Strengthening the patient voice in NICE's decision-making

Progress report

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. Patient involvement is a vital part of NICE's approach to health technology assessment (HTA), providing crucial insight into the lived experience of a particular condition and the prospective impact of a new treatment under consideration.

For rare disease treatments this process is even more important. 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 especially crucial that people living with the condition and their family have the opportunity to input into NICE's decision-making processes.

In June 2023, the Specialised Healthcare Alliance (SHCA), in partnership with Genetic Alliance UK, published a report, *Strengthening the patient voice in NICE's decision-making*, which set out our members' experiences of working with NICE across the HTA process – looking at what works well and where patient groups, particularly smaller organisations representing patients with ultra rare conditions, need further support.

This report sets out the progress made against our 2023 recommendations, outlining where NICE has strengthened patient involvement based on feedback from our members. It also describes where our members feel more progress is needed to deliver on NICE's vision "*to have a best-practice approach to involvement and engagement, to improve the impact of our guidance and ensure the best care for people and communities*."

NICE's new strategy for involvement and engagement

The SHCA and Genetic Alliance UK welcomed NICE's collaborative approach to engagement following the publication of our 2023 report, with NICE carefully considering how it can work differently to implement the recommendations we made. In 2024, NICE published a new three-year strategy for involvement and engagement, *Working alongside people and communities at NICE.*

The strategy sets out five areas of focus and 12 guiding principles. Areas of focus include topics we raised in our 2023 report, such as delivering impactful involvement and engagement, implementing tailored approaches to engagement, and supporting productive partnerships with people and communities.

Whilst the strategy is still being implemented, NICE has provided an update that six initial actions have been completed – including implementing the revised non staff payment policy

and setting up the working alongside people and communities' oversight group. Additional work plans are now being delivered linked to the five areas of focus.

Measuring progress

We shared a survey with the SHCA's membership, which received 11 responses, and carried out six one-on-one interviews with patient groups representing people with rare conditions who have recently worked with NICE on a technology appraisal. Across the survey and interviews, we explored members' experiences of working with NICE on a technology appraisal and their reflections on NICE's strategy for involvement and engagement.

Results from our survey are included throughout. Whilst 11 responses were received, responses to each question were not mandatory and the response rate varies by question.

Based off members' reflections, this report assesses the progress made by NICE against the recommendations we made in our 2023 report, which were organised around three core themes:

- Theme one: Working with the NICE Patient Involvement Programme (PIP) team
- Theme two: Increasing support for patients in providing written submissions
- Theme three: Ensuring NICE committee meetings are made more accessible to rare disease patient groups

Theme one: Working with the NICE PIP team

Our 2023 report recommended that NICE:

- At the start of a HTA for a rare disease treatment, works 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
- Develop and publish online a short, succinct step-by-step guide to the HTA process for patient organisations representing rare disease patients, outlining what is expected from patient organisations from the beginning
- Undertakes a review of the effectiveness of summaries of information for patients that are included in company submissions

The SHCA members we spoke to welcomed the support provided by the NICE PIP team and the public involvement advisor in helping them navigate NICE's processes. Members who had attended, or nominated a patient expert to attend, workshops and training sessions run by NICE noted they were invaluable in informing them about what to expect from the different stages of an appraisal and how to prepare for them, particularly for committee meetings. One member noted the significant progress made over the last decade in strengthening the support provided by NICE throughout the appraisal process, with regular meetings now offered at different touchpoints.

PIP refers to the Patient Involvement Programme team at NICE that develops and supports patients, service users, carers and public involvement. Support ranges from informal advice to providing workshops and supporting engagement with the HTA process.

⇒ 8 out of 9 (89%) respondents replied *"about the same"* when asked if they had found NICE and its work easier to engage with since 2023.

However, members felt there are areas where support provided by the PIP team can be strengthened and made more consistent. Some of those we spoke to said that the reactive support provided by the PIP team was very good, with individual advisors quick to get back to requests, but proactive support could be improved. This means that smaller patient groups, particularly those who have not worked with NICE before, may not be aware of the extent of support available, leaving them feeling left to their own devices.

Similarly we heard how, whilst it is welcome that the PIP team offers flexibility in providing extra time where meeting deadlines is difficult, this is again an area that can disadvantage patient groups less familiar with working with NICE – who will be less likely to understand that requesting deadline extensions and asking for additional support is something they can do. One member suggested the Scottish Medical Consortium's (SMC's) Patient and Clinician Engagement (PACE) meeting was more effective at ensuring patient groups are provided with proactive support, as there was an impression NICE's PIP advisors look to avoid steering patient groups in the evidence provided, which can be frustrating.

