

GSK Public policy positions

Compassionate use

The Issue

GSK is a science-led global healthcare company dedicated to researching, developing and manufacturing medicines, vaccines and consumer healthcare products to help people do more, feel better and live longer.

An essential element of our work is to develop our medicines through conducting clinical trials, where we work 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 in the interest of patients to have access to our investigational medicines and where enrolment into a clinical trial is not an option. Under these circumstances, and where patients have serious or life-threatening diseases or conditions with no satisfactory alternative treatment options, regulators may grant permission for companies to provide a treating HCP with an investigational medicine. Such use of an investigational 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 compassionate use of our investigational pharmaceutical products and vaccines (referred to as investigational medicines) and the criteria we use to assess requests from HCPs.

GSK's Position

- GSK recognises that there may be circumstances when it is appropriate for HCPs to give their patients investigational medicines to treat life threatening or seriously debilitating diseases/conditions where no satisfactory alternatives exist.
- GSK considers the need for compassionate use for all our investigational medicines early on in the planning of our research programmes. Recommendations based on these considerations are approved by our Chief Medical Officer.
- We support compassionate use for patient populations meeting specific criteria managed under formal programmes, as well as for named (or individual) patients, where appropriate and subject to fulfillment of our criteria.
- All requests for compassionate use of a GSK investigational medicine, which must be made by an HCP, will be considered by GSK and we will work with the requestor to support them through the request process.

- All HCP requests for compassionate use of a GSK investigational 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 benefit to the patient justifies the risk.
- Other key considerations include:
 - Whether sufficient information to inform appropriate use of the GSK investigational medicine exists.
 - Any concern that compassionate use of the investigational medicine might compromise any related clinical trial or regulatory pathway.
 - That use will be in a country where appropriate medical oversight exists, and where GSK intends to seek regulatory approval and make the medicine generally available.
 - That the proposed compassionate use complies with local laws and regulations.
- These criteria help ensure GSK has a consistent approach for our decisions on compassionate use.
- We fulfil all regulatory requirements to post details of our compassionate use activities on various independent registers, including [ClinicalTrials.gov](https://www.clinicaltrials.gov) and the [European Medicines Agency Compassionate Use](https://www.ema.europa.eu/en/medicines/compassionate-use) website.

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 compassionate 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 to use, 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 investigational medicines available for compassionate use.

Patients interested in accessing a GSK investigational medicine for compassionate use should talk to their doctors.

HCPs interested in submitting a request for compassionate use of GSK investigational medicines should follow this [link](#).

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