



Highly specialised technologies: NICE prioritisation board routing criteria

Education

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1 The vision

The Highly Specialised Technologies (HST) Programme evaluates technologies for ultra-rare diseases ([defined in NICE strategic principles](#), and previously referred to as 'very rare'). These ultra-rare diseases are often very severe and debilitating and need specific consideration by the HST Programme. The vision for the HST Programme was last consulted on in 2021. Specifically, it evaluates technologies that:

- meet the definition of an HST, as described in legislation in [Schedule 4 of the NHS Commissioning Board and Clinical Commissioning Groups \(Responsibilities and Standing Rules\) Regulations 2012](#), or may potentially need nationally coordinated delivery approaches, and
- need consideration using the methods and processes of the HST Programme, as identified through the HST routing criteria.

NICE's standard technology appraisals methods and processes are designed to be flexible, and adaptable for all technologies and conditions. They are therefore suitable for most technologies that treat rare diseases and small populations.

The HST Programme is designed to be used in exceptional circumstances. Its purpose is to evaluate technologies for ultra-rare diseases that have:

- small numbers of patients
- limited or no treatment options
- challenges for research and difficulties with collecting evidence because of the uniqueness of the disease.

The HST Programme aims to:

- encourage research on, and innovation for, ultra-rare diseases when there are challenges in generating an evidence base that is robust enough to bring the product to market
- secure fairer and more equitable treatment access for very small populations with ultra-rare diseases

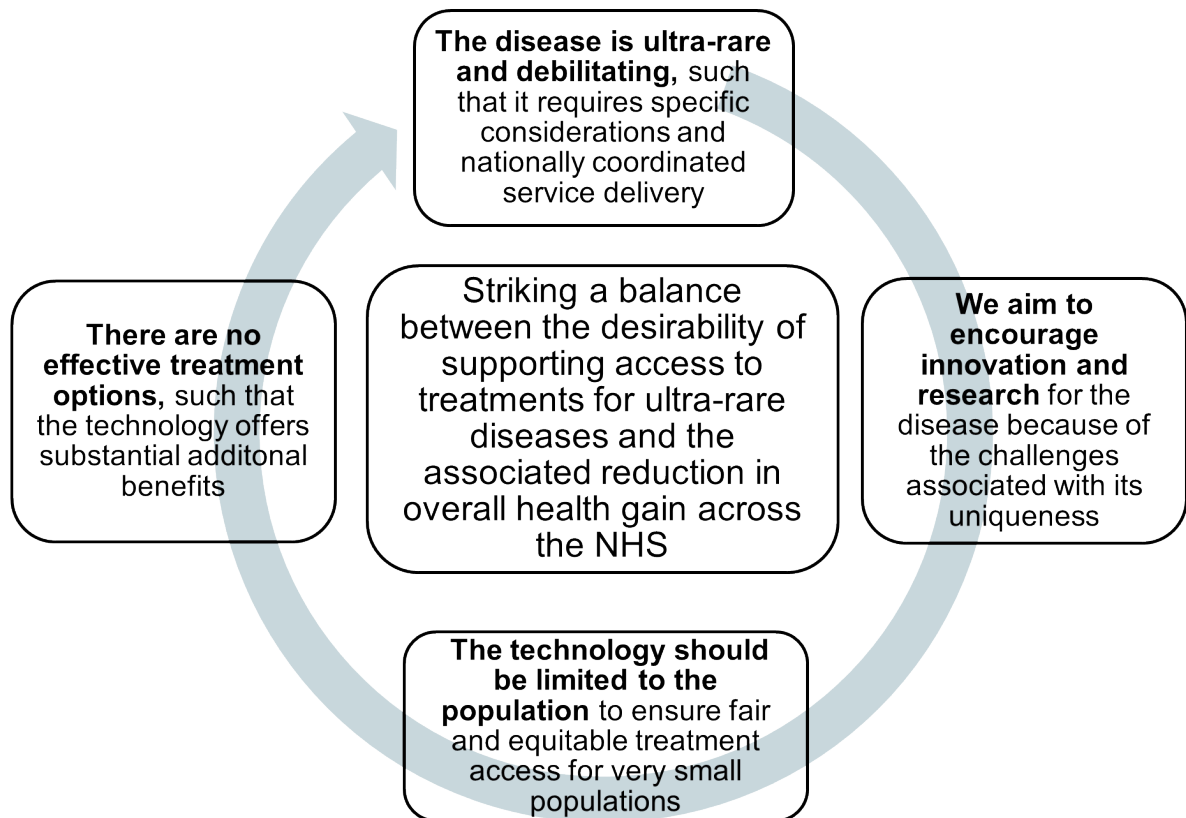
- recognise that an approach that maximises health gain for the NHS may not always be acceptable because it could deliver results that are not equitable.

