**NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE**

**A guide for patient organisations**

**Completing an organisation submission following a period of Managed Access for Technology Appraisals or Highly Specialised Technologies**



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# Introduction

## About this guide

This guide is for organisations that represent people (and their carers and families) with the condition for which the treatment is being re-evaluated following a period of **managed access**.

For simplicity, NICE calls these organisations ‘patient organisations’.

Understanding the experiences of people living with a condition, and their carers and families, helps NICE committees to make their decisions. We need your help to collect and present important information about what it is like for a person to live with a condition, and to have specific medicines or treatments. You can also provide us with information on what it is like to have care for a specific condition in the NHS in England. Your input is essential to help us consider the experiences and views of people directly affected by the recommendations we make. We very much appreciate the time and effort you put into completing a submission.

## Help and support

Members of the NICE public involvement team are available to guide you through the submission process. We can read through your draft submission and highlight any areas that could be strengthened. If you have any questions, we are happy to support you. **Please contact** [**PIP@nice.org.uk**](mailto:PIP@nice.org.uk)**.**

Resources available elsewhere are detailed below.

## What is involved in a submission?

To submit information from your patient organisation, you need to complete a patient organisation submission form. This provide us with suitable information from people living with the condition (and their families and carers) and if known the experiences of patients during the **Managed Access Agreement**.

Strong submissions contain facts, information and summaries of experiences, and give a concise, accurate and balanced overview of patients’ and carers’ perspectives.

Your submission will be published on the NICE website, as are all documents providing evidence for a technology appraisal.

## Why provide a submission?

People living with a condition have knowledge, perspectives and experiences that are unique, and can provide essential evidence for the appraisal process. They can provide insight into what it is like to live with that condition, and the advantages and disadvantages of treatments. Your submission could identify important aspects of the treatment or condition that may not be:

* That might not have been captured during the period of **Managed Access**
* Represented in research published in peer reviewed journals
* well captured in quality-of-life measures or other outcome measures used in clinical trials and other research studies
* immediately understood by the NICE committee.

The submission is also an opportunity to identify the priorities and preferences of patients, and what the added value of a particular treatment might be to them.

## How your submission is used in the appraisal

The input of people with the condition, and their families and carers, may be used in many different ways during an appraisal, including to:

* identify outcomes that are important to patients
* represent patient expectations and direct experience with the treatment being appraised
* help in the critical appraisal of evidence, by assessing its relevance to patients
* help interpret other evidence, such as that provided by the company selling the treatment
* provide new evidence.

## Planning and completing a submission following a period of managed access

When planning your submission, you should decide whether you need to gather new information from people with the condition, and their family and carers, or whether you already have the necessary information to complete the submission form.

For example, your organisation may have been involved in **the managed access** oversight meetings during the period of **managed access**.

If you need to gather new information, we suggest you start doing so as soon as you hear from us that an appraisal has been referred by the Department of Health and when it is due start. When the appraisal starts, you will be sent an invitation to participate. This will contain a link to [NICEdocs](https://appraisals.nice.org.uk/) (our online document-sharing platform), which will include the patient organisation submission form. You have 8 weeks to complete a submission.

## What to include in your submission

We want to understand the experiences of people living with the health condition for which the treatment being appraised is used, and of people caring for them. It is important to report on the experiences of many people living with the condition, rather than only people with exceptional experiences. It is also important to focus on how the condition affects the quality-of-life of people with the condition, and their families and carers, rather than clinical or cost effectiveness because these latter issues are comprehensively covered by other stakeholders.

Please remember to be clear and concise. Your submission will have the most impact if it covers a range of experiences, is balanced and acknowledges both advantages and disadvantages of the new treatment.

Try to include both quantitative (numbers from surveys) and qualitative (quotes about the experiences of people with the condition, and their families and carers) data throughout your submission.

## What not to include in your submission

**Clinical or scientific evidence:** As part of the process for assessing the treatment, the scientific evidence will be provided either by the company whose treatment this is or by the academic review group.

