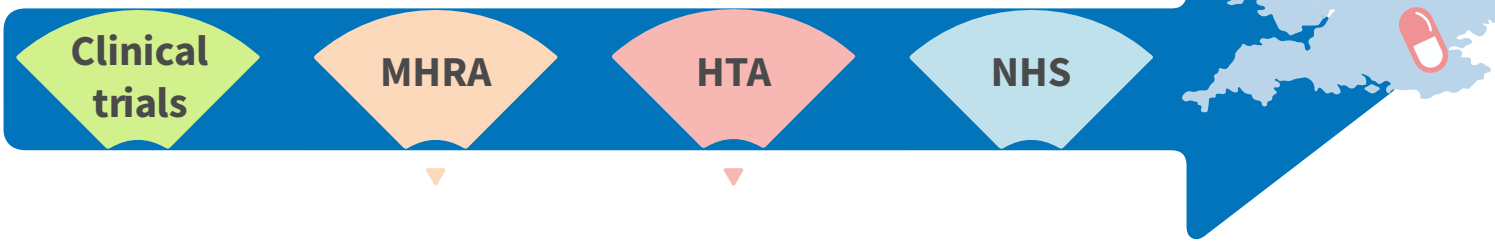
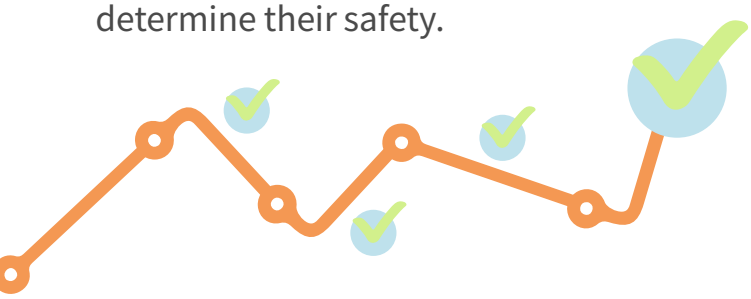
Access to rare disease medicines in the UK

An ‘orphan medicine’ is a name given to a medicine that treats a rare condition, a condition that affects less than 1 in 2,000 people.



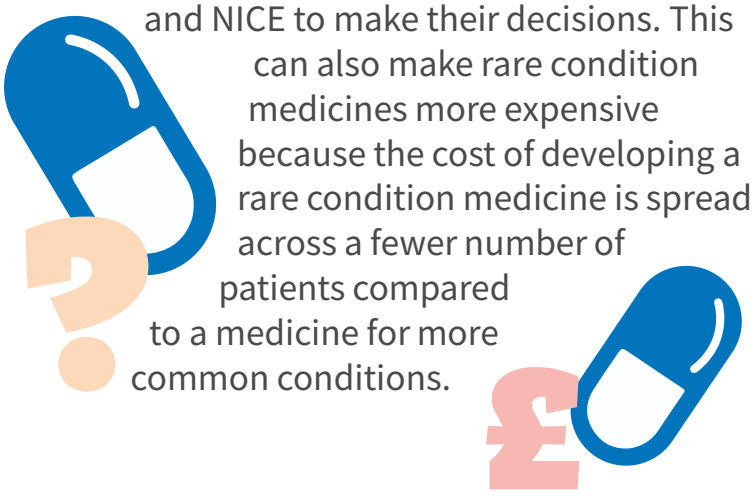
The Medicines Healthcare Regulatory Agency (MHRA) offers marketing authorisations to medicines and medical devices in the UK by assessing scientific data, such as clinical trial data, to determine their safety.



In the UK, medicines also go through a Health Technology Assessment (HTA) which decides whether or not a medicine is good value for money and should be provided on the NHS or not. The relevant organisations that carry out these assessments in the UK are the National Institute of Health and Care Excellence (NICE) for England, the Scottish Medicines Consortium (SMC) and the All Wales Medicines Strategy Group (AWMG). It is common for the AWMG to follow the same decision as NICE.