The HST Programme acknowledges that:

- It is important for NICE to apply appropriate constraints to the ultra-rare disease technologies that can potentially be routed to the HST Programme. This is because the HST Programme is a deliberate departure from the standard technology appraisal process. It values the benefits of HSTs more by having a much higher incremental cost-effectiveness ratio (ICER) threshold for the reasons outlined.
- Each time NICE routes a topic to the HST Programme, and if the technology is recommended, it is deciding that the NHS must commit to allocating resources that would otherwise have been used for activities that would be expected to generate greater population health benefits.
- NICE has sought to strike a balance between the desirability of supporting access to treatments for ultra-rare diseases and the resulting inevitable reduction in overall health gain across the NHS. Both considerations are valid and important, and neither can be given absolute priority over the other. So, the HST routing criteria and their anticipated application do not intentionally seek to capture every case in which there are challenges in generating an evidence base, or when there is a small population with an ultra-rare disease.
- This approach ensures that technologies routed to the HST Programme fulfil the vision of the programme and manages the displacement in the wider NHS. How the context of the HST vision is linked to the HST routing criteria is illustrated in figure 1.

It can be challenging to identify the exceptional circumstances in which the HST methods and processes should be used. This is because of difficulty in getting the information needed at the point of a routing decision. Proxy information is often relied on and used to make judgements. The HST routing criteria identify which technologies should be routed to the HST Programme. These criteria help the NICE prioritisation board make judgements that are as informed, justifiable, consistent, predictable, and transparent as possible. NICE's capacity to develop HST guidance can react to need. There is no limit on the number of technologies that can be routed to the HST Programme.

Figure 1: Summary of the vision of the HST Programme



2 HST routing criteria

Technologies will be considered eligible for routing to the HST Programme if the [NICE prioritisation board](#) agrees that all 4 routing criteria have been met (see [section 3](#)). Each criterion has a set of definitions that help to explain how it will be assessed. The criteria should not be assessed in isolation. The NICE prioritisation board will always consider all the definitions when assessing each routing criterion.

2.1 Criterion 1: The disease is ultra-rare and debilitating

Description of the HST Programme's vision

The rarer a disease is, the more challenging it is to do research and generate an evidence base that is robust enough to bring an effective technology to market. The HST Programme's vision aims to encourage research when it is most challenging.

Not all ultra-rare diseases are debilitating. The vision focuses on ultra-rare diseases that cause ongoing debilitating symptoms and have an exceptional burden on the people with them, and on their carers and families. This is to justify prioritising access to HST technologies over overall population health.

Routing criterion 1

The disease is ultra-rare and debilitating, that is,

- 1A: it is defined as having a point prevalence of 1:50,000 or less in England ([NICE strategic principles for rare disease](#)).
- 1B: it is lifelong after diagnosis with current treatment, and has an exceptional negative impact and burden on people with the ultra-rare disease, and their carers and families.

Definitions

These definitions have been developed to help define what an ultra-rare disease is, and

the debilitating nature of the disease. Relevant information should be collected during scoping by NICE (from the company, and other research or academic sources) to explain how each definition is considered by the [NICE prioritisation board](#).