When all the evidence has been presented to the committee any gaps in the evidence will be highlighted during the consultation on the evidence. Please signpost us to it then.

**Summarised or reworded information from sources other than patients or caregivers (for example, clinicians, other healthcare professionals, pharma companies or other stakeholders):** The purpose of a patient group submission is to collect input from people living with the condition and their carers. The patients’ views, opinions and experiences are unique. Please spend your time and effort on this perspective.

Input from clinicians, pharmaceutical companies and other stakeholders is sought separately.

**The same message repeated under different template headings:** Sometimes it may be difficult to limit information to only 1 section of the template. Please ensure that you are answering the specific question under each section and not repeating information. We want to ensure that we get only the most relevant information so that the committee has the best evidence and information possible to make their decision.

If you would like advice about completing the template, please contact the PIP team at NICE.

**Lobbying or advocacy:** The NICE committee makes its decisions based on evidence. Lobbying or advocacy in submissions detracts from your key points and may be undermine your submission.

**An unbalanced submission:** This could be not including the experiences of a range of people living with the condition, or both positives and negatives of the new treatment; either of these are likely to weaken your submission because it may be seen as incomplete or potentially biased.

## Information available elsewhere

* [Patient involvement](https://www.nice.org.uk/about/nice-communities/public-involvement/develop-NICE-guidance) guides (apart from submissions) in the rest of the technology appraisal process – this is available on the NICE website.
* The [methods and processes](https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance) for NICE technology appraisals.
* Supplementary information for patient and carer groups on taking part in Health Technology Assessments – provided by HTAi PCIG and including:
  + an introduction to Health Technology Assessments and the role of patients (a webinar)
  + help conducting surveys
  + other videos and guides.

See: [For patients and patient groups](https://htai.org/interest-groups/pcig/resources/for-patients-and-patient-groups/)

# Completing the form

Not all questions or suggestions in the form will be relevant to every submission. It is OK to leave a section blank.

## Section 1 - About you

1. **Your name**

If you are submitting on behalf of an organisation, your name will be removed from the submission when published on the NICE website.

1. **Name of organisation**

Please give the name of the organisation that is making the submission. If you wish to make a joint submission with 1 or more other organisations, please put all the names in this box.

1. **Job title or position**

If you are an employee or volunteer of the organisation, please give details.

**4a. Provide a brief description of the organisation**

Briefly describe how you interact with people with the condition, and their carers and families, for example, through: newsletters, a website, social media, local events, helplines or support groups.

Please describe the organisational aims and objectives (or vision and mission).

**4b. Has the organisation received any funding from the company/companies of the treatment and/or comparator products in the last 12 months?**

If you have received funding from the company who manufactures the treatment and /or comparator products in the last 12 months please list all of them, and state how much they give.

**4c.Do you have any direct or indirect links with, or funding from, the tobacco industry?**

Please state if you have any links with the tobacco industry and, if so, explain what these are.

**5. How did you gather information about the experiences of patients and carers to include in your submission?**

Please give an overview of the sources you used, for example, information may have been gathered from: one-to-one discussions with colleagues, people with the condition and those caring for them; the **managed access** oversight meetings (if involved); telephone helplines; focus groups; online forums; published or unpublished research; user-perspective literature; or an organisational survey.

We recognise that there may sometimes be reasons why you may not have been able to consult directly with patients on this treatment.

## Section 2 - Living with the condition and current treatment

6. What is it like to live with the condition?

Provide clear facts, information and summaries to give a concise and balanced overview of a range of patients’ and carers’ experiences.

Consider the experience of living with the condition and the impact on has on quality of life such as:

* ability to work or gain an education,
* emotional health and well-being,
* adaptations to your home,
* financial impact,
* relationships
* diet,
* exercise,
* travel.

For children, consider their

* ability to go to school,
* develop emotionally,
* form friendships and participate in school and social life,
* Is there any impact on their siblings?