Challenges

Due to the small numbers of people affected by each rare condition, it can sometimes be difficult to get enough evidence, causing uncertainty for the MHRA and NICE to make their decisions. This can also make rare condition medicines more expensive because the cost of developing a rare condition medicine is spread across a fewer number of patients compared to a medicine for more common conditions.



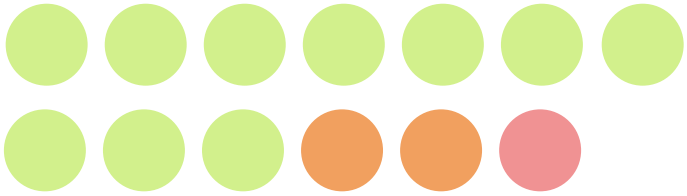
The current processes for assessing medicines are generally better suited for common conditions or conditions that affect people for a short period of time (acute illnesses) compared to life-long conditions (chronic illnesses), which many rare conditions are. Since February 2022, NICE have changed the way they carry out their assessments on new medicines following a long review. It will take some time to understand how effective these changes have been but they are aimed at being better able to handle the uncertainty that comes with rare condition medicines.

Rare condition medicines in the UK

There are over 7,000 rare conditions and only **1 out of 20** rare conditions have an approved treatment or medicine to help. Where there is an approved treatment, these are mostly for rare cancers.

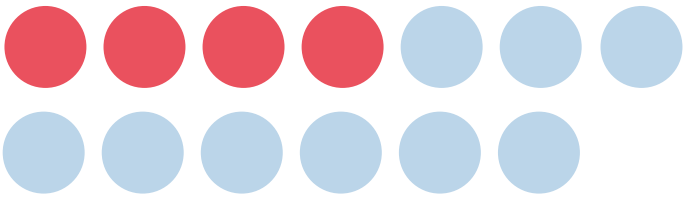
During this period it took, on average, 12 months for a rare condition medicine to be assessed by NICE.

Since NICE's review, there have been **13** rare condition medicines that have completed the HTA process (between 1 February 2022 and 31 October 2023). Of those, **10** were recommended, **2** were recommended with restrictions and **1** was not recommended.



NICE has two main HTA pathways to assess a medicine's cost effectiveness. The **Single Technology Appraisal route (STA)** and the **Highly Specialised Technology Appraisal route (HST)**. The STA pathway is the standard pathway whereas the HST pathway is designed to assess rare condition medicines that meet certain criteria.

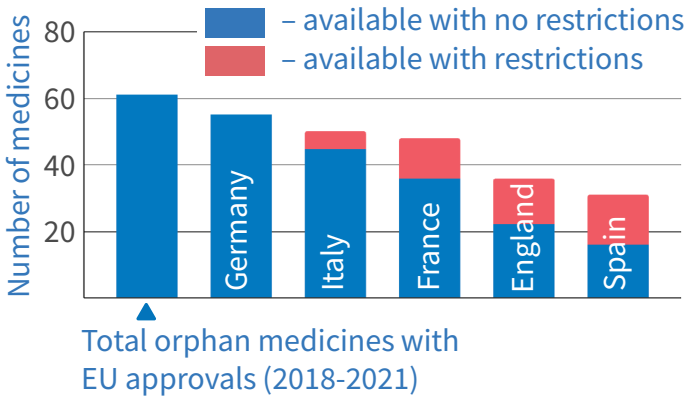
Of the 13 rare condition medicines that have completed the NICE appraisal since February 2022, only **4** medicines were routed via the HST pathway, the remaining **9** were assessed via the STA pathway.



Medicines that treat severe conditions and are going through the STA process may be eligible to have a modifier applied that allows a higher cost threshold. Of the 9 rare condition medicines that were routed via the STA pathway, 2 of them had the severity modifier applied.

How does the UK compare to other countries?

According to a recent survey* 61 orphan medicines received EU approvals between 2018 and 2021. Of those, 36 orphan medicines were made available in England, compared to 55 in Germany, 50 in Italy, 48 in France, and 31 in Spain.



We'd like to thank MAP Patient Access Ltd for giving us the data presented in this factsheet.

* IQVIA EFPIA Patients W.A.I.T. Indicator 2022 Survey