Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

Valuing rarity
Learnings from European HTA systems

Introduction

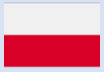
In August 2024, the SHCA published a report setting out the impact of the 2022 NICE methods and process review on access to rare disease treatments. We found that **many rare disease patients are still waiting too long to access new treatments**, and in some cases are missing out altogether on potentially life-changing treatments available in other European countries.

Informed by the findings and engagement with SHCA members, both through one-on-one interviews and a survey shared with the full membership, the SHCA called for a specific focus on rarity in future modular updates to NICE's methods, alongside a *NICE Listens* exercise to understand the social value associated with treating rare diseases. 2026 presents an important opportunity for NICE to review its approach to valuing rarity, building on the welcomed developments committed to at the end of 2025:

- The Medicines and Healthcare products Regulatory Agency (MHRA) will introduce a new set of regulations to speed up the time it takes to test, manufacture and approve rare disease treatments in the UK[1]
- NICE will introduce the first increase to the cost-effectiveness thresholds used in the assessment of new treatments through its standard technology appraisal process since it was established in 1999[2]
- As part of the changes to cost-effectiveness thresholds, NICE will introduce a new value set for use within EQ-5D-5L (the tool NICE uses to assess quality of life and inform calculations about the quality of life gain associated with a treatment)[2]

While the increase in the standard threshold represents a major change in NICE's ways of working, it is unlikely to materially affect access to treatments for rare diseases on its own. The creation of a new regulatory approach to rare disease treatments presents an opportunity to reconsider the reforms that are needed across the whole adoption pathway to unlock better access to new medicines.

To support NICE and the MHRA in reforming the way that rare disease treatments are assessed in England, this report sets out four case studies of changes introduced in other European countries, which provide examples of potential avenues that could be explored in England.


Reforming the way rare disease treatments are valued in HTA


Streamlining early access for new innovations in rare disease treatments


Placing additional value on rare in HTA


A new access pathway for ultra-rare conditions

The SHCA has welcomed NICE's constructive approach to engagement, including through the recent changes to the HST routing criteria. We look forward to continuing to work with NICE and the MHRA to consider how planned reform to the access environment in 2026 can incorporate learnings from the case studies set out in resolving the challenges facing the assessment of rare disease treatments in England.

The SHCA is a coalition of over 140 charities and corporate supporters which advocates on behalf of people living with rare and complex conditions.

Background

The UK Rare Diseases Framework defines a rare disease as occurring in fewer than 1 in 2,000 people, affecting over 3.5 million people in the UK.[3] A very rare disease is defined by NICE as occurring in fewer than 1 in 50,000 people, affecting fewer than 1,100 people.[4] Treatments for very rare conditions that meet a set of strict criteria are assessed through NICE's Highly Specialised Technologies (HST) pathway, whereas all other rare disease treatments are assessed through the Single Technology Appraisal (STA) pathway.[4]

For a rare disease treatment to be approved through the STA programme, NICE needs to find that it is cost-effective in consideration of how a disease affects people's quality of life and the length of life they will gain as a result of an intervention, expressed as quality-adjusted life years (QALYs). Following the changes committed to in December 2025, treatments that cost between £25,000 and £35,000 per QALY may be deemed as cost-effective under an STA if they meet certain criteria (an increase from the previously used thresholds of £20,000 and £30,000).[5]

The HST pathway has a much higher cost-effectiveness threshold of £100,000-£300,000 per QALY, in recognition of the challenges that exist in developing treatments for ultra-rare conditions, including the higher development costs and the difficulties in generating evidence.