Consider what aspects of the condition are the most important to control, for example, symptoms that limit social interaction or ability work.

Consider whether caring for somebody with this condition affect the carer’s own health to the extent that they need to seek NHS treatment themselves.

If experiences vary between different groups of people living with the condition, please explain this in question 18 (‘patient populations’).

EQ-5D is NICE’s preferred measurement to assess health-related quality of life. It is a standardised 5-dimension instrument used to measure health outcomes. It is completed by the person having a treatment themselves and is quick to use.

Consider the 5 aspects that are often considered for EQ‑5D:

1. mobility – this might be overall, or of particular parts of the body
2. self-care – such as the ability to get dressed, wash, eat and live independently
3. usual activities – work, education, family and social life
4. pain and discomfort – explore what this means in real terms about the ability to do things
5. anxiety and depression – describe what the impact of the condition is on mental health.

However, these 5 aspects may not cover everything particular to people with this condition (or may not cover everything adequately). So, please also consider other physical symptoms. Examples might include:

* tiredness and fatigue, such as the severity and the effects
* diarrhoea incontinence, such as the severity and the effects
* difficulty breathing, eating, drinking or sleeping.

7. What do carers experience when caring for someone with the condition?

This is your opportunity to highlight the important aspects of the condition for carers and families.

Consider the experience of living with the condition and the impact on daily life (physical and emotional health, ability to work, adaptations to your home, financial impact, relationships and social life).

* What do carers have to do for the patient(s) on a daily basis (such as washing, lifting, avoidance of harm, treatment administration etc)?
* What are the most challenging aspects for carers (e.g. physical challenges, emotional effects, anxiety, guilt, sleep deprivation, stress, depression, need to give up work, costs of caring, need to strictly adhere to treatment timetable limiting other activities, impact on social life)?
* Who is involved in the care and support of the patient; consider impacts on extended family, friends etc).
* What are the hard or demanding things carers/families don’t complain about?
* What are the challenges faced by the family (e.g. impacts on family life for siblings, genetic implications – observing a family member with a condition you know you have, decision whether to have further children)

8. What do patients and carers think of the current treatments and care available on the NHS in England?

This section includes your experience of current practice in the NHS, outcomes important to people living with the condition, risks and benefits of current treatments (Some of these may be used as comparators for the treatment and they will be listed in the scope for this appraisal).

**General questions about the current treatments:**

* + What do people living with the health condition, and the people caring for them, most want from any treatment?
  + What is most important to them and why?
  + How far do the current treatments meet those needs?
  + Do the treatments improve the conditions and symptoms, and do they have any side effects?
  + Are there any treatments being used currently that people living with the condition would like to replace with the new treatment? If so, please tell us what they are and why? For example, is this individual choice about treatment benefits and side effects, or does it depend on a particular group of people with different characteristics? (See also later questions 18 & 20 about patient populations and equalities.)
  + If you know of any differences in opinion between patients about the benefits of the treatment being appraised, please tell us about them.

9. Considering all treatments available to patients are there any unmet needs (that current treatments do not provide) for patients with this condition?

How much do the current treatments control or reduce the most challenging aspects of the condition?

Are there any important aspects of the condition that current treatments do not control at all?

## Section 3 - Experience, advantages, and disadvantages of the treatment during the Managed Access Agreement (MAA)

In this section we are asking you to share details of patient experience of treatment during the managed access agreement and whether there are any advantages or disadvantages of this treatment

10.What are patients’ and carers’ experience of accessing and having the treatment?

Please consider patient experience of

* Access to treatment centres (how easy is it for patients to access treatment centres),
* Clinical assessments to start treatment,
* Ongoing assessments,
* The treatment (including):
  + How the treatment is delivered,
  + Frequency of treatment,
  + Frequency of visits to the hospital (if applicable),
  + Is the treatment administered at home or away from hospital,
* Any treatment stopping criteria,
* Any other issues not listed above.