- 1A of routing criterion 1 is about defining the ultra-rare 'disease', not about the symptoms associated with the ultra-rare disease (regardless of whether the symptom or set of symptoms are the dominating feature). 1B of routing criterion 1 is about the characteristics of the ultra-rare disease.
- 'Disease' refers to a condition for which a diagnosis can be made using the International Classification of Diseases (ICD-11) developed by the World Health Organization (WHO) as a guiding tool. Diagnosis is based on a unique set of signs and symptoms (characteristics) identified using:
 - clinical examination
 - patient history
 - imaging or laboratory tests that are, or can be made, available in the NHS in England.
- 'Disease' does not refer to subgroups based on age, sex, severity, or genetic subtype. These will only be considered if they are clinically meaningful.
- 'Point prevalence' refers to the point prevalence of the 'disease' in England. It counts the number of people with a diagnosis of the disease thought to be alive in England (numerator) on a given index date compared with the total population of England (denominator) at that time ([NHS England](#)).
- 'Lifelong' indicates that the disease needs ongoing clinical management, supportive care, or both.
- 'Exceptional negative impact' refers to shortened length of life or severely impaired quality of life. The precise assessment of this will require an element of subjective judgement.

2.2 Criterion 2: NICE aims to encourage innovation and research

Description of the HST Programme's vision

This criterion is designed to uphold the HST Programme's vision to encourage innovation and research into ultra-rare and debilitating diseases for which there is poor service provision within the NHS (for example, delay in diagnosis, no treatment options beyond supportive care). Without these incentives from the HST Programme, the technology may not be available either after launch, or during development or testing of the technology in England. The availability of the innovation can also reshape NHS services and advance awareness.

Routing criterion 2

The technology is an innovation for the ultra-rare disease.

Definitions

These definitions have been developed to help define an innovative technology. Information about the technology should be collected by NICE from relevant sources (for example, the Medicines and Healthcare products Regulatory Agency [MHRA], ongoing trials, registries) to explain how each definition is considered.

- 'Innovation' refers to a technology or medicine such as an advanced therapy medicinal product (ATMP), a new chemical or biological entity, or a novel drug device combination that brings additional health gains to people with the ultra-rare disease (compared with existing treatment or best supportive care).
- To ensure the technology is an innovation for the ultra-rare disease:
 - the technology should not be a repurposed technology
 - the indication for the technology should not be a significant extension of an indication from another population or disease.
- A repurposed technology means new uses for medicines that are outside the scope of the existing licence for the medicine. This typically involves taking an existing

medicine that already has a marketing authorisation or licence for human use for a particular condition and then using it to treat another condition. This can also include generic treatments or treatments that have had marketing authorisation withdrawn and the developer is seeking a new indication.

2.3 Criterion 3: The technology should be limited to the population in its licensed indication

Description of the HST Programme's vision

This criterion is designed to establish the acceptability of the technology as an effective use of NHS resources, considering the significantly higher ICER threshold. So, the eligible population needs to be small. This is to strike a balance between the desirability of supporting access to treatments for ultra-rare diseases and the inevitable reduction in overall health gain across the NHS because of a higher ICER threshold. A small subpopulation within a population with a common disease would not be suitable for the HST Programme.

Routing criterion 3

No more than 300 people in England are eligible for the technology in its licensed indication, and the technology is not an individualised medicine.

Definitions

These definitions have been developed to help define what kind of licensed indication is suitable for a technology to be considered for routing to the HST Programme, and to help explain what an individualised medicine is. Relevant information about the licensed indication of the technology should be collected by NICE to explain how each definition is considered.

- 'Eligible' refers to everyone who could have the technology under its marketing authorisation (obtained or in the process of being obtained) in England.
- The 'technology' should only be developed for the ultra-rare disease, so the eligible population is small. The technology:

- has to be the first licensed treatment indicated for the ultra-rare disease under consideration
 - should not be an extension of an indication from another:
 - ◇ related population or disease, or
 - ◇ subgroup of people with the same ultra-rare disease under consideration
 - is unlikely to be suitable for other subgroups of the population with the ultra-rare disease in the future who are outside of its first indication.
- 'Individualised medicine' refers to a medicine that is developed based on a person's unique genetic profile (n of 1), or on the genetic profile of monozygotic twins or triplets.

