



Our position on Treatment Use of Unlicensed Medicines

What is the issue?

GSK is uniting science, talent and technology to get ahead of disease together.

An essential element of our work is to develop our medicines through conducting clinical trials, working with Healthcare Professionals (HCPs), patients and healthy volunteers to evaluate an investigational medicine's efficacy and safety prior to securing regulatory approval.

Regulators will only authorise a new medicine if these trials, together with other research data, demonstrate a medicine has a favourable risk/benefit profile. And a medicine can only be marketed and made available to patients after it has been authorised. We therefore ensure our resources are applied to the efficient conduct of these trials, in order to bring our medicines as quickly as possible to those who need them.

There will, however, be occasions when it may be appropriate for patients to have access to our unlicensed medicines outside of a clinical trial. Under these circumstances, and where patients have serious or life-threatening diseases or conditions with no option to join a clinical trial and no satisfactory alternative treatment options, a company may provide a treating HCP with an unlicensed medicine. Such treatment use of an unlicensed medicine outside a clinical trial is often called "compassionate use" or "expanded access" (but may go by other names).

This paper sets out GSK's approach to the treatment use of our unlicensed medicines and the criteria we use to assess requests from HCPs.

What is GSK's view?

- We recognise that there may be circumstances when it is appropriate for HCPs to give a patient an unlicensed medicine to treat a life threatening or seriously debilitating disease/condition where no satisfactory alternatives exist.
- GSK considers the appropriateness of treatment use for all our investigational medicines early on in the planning of our research programmes. GSK also considers requests for treatment access to a medicine that is licensed in some countries but not licensed where treatment would occur. Decisions to supply unlicensed medicines for treatment use decisions are made by our senior medical staff based solely on medical and ethical criteria.
- We consider treatment use for patient populations meeting specific criteria managed under formal programmes, as well as for individual patients (often called "named patient access") where appropriate and if our criteria are met.
- All requests for treatment use of a GSK medicine must be made by an HCP.
- All HCP requests for treatment use of a GSK medicine are considered against the following criteria:
 - The illness being treated is life threatening or seriously debilitating
 - There are no satisfactory alternative treatments (confirmed by the HCP)
 - There is sufficient evidence to believe the potential benefit to the patient justifies the potential risk.
- Other key considerations include:
 - Whether sufficient information to inform appropriate use of the GSK medicine exists, especially with regard to dosage or treatment duration for the patient

- Whether treatment use of the unlicensed medicine might somehow compromise any related clinical trial or regulatory pathway
- That use will be in a country where appropriate medical capability for treatment use exists
- That use in larger access programmes will only take place in countries where GSK intends to seek regulatory approval and to make the medicine available; the same limitation will not necessarily apply for named (or individual) patients
- Ethical considerations relating to fairness to similar patients
- That the proposed treatment use complies with local laws and regulations.
- These criteria help ensure GSK has a consistent approach for our decisions on treatment use of our unlicensed medicines.
- We fulfil all regulatory requirements to make public information about our treatment use of our unlicensed medicines.
- Information on whether a particular medicine is available for treatment use in the US, and, if available, the patient eligibility criteria, may be found on <https://www.clinicaltrials.gov/> by searching for the medicine.

Background

Information about a medicine's efficacy and safety may be limited at the stage at which an investigational medicine is requested and provided for treatment use. Studies relating to the toxicity of an investigational medicine will generally have been completed and analysed, and early studies looking at how the medicine is handled by the body will have been completed. However, there may still be uncertainty about the best way to give the medicine to patients, such as the exact dose, the dose frequency, and the medicine's efficacy and safety profile (which side-effects it can cause), which may not yet have been fully established. These uncertainties mean that very careful assessments must be undertaken before making unlicensed medicines available for treatment use.

Patients interested in accessing a GSK medicine for treatment use should talk to their HCPs.

HCPs interested in submitting a request for treatment use of GSK medicines should follow [GSK Managed Access Request Portal](#)