⇒ 9 out of 9 (100%) were unsure as to whether NICE's processes have become easier to engage with for patients from ethnic minorities, and those who face obstacles such as language, cultural or educational barriers.

Members also expressed frustration at NICE's payment process for stakeholders involved in appraisals. This is an area covered in the new strategy, in which NICE commits to implementing a fair and transparent payment policy to underpin involvement and engagement activity. However, there was confusion if this was likely to lead to a change in payment policy, or instead referred to the existing payment made to patient groups for their involvement in an appraisal.

The members we spoke to were unanimous that the current payment model for patient groups is unsatisfactory and in no way proportionate to the significant time investment patient groups make in appraisals.

"The payment is simply a drop in the ocean given the time and resources required of a patient organisation when participating in lengthy NICE appraisal processes."

However members did say the payment provided to patient representatives to attend training sessions and scoping workshops was a more proportionate reimbursement for the disruption caused to their day-to-day lives from being involved in a HTA.

A further concerning area of feedback related to the communications provided by NICE is at the end of an appraisal, particularly the period where a patient group is told the outcome of an appraisal in confidence. In one instance, we heard how a patient representative nominated by a patient group was strongly warned that they could not communicate the decision to the patient group – as they were not registered as a stakeholder in the appraisal. In another instance, a patient group received an email threatening them from being excluded from future appraisals after an embargoed announcement was leaked – though they were not aware of the leak.

We heard how the language used in communication on appraisal outcomes can be very traumatic for both patient groups and patient representatives, and whilst they recognise the importance of

embargoes on appraisal consultation documents – NICE should be sensitive to this in their communication. Members felt that the current approach to engagement at this stage is not consistent with the spirit of collaboration and treating patient groups as trusted partners in the HTA process.

"The communication about when we will receive information (such as publication of final guidance) has remained very unreliable. We understand that there must be lots of competing pressures behind the scenes but it's very frustrating not to be able to plan our workload to respond/inform the community in a timely fashion."

Theme two: Increasing support for patients and patient representatives in providing written evidence

Our 2023 report recommended that NICE:

- Offers tailored support to patient groups on the areas written submissions should cover, including reviewing submissions where appropriate, and make clear that patient groups can seek extensions to deadlines if required
- Encourages public involvement advisors to supplement the webinars that NICE runs by explaining to individual patient groups how their evidence forms an important part of the HTA process
- Requires committees to clearly and consistently set out how patient input and evidence has shaped their decisions in written guidance
- Undertakes a review of the effectiveness of summaries of information for patients that are included in company submissions

Members welcomed the initial support provided by the PIP team on written submissions, with guidance offered on the areas that should be covered and types of information included.

NICE defines which topics to assess, the scope of the HTA and who is consulted during it. Written evidence is then a key part of the appraisal process. All non-company consultees are provided with eight weeks to submit a statement on the potential clinical and cost effectiveness of a treatment.

We also heard that NICE should provide more tailored support for patient groups who have no experience of working on an appraisal – for example, outlining what types of evidence to include that will be most relevant to a committee's decision-making process. One SHCA member noted how they only learned after the deadline what types of information could be included, and felt they missed an opportunity to make their response more impactful. We also heard the value of the 'summaries of information' provided by companies for patients was limited, as they tended to be too brief to act as an adequate substitute for engaging with the full details of an appraisal.

⇒ 5 out of 8 (63%) felt the support provided by NICE to assist with the submission of evidence in technology appraisals has remained about the same since 2023, whilst 3 felt it had improved.

Members reflected on how NICE could make its resources more interactive and easier to engage with. We were told the forms that patient groups are required to fill in on conflicts, whilst important, are difficult to complete, and the file types used are hard to navigate. We heard of one patient expert who pulled out of a committee meeting because they became frustrated at the process and unable to manage the time burden created by the evidence process. Another

SHCA member described how they ran a survey of their patients in partnership with the PIP team, and the process was welcomed in creating better evidence. However they noted it was difficult to access the different appraisal documents on NICE's website, and engagement could have been even stronger if this process was more accessible.

"It can be overwhelming when one of these lands on your plate. Templates from NICE on evidence and patient surveys can go a long way in helping manage the process."

Linked to the recommendation made in our 2023 report, a common theme from our conversations with members was doubt as to the impact written submissions from patient groups have in the decision-making process. In instances where an appraisal concludes with a positive recommendation, members noted they do not receive any insight as to the role of their evidence in contributing to that positive decision. One SHCA member felt that their evidence helped make the impact of a condition more real to a committee, but ultimately had little role in shifting a negative recommendation to a positive one. We heard how even a paragraph in the final appraisal document on the role of patient written and oral evidence would help patient groups feel the significant time investment had been justified, as well as supporting them to refine future evidence submissions.

"You do your submission then never hear if it was impactful or made any difference. It goes into a black hole."