2.4 Criterion 4: There are no effective treatment options

Description of the HST Programme's vision

This criterion is designed to address the lack of effective treatment and access to NHS services for some ultra-rare diseases. To justify prioritising treatment access for ultra-rare diseases over overall population health, the technology under consideration should be anticipated to provide substantial health benefits to people with the disease over existing clinical management and supportive care.

Routing criterion 4

The technology is likely to offer substantial additional benefit for people with the ultra-rare disease over existing established clinical management, and the existing established clinical management is considered inadequate.

Definitions

These definitions have been developed to help define what is substantial additional benefit, and to help to explain the meaning of no other treatment options. Relevant information should be collected by NICE to explain how each definition is considered.

- 'Substantial additional benefit' means that the technology is likely to:
 - significantly redress the reduced length of life, or
 - is likely to demonstrate substantial improvements in the severely impaired quality of life attributable to the ultra-rare disease, as exemplified by research data on clinically relevant measures, for example, patient-reported outcome measures (PROMs).

- 'The technology' means that:
 - if the technology is a disease-modifying treatment (including curative treatment), there is no other disease-modifying treatment available in the NHS in England for the same ultra-rare disease at the time of the routing decision, or
 - if the technology treats a symptom or set of symptoms unique to the ultra-rare disease, there is no other treatment available in the NHS in England for the same symptom for which the technology is indicated at the time of the routing decision.

3 Routing deliberation and decision

To ensure transparency and consistency of routing decisions, an HST routing assessment checklist (see [appendix A](#)) will be used to collate all relevant information by the NICE technical team. This information will be presented by the NICE technical team to the NICE prioritisation board for discussion and deliberation. At the end of the discussion and deliberation, the NICE prioritisation board members will vote on each routing criterion as 'met' or 'not met.' All 4 HST routing criteria need to be voted by the NICE prioritisation board as 'met' (that is, with a majority vote being met) for the technology to be routed to the HST Programme. If the NICE prioritisation board thinks more information is needed to reach a decision, the decision will be deferred while relevant information is obtained. Rationales for the routing decisions will be captured in the HST routing assessment checklist. If there are split votes (50% compared with 50%) for any individual criterion, the NICE prioritisation board chair will have the deciding final vote.

All HST routing decisions will be available on the [NICE prioritisation board webpage](#). This will also have the rationales for the decisions supplemented by the completed HST routing assessment checklist, and the timeframe for participating in the clarification process.

4 HST routing decision clarification process

The aim of the clarification process is to explain NICE's reasons for its HST routing decision(s) that are queried by stakeholders. For more details on the clarification process, see [section 12 of the NICE-wide topic prioritisation: the manual \(PMG46\)](#).

5 Review of the HST routing criteria

NICE will review the HST routing criteria 3 years after its publication to assess the need of further refinement. In exceptional circumstances, the review may be started sooner than the 3 year interval.

Appendix A: Highly specialised technology (HST) routing assessment checklist

Introduction

The NICE HST routing assessment checklist highlights when a technology meets or does not meet the criteria for routing it to the HST Programme. All 4 criteria need to be met for a technology to be routed.

HST routing criteria checklist

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
<p>Criterion 1</p> <p>The disease is ultra-rare and debilitating, that is:</p> <ul style="list-style-type: none"> • 1A: It is defined as having a point prevalence of 1:50,000 or less in England (NICE strategic principles for rare disease). • 1B: It is lifelong after diagnosis with current treatment, and has an exceptional negative impact and burden on people with the ultra-rare disease, and their families and carers. 	<p>'Disease' refers to a condition for which a diagnosis can be made using the International Classification of Diseases (ICD-11) developed by the World Health Organization (WHO) as a guiding tool. Diagnosis is based on a unique set of signs and symptoms (characteristics) identified using:</p> <ul style="list-style-type: none"> • clinical examination • patient history • imaging or laboratory tests that are, or can be made, available in the NHS in England. <p>'Disease' does not refer to subgroups based on age, sex, severity, or genetic subtype. These will only be considered if they are clinically meaningful.</p> <p>Have these definitions been met or not met? [Yes or No]</p> <p>Notes and rationales:</p> <p>'Point prevalence' refers to the point prevalence of the 'disease' in England. It counts the number of people with a diagnosis of the disease thought to be alive in England (numerator) on a given index date compared with the total population of England (denominator) at that time (NHS England).</p> <p>Has this definition been met or not met? [Yes or No]</p>

