

Do you agree with the draft guidance that doctors can prescribe off-label or unlicensed medicines if satisfied, on the basis of authoritative clinical guidance, that it is as safe and effective as an appropriately licensed alternative?

The guidance: "you may prescribe off-label or unlicensed medicines [...] if [...] you are satisfied, on the basis of authoritative clinical guidance, that it is as safe and effective as an appropriately licensed alternative" implies parity between clinical guidance and market authorisation; and if there are equal medicines in each position, then the prescribing physician may choose between the options based on some criteria other than efficacy and safety.

Genetic Alliance UK believes that the current system for ensuring that the benefits outweigh the risks of medicines should be adhered to as much as possible. Progress toward market authorisation can be time-consuming, but it should be a target for all efficacious medicines. It is certainly within the interests of patients that such medicines be available before they achieve this status, but we do not believe that the licensing systems should be side-stepped on a more regular basis.

Therefore we would propose that unlicensed or off-label medicines are only used in place of an existing licensed treatment when there is a significant benefit to the patient concerned over and above that provided by the licensed treatment.

Do you agree with the guidance at paragraph 60 that it may not be necessary to draw patients' attention to the licensing status of medicines routinely used off-label and for which there is authoritative clinical guidance?

Genetic Alliance UK believes that patients should be able to access all relevant information in order to make informed decisions regarding their care. Many patients, especially those affected by the severe, complex, and/or rare conditions that are supported by our members choose to research their treatments online, either with their peers, or with information portals such as those offered by the European Medicines Agency and the Medicines and Healthcare products Regulatory Agency. Receipt of incomplete or conflicting information could concern patients who find that their treatment does not have a market authorisation.

Do you have any other comments on the Prescribing off-label and unlicensed medicines section?

Current usage of off-label and unlicensed medicines is patchy in the UK. This is perhaps because some doctors are more willing to prescribe off-label or unlicensed medicines than others, or because they are unwilling to prescribe a treatment without data to inform their decision. A framework for data collection that allows for the evaluation of off-label or unlicensed medicines could provide evidence to support prescribing decisions, and would facilitate better surveillance of this form of medicine use.

While Genetic Alliance UK believes that medicines should be available to patients who need them as soon as possible, we would caution against the fostering of a culture where prescribing off-label and unlicensed treatments becomes commonplace. Patient safety is likely to suffer from such a culture. Instead steps should be taken to change the regulatory environment that necessitates such measures. Until that can be achieved, guidance for prescribing should endorse the system of licensing as the best means to protect patient safety.