The Rare Diseases Framework acknowledges the challenges NICE and other HTA bodies face in evaluating rare disease treatments, due to small patient populations and accompanying uncertainty. Additionally, as approved medicines are only available for around 5% of rare diseases, many rare diseases have no current treatment alternatives. The absence of suitable comparators makes it harder to demonstrate both the impact of a treatment on the quality and length of life and the cost-effectiveness of a new treatment compared to the very low-cost status quo.[6]

These challenges mean that the changes to STA thresholds, whilst welcome, will not come close to addressing the systemic challenges affecting the assessment and approval of rare disease treatments in England. The SHCA's report on valuing rarity in 2024 found that 92% of SHCA members and corporate supporters who responded to our survey did not consider NICE's methods and processes to be suitable for the appraisal of rare disease treatments. The findings illustrated the growing frustration from the rare diseases community that patients in England are at risk of missing out on access to potentially life changing therapies.

The SHCA welcomes the commitment from the MHRA to reform the regulation of rare disease treatments in recognition of these challenges, and its commitment to working with the community in developing solutions. It is encouraging that the MHRA is seeking to address the significant unmet needs in rare disease treatments and has formally acknowledged the need for a bespoke approach that reflects the challenges associated with developing new therapies. The MHRA has stated that these reforms aim "*to simplify evidence requirements and facilitate discussion with HTA assessment methodologies or NHS commissioning and reimbursement frameworks to facilitate ultimate patient access*".[7]

It is essential that NICE, the NHS and Government work with the MHRA to ensure that the creation of any new regulatory pathway is part of broader system-level reforms that unlock current barriers to access. In support of the MHRA's work in considering reforms to the regulation of rare disease treatments, **this report sets out examples of international approaches to the assessment of rare disease treatments that show how alternative approaches could help to address these challenges**.

Reforming the way rare disease treatments are valued in HTA

Background
Poland has historically lagged behind many European countries in access to innovative therapies, including rare disease treatments. It has consistently placed in the lower half of Europe in access to rare disease treatments. Only 38% of new innovative medicines (including orphan and rare disease medicines) are reimbursed, compared with an EU average of 42%.[8]

In recognition of this, in 2024 Poland introduced a new Rare Disease Plan for 2024–2025, with the aim of improving the quality of life for patients with rare diseases and their families.[9] The Plan has recently been extended by a further year, and a range of reforms have been introduced across the care and treatment pathway, with specific funding allocated to delivery of the plan.

Policy change
One of the reforms introduced is to the way treatments for rare diseases are valued and assessed, adapting the reimbursement pathway to enable faster patient access to new innovative treatments. Under the reforms, rare disease treatments will be treated differently in the reimbursement procedure, with regulations introduced to expand the evaluation criteria used in assessment to consider therapeutic benefit and societal value in addition to price, recognising the fact that in many rare diseases there is no existing treatment option.[9,10] The Minister of Health is introducing a Multi-Criteria Decision Analysis (MCDA) framework to deliver on this, and whilst final details are yet to be announced, it is expected to include unmet need, severity of a condition and societal value.

The role of patient groups in HTA is also being expanded, so that they are able to participate in discussions with the Economic Commission alongside the manufacturer, providing additional evidence on the prospective impact of a new treatment on quality of life. This involves supporting the Polish Economic Commission in an advisory capacity, to provide qualitative evidence on unmet need and lived experience, as opposed to the commercial aspect of negotiations.[9]

Additionally, to address the high number of rare disease treatments approved with only conditional access (around 85% of rare disease treatments approved in Poland are only available with restrictions)[11], new regulations will also prevent Poland's HTA Agency from issuing 'conditional' recommendations on access – with access instead provided for an indefinite time.[9] There is no formal reassessment window, with reassessment triggered by new evidence as opposed to being tied to a specific time period. This could be a result of additional real-world data or a comparator treatment becoming available, or a wider development that impacts clinical and cost-effectiveness.

Outcomes
The above changes to the assessment of rare disease treatments are in the process of being implemented, so their impact on the HTA process remains uncertain. However Poland's growing focus on the access environment has led to steady progress in EFPIA's indicators over the last five years. Poland has also achieved breakthroughs in the treatment of cystic fibrosis and SMA following new access details.