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
	<p>Notes and rationales:</p> <p>'Lifelong' indicates that the disease needs ongoing clinical management, supportive care, or both.</p> <p>Has this definition been met or not met? [Yes or No]</p> <p>Notes and rationales:</p> <p>'Exceptional negative impact' refers to shortened length of life or severely impaired quality of life. The precise assessment will require an element of subjective judgement.</p> <p>Has this definition been met or not met? [Yes or No]</p> <p>Notes and rationales:</p>

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
<p>Criterion 2</p> <p>The technology is an innovation for the ultra-rare disease.</p>	<p>'Innovation' refers to a technology or medicine such as an advanced therapy medicinal product (ATMP), a new chemical or biological entity, or a novel drug device combination that brings additional health gains to people with the ultra-rare disease (compared with existing treatment or best supportive care).</p> <p>Has this definition been met or not met?</p> <p>[Yes or No]</p> <p>Notes and rationales:</p> <p>To ensure the technology is an innovation for the ultra-rare disease:</p> <ul style="list-style-type: none"> • the technology should not be a repurposed technology • the indication for the technology should not be a significant extension of an indication from another population or disease. <p>Has this definition been met or not met?</p> <p>[Yes or No]</p> <p>Notes and rationales:</p>

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
<p>Criterion 3</p> <p>No more than 300 people in England are eligible for the technology in its licensed indication, and the technology is not an individualised medicine.</p>	<p>'Eligible' refers to everyone who could have the technology under its marketing authorisation (obtained or in the process of obtaining) in England.</p> <p>Has this definition been met or not met? [Yes or No]</p> <p>Notes and rationales:</p> <p>The 'technology' should only be developed for the ultra-rare disease, so the eligible population is small. The technology:</p> <ul style="list-style-type: none"> • has to be the first treatment for the 'licensed indication' for the ultra-rare disease under consideration • should not be an extension of an indication from another: <ul style="list-style-type: none"> – related population or disease, or – subgroup of people with the same ultra-rare disease under consideration. • is unlikely to be suitable for other subgroups of the population with the ultra-rare disease in the future who are outside of its first indication. <p>Has this definition been met or not met? [Yes or No]</p> <p>Notes and rationales:</p>

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
	<p>'Individualised medicine' refers to a medicine that is developed based on a person's unique genetic profile (n of 1), or on the genetic profile of monozygotic twins or triplets.</p> <p>Has this definition been met or not met?</p> <p>[Yes or No]</p> <p>Notes and rationales:</p>

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
<p>Criterion 4</p> <p>The technology is likely to offer substantial additional benefit for people with the ultra-rare disease over existing established clinical management, and the existing established clinical management is considered inadequate.</p>	<p>'Substantial additional benefit' means that, the technology is likely to:</p> <ul style="list-style-type: none"> • significantly redress the reduced length of life, or • is likely to demonstrate substantial improvements in the severely impaired quality of life attributable to the ultra-rare disease, as exemplified by research data on clinically relevant measures, for example, patient-reported outcome measures (PROMs). <p>Has this definition been met or not met? [Yes or No]</p> <p>Notes and rationales:</p> <p>'The technology' means that:</p> <ul style="list-style-type: none"> • if the technology is a disease-modifying treatment (including curative treatment), there is no other disease-modifying treatment available in the NHS in England for the same ultra-rare disease at the time of the routing decision, or • if the technology treats a symptom or set of symptoms unique to the ultra-rare disease, there is no other treatment available in the NHS in England for the same symptom for which the technology is indicated at the time of the routing decision. <p>Has this definition been met or not met? [Yes or No]</p>

Highly specialised technologies: NICE prioritisation board routing criteria

Routing criteria	Descriptions of how the criteria are met or not met through assessing the definitions
	Notes and rationales:
	Overall routing decision: [highly specialised technology or technology appraisal] Other comments:

Appendix B: Routing process for highly specialised technologies (HST)

[Appendix B is available as a downloadable pdf.](#)

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