

The Specialised Healthcare Alliance spring survey results

Introduction

In May 2019 the Specialised Healthcare Alliance (SHCA) ran a survey to gather views on different processes for assessing treatments for rare and complex conditions in England and the devolved nations. Given the wide range of experiences amongst the Alliance's members and corporate supporters, the survey included questions on the following:

- NICE's assessment processes, including highly specialised technology evaluations (HST) and single technology appraisals (STA)
- NHS England's policy development and prioritisation process
- Processes used across the devolved nations

This briefing includes the headline findings from the survey, key trends across the 2018 and 2019 surveys and specific feedback from respondents on each of the processes listed above. In total the survey received 39 responses, 90% of which were from patient organisations or charities.

Headline findings

The survey revealed some common themes and concerns:

- Greater flexibility for rare disease treatments continues to be identified as the major area for improvement in the system, across all assessment processes, with 89% of respondents calling for more flexibility to reflect the small population sizes of rare diseases
- NICE processes for evaluation are considered significantly more transparent than those of NHS England, with 100% of respondents saying they find the NHS England prioritisation process either not very or not transparent, compared to just 17% when asked about the NICE STA process
- When asked to consider reforms to the assessment process, 67% said it was not very fair or not fair to have a system where rare disease treatments are assessed by different methodologies and budgets depending on the route they are scoped into
- There is support for Scotland's recent efforts to improve access to treatments

Trends across the 2018 and 2019 surveys

A number of questions were repeated from the 2018 SHCA survey to allow for an assessment of changes year-on-year. Of particular note:

- There is growing concern that cost-effectiveness threshold for NICE appraisals could have an impact on patients' access to medicines, up from 95% to 100% of respondents in 2019 noting they were either somewhat concerned or very concerned
- Respondents have found it increasingly difficult to access information on the prioritisation process, with over 90% of 2019 respondents noting it was either challenging or very challenging, up from 50% in 2018

NICE's HST and STA processes

- Over 80% of respondents had confidence in the decisions of the HST and STA processes
- 90% of respondents felt their contributions were either valued or very valued in the HST process, compared to 66% in 2018
- 83% of respondents felt the STA process was either somewhat or very transparent
- However, 54% of respondents were not confident or not very confident in the suitability of the STA process for treatments of rare conditions that do not qualify for an HST

There is confidence amongst members in NICE's approach to the process of assessment. Respondents found the processes transparent, felt their views were valued and had confidence in the decisions.

However, there are significant concerns around the scoping criteria for deciding whether a rare disease treatment should be assessed through the STA process and the suitability of the STA process for those treatments that do not qualify for an HST.

While there was uncertainty as to what the most suitable assessment path would be in the future, there was strong agreement from 89% of respondents that any new appraisal process should have greater flexibility to reflect the small population sizes of rare diseases.

NHS England's prioritisation process

- Over 70% of respondents were not very confident or not confident in the decisions of the prioritisation process
- 80% of respondents found it challenging or very challenging to provide information to the process
- 67% of respondents felt their contribution was undervalued
- 60% of respondents faced delays

Members continue to report challenges in their interactions with the prioritisation process. There continues to be a lack of confidence in the decision-making process, with members calling for greater transparency, flexibility and support for small patient organisations looking to be involved. While patient groups understand the difficult task facing NHS England, they do not believe the current process is suitable. As one respondent noted, “as a patient organisation we agree with the prioritisation thesis but not the process.”

The access environment across the devolved nations

- 50% of respondents thought Scotland provided the best access to treatment for patients with rare and complex conditions
- In comparison 6% of respondents felt England and Wales were the best respectively, with no one considering Northern Ireland to have the best system in place

The progress made in Scotland in recent years was recognised by respondents, with over 60% noting that the changes since 2014 had improved access to treatment and 60% feeling the introduction of the new ultra-orphan pathways will deliver future improvements. While this progress is reflected in respondents’ views on which system provides the best access, there was still some confusion, with 38% of respondents noting they were unsure.

Conclusion

The SHCA is committed to working collaboratively to improve the access environment for patients with rare and complex conditions across the UK.

We hope this survey provides a helpful insight into this complex environment, particularly in light of the upcoming transfer of activity from NHS England to NICE.

We would welcome the opportunity to discuss the survey findings with representatives from NICE, NHS England, the Department of Health and Social Care and the devolved nations and look forward to sharing our ideas on how some of the concerns raised in this survey could be addressed through opportunities such as the NICE methods and processes review.

About the SHCA

The Specialised Healthcare Alliance is a coalition of patient-related groups and corporate supporters with a strong record in campaigning on behalf of people with rare and complex conditions in need of specialised care.

SHCA, July 2019