11. What do patients or carers think are the advantages of the treatment?

Please explain any advantages that people living with the health condition, and those caring for them, think this treatment has over other NHS treatments in England.

**If you have been unable to find anybody who has had the treatment, please tell us because we understand how difficult it can be to answer this question without their input.**

Please consider:

* + - what the expectations of the treatment were and whether these expectations were met
    - What tangible impacts did the treatment have for example:
      * improvement of a disabling symptom or physical symptoms,
      * course, outcome or stabilisation of the condition,
      * maintenance or improvement of mobility,
      * reduction in pain,
      * reduction in other medications,
      * ease of use (for example, tablets rather than injection),
      * where the treatment is adminstered (for example, at home or in hospital),
      * improvements in mental health,
      * fewer emergency hospital visits,
      * quality of life (such as lifestyle, work, school attendance, ability to socially interact),
      * for other people (for example, family, friends, and employers),
      * any other issues not listed above.
    - If there were unexpected benefits that were not reported/discussed in the original appraisal (if known).
    - If you know of any differences in opinion between patients or carers about the benefits of the treatment being appraised, please tell us about them.
    - If you are familiar with [EQ‑5D](https://euroqol.org/), are there benefits from this treatment that are not adequately captured using the EQ‑5D tool? Please explain what these are and why they are important to patients.

12. What do patients or carers think are the disadvantages of the technology?

**If you have been unable to find anybody who has taken the treatment, please tell us because we understand how difficult it can be to answer this question without their input.**

Please list any concerns people living with the condition, and those caring for them, have about this treatment.

Please consider:

* + - aspects of the condition that this treatment cannot help with, or might make worse
    - any difficulties in taking or using this treatment (for example, injection rather than tablets)
    - any side effects (for example, type or number of problems, how often they occur, how long they last, how severe they are). Please describe which side effects people living with the condition might be willing to accept or tolerate and which would be difficult to accept or tolerate and why
    - any concerns about where this treatment has to be used (for example, in hospital rather than at home)
    - any negative effect on others (for example, family, friends and employers)
    - any financial impact on the people living with the condition or their family (for example, the cost of travel to hospital or paying a carer)
    - Were there side effects that were not listed on the patient information leaflet?
      * How were side effects managed?
      * Were side effects acceptable or did they lead to treatment discontinuation?
      * What were patients’ views of the side effects?
      * Were there differences in benefits or side effects across different types of patients?
    - Were there any challenges taking this treatment alongside the other care and management necessary for the condition?
    - Did patients stop treatment for reasons other than not meeting the continuation criteria?
    - any other issues not listed above.
  + If you know of any differences in opinion between people living with the health condition, and those caring for them, about the disadvantages of this treatment, please tell us about them.

13. What place do you think this treatment has in future NHS treatment and care for the condition?

Consider how this treatment has impacted patients and how it fits alongside other treatments and care pathway.

## Section 4 - Patients views on assessments used during the MAA

In this section we are asking you to share experiences of measurements, tests and assessments experienced by patients and families during the period of the MAA

If any of the questions in this section are not relevant to your organisation please enter “not applicable or not known”.

14. Results from tests and assessments are used to help reduce uncertainty about the effectiveness of treatment. How well do you think the tests and and assessments worked in measuring the effectiveness of the treatment?

If you are familiar with the tests and assessments used, please tell us how well or not they addressed the uncertainties outlined in the **managed access arrangement.**

Consider

* Which uncertainties that were addressed well
* If there were uncertainties that weren’t addressed well and if so tell us why (if known)
* Do you feel the data collected were on the right outcomes?
* Do you feel the way the MAA has operated and the information that has been gathered addresses the uncertainties raised in the original appraisal?
* What gaps in evidence might still remain?

