes manual and topic selection manual for its health technology evaluation programmes
[2] NICE, 2020, Improving meaningful public involvement in NICE medicines and technologies guidance