St Luke's Cancer Alliance

NHS

Position Statement

Access to Unlicensed Investigational Drugs Outside of Clinical Trials via Expanded Access or Compassionate Use Programmes

Background

The primary intent of an expanded access programme (EAP) /compassionate use programme (CUP) is to provide access to a new drug for people with a life-threatening or serious disease for which there is no good alternative treatment and who are not eligible for entry into a relevant clinical trial(s) .
A secondary purpose is to generate additional information about the drug, especially its safety.
This process results in facilitating access to the drug before it is licensed. Use of an EAP is legitimate only if clinical investigators are actively studying the new treatment in well-controlled studies, or all studies have been recently completed or closed to recruitment. There must be evidence that the drug is an effective treatment in the patient group to be treated under the EAP and the drug must not expose patients to unreasonable risks.

This position statement encompasses the use of an unlicensed medicine through expanded access or compassionate use programmes – it does not encompass 'specials' or 'off-label' use outside these programmes.

Position

The St Luke's Cancer Alliance discourages access to investigational drugs outside of clinical trials. However, in principle we believe that cancer patients for whom licensed drugs offer no further treatment options should have access to an investigational drug through an EAP, provided the following conditions are met:

- All treatment possibilities with licensed drugs have been considered and it is agreed that they offer no further clinically appropriate options.
- The patient is not eligible for entry into any relevant clinical trial currently open in that Trust.
- The prescriber believes the risk/benefit profile of the new therapy is favourable to the patient.
- The prescriber takes full responsibility for the use of this medicine for an individual patient.
- **Ideally the manufacturer should guarantee in writing that the medicine will be provided free of charge for the full duration of the patient's treatment.**
- For any programme where the free stock ceases post-licensing for existing patients, or there is any uncertainty of continued free supply pre-licensing, this must be included in the consent process and explicitly documented in the patient's notes.
  An ongoing funding source should be attempted to be identified at the point that free stock ceases, although continuation of funding can in no way be guaranteed. If no funding source is identified, treatment would have to be discontinued.
- ***Before discussion with the patient,*** the prescriber has discussed the option of the EAP with the oncology/haematology pharmacist or Trust Chief Pharmacist and has made a successful Drug and Therapeutics application to the Alliance Chemotherapy Group, as well as the Trust Drug & Therapeutics Committee.
- The prescriber explains to the patient/carer:
  - The medicine is not licensed for use;
  - The intended benefits and risks of taking the medicine;
- Where a patient information leaflet (PIL) is unsuitable or is not available an information sheet should be produced in accordance with local procedures.
- The prescriber obtains and records the patient's consent to treatment before prescribing.

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It should also be noted that

- Other than in very exceptional circumstances such as life threatening emergencies, *no* systemic anti-cancer therapy whether licensed or unlicensed, should be introduced into clinical practice for the first time without the prior approval of the Alliance Chemotherapy Group (or Trust DTC where more urgent) and relevant commissioning bodies.
- Whilst the medicine may be provided free of charge via an EAP, other associated costs e.g. additional attendance costs and / or investigations may not be covered. Therefore it must be ensured that the Commissioning representative on the Alliance Chemotherapy Group is included in the approval process and subsequently that funding arrangements are in place to pick up any associated additional costs.
- Before initiating the first supply of systemic anti-cancer therapy for a patient under an EAP, pharmaceutical companies must confirm to the Alliance Chemotherapy Group (or Trust DTC) in writing, the proposed arrangements for the continued supply of the drug for any patients who have already started treatment when a licence for the drug is granted.
- Pharmaceutical companies should not take participation in an EAP to mean that funding for continued use of the drug once licensed will be automatically agreed.
- Pharmaceutical companies must not enter into any agreement to supply drugs to individual clinicians. Drug supplies must ***always*** be made exclusively via the established pharmacy department supply chain.
- Supplies of drugs must be delivered to the pharmacy department. Drugs must ***never*** be delivered directly to clinicians, clinical nurse specialists or other non-pharmacy staff.
- Pharmacists obtaining unlicensed drugs should follow the 'Guidance For The Purchase And Supply Of Unlicensed Medicinal Products' [(1)] - there should also be local policy and SOPs for doing this.
- In line with the requirements of the Medicines Management Framework and Standards for Better Health [(2,3,4)], Trusts should have systems for the managed entry of new licensed medicines into local health economies and for review of review use of unlicensed medicines.

Acknowledgement:
This document is based on the following documents:

Joint Position Statement: Access to Unlicensed Investigational Drugs Outside of Clinical Trials; Cancer Network Pharmacists' Forum & British Oncology Pharmacy Association; May 2006.

Access to Unlicensed Investigational Drugs Outside of Clinical Trials via Expanded Access of Compassionate Use Programmes; Kent and Medway Area Prescribing Committee; Version 3 January 2008.

References:

1. Guidance For The Purchase And Supply Of Unlicensed Medicinal Products, Notes For Prescribers And Pharmacists; NHS Pharmaceutical Quality Assurance Committee; Third Edition June 2004

2. Standards for Better Health, Department of Health, April 2005

3. DoH. Medicines Management in NHS Trusts: Hospital Medicines Management Framework, 2003

4. The supply of unlicensed relevant medicinal products for individual patients; MCA Guidance Note No. 14 (Previously MAL 14), revised May 2005