Ataxia UK – UK Medicines Approval Process webinar - FAQs

This FAQs document is connected to the UK medicines approval process educational webinar hosted by Ataxia UK in July 2024. The webinar explains how patient groups such as Ataxia UK and people affected with ataxia can get involved and have an impact in the decision-making process.

The webinar is aimed at people with ataxia and their carers or families. This webinar is sponsored by Biogen. Biogen has not had any involvement in its content, production or delivery.

1. What is patient access to medicines?

Successful patient access means a new treatment is available to patients.

Healthcare systems, such as the NHS in the UK, have processes for deciding which new treatments will be available to patients.

2. Who decides which new medicines will be available to NHS patients?

Before a medicine can be routinely used by the NHS it needs to be approved by the Medicines and Healthcare products Regulatory Agency (MHRA) for use in a particular condition or group of patients. This medicine then has a Marketing Authorisation, or license, for this condition/group of patients.

The UK has different agencies that decide whether or not to make a new medicine available to NHS patients. Most new medicines go through a Health Technology Assessment (HTA), which looks at whether a new treatment provides value for money for the NHS.

In Scotland, the Scottish Medicines Consortium (SMC) makes recommendations about most new medicines and the NHS in Scotland usually implements these recommendations.

In England and Wales, the National Institute for Health and Care Excellence (NICE) assesses most new medicines. NICE produces guidance which the NHS implements. The NHS in Northern Ireland also usually adopts NICE guidance.

The All Wales Medicines Strategy Group (AWMSG) may look at treatments that NICE has not assessed. These decisions are implemented by the NHS.

3. How long does a decision take?

NICE and the SMC aim to make decisions as quickly as possible and to have developed a draft decision within 6 months of a Marketing Authorisation. It can sometimes take longer to reach a final decision if more information is needed or the manufacturer is agreeing financial arrangements that would make the medicine better value for money.

4. What happens in an HTA?

In an ideal world, access would mean that all patients who could benefit from a treatment could receive it when and where they need it, at a price that is affordable to the healthcare system.

However, in reality there is a limited budget to deliver healthcare services and difficult decisions must be made about how to prioritise. If a new treatment is introduced, something else may need to change or stop to make space for it; the cost impacts of new treatments are evaluated through health economic analyses. Health technology assessments (HTAs) are used to give decision-makers (known as HTA bodies) important information to decide which treatments or services should be paid for (known as reimbursement).

Health Technology Assessments, or appraisals, review the evidence for the clinical and cost effectiveness of a new medicine. They will look at:

- The unmet need of the target patient population
- The benefits of a new medicine
- The benefits of current clinical care (the comparator)
- The cost of current care
- The costs of introducing a new medicine

The value of a treatment can go beyond its clinical outcomes and financial cost. NICE and the SMC consider the value that the treatment can offer to patients in terms of improving health-related quality of life (HRQoL) (a measure of someone's perceived physical and mental health over time) or being more convenient (for example, an oral treatment instead of an injection). They may also consider additional factors such as healthcare resources (for example, time needed from doctors or nurses to administer the treatment).

5. What can Ataxia UK do?

Ataxia UK can be a stakeholder in the process to ensure that the views of patients are represented.

Some examples include:

- Gathering evidence about the impact of the condition, current treatments and (if possible) the new treatment
- Representing the unique insights of patients by making written submissions and attending meetings
- Identifying and working with clinical experts
- Collaborating with other Patient Groups

6. What can the Ataxia community do?

The community can get involved through opportunities that Ataxia UK provides such as webinars and surveys. The community can also respond to any public consultations about the new medicine.

7. What does the company do?

A company that wants to sell a new medicine in the UK will need to have a marketing authorisation approved by the MHRA. They will also need to provide an evidence submission, or value dossier, to HTA agencies. This will include evidence about:

- Who the treatment is for, and how these patients will be identified
- Current clinical care and clinical outcomes
- The benefits of a new treatment
- Health-related Quality of Life
- The impact of the new treatment on NHS services and budgets

8. What about private patients?

If a medicine is approved by the Medicines and Healthcare products Regulatory Agency (MHRA) for use in a particular condition or group of patients, this medicine then has a Marketing Authorisation for this condition/group of patients and can immediately be prescribed in a private healthcare setting. The NHS would not usually pay for this treatment before an HTA has been completed. Instead, the treatment would be paid for directly by the individual or family or through private health insurance.

9. What happens if I live/travel outside the UK?

If you live outside the UK, the NHS will not usually pay for the cost of treatment in this country. You may have alternative healthcare arrangements in that country and could contact your clinical team for information about the availability of a medicine.

If you travel from the UK to another country to receive a medicine, you should first check how much you will have to pay for the medicine as it will not usually be paid for by the NHS.