Streamlining early access for new innovations in rare disease treatments

Background
The French HTA system has a strong track record in supporting access to the latest innovations in rare disease treatments, in comparison to many European peers. In February 2025, France launched its fourth National Plan for Rare Diseases for 2025–2030. The plan introduced several commitments aimed at improving care for people with rare diseases, speeding up research, and supporting access to new innovation.[12] Actions linked to access include supporting the use of real-world evidence in strengthening early and compassionate access schemes.

In 2021, France reformed its early access scheme for treatments with higher levels of uncertainty, introducing a new Autorisation d'Accès Précoce (AAP) framework – consolidating existing early access schemes into a single new framework.[13] This included the previous Autorisation Temporaire d'Utilisation (ATU), which enabled temporary reimbursement use for drugs without a marketing authorisation. Similar to other early access schemes, the framework is intended to provide interim access whilst further evidence is collected on a prospective new treatment and long term reimbursement finalised.

Policy change
The AAP framework covers treatments that have the potential to provide significant patient benefit, have a robust development plan, and address unmet needs – making the scheme particularly applicable to new rare disease treatments.[13]

Whilst the previous ATU scheme had no legal requirement on decision-making timelines, the new AAP scheme sets out an expectation that applications are evaluated within three months – with a reported median timeline to date of 78.5 days.[14] Whilst companies are able to set prices freely as part of the scheme to speed up review timelines, a payback mechanism ensures that if the final negotiated price is lower than the price set for the AAP access, the manufacturer pays back the difference. There are two routes for treatment both for medicines that are yet to receive a MA but have strong preliminary data, and those that have received a MA but are not yet reimbursed.[15]

Outcomes
Following the introduction of the new AAP scheme, between the second half of 2021 and the first half of 2024, a total of 288 early access decisions were issued, including 76 for pre MA, 96 for post MA, and 116 for renewals. There was a 70% approval rate of treatments considered in the first two categories.

There is no published breakdown of access data under the scheme for rare diseases specifically. However, France consistently performs strongly in the EFPIA WAIT indicator on both availability and time to access – with a 68% rate of availability for rare disease treatments, compared to 52% in England.[8]

Placing additional value on rare in HTA

Background
Germany has the highest rate of full availability of rare disease treatments in Europe. Its 89% rate of full availability is more than double that of the EU average (42%) and significantly higher than England's (52%).[8]

Policy change
Germany's HTA system places specific value on treatments for rare diseases, recognising the challenges of research and development in these treatments and the high levels of uncertainty that often accompany them. Under the Arzneimittelmarktneuordnungsgesetz (AMNOG) framework introduced in 2011, medicines designated as rare disease treatments are automatically deemed to demonstrate an additional benefit at launch by virtue of their rarity. As a result, manufacturers are not required to submit comparative clinical evidence as part of the initial HTA assessment.[16]

This applies to all rare disease treatments, regardless of sales volume, enabling immediate reimbursement following marketing authorisation by the European Medicines Agency (EMA). A full benefit assessment is only triggered once annual statutory health insurance sales exceed €30 million.[17] Similar to France, companies freely set prices for treatments under consideration for six months while assessment is carried out of the medicine's additional benefit compared to the standard of care. A payback mechanism then applies if the final agreed price is lower. However unlike in France this applies to all new innovative treatments, as opposed to those specifically addressing conditions with unmet need.

Outcomes
The automatic recognition of additional benefit for rare disease treatments reduces the burden of generating evidence and mitigates the disadvantages associated with small patient populations and limited comparative data. The early access model helps to provide fast access to treatments for patients, whilst real-world data is collected to manage uncertainty.

A new access pathway for ultra-rare conditions

Background
Prior to 2018, rare and very rare disease treatments in Scotland were assessed through the standard Scottish Medicines Consortium (SMC) appraisal process, which was applied equally to all treatments regardless of population size. The introduction of the SMC's ultra-orphan pathway helped to address some of these challenges, following recognition standard HTA processes were not suitable for medicines treating very small patient populations.[18] Previously access to treatment in ultra-rare conditions was often reliant on Individual Patient Treatment Requests (IPTR) or the Peer Approved Clinical System (PACS) when medicines were not accepted by SMC.