15. Were there were any tests or assessments that were difficult or unhelpful from a patient’s or carer’s perspective?

Consider

* Duration of assessment or test
* Type of assessment or test
* Frequency of assessment or test
* Difficulty of assessment or test

16. Do patients and carers consider that their experiences were captured adequately in the MAA tests and assessments?

Please consider

* Clinical outcomes,
* Emotional and psychological outcomes
* whether there is a variety opinion

17. What outcomes do you think have not been assessed or captured in the MAA data?

Were there any outcomes for patients that were not assessed or captured as part of the **managed access agreement**?

## Section 5 - Patient population

In this section we are asking you to consider patients who may benefit from the treatment and tell us about patient who declined treatment.

18. Are there any groups of patients who might benefit more or less from the treatment than others? If so, please describe them and explain why.

* + Are there challenges in managing this condition when people with the condition also have other medical conditions? Please give examples of those that you think are the most important.
  + Are there any groups of people with the condition who might benefit more from this treatment than others? If so, please describe them and explain why.
  + Are there any groups of people with the condition who might benefit less from this treatment than others? If so, please describe them and explain why.
  + If you know, consider whether people treated in the NHS for this condition are different from those in the trial or trials and, if so, why.

**(We note that this might link with the equality section below.)**

19. Were there people who met the MAA eligibility criteria who decided not to start treatment?

Please state (if known)

* proportion of eligible patients who did not start the treatment
* their reasons for this, for example
  + were they offered an alternative treatment

## Section 6 - Equality

20. Are there any potential [equality issues](https://www.nice.org.uk/about/who-we-are/policies-and-procedures/nice-equality-scheme) that should be taken into account when considering this condition and treatment?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others.

Protected characteristics are:

* age
* being or becoming a transsexual person
* being married or in a civil partnership
* being pregnant or having a child
* disability
* race including colour
* nationality
* ethnic or national origin
* religion
* belief or lack of religion or belief
* sex and sexual orientation.

Are there groups of people with the condition who would have difficulties using the new treatment?

* Consider practical issues such as mobility, manual dexterity if self-injecting, cognitive ability, other health conditions, acceptability of side effects, and religious or cultural reasons.
* Consider whether the equalities issues are different from the comparator treatment (if applicable) and why.
* What evidence do you think would help the committee to identify and consider such effects on people with the condition?

## Section 7 - Other issues

21. Are there any other issues that you would like the committee to consider?

These might include:

Any further reflections about the uncertainties outlined the **Managed Access Agreement** that you think are important that have not been addressed elsewhere in your submission.

**Innovation**

What makes this treatment significantly different from other treatments for this condition?

Many treatments considered by NICE are ‘new. This does not necessarily mean that they are ‘innovative’ in the NICE sense. By ‘innovative’ we mean, for example, that they are: the first treatment of their type; they have a significant different mechanism of working; they are taken in a different way; or they have a significantly different side effect profile. They are considered to be ‘a step change’ treatment.

**Research evidence on patient or carer views of the treatment**

* Is your organisation familiar with the published research literature for the treatment?
* If you are aware of any relevant research on patient or carer views of the condition or existing treatments (for example, qualitative studies, surveys and polls), please provide references to the relevant studies.

## Section 8 - Key messages

In no more than 5 bullet points, please summarise the key messages of your submission.

* When somebody reads your submission, which are the most important messages you would like them to remember?
* If you had to choose up to 5 things from your submission to be presented to the committee, what would you want them to be?
* You may have fewer than 5 key messages.
* For each bullet point, summarise your message into a short sentence or phrase.
* The aim is to highlight and reinforce something that you explained more fully earlier in your submission.
* The more succinct your summarised key message, the more impact it is likely to have.

**Acknowledgements**

We would like to thank the patient organisations who have given feedback on the Managed Access Agreements patient organisations submission template and IMPACT HTA whose guide “[patient group submissions template for re-appraisal after an OBMEA](https://8c3e11d9-5f36-452f-abe3-c95befd6e85d.filesusr.com/ugd/e1a359_9937a09bdf2141d6aaca097b488c233f.docx?dn=210331%20IMPACT_HTA_%20WP10%20OBMEA%20Patient%20Gr)” was referenced when producing this guide.