Theme three: Ensuring NICE committee meetings are accessible

Our 2023 report recommended 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, and ensures 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

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 our discussions included:

- Delivering consistency in how committee meetings are run and patient evidence handled
- Strengthening the support patient representatives receive to handle difficult conversations in meetings
- Ensuring patient groups have sufficient notice on timelines and making meetings more accessible

Following the finalisation of the appraisal 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.

⇒ 5 out of 11 (45%) felt their views and feedback had been listened to by NICE, both in relation to written submissions and committee meetings, whilst 5 felt unsure. One respondent felt they had not been listened to.

A majority of the SHCA members we spoke to had positive experiences of working with NICE committee chairs, who took time before the meeting to ensure the patient representative had sufficient support, felt comfortable, and was well briefed on the format and their role. It is especially welcomed when the chair schedules a meeting with the patient group and experts before the meeting to provide an opportunity to ask questions.

Unfortunately we did also hear instances where these standards slipped, particularly where a meeting was delayed from a previous session on a different treatment. We also heard of several instances where committee chairs had been more assertive in responding to patients if they felt the evidence provided was less relevant, and this can be difficult for patients to manage when they have made often significant sacrifices to attend a meeting.

SHCA members outlined how training should be improved for committee members in how they interact with and ask questions of patient experts, with one noting a formal complaint was made after a committee member cut off a patient representative. Even in more successful examples, a consistent theme of our discussions was the need to ensure patients are supported to handle often very traumatic conversations on the prospective impact of a treatment. For patients who have lost family members to the condition in question, the frank analysis of the cost effectiveness of a treatment can be triggering.

"We understand NICE have to hold cold economic discussions but when you have patients there it is important to make an allowance for that and understand how raw the patient experience can be."

One patient group we spoke to said they continue to make the case for reforming the process for giving evidence so that it is more accessible and available in different formats. They noted that having one-on-one conversations with patient representatives can be both more beneficial to committees in supporting insights and more manageable for patients than presenting in large meetings.

We heard how, in response to feedback that committee discussions can be insensitive to the patient experience, NICE has offered, in future, to provide patient representatives with the opportunity to travel to NICE's offices to listen to the meeting and present with the PIP team present. Whilst this response to feedback is welcomed, given the impracticalities for patients who would have to travel long distances to do this, NICE should continue to work with patient groups to consider different forms of support that will help to address the above concerns.

In line with the findings from our 2023 report, scheduling of committee meetings continued to be a recurrent theme in our engagement – both on meetings being scheduled at short notice and slippages to timings on the day from overrunning meetings. This can be very inconvenient for charity and patient representatives, given the various competing pressures on their time, and members felt it often led to their sessions being rushed through as a result.

One example included a meeting where the treatment under consideration was the fifth session being carried out of six conducted on the day, with significant delays from preceding sessions. The patient group involved suggested NICE are too optimistic on what can be covered, and longer breaks should be inserted into schedules so that committee chairs are able to rest between sessions.

We heard concern that the above issues could become more pronounced with NICE's intention to streamline the HTA process and shorten timelines. Whilst this is welcomed in addressing the current lengthy gaps between committee meetings, NICE must ensure it does not minimise opportunities for patient groups to feed in, and exacerbate existing challenges around meeting format.

Conclusion

The SHCA and Genetic Alliance UK welcomed the constructive approach to engagement following our 2023 report and the subsequent publication of NICE's new three-year strategy for involvement and engagement, *Working alongside people and communities at NICE*. This progress report is intended to be a positive contribution towards that strategy and the delivery of NICE's vision to have a best-practice approach to involvement and engagement.

It is clear that NICE has already made important progress in strengthening the way patient groups are able to engage with, and contribute to, an HTA, from establishing more regular touch points with the PIP team, to working in partnership with patient groups on evidence generation, and reflecting on feedback on the way committees are run to ease the emotional burden on patients.

However, our members are also clear thar there remains work to do in delivering on each one of our 2023 recommendations. In particular, as immediate areas of focus:

⇒ NICE should continue to reflect on how the PIP team can provide more proactive and tailored support to smaller patient groups, who have little or no experience of the HTA process.
⇒ Communication on how patient evidence forms an essential part of the decision-making process should be strengthened, so that patient groups understand how the time they invest in HTA supports the work of NICE committees.
⇒ NICE should consider how the ways in which patient representatives provide evidence to committees can be diversified, to support them to handle the emotional impact of contributing to committee meetings, and the impact on their day-to-day lives, given the sacrifices that need to be made to attend them.

The SHCA and Genetic Alliance UK look forward to working with NICE to support the implementation of the involvement and engagement strategy, and to consider how the recommendations made in our report can be delivered.

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