How new medicines and treatments become available in the UK

Once a medicine or other treatments (a procedure or device) have been developed, they have to go through several stages before becoming available to be prescribed to patients.

There is no one route through these stages that all medicines and treatments go through on their way to being available.

Here we describe the key stages and routes, and what the implications are for patients as a medicine or other treatment go through them.

Stage one:
Licensing

New medicines and treatments go through a rigorous process of testing during the development process (e.g. clinical trials). They must be proved to be safe and have some level of effectiveness at treating a condition before they can be licensed for use.

In the UK, the organisation that makes this assessment and grants these licences is the Medicines and Healthcare products Regulatory Agency (MHRA). Its licences declare what health condition the medicine and treatment should be used for and the recommended dosage.

This process can take several months to several years.

Medicines or treatments that have been licensed by the MHRA are available for healthcare professionals to prescribe privately, which means that the patient has to pay for them. In order for them to be available on the NHS as well, they need to go through another stage of assessment (see stage two below).

Note: Unlicensed medicines and treatments

There are times when a healthcare professional may recommend to a patient a medicine or treatment that has been licensed for another condition, but not for the patient's condition. This is known as 'off-label prescription'. This can happen if there is evidence that the medicine or treatment prescribed off-label can be beneficial at treating that condition (e.g. through a clinical trial).

Duchenne UK

Stage two:

Approval for use on the NHS

There are two main routes that drugs and treatments can go down to be accessed through the NHS in England; one through the MHRA, and one through NICE (National Institute for Health and Care Excellence). The NICE health technology appraisal route is further divided into multiple strands, two of which are most relevant to Duchenne muscular dystrophy. The stages and implications of the different routes are outlined below.

Route	**The early access to medicines scheme (EAMS)**	**Technology appraisal (TA)**	
		Single technology appraisal (STA)	Highly specialised technology (HST) evaluations
Who approves medicines and treatments on this route	MHRA	NICE	
What this route does	EAMS allows drugs and treatments to be prescribed by healthcare professionals to patients while MHRA is still assessing them and before it has granted them a full licence.	The STA assesses a medicine or treatment and decides if it can be available to be prescribed on the NHS in England. It takes into account the need for it, its effectiveness at treating the condition, and its cost. The Scottish Medicines Consortium (SMC) undertakes a similar process for NHS Scotland. While there aren't health technology assessments for the NHS in Wales and Northern Ireland, there is a process to decide on their use for them which often follows the NICE decision.	HST assessments are for very rare diseases, with a patient population in England of around 1,100.

			
Timing	Drugs and treatments are approved via EAMS usually in about 12 to 18 months before they are granted a full licence by the MHRA.	This process usually takes about six months to a year.	This process usually takes several months.
Why drugs and treatments go down this route	EAMS is intended to grant early access to medicines before they have been fully assessed by regulatory bodies. Drugs and treatments go down this route for two important reasons. The first is that there is a clear unmet medical need, which means that there is currently no effective treatment for the condition that they aim to treat. The second is that the medicine or treatment has been designated as a promising innovative medicine (PIM). Medicines and treatments that are designated as PIM are those that the early data from their trials show that they have a significant benefit for patients in life-threatening or seriously debilitating conditions.	Most medicines and treatments go down this route. It is usually those that don't qualify for the HST route. A medicine or treatment may go down this route and the EAMS route at the same time.	HST assessments are for medicines and treatments for very rare conditions. They can be for new drugs and treatments or for existing drugs and treatments which have been found to be effective at treating a new condition. They are for medicines and treatments that significantly improve both the quality and the quantity of life of the person with the condition. As a result, the economic assessment of these treatments are easier to pass. A medicine or treatment may go down this route and the EAMS route at the same time.
What does medicines and treatments approved through this route mean for patients	Medicines and drugs being approved via the EAMS route can be available sooner than the those that go through a full licencing process. However, they are likely to have greater restrictions on who and how they can be prescribed to. Because approval to go through EAMS is based on early data from clinical trials on those drugs, it can be cancelled if further data shows the drug to be less effective or not safe. Because the drug or treatment does not have a full MHRA licence, some healthcare professionals may be reluctant to recommend it to patients.	It means that they are available to be recommended on the NHS by healthcare professionals to patients, and that this approval will outline who can be prescribed it and how.	It means that they are available to be recommended on the NHS by healthcare professionals to patients, and that this approval will outline who can be prescribed it and how.