Policy change
Under the pathway, if a treatment is validated by the SMC as ultra-orphan and found to be clinically effective it will be made available on the NHS for at least three years while further information on effectiveness is gathered. Unlike the HST programme in England, there are no cost-effectiveness thresholds associated with the pathway. Instead, the SMC applies greater flexibility in its economic assessment, recognising the high levels of uncertainty associated with very small patient populations

- with decisions not driven by a fixed ICER threshold. After the interim funding period, the manufacturer is required to make a full resubmission for the SMC to make a final decision on whether the medicine will be made routinely available.[18]

Whilst limited availability places natural limitations on the pathway's effectiveness, it does have benefits in enabling access over that of the HST pathway. The SMC accept a greater level of uncertainty in the economic case when assessing rare disease treatments, including consideration of whether they substantially increase life expectancy and/or quality of life. Mandatory Patient and Clinician Engagement (PACE) meetings are also a key feature of the pathway that enables wider data to be captured around lived experience, unmet need and non-therapeutic benefits.

PACE meetings bring together patient organisations and clinicians to provide structured qualitative evidence on lived experience, disease severity, unmet need and the wider impact of treatment on patients and families. Evidence from these meetings is then formally incorporated into SMC decision-making, ensuring that factors which are difficult to quantify — such as loss of independence, burden on carers and the absence of alternative treatment options — are formally considered alongside clinical and economic data.

Outcomes
As a result of limited eligibility the ultra-orphan pathway has had more limited impact in transforming access to rare disease treatments, with additional flexibility and earlier access only applicable to treatments for ultra-rare conditions. As a result EFPIA data shows that only 44% of rare disease treatments have full availability in Scotland, compared to 42% in Europe.

Conclusion

The UK Rare Diseases Framework has set out a goal to improve access to specialist care, treatments and drugs. Exciting progress has been made in some areas in delivering against this priority, and 2026 could be a significant year in addressing some of the structural challenges affecting the assessment of rare disease treatments set out in the SHCA's 2024 report.

The SHCA welcomes the commitments made by the Government to improve access to innovation, from the MHRA's plans to accelerate access to new rare disease treatments to the wider reforms to the UK commercial environment. This report is intended to be a positive contribution towards these reforms, looking at how they can deliver progress in the assessment of rare disease treatments specifically. If these opportunities are missed, there is a risk that the challenges set out in our 2024 will remain unaddressed and rare disease patients will continue to miss out on the latest breakthroughs in innovation.

Linked to the above developments, now is an opportune time for NICE to study the evidence base for genuine reform to the rare disease assessment pathway by commissioning primary research on the social value associated with treating rare diseases, utilising the NICE Listens programme. The SHCA is calling for a new UK Rare Diseases Framework to be committed to at the end of 2026. Future England rare diseases action plans would create an important vehicle for new actions to be committed to on improving access to care, treatment and drugs.

This paper sets out what alternative systems to the assessment of rare disease treatments could look like, applying learnings from other European approaches to valuing rarity:

⇒ Reforming the way rare is valued in HTA, placing additional value on therapeutic benefit and societal value, as well as unmet need

⇒ Utilising early access schemes for rare disease treatments with higher levels of uncertainty that have the potential to provide significant patient benefit. In England this could be through reform

to the IMF to facilitate the approval of rare disease treatments that are candidates for managed access agreements

⇒ Introducing a specific framework for applying flexibility in the assessment of rare disease treatments. The NICE methods review recommended that increased flexibility is applied to treatments where there are high levels of uncertainty, this has not been delivered in practice

The SHCA looks forward to working with NICE, NHS England and the MHRA in supporting the delivery of reforms committed to and considering how learnings from European HTA systems can be applied.

January 2026

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