1. Purpose of policy

This document outlines the principles and guidance notes for medicines access schemes, both those schemes for unlicensed medicines and those for medicines that are licensed and either awaiting evaluation by the Scottish Medicines Consortium (SMC) or following acceptance by SMC. It covers the authorisation and prescribing of medicines accessed via such schemes.

2. Scope

This policy applies to all medical, nursing and healthcare staff in the Acute Division of NHSGGC (including adult, paediatric and mental health services) involved in the procurement, prescribing, supply and administration of medicines. For the purpose of this guidance, this includes those with honorary contracts, partnership and agency staff.

3. Terminology

The names used for medicines access schemes can vary greatly, and in many cases are decided by individual pharmaceutical manufacturers. The following terms are those used most frequently.

3.1 Unlicensed medicines schemes

Unlicensed medicines schemes aim to make available a medicine that has not yet received a product licence (marketing authorisation) within the UK for its intended use.

3.1.1 Compassionate use programmes

The European Medicines Agency (EMA) defines compassionate use as:
"…a treatment option that allows the use of an unauthorised medicine. Compassionate-use programmes are for patients in the European Union (EU) who have a disease with no satisfactory authorised therapies or cannot enter a clinical trial". [1]
They are intended to facilitate the availability to patients of new treatment options under development". Compassionate use programmes put in place by the EMA are for medicines expected to benefit patients:

- with life-threatening, long-lasting or seriously disabling illnesses
- who are seriously ill and who cannot be treated satisfactorily with licensed medicines, or who have a disease for which no medicine has yet been licensed
- that are not eligible for ongoing clinical trials

3.1.2 Expanded access programmes

Expanded access is essentially the same as compassionate use.

3.1.3 Named Patient Supply

Whereas compassionate use and expanded access programmes are instigated by a product manufacturer, named patient supply requests tend to be initiated by a prescriber making a direct approach to a manufacturer

[1] European Medicines Agency. Accessed via www.ema.europa.eu on 21/04/2020

3.1.4 Early Access to Medicine Schemes (EAMS)

EAMS [2] are managed by the Medicines and Healthcare Products Regulatory Agency (MHRA) and aim to give patients with life-threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet need. EAMS are a formalised variant of an expanded access programme where the MHRA has carried out a risk: benefit assessment in the format of a Public Assessment Report. Formal arrangements for patient monitoring and provision of data are agreed nationally, and in addition, the ADTC Collaborative within Healthcare Improvement Scotland generally provides operational guidance to inform healthcare professionals before they enter into an agreement.

3.2 Post-licence free of charge access schemes (FOC Schemes)

These are schemes for medicines and indications that have marketing authorisation that offer medicines free-of-charge or for a small nominal fee up until the point of SMC assessment and in some cases, after acceptance by SMC. Unlike EAMS, these medicines have not been identified by the MHRA as providing significant advantage over existing treatments for serious or life-threatening conditions.

General Principles

1. Where a medicines access scheme is being considered, formal schemes that are endorsed for implementation in NHSScotland are preferable and pharmaceutical companies should be encouraged to engage with National Services Scotland or Healthcare Improvement Scotland (HIS) to progress these. These may include EAMS programmes where Scottish Operational Guidance has been produced by the Area Drug and Therapeutics Committee Collaborative (ADTCC) and post-licence (pre-HTA) free-of-charge schemes that have been endorsed by National Procurement in accordance with national guidance.

2. All schemes, whether nationally approved or not, must adhere to relevant local policies and processes e.g. ULM, IPTR, PACS 2. Any other scheme that is not formally endorsed on a national level must be considered locally and approved prior to any commitment. Who considers and approves these schemes will differ dependent on the setting where the medicine is intended to be used, the type of scheme and the operational and financial aspects.

3. The relevant specialty should determine the most suitable approach for consideration of such schemes within the principles outlined within this policy. Examples of groups and individuals who may be involved includes:

   - Clinical Directors
   - Chiefs of Medicine
   - Senior Pharmacists (e.g. Lead Clinical Pharmacist)
   - General Managers
   - Lead Clinicians for the speciality in question
   - Pharmacy Distribution Centre (PDC) / Homecare Team
   - Regional Cancer Prescribing Subgroup
   - Acute Services Prescribing Management Group

[2] Medicines and Healthcare products Regulatory Agency (MHRA). Accessed via www.gov.uk on 21/04/2020

4. Persons or groups evaluating the suitability of schemes that are not formally endorsed should consider the following:

    4.1. Are there no viable licensed and/or routinely available alternatives?

    4.2. Are the contract terms for the scheme reasonable, including supply arrangements, associated costs* and the conditions that apply upon closure of the scheme or following completion of supply? (*The preference would be for any costs to be met or any agreed medicine costs to remain in place up until the point of licence and SMC acceptance.)

    4.3. Does the use of the medicine require the co-administration of other medicines or devices or involve the need for additional services or resources?

    4.4. Is the workload, including administrative burden, reasonable and manageable within existing resources?

    4.5. Is the patient/ patient population for whom the medicine is being requested/proposed clearly defined?

    4.6. If the scheme has been considered and not recommended at a national level, does the local situation justify a different outcome?

    4.7. For unlicensed medicines, is there a clinical need to consider access to this medicine now or can access wait until the medicine in question is licensed and has been through due process?

    4.8. For pre-HTA medicines, is there a clinical need to consider access to this medicine now or can access wait until the medicine in question has been through due process?

    4.9. For post-HTA medicines, do the benefits of implementing the scheme justify the administrative burden and will this act as an unacceptable incentive to prescribe?

    4.10. Is there an expectation from the company to share patient data or information as part of the scheme? If so, are the requirements clearly stated and reasonable?

5. Pharmacy will be involved in all situations when enrolment in a medicines access scheme is being considered as there is a requirement to ensure that all medicines use is subject to appropriate medicolegal operational aspects. Typically, both local pharmacy departments and the Pharmacy Distribution Centre (PDC) will be involved in enrolment.

6. All medicines must be procured through the standard NHSGG&C Pharmacy procurement systems and early communication with the Pharmacy Distribution Centre (PDC) Unlicensed Medicines Team is advised. The PDC will be the formal receipt area of any medicines obtained via such schemes. Individual clinicians must not negotiate direct supplies to themselves or to their patients.

7. Where the scheme is to provide access to an unlicensed medicine, the policy requirements for procurement, authorisation, supply and prescribing are as those described in the NHSGGC Unlicensed Medicines Policy.

8. Where the scheme is to provide access to a licensed medicine where the SMC has not yet published advice on use in NHS Scotland, or use is outwith such advice, the appropriate process (e.g. PACS2 or similar) will be followed prior to enrolment in the scheme.

9. Where access to a medicine via compassionate use or expanded access scheme ends following a Marketing Authorisation being granted, but the medicine has still not been considered by SMC, the appropriate process to allow continued access will be followed (e.g. PACS2 or similar).

10. Consideration must be given to whether participation in the scheme will set a precedent, either for an individual patient or for future patients. This may include taking into account the potential scale of use for the medicine in question or number of patients who may be treated.

Authorisation:

Recognising the potential for setting precedence for future use, formal initial authorisation for any access scheme that is not formally endorsed for implementation in NHSScotland will come from the relevant Chief of Medicines (COM). The exception to this is when the access scheme is for a post-HTA licensed medicine when authorisation from the Clinical Services Manager (CSM) or Clinical Director (CD) is considered sufficient. In considering the initial authorisation, the COM/CSM/CD will consult with an appropriate senior pharmacist, General Manager and Lead Clinician for the specialty where the medicine is intended to be used.

Following approval, the agreement with the pharmaceutical company can be signed and individual patient recruitment can commence.

Specific considerations and required documentation: Endorsed unlicensed medicines schemes (e.g. EAMS)

Healthcare Improvement Scotland, via the ADTC Collaborative will aim to issue operation guidance for the implementation of the EAMS which will include a clear and appropriate exit strategy and guidance on information governance. In the absence of operational guidance, the advice in the general principles section for considering the scheme should be followed.

An Unlicensed Medicines Form (ULM Form) should be completed and approved prior to registering any patient for an EAMS.

Required documentation: Compassionate Use, Expanded Access Schemes or Named Patient Supply

An Unlicensed Medicines Form (ULM Form) should be completed and approved prior to registering any patient for an unlicensed medicine scheme. Following COM approval, the agreement with the pharmaceutical company can be signed off and individual patient recruitment can begin

Required documentation: Pre-HTA licensed medicine access schemes

An IPTR or PACS 2 (whichever is appropriate) should be completed and approved prior to registering any patient.

Required documentation: Post-HTA licensed medicine access schemes

No paperwork is routinely required to access medicines accepted for use by SMC.

Good Practice Points:

Consideration should be given of whether the medicine needs to be included in the following year's horizon scanning exercise. If other medicines or services are required this may need wider service discussion or consideration of the implications of the in-year budget.

For unlicensed medicine schemes or pre-HTA schemes, often the initial cost of medicines are either free of charge, or at a nominal cost. There may be future cost implications that arise when access to the medicine via the scheme ceases. In agreeing terms with the pharmaceutical company in regards to funding of treatment the following statement is suggested:

"x" Pharmaceutical Company commits that after the initiation of therapy the medicine will be provided free of charge until the date of public communication of Scottish Medicines Consortium advice to NHS Scotland. If the drug is not recommended by SMC, then the company will continue to supply the drug free of charge to these patients, until discontinuation on medical grounds, dependent on drug availability.

Supply

Arrangements for supply should be clearly defined. In most cases compassionate/ expanded access medicines will have to be supplied from the hospital. It is important to ensure that there is appropriate discussion with both the on-site pharmacy department and the Pharmacy Distribution Centre to arrange this.

Request for patients from neighbouring health boards

Where a patient who resides in a health board other than NHS GG&C, engagement with the patient's home board to assist in any financial planning is imperative, considering the potential for future cost implications. The West of Scotland Health Boards have an agreement on the management of medicines accessed via PACS2, IPTRs and the Unlicensed Medicines (ULM) process within each other's Boards.

Details on specific engagement with the patient's home health board can be found within the relevant policies for IPTRs, PACS 2 requests